As filed with the Securities and Exchange Commission on September 2, 2020.

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

Taysha Gene Therapies, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**2836**	**84-3199512**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

2280 Inwood Road
Dallas, TX 75235
(214) 612-0000
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

RA Session II
President and Chief Executive Officer
Taysha Gene Therapies, Inc.
2280 Inwood Road
Dallas, TX 75235
(214) 612-0000
(Name, address, including zip code, and telephone number, including area code, of agent for service)

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Approximate date of commencement of proposed sale to the public: As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act. ☐

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities To Be Registered	**Proposed Maximum Aggregate Offering Price(1)**	**Amount of Registration Fee(2)**
Common Stock, $0.00001 par value per share	$100,000,000	$12,980

(1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended. Includes the offering price of additional shares that the underwriters have the option to purchase.

(2) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum aggregate offering price.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell these securities nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated September 2, 2020

PRELIMINARY PROSPECTUS

Shares

Common Stock

This is an initial offering of shares of common stock of Taysha Gene Therapies, Inc.

We are offering shares of our common stock.

Prior to this offering, there has been no public market for our common stock. It is currently estimated that the initial public offering price per share will be between $ and $. We have applied to list our common stock on The Nasdaq Global Market under the trading symbol "TSHA."

We are an "emerging growth company" as defined under U.S. federal securities laws and, as such, will be subject to reduced public company reporting requirements for this prospectus and future filings.

Investing in our common stock involves risks. See "Risk Factors" beginning on page 15 to read about factors you should consider before buying shares of our common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

	Per Share	Total
Initial public offering price	$	$
Underwriting discounts(1)	$	$
Proceeds, before expenses, to Taysha Gene Therapies, Inc.	$	$

(1) See the section titled "Underwriting" for a description of the compensation payable to the underwriters.

To the extent that the underwriters sell more than shares of common stock, the underwriters have the option to purchase up to an additional shares from us at the initial price to the public less the underwriting discount.

The underwriters expect to deliver the shares against payment in New York, New York on , 2020.

Goldman Sachs & Co. LLC **Morgan Stanley** **Jefferies**

Chardan

Prospectus dated , 2020

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Neither we nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

For investors outside of the United States: we have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. Persons outside of the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside of the United States.

All trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus may be referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert their rights thereto.

PROSPECTUS SUMMARY

This summary highlights, and is qualified in its entirety by, information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and the related notes appearing elsewhere in this prospectus, before making an investment decision. As used in this prospectus, unless the context otherwise requires, references to "we," "us," "our," "the company," "Taysha" and "Taysha Gene Therapies" refer to Taysha Gene Therapies, Inc., together with its consolidated subsidiaries.

Taysha Gene Therapies, Inc.

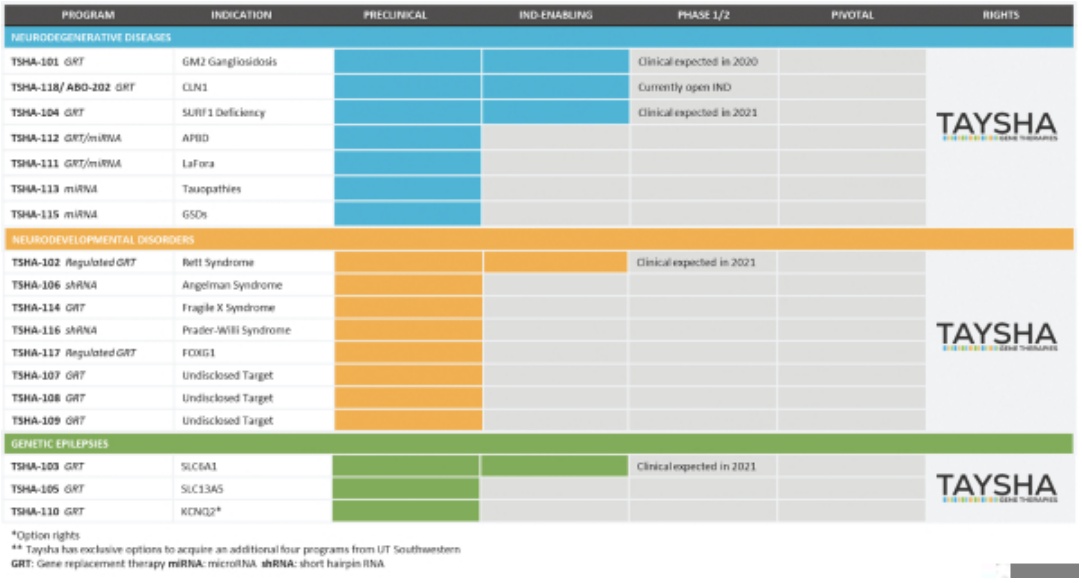
Overview

We are a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations. We were founded in partnership with The University of Texas Southwestern Medical Center, or UT Southwestern, to develop and commercialize transformative gene therapy treatments. Together with UT Southwestern, we are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates, with exclusive options to acquire four additional development programs at no cost. By combining our management team's proven experience in gene therapy drug development and commercialization with UT Southwestern's world-class gene therapy research capabilities, we believe we have created a powerful engine to develop transformative therapies to dramatically improve patients' lives. We expect to initiate a Phase 1/2 clinical trial of TSHA-101 for the treatment of GM2 gangliosidosis, under a Clinical Trial Application, or CTA, in Canada by the end of 2020. In addition, we plan to submit investigational new drug applications, or INDs, for four programs to the U.S. Food and Drug Administration, or the FDA, by the end of 2021: TSHA-101, TSHA-102 (Rett syndrome), TSHA-103 (SLC6A1 haploinsufficiency) and TSHA-104 (SURF1 deficiency). We are also developing TSHA-118 (formerly ABO-202) for the treatment of CLN1 disease (or infantile Batten disease) and intend to initiate a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. In addition to our product pipeline candidates, we are building a platform of next-generation technologies to optimize key components of our AAV-based gene therapies, including redosing, transgene regulation and capsid development.

The fundamental components of our approach to gene therapy development are an adeno-associated virus serotype 9, or AAV9, capsid, intrathecal delivery and an efficient manufacturing process. We use an AAV9 capsid to deliver therapeutic genes engineered to replace a mutated gene, enhance the expression of a silenced gene or decrease the expression of a gene, depending on the underlying biology of the specific disease. We use intrathecal administration, which involves direct delivery of our gene therapies to the cerebrospinal fluid, or CSF, to facilitate optimal biodistribution and cell transduction within the central nervous system, or CNS. Our flexible manufacturing processes allow us to produce our gene therapy product candidates efficiently at scale. Through our partnership with UT Southwestern, we have access to a Good Manufacturing Practice-, or GMP-, compliant manufacturing suite that utilizes a suspension HEK293 process to produce AAV9. We also intend to establish our own commercial-scale, GMP-compliant manufacturing facility to meet demand in the event that our product candidates receive marketing approval.

Our Pipeline

We are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates for monogenic diseases of the CNS in both rare and large patient populations, with exclusive options to acquire four additional development programs at no cost. Our current pipeline, including the stage of development of each of our product candidates, is represented in the table below.



Our portfolio of gene therapy candidates targets broad neurological indications across three distinct therapeutic categories, which together have the potential to address over 500,000 patients in the United States and the European Union:

- **Neurodegenerative diseases**, which refer to diseases that are characterized by the progressive degeneration of the structures and functions of the CNS.
- **Neurodevelopmental disorders**, which refer to a group of conditions with onset during the time when the brain is developing and are a reflection of disabilities associated primarily with the functioning of the neurological system and brain.
- **Genetic epilepsies**, which refer to disorders with recurrent seizures associated with abnormal development of the brain.

Our most advanced product candidates include:

- **TSHA-101**, which is being developed for the treatment of GM2 gangliosidosis, a lysosomal storage disorder and family of severe neurodegenerative diseases that includes Tay-Sachs disease and Sandhoff disease. We are developing TSHA-101 as a bicistronic *HEXB*P2A-*HEXA* transgene packaged into an AAV9 vector under the control of a CAG promoter. We have conducted preclinical studies evaluating safety and biodistribution, and plan to initiate a Phase 1/2 clinical trial under a CTA in Canada by the end of 2020. We plan to submit an IND for TSHA-101 to the FDA by the end of 2021. TSHA-101 has received orphan drug designation and rare pediatric disease designation from the FDA for the treatment of GM2 gangliosidosis.

- **TSHA-118** is a self-complementary AAV9 viral vector that expresses human codon-optimized *CLN1* complementary deoxyribonucleic acid under control of the chicken ß-actin hybrid promoter. Preclinical studies evaluating safety and biodistribution have been conducted, and we plan to conduct a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. TSHA-118 has received orphan drug designation from the FDA and the European Medicines Agency, fast track designation from the FDA and rare pediatric disease designation from the FDA for the treatment of CLN1 disease.
- **TSHA-102**, which is being developed for the treatment of Rett syndrome, one of the most common genetic causes of severe intellectual disability, characterized by rapid developmental regression and in many cases caused by heterozygous loss of function mutations in *MECP2*, a gene essential for neuronal and synaptic function in the brain. TSHA-102 is constructed from a neuronal specific promoter, MeP426, coupled with the *miniMECP2* transgene, a truncated version of *MECP2*, and miRNA-Responsive Auto-Regulatory Element, or miRARE, our novel miRNA target panel, packaged in self-complementary AAV9. We plan to submit an IND for TSHA-102 to the FDA by the end of 2021.
- **TSHA-103**, which is being developed for the treatment of SLC6A1 haploinsufficiency disorder, one of the most common monogenic causes of epilepsy characterized by myoclonic atonic seizures, autism spectrum disorder and intellectual disability. TSHA-103 is constructed from a codon-optimized version of the human *SLC6A1* gene packaged within a self-complementary AAV9 viral vector under the control of a JeT promoter. We plan to submit an IND for TSHA-103 to the FDA by the end of 2021.
- **TSHA-104**, which is being developed for the treatment for Surfeit locus 1, or SURF1, deficiency. TSHA-104 is constructed from a codon-optimized version of the human *SURF1* gene packaged within a self-complementary AAV9 viral vector under the control of a modified version of the chicken ß-actin, or CBA, promoter CBA hybrid intron. We plan to submit an IND for TSHA-104 to the FDA by the end of 2021.

Our Strategic Partnership with The University of Texas Southwestern Medical Center

Our partnership with UT Southwestern is differentiated from traditional collaborations between industry and academia due to our access to UT Southwestern's faculty, manufacturing facility and integrated research and clinical care approach, which, together, we believe will enable us to advance our development programs with speed and scale. Under the terms of our collaboration and license agreement with UT Southwestern, we hold an exclusive, worldwide royalty-free license to discover, develop and commercialize gene therapies for our pipeline. Within the framework of our partnership, UT Southwestern will conduct discovery and preclinical research, lead IND-enabling studies, manufacture GMP vectors for use in preclinical studies and clinical trials and execute natural history studies to support the development of our product candidates. We are responsible for all clinical development, regulatory, strategy, commercial manufacturing and commercialization.

Through our partnership, we are able to leverage the collective expertise of UT Southwestern researchers, clinicians, and investigators with decades of experience in conducting cutting-edge research and providing clinical care, including in the neurodegenerative disease, neurodevelopmental disorder and genetic epilepsy therapeutic categories. Furthermore, UT Southwestern's state-of-the art, GMP viral vector manufacturing facility consists of a full process development laboratory and 500 liter GMP suite with the capacity to support multiple preclinical and early clinical development efforts in parallel.

Collectively, UT Southwestern faculty members have received six Nobel Prizes, and the faculty includes 24 members of the National Academy of Sciences, 16 members of the National Academy of Medicine, and 13 Howard Hughes Medical Institute Investigators. The UT Southwestern Gene Therapy Program is led by Steven Gray, Ph.D., and Berge Minassian, M.D. Dr. Gray's core expertise is in AAV-based gene therapy vector engineering and optimizing approaches to deliver therapeutic transgenes to the CNS. Dr. Minassian is a pediatric neurologist whose clinical specialties are epilepsy, neurodegenerative diseases and neurodevelopmental conditions.

Our Strategy

We are building a patient-centric business with the goal of developing AAV-based gene therapies for the treatment of monogenic diseases of the CNS in both rare and large patient populations. We are focused on executing the following elements of our strategy:

- **Build a sustainable gene therapy company.** Our goal is to build a gene therapy company with a sustainable pipeline of product candidates and a consistent stream of new commercial product launches. To that end, we are focused on rapidly advancing our current pipeline of AAV9-based gene therapies while actively developing our next generation platforms to discover and develop additional product candidates.
- **Advance our lead product candidates through clinical trials to commercialization.** Our product portfolio currently consists of 18 gene therapy product candidates targeting a diverse set of rare and prevalent CNS indications, with exclusive options to acquire four additional development programs from UT Southwestern at no cost. We intend to develop, seek regulatory approval and commercialize each product candidate in our portfolio. We expect to initiate a Phase 1/2 clinical trial of TSHA-101, a neurodegenerative product candidate, by the end of 2020, and we plan to submit INDs to the FDA for four programs by the end of 2021: TSHA-101, TSHA-102, TSHA-103 and TSHA-104. We also intend to conduct a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. If our clinical trials are successful, we plan to discuss expedited regulatory approval strategies with regulatory authorities.
- **Leverage our relationship with UT Southwestern.** We are anchored by a differentiated strategic partnership with UT Southwestern that allows us to access a highly experienced team of researchers and clinicians with deep experience in the underlying biology and treatment of monogenic CNS disorders and the patient populations that they treat. We believe that our partnership with UT Southwestern provides us with a significant advantage to rapidly discover, develop and commercialize novel gene therapies.
- **Utilize scalable manufacturing technologies.** A critical component of the development of any complex biological therapy, including gene therapies, is the ability to manufacture the therapy efficiently at scale. Through our partnership with UT Southwestern, we have access to a flexible, scalable and well-characterized GMP manufacturing suite that utilizes a suspension HEK293 process to produce AAV9, which we believe will enable us to produce material suitable for clinical trials in a cost and time-efficient manner. We believe the capacity offered by UT Southwestern's manufacturing facility will be sufficient to meet the clinical demand for our full pipeline of product candidates. Because we currently utilize the AAV9 capsid across our product portfolio, our product candidates differ primarily by their therapeutic transgene, and we believe that minimal changes to our optimized upstream and downstream processes are required to manufacture each product candidate. In addition, we intend to establish our own commercial scale GMP-compliant manufacturing facility to meet commercial demand if our product candidates receive marketing approval.

- **Develop our next generation platform technologies.** In addition to our pipeline of AAV9-based gene therapies, we are actively developing three distinct platform technologies to enable the discovery, development, and rapid translation of new gene therapies: a proprietary technology to allow redosing of AAV-based gene therapies, transgene regulation (miRARE) and novel capsid development.
- **Evaluate strategic opportunities to accelerate development timelines and maximize the value of our product candidate pipeline.** We are evaluating opportunities to maximize the value of our product candidate pipeline, including through a joint venture or other structure in China. We believe these structures may provide us with the opportunity to leverage the financial and other resources of partners to advance the development of our product candidate pipeline in a key geography such as China.

Our Approach

We utilize AAV9, an AAV serotype with a unique ability to cross the blood-brain barrier and transduce cells of the CNS. AAV9 has been widely characterized across numerous preclinical studies and more than 15 ongoing or completed clinical trials and has a well-characterized biodistribution, safety, tolerability and efficacy profile. In 2019, the FDA approved Zolgensma, the first systemic gene therapy that utilizes AAV9, for the treatment of spinal muscular atrophy Type 1 in infants.

Intrathecal administration refers to the injection of a therapy directly into the CSF. The procedure is routinely performed in an outpatient setting and is generally well tolerated. We intend to administer our product candidates intrathecally, as we believe that intrathecal administration confers several advantages for the delivery of gene therapies to the CNS. In comparison to intravenous administration, intrathecal administration allows for a lower dose of the therapy, as the vector is confined to the CNS with limited uptake into off-target tissues. Because the CNS is immune-privileged, intrathecal gene therapy may be administered even in the presence of pre-existing antibodies to AAV. Finally, intrathecally delivered gene therapies have limited exposure to peripheral organs, which enables a higher concentration of vector to be delivered to the diseased tissue of interest. A growing body of literature supports the safety and relevance of lumbar intrathecal injection to deliver AAV9 to the CNS and achieve favorable biodistribution and transgene expression profiles. In comparison to other AAV serotypes, AAV9 administration through lumbar intrathecal injection has been shown to result in superior transduction of multiple cells within the CNS.

We use a scalable production process for our product candidates using a suspension cell culture process in which mammalian HEK293 cells are transiently transfected with plasmid DNA. Our production process, including all process development, product characterization, analytical capabilities and purification techniques, is designed to efficiently scale to support our clinical and commercial development needs. The utilization of the AAV9 capsid across our product portfolio allows us to manufacture each product with minimal changes to our optimized upstream and downstream process, since each of our product candidates differ primarily by their therapeutic transgene.

Our Therapeutic Strategy

We design our product candidates based on the underlying biology of the disease target and the characteristics that we believe will result in maximum therapeutic benefit for patients:

- **Gene replacement therapies.** To treat diseases or disorders caused by a missing gene or limited expression of a gene due to loss-of-function mutations, we design our product candidates to replace the gene of interest. In general, these product candidates are comprised

of a codon-optimized DNA transgene that encodes the wild type gene of interest, coupled with a promoter selected to ensure expression in the cell or tissue-type of interest.

- **Regulated gene replacement therapies.** In a number of disorders, including Rett syndrome and FOXG1 syndrome, the expression of a therapeutic transgene needs to be regulated. In these disorders, high doses of transgene-expressing vectors may be harmful, while low doses may avoid toxicity but be sub-therapeutic. For disorders that require replacement of dose-sensitive genes, we have combined high-throughput microRNA, or miRNA, profiling and genome mining to create miRARE, our novel miRNA target panel. This approach is designed to enable our product candidates to maintain safe transgene expression levels in the brain. Importantly, this built-in regulation system is fully endogenous, and therefore does not require any additional exogenous drug application.
- **Vectorized miRNA gene therapies.** In certain diseases within our pipeline, including Lafora disease, adult polyglucosan body disease and tauopathies, the goal of our product candidates is to silence the expression of genes that are involved in or considered to be the root cause of disease onset and progression. To accomplish this, we design transgenes that express miRNA, which are small, non-coding sequences of RNA that result in silencing of gene expression.
- **Vectorized shRNA gene therapies.** In certain diseases such as Prader-Willi syndrome and Angelman syndrome, the goal of our product candidates is to activate a constitutively silenced gene to generate a therapeutic effect under control of the endogenous promoters of the cell. We utilize transgenes that express short-hairpin RNA, or shRNA, which, upon binding to the target of interest, are designed to reactivate a silenced gene.

Our Next Generation Platform Technologies

In addition to our pipeline of AAV9 product candidates, we are building a suite of platforms to develop next-generation technologies that can optimize key components of an AAV-based gene therapy.

Novel Route of Administration to Allow Redosing

We are advancing a novel AAV dosing platform with the potential to facilitate redosing by administering AAV-based gene therapies directly to the vagus nerve. In preclinical studies in adult rats, we observed that AAV9 delivery to the vagus nerve resulted in efficient targeting of the vagal neurons. In preclinical studies in dogs, AAV delivery to the vagus nerve was well tolerated at all doses. Post-mortem analysis showed that vagal nerve fibers and neurons were microscopically normal.

We believe that direct administration of our AAV9 therapies to the vagus nerve could be useful to treat the peripheral and autonomic manifestations of the CNS diseases in our pipeline. We plan to further evaluate the safety and feasibility of this approach in non-human primates, or NHPs.

Regulated Transgene Expression Using miRARE

In a number of disorders, including Rett syndrome and FOXG1 syndrome, the expression of a therapeutic transgene needs to be regulated. In these disorders, high doses of transgene-expressing vectors may be harmful, while low doses may avoid toxicity but be sub-therapeutic. For disorders that require replacement of dose-sensitive genes, we have combined high-throughput miRNA, profiling and genome mining to create miRARE, our novel miRNA target panel. This approach is designed to enable our product candidates to maintain safe transgene expression levels in the brain.

Importantly, this built-in regulation system is fully endogenous, and therefore does not require any additional exogenous drug application.

Novel Capsid Identification

We are developing a novel AAV capsid platform that utilizes machine learning, capsid shuffling and directed evolution to improve targeted delivery. Our approach allows us to identify capsids with improved properties in mice and NHPs in parallel to maximize their translational relevance. We are utilizing single-molecule, real-time sequencing analysis for high throughput characterization of these capsids.

We believe that our approach will allow us to rapidly identify new capsids to drive new product candidates for CNS disorders with novel biodistribution and transduction profiles into our development pipeline.

Our History and Team

We believe that we have established a unique position in advancing the development of gene therapies. Our scientific founders, Dr. Steven Gray, who serves as our Chief Scientific Advisor, and Dr. Berge Minassian, who serves as our Chief Medical Advisor, have extensive experience in developing gene therapies and conducting clinical trials for complex CNS diseases. Our management team has significant experience in discovering, developing, manufacturing, and commercializing gene therapies. The members of our leadership team have specialized expertise developed at companies including Audentes Therapeutics, AveXis, BioMarin, PTC Therapeutics, Rocket Pharmaceuticals and Sanofi-Genzyme. Our board of directors played an integral role in the formation of our company and is comprised of Sean Nolan, the chairman of our board of directors and former Chief Executive Officer of AveXis, Phillip B. Donenberg, the former Chief Financial Officer of AveXis, Paul Manning of PBM Capital, Sukumar Nagendran, M.D., the former Chief Medical Officer of AveXis, and RA Session II, our President, Chief Executive Officer and Founder. Since our inception, we have raised an aggregate of $126.0 million of gross proceeds from the sale of preferred stock, including from leading institutional and life science investors such as PBM Capital, certain funds managed by Fidelity Management & Research Company LLC, Nolan Capital, GV (formerly Google Ventures), Invus, Casdin Capital, Franklin Templeton, Octagon Capital, Perceptive Advisors LLC, Sands Capital, ArrowMark Partners, Venrock Healthcare Capital Partners and other mutual fund and institutional investors.

Risks Associated with Our Business

Our business is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section titled “Risk Factors” and include, among others:

- We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.
- Even if this offering is successful, we will need substantial additional funding to meet our financial obligations and to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy.
- We are very early in our development efforts and all of our product candidates are in preclinical development. If we are unable to successfully develop, receive regulatory approval for and

commercialize our product candidates for these or any other indications, or successfully develop any other product candidates, or experience significant delays in doing so, our business will be harmed.

- Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.
- The regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed.
- We have not yet tested any product candidates in clinical trials. Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials.
- We may not be successful in our efforts to build a pipeline of additional product candidates or our next-generation platform technologies.
- Our business and operations may be adversely affected by the evolving and ongoing COVID-19 global pandemic.
- Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.
- We currently rely exclusively on our collaboration with UT Southwestern for our preclinical research and development programs, including for discovering, preclinically developing and conducting all IND-enabling studies for our lead product candidates and our near-term future pipeline. Failure or delay of UT Southwestern to fulfill all or part of its obligations to us under the agreement, a breakdown in collaboration between the parties or a complete or partial loss of this relationship would materially harm our business.
- Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates.
- We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.
- Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain. We are aware of issued patent or patents issued to REGENXBIO Inc., or REGENX, that claim AAV vectors that have an AAV9 capsid serotype. If we commercialize any of our product candidates prior to the expiry of those patents in 2026 without a license, REGENX could bring an action claiming infringement.
- If we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market.

Implications of Being an Emerging Growth Company and a Smaller Reporting Company

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For so long as we remain an emerging growth company, we may take advantage of relief from certain reporting requirements and other burdens that are otherwise applicable generally to public companies. These provisions include:

- reduced obligations with respect to financial data, including only being required to present two years of audited financial statements, in addition to any required unaudited interim financial

statements with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;

- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements;
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements; and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor’s report on financial statements.

We may take advantage of these provisions until we no longer qualify as an emerging growth company. We will cease to qualify as an emerging growth company on the date that is the earliest of: (i) December 31, 2025, (ii) the last day of the fiscal year in which we have more than $1.07 billion in total annual gross revenues, (iii) the date on which we are deemed to be a “large accelerated filer” under the rules of the U.S. Securities and Exchange Commission, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, or (iv) the date on which we have issued more than $1.0 billion of non-convertible debt over the prior three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. We have taken advantage of certain reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different than you might obtain from other public companies in which you hold equity interests.

In addition, under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to take advantage of the extended transition period to comply with new or revised accounting standards and to adopt certain of the reduced disclosure requirements available to emerging growth companies. As a result of the accounting standards election, we will not be subject to the same implementation timing for new or revised accounting standards as other public companies that are not emerging growth companies, which may make comparison of our financials to those of other public companies more difficult. As a result of these elections, the information that we provide in this prospectus may be different than the information you may receive from other public companies in which you hold equity interests. In addition, it is possible that some investors will find our common stock less attractive as a result of these elections, which may result in a less active trading market for our common stock and higher volatility in our share price.

We are also a “smaller reporting company,” meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging

growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Corporate Information

We were incorporated under the laws of the State of Texas in September 2019. In February 2020, we converted to a Delaware corporation. Our principal executive offices are located at 2280 Inwood Road, Dallas, Texas 75235 and our telephone number is (214) 612-0000. Our website address is *www.tayshagtx.com*. The information contained on, or accessible through, our website is not incorporated by reference into this prospectus. We have included our website in this prospectus solely as an inactive textual reference.

The Offering

Common stock offered by us	shares
Underwriters' option to purchase additional shares	shares
Common stock to be outstanding immediately after this offering	shares (or shares if the underwriters exercise in full their option to purchase additional shares)
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $ million, (or approximately $ million if the underwriters exercise in full their option to purchase up to additional shares of common stock), assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We currently intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, to and the remainder for working capital and other general corporate purposes. See the section titled "Use of Proceeds" beginning for additional information.
Risk factors	You should read the section titled "Risk Factors" for a discussion of factors you should consider carefully, together with all the other information included in this prospectus, before deciding to invest in our common stock.
Proposed Nasdaq Global Market symbol	"TSHA"

The number of shares of our common stock to be outstanding after this offering is based on 25,647,048 shares of our common stock outstanding as of June 30, 2020, after giving effect to the conversion of all outstanding shares of our convertible preferred stock, including the convertible preferred stock issued in July and August 2020, into an aggregate of 15,647,048 shares of common stock, and excludes:

- 15,000 shares of our common stock issuable upon the exercise of options under our 2020 Equity Incentive Plan, or the Existing Plan, granted subsequent to June 30, 2020, at an exercise price of $16.23 per share;
- 705,882 shares of our restricted common stock awarded under the Existing Plan subsequent to June 30, 2020;
- 2,615,935 shares of common stock issuable upon the vesting of restricted stock units awarded under the Existing Plan subsequent to June 30, 2020;
- 192,595 shares of our common stock reserved for future issuance under the Existing Plan, which shares will cease to be available for issuance at the time our 2020 Stock Incentive Plan, or the New Plan becomes effective and will be added to, and become available for issuance under, the New Plan;

- shares of our common stock reserved for future issuance under our New Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the New Plan; and
- shares of our common stock reserved for future issuance under our 2020 Employee Stock Purchase Plan, or the ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

Unless otherwise indicated, all information contained in this prospectus, including the number of shares of common stock that will be outstanding after this offering, assumes or gives effect to:

- the conversion of all outstanding shares of our convertible preferred stock on a one-for-one basis into an aggregate of 15,647,048 shares of our common stock, which will occur upon the closing of this offering;
- a -for- stock split of our common stock effected on ;
- the filing and effectiveness of our amended and restated certificate of incorporation upon the closing of this offering;
- no exercise of the outstanding options referred to above after June 30, 2020; and
- no exercise by the underwriters of their option to purchase additional shares of our common stock.

SUMMARY FINANCIAL DATA

The following tables set forth our summary statement of operations data for the period from September 20, 2019 (the date of our inception) through December 31, 2019. We have derived the statement of operations data for the period from September 20, 2019 (the date of our inception) through December 31, 2019 from our audited financial statements appearing elsewhere in this prospectus. The statement of operations data for the six months ended June 30, 2020 and the balance sheet data as of June 30, 2020 have been derived from our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as the audited financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, considered necessary for a fair presentation of the financial information in those statements.

You should read the following summary financial data together with our financial statements and the related notes thereto included elsewhere in this prospectus and the sections of this prospectus titled “Selected Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” The summary financial data in this section are not intended to replace our financial statements and are qualified in their entirety by our financial statements and related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected in the future.

	Period from September 20, 2019 (Date of Inception) through December 31, 2019	Six Months Ended June 30, 2020
Statement of Operations Data:		
(in thousands, except share and per share data)		
Operating expenses:		
Research and development	$ 987	$ 8,576
General and administrative	128	1,018
Total operating expenses	1,115	9,594
Loss from operations	(1,115)	(9,594)
Other expense:		
Change in fair value of preferred stock tranche liability	—	(17,030)
Interest expense	—	(27)
Total other expense	—	(17,057)
Net loss	$ (1,115)	$ (26,651)
Net loss per common share, basic and diluted	$ (0.13)	$ (2.67)
Weighted average common shares outstanding, basic and diluted	8,834,951	10,000,000
Pro forma net loss per common share, basic and diluted (unaudited)(1)		$ (1.91)
Weighted average shares outstanding used in computing pro forma net loss per share (unaudited)(1)		13,924,176

(1) See Note 6 to our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus for an explanation of the method used to calculate historical and pro forma net loss per share and historical and pro forma weighted average common shares outstanding.

	As of June 30, 2020		
	Actual	Pro Forma(1)	Pro Forma As Adjusted(2)
Balance Sheet Data:			
(in thousands)			
Cash and cash equivalents	$ 11,200	$118,252	$
Working (deficit) capital(3)	(8,791)	115,437	
Total assets	11,318	118,370	
Preferred stock tranche liability	17,176	—	
Convertible preferred stock	18,014	—	
Accumulated deficit	(27,766)	(27,766)	
Total stockholders' (deficit) equity	(26,786)	115,456	

(1) The pro forma column reflects (i) the receipt of $107.1 million in aggregate net proceeds from the issuance and sale of our Series A and Series B convertible preferred stock in July and August 2020 and the related settlement of the convertible preferred stock tranche liability and (ii) the conversion of all of the outstanding preferred shares of our convertible preferred stock into an aggregate of 15,647,048 shares of our common stock upon completion of this offering, including the conversion of 3,800,000 shares of Series A convertible preferred stock issued in July 2020 and 5,647,048 shares of Series B convertible preferred stock issued in July and August 2020, as if such conversion had occurred on June 30, 2020.

(2) The pro forma as adjusted column reflects the pro forma adjustments set forth above and the sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting fees and commissions and estimated offering expenses payable by us. This pro forma as adjusted information is illustrative only and will depend on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase or decrease in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase or decrease the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions. Each increase or decrease of 1.0 million in the number of shares we are offering would increase or decrease the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by $ million, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions.

(3) We define working (deficit) capital as current assets less current liabilities. See our financial statements appearing elsewhere in this prospectus.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including our financial statements and related notes, before deciding whether to purchase shares of our common stock. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties that we are unaware of, or that we currently believe are not material, may also become important factors that affect us. If any of the following risks are realized, our business, financial condition, results of operations and prospects could be materially and adversely affected. In that event, the price of our common stock could decline, and you could lose part or all of your investment.

Risks Related to our Financial Position and Capital Needs

We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.

Since our inception, we have incurred significant net losses, and we expect to continue to incur significant expenses and operating losses for the foreseeable future. Our net losses were $1.1 million and $26.7 million for the period from September 20, 2019 (the date of our inception) through December 31, 2019 and for the six months ended June 30, 2020, respectively. As of June 30, 2020, we had an accumulated deficit of $27.8 million. We have financed our operations with $126.0 million in gross proceeds raised in our private placements of convertible preferred stock through August 2020. We have no products approved for commercialization and have never generated any revenue from product sales.

All of our product candidates are still in the preclinical testing stage. We expect to continue to incur significant expenses and operating losses over the next several years. We expect that it could be several years, if ever, before we have a commercialized product. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue to advance the preclinical and clinical development of our product candidates and preclinical and discovery programs;
- conduct our planned clinical trials of TSHA-101 and TSHA-118, as well as initiate and complete additional clinical trials of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and future product candidates;
- seek regulatory approval for any product candidates that successfully complete clinical trials;
- continue to develop our gene therapy product candidate pipeline and next-generation platforms;
- scale up our clinical and regulatory capabilities;
- manufacture current good manufacturing practices, or cGMP, material for clinical trials or potential commercial sales;
- establish and validate a commercial-scale cGMP manufacturing facility;
- establish a commercialization infrastructure and scale up internal and external manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;

- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing quality control, regulatory, manufacturing and scientific and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses in operating as a public company.

To date, we have not generated any revenue. To become and remain profitable, we must succeed in developing and eventually commercializing product candidates that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, obtaining regulatory approval, and manufacturing, marketing and selling any product candidates for which we may obtain regulatory approval, as well as discovering and developing additional product candidates. We are only in the preliminary stages of most of these activities and all of our product candidates are in preclinical development. We may never succeed in these activities and, even if we do, may never generate any revenue or revenue that is significant enough to achieve profitability.

Even if we achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our development efforts, obtain product approvals, diversify our offerings or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We have a limited operating history and no history of commercializing products, which may make it difficult for an investor to evaluate the success of our business to date and to assess our future viability.

We are a preclinical-stage gene therapy company with a limited operating history. We commenced operations in 2019, and our operations to date have been largely focused on organizing and staffing our company, business planning, raising capital and entering into collaboration and license agreements for conducting preclinical research and development activities for our product candidates and gene therapy pipeline. To date, we have not yet demonstrated our ability to successfully complete clinical trials, including pivotal clinical trials, obtain regulatory approvals, manufacture a product on a commercial scale, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We will need to develop commercial capabilities, and we may not be successful in doing so.

Even if this offering is successful, we will need substantial additional funding to meet our financial obligations and to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to curtail our planned operations and the pursuit of our growth strategy.

Our operations have consumed substantial amounts of cash since inception. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive

and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. We expect to continue to incur significant expenses and operating losses over the next several years as we conduct clinical trials of our product candidates, initiate future clinical trials of our product candidates, advance our preclinical programs, seek marketing approval for any product candidates that successfully complete clinical trials and advance any of our other product candidates we may develop or otherwise acquire. In addition, our product candidates, if approved, may not achieve commercial success. Our revenue, if any, will be derived from sales of products that we do not expect to be commercially available for a number of years, if at all. If we obtain marketing approval for any product candidates that we develop or otherwise acquire, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. We also expect an increase in our expenses associated with creating additional infrastructure to support operations as a public company.

As of June 30, 2020, we had cash and cash equivalents of $11.2 million. In July and August 2020, we received $107.1 million in aggregate net proceeds from the issuance and sale of our Series A and Series B convertible preferred stock financings. We believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to fund our operating expenses and capital requirements into . This estimate is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect. Changes may occur beyond our control that would cause us to consume our available capital before that time, including changes in and progress of our development activities, acquisitions of additional product candidates, and changes in regulation. Our future capital requirements will depend on many factors, including:

- the scope, progress, costs and results of discovery, preclinical development, laboratory testing and clinical trials for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and future product candidates;
- the extent to which we develop, in-license or acquire other product candidates and technologies in our gene therapy product candidate pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development;
- the number and development requirements of product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs of establishing and maintaining our own commercial-scale cGMP manufacturing facility;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales, and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval; and
- the costs of operating as a public company.

We will require additional capital to achieve our business objectives. Additional funds may not be available on a timely basis, on favorable terms, or at all, and such funds, if raised, may not be sufficient

to enable us to continue to implement our long-term business strategy. Further, our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions and the recent disruptions to and volatility in the credit and financial markets in the United States and worldwide resulting from the ongoing COVID-19 pandemic. If we are unable to raise sufficient additional capital, we could be forced to curtail our planned operations and the pursuit of our growth strategy.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial revenue, we may finance our cash needs through a combination of equity offerings, government or private party grants, debt financings and license and collaboration agreements. We do not currently have any other committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams or product candidates, grant licenses on terms that may not be favorable to us or commit to future payment streams. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Risks Related to the Development of our Product Candidates

We are very early in our development efforts and all of our product candidates are in preclinical development. If we are unable to successfully develop, receive regulatory approval for and commercialize our product candidates for these or any other indications, or successfully develop any other product candidates, or experience significant delays in doing so, our business will be harmed.

We are very early in our development efforts and all of our product candidates are still in preclinical development. We expect to commence a Phase 1/2 clinical trial of TSHA-101 by the end of 2020 under a Clinical Trial Agreement, or CTA, with Health Canada, and we intend to initiate a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. Each of our programs and product candidates will require additional preclinical and/or clinical development, regulatory approval, obtaining manufacturing supply, capacity and expertise, building a commercial organization or successfully outsourcing commercialization, substantial investment and significant marketing efforts before we generate any revenue from product sales. We do not have any products that are approved for commercial sale, and we may never be able to develop or commercialize marketable products.

Our ability to generate revenue from our product candidates, which we do not expect will occur for several years, if ever, will depend heavily on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidates that we develop or otherwise may acquire will depend on several factors, including:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable, and clinical trials;

- effective investigational new drug applications, or INDs, from the U.S. Food and Drug Administration, or the FDA, or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for our product candidates;
- sufficiency of our financial and other resources to complete the necessary preclinical studies and clinical trials;
- successful enrollment and completion of clinical trials, including under the FDA's current Good Clinical Practices, or GCPs, and current Good Laboratory Practices;
- successful development of, or making arrangements with third-party manufacturers for, our commercial manufacturing processes for any of our product candidates that receive regulatory approval;
- receipt of timely marketing approvals from applicable regulatory authorities;
- launching commercial sales of products, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of our products, including method of administration, if approved, by patients, the medical community and third-party payors, for their approved indications;
- the prevalence and severity of adverse events experienced with TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidates;
- the availability, perceived advantages, cost, safety and efficacy of alternative therapies for any product candidate, and any indications for such product candidate, that we develop;
- our ability to produce TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidates we develop on a commercial scale;
- obtaining and maintaining patent, trademark and trade secret protection and regulatory exclusivity for our product candidates and otherwise protecting our rights in our intellectual property portfolio;
- maintaining compliance with regulatory requirements, including cGMPs, and complying effectively with other procedures;
- obtaining and maintaining third-party coverage and adequate reimbursement and patients' willingness to pay out-of-pocket in the absence of such coverage and adequate reimbursement; and
- maintaining a continued acceptable safety, tolerability and efficacy profile of the products following approval.

If we are not successful with respect to one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize the product candidates we develop, which would materially harm our business. If we do not receive marketing approvals for any product candidate we develop, we may not be able to continue our operations.

We intend to identify and develop novel gene therapy product candidates, which makes it difficult to predict the time, cost and potential success of product candidate development.

Our strategy is to identify, develop and commercialize gene therapy product candidates using an adeno-associated virus serotype 9, or AAV9, capsid for intrathecal delivery of therapeutic transgenes to certain kinds of cells. Our future success depends on the successful development of these novel therapeutic approaches. To date, very few products that utilize gene transfer have been approved in

the United States or Europe and no gene therapy products that utilize an intrathecal method of administration have been approved. There have been a limited number of clinical trials of gene transduction technologies, with only two product candidates ever approved by the FDA.

Although AAV9 has been tested in numerous clinical trials and is used in two currently approved products, we cannot be certain that our AAV9 product candidates will successfully complete preclinical studies and clinical trials, or that they will not cause significant adverse events or toxicities. We also cannot be certain that we will be able to avoid triggering toxicities in our future preclinical studies or clinical trials or that our intrathecal method of administration will not cause unforeseen side effects or other challenges. Any such results could impact our ability to develop a product candidate, including our ability to enroll patients in our clinical trials. As a result of these factors, it is more difficult for us to predict the time and cost of product candidate development, and we cannot predict whether the application of our approach to gene therapy, or any similar or competitive programs, will result in the identification, development, and regulatory approval of any product candidates, or that other gene therapy programs will not be considered better or more attractive. There can be no assurance that any development problems we experience in the future related to our current gene therapy product candidates or any of our research programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays and challenges in achieving sustainable, reproducible, and scalable production. Any of these factors may prevent us from completing our preclinical studies or clinical trials or commercializing any product candidates we may develop on a timely or profitable basis, if at all.

Because gene therapy is novel and the regulatory landscape that governs any product candidates we may develop is rigorous, complex, uncertain and subject to change, we cannot predict the time and cost of obtaining regulatory approval, if we receive it at all, for any product candidates we may develop.

The regulatory requirements that will govern any novel gene therapy product candidates we develop are not entirely clear and are subject to change. Within the broader genetic medicine field, very few therapeutic products have received marketing authorization from the FDA or the European Medicines Agency, or the EMA. Even with respect to more established products that fit into the categories of gene therapies or cell therapies, the regulatory landscape is still developing. Regulatory requirements governing gene therapy products and cell therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial overlap in those responsible for regulation of existing gene therapy products and cell therapy products. For example, in the United States, the FDA has established the Office of Tissues and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review.

Our product candidates will need to meet safety and efficacy standards applicable to any new biologic under the regulatory framework administered by the FDA. In addition to FDA oversight and oversight by institutional review boards, or IRBs, under guidelines promulgated by the National Institutes of Health, or NIH, gene therapy clinical trials are also subject to review and oversight by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment. While the NIH guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The same applies in the European Union. The EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety, and efficacy of advanced-therapy medicinal products. Advanced-therapy medicinal products include gene therapy medicines, somatic-cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any gene therapy product candidate we may develop, but that remains uncertain at this point.

Adverse developments in preclinical studies or clinical trials conducted by others in the field of gene therapy and gene regulation products may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for approval of any product candidates we may develop or limit the use of products utilizing gene regulation technologies, either of which could harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty, and intended use and market of the potential products. The regulatory approval process for product candidates such as ours can be more expensive and take longer than for other, better known, or more extensively studied pharmaceutical or other product candidates. Further, as we are developing novel potential treatments for diseases in which, in some cases, there is little clinical experience with potential new endpoints and methodologies, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results, and the resulting clinical data and results may be more difficult to analyze. In addition, we may not be able to identify or develop appropriate animal disease models to enable or support planned clinical development. Any natural history studies that we may conduct or rely upon in our clinical development may not be accepted by the FDA, EMA or other regulatory authorities. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing gene regulation technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays, or other impediments to our research programs or the commercialization of resulting products. Further, approvals by one regulatory agency may not be indicative of what other regulatory agencies may require for approval.

The regulatory review committees and advisory groups described above and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional preclinical studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates, or lead to significant post-approval limitations or restrictions. As we advance our research programs and develop future product candidates, we will be required to consult with these regulatory and advisory groups and to comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of any product candidates we identify and develop. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of an increased or lengthier regulatory approval process or further restrictions on the development of our product candidates can be costly and could negatively impact our ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

Preclinical studies and clinical trials are expensive, time-consuming, difficult to design and implement and involve an uncertain outcome. Further, we may encounter substantial delays in completing the development of our product candidates.

All of our product candidates are in preclinical development and their risk of failure is high. The clinical trials and manufacturing of our product candidates are, and the manufacturing and marketing of our products, if approved, will be, subject to extensive and rigorous review and regulation by numerous government authorities in the United States and in other countries where we intend to test and market our product candidates. Before obtaining regulatory approvals for the commercial sale of any of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. In particular, because our product candidates are subject to regulation as biological products, we will need to demonstrate that they are safe, pure and potent for use in their target indications. Each product candidate must demonstrate an adequate risk versus benefit profile in its intended patient population and for its intended use.

Clinical testing is expensive and can take many years to complete, and is subject to uncertainty. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Failure can occur at any time during the clinical trial process. Even if our future clinical trials are completed as planned, we cannot be certain that their results will support the safety and effectiveness of our product candidates for their targeted indications or support continued clinical development of such product candidates. Our future clinical trial results may not be successful.

In addition, even if such trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more trials could be required before we submit our product candidates for approval. This is particularly true for clinical trials in very rare diseases, such as with TSHA-101 for the treatment of GM2 gangliosidosis, TSHA-118 for the treatment of CLN1 disease (or infantile Batten disease), and TSHA-102 for the treatment of Rett syndrome, where the very small patient population makes it difficult or impossible to conduct two traditional, adequate and well-controlled studies, and therefore the FDA or comparable foreign regulatory authorities are often required to exercise flexibility in approving therapies for such diseases. Moreover, results acceptable to support approval in one jurisdiction may be deemed inadequate by another regulatory authority to support regulatory approval in that other jurisdiction. To the extent that the results of the trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, we may be required to expend significant resources, which may not be available to us, to conduct additional trials in support of potential approval of our product candidates.

To date, we have not initiated or completed any clinical trials required for the approval of our product candidates. We may experience delays in conducting any clinical trials and we do not know whether our clinical trials will begin on time, need to be redesigned, recruit and enroll patients on time or be completed on schedule, or at all. Events that may prevent successful or timely completion of clinical development include:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of clinical trials;
- delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for advanced clinical trials, including our natural history studies;
- delays in developing suitable assays for screening patients for eligibility for trials with respect to certain product candidates;
- delays in reaching agreement with the FDA, EMA or other regulatory authorities as to the design or implementation of our clinical trials;

- obtaining regulatory approval to commence a clinical trial;
- reaching an agreement on acceptable terms with clinical trial sites or prospective contract research organizations, or CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- obtaining IRB approval at each trial site;
- recruiting suitable patients to participate in a clinical trial;
- having patients complete a clinical trial or return for post-treatment follow-up;
- clinical sites, CROs or other third parties deviating from trial protocol or dropping out of a trial;
- failure to perform in accordance with the FDA's GCP requirements, or applicable regulatory guidelines in other countries;
- addressing patient safety concerns that arise during the course of a trial, including occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- adding a sufficient number of clinical trial sites; or
- manufacturing sufficient quantities of product candidate for use in clinical trials.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates or significantly increase the cost of such trials, including:

- we may experience changes in regulatory requirements or guidance, or receive feedback from regulatory authorities that requires us to modify the design of our clinical trials;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we or our investigators might have to suspend or terminate clinical trials of our product candidates for various reasons, including non-compliance with regulatory requirements, a finding that our product candidates have undesirable side effects or other unexpected characteristics, or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate and we may not have funds to cover the costs;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; and
- any future collaborators that conduct clinical trials may face any of the above issues, and may conduct clinical trials in ways they view as advantageous to them but that are suboptimal for us.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- incur unplanned costs;
- be delayed in obtaining marketing approval for our product candidates or not obtain marketing approval at all;
- obtain marketing approval in some countries and not in others;
- obtain marketing approval for indications or patient populations that are not as broad as intended or desired;
- obtain marketing approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings or Risk Evaluation and Mitigation Strategies, or REMS;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trials are being conducted, by the Data Safety Monitoring Board for such trial or by the FDA, EMA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA, EMA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

All of our product candidates will require extensive clinical testing before we are prepared to submit a biologics license application, or BLA, or marketing authorization application, or MAA, for regulatory approval. We cannot predict with any certainty if or when we might complete the clinical development for our product candidates and submit a BLA or MAA for regulatory approval of any of our product candidates or whether any such BLA or MAA will be approved. We may also seek feedback from the FDA, EMA or other regulatory authorities on our clinical development program, and the FDA, EMA or such regulatory authorities may not provide such feedback on a timely basis, or such feedback may not be favorable, which could further delay our development programs.

We cannot predict with any certainty whether or when we might complete a given clinical trial. If we experience delays in the commencement or completion of our clinical trials, or if we terminate a clinical trial prior to completion, the commercial prospects of our product candidates could be harmed, and our ability to generate revenues from our product candidates may be delayed or lost. In addition, any delays in our clinical trials could increase our costs, slow down the development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may harm our business, financial condition and results of operations. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

The regulatory approval processes of the FDA, EMA and comparable foreign authorities are lengthy, time consuming and inherently unpredictable. If we are not able to obtain required regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval or other marketing authorizations by the FDA, EMA and comparable foreign authorities is unpredictable, and it typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, and the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that we may never obtain regulatory approval for any product candidates we may seek to develop in the future. Neither we nor any current or future collaborator is permitted to market any drug product candidates in the United States until we receive regulatory approval of a BLA from the FDA, and we cannot market it in the European Union until we receive approval for a MAA from the EMA, or other required regulatory approval in other countries. To date, we have had only limited discussions with the FDA regarding clinical development programs or regulatory approval for any product candidate within the United States. In addition, we have only had limited discussions with Health Canada, and no discussions with the EMA and other comparable foreign authorities, regarding clinical development programs or regulatory approval for any product candidate outside of the United States.

Prior to obtaining approval to commercialize any drug product candidate in the United States or abroad, we must demonstrate with substantial evidence from well-controlled clinical trials, and to the satisfaction of the FDA or foreign regulatory agencies, that such product candidates are safe, pure and effective for their intended uses. Results from preclinical studies and clinical trials can be interpreted in different ways. Even if we believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. The FDA may also require us to conduct additional preclinical studies or clinical trials for our product candidates either prior to or after approval, or it may object to elements of our clinical development programs.

Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval and marketing authorization process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval and marketing authorization to market our product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

We have invested a significant portion of our time and financial resources in the development of our preclinical product candidates. Our business is dependent on our ability to successfully complete preclinical and clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and any future product candidates in a timely manner.

Even if we eventually complete clinical testing and receive approval of a BLA or foreign marketing application for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates, the FDA, EMA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-marketing clinical trials. The FDA, EMA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited indication or patient population than we originally request, and the FDA, EMA or applicable foreign regulatory agency may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any delay in obtaining, or inability to obtain, applicable regulatory approval or other marketing authorization would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

In addition, the FDA, EMA and other regulatory authorities may change their policies, issue additional regulations or revise existing regulations, or take other actions, which may prevent or delay approval of our future products under development on a timely basis. Such policy or regulatory changes could impose additional requirements upon us that could delay our ability to obtain approvals, increase the costs of compliance or restrict our ability to maintain any marketing authorizations we may have obtained.

We have not yet tested any product candidates in clinical trials. Success in preclinical studies or earlier clinical trials may not be indicative of results in future clinical trials.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Preclinical tests and Phase 1 and Phase 2 clinical trials are primarily designed to test safety, to study pharmacokinetics and pharmacodynamics and to understand the side effects of product candidates at various doses and schedules. Success in preclinical or animal studies and early clinical trials does not ensure that later large-scale efficacy trials will be successful nor does it predict final results. For example, we may be unable to identify suitable animal disease models for our product candidates, which could delay or frustrate our ability to proceed into clinical trials or obtain marketing approval. Our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. Further, both our planned Phase 1/2 clinical trial of TSHA-101 and our planned Phase 1/2 clinical trial of TSHA-118 will involve a small patient population. Because of the small sample sizes, the results of these trials may not be indicative of results of future clinical trials. Further, although other gene therapy clinical trials conducted by others also utilized AAV9 vectors, these trials should not be relied upon as evidence that our planned clinical trials will succeed.

Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

Interim "top-line" and preliminary results from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim top-line or preliminary results from our clinical trials. Interim results from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top-line results also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

Our preclinical studies and clinical trials may fail to demonstrate the safety and efficacy of our product candidates, or serious adverse or unacceptable side effects may be identified during the development of our product candidates, which could prevent or delay regulatory approval and commercialization, increase our costs or necessitate the abandonment or limitation of the development of some of our product candidates.

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are safe, pure and effective for use in each target indication, and failures can occur at any stage of testing. Preclinical studies and clinical trials often fail to demonstrate safety or efficacy of the product candidate studied for the target indication. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation.

While new AAV vectors have been developed to reduce side effects previously reported in third-party gene therapy treatments, and AAV9 has been generally well tolerated in clinical trials and in approved products, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration, which, while not necessarily adverse to the patient's health, could substantially limit the effectiveness of the treatment. For example, in previous third-party clinical trials involving other AAV vectors for gene therapy, some subjects experienced the development of a T-cell antibody response, whereby after the vector is within the target cells, the cellular immune response system triggers the removal of transduced cells by activated T-cells. Other preclinical studies have suggested that high dosages of AAV administration may result in toxicity due to degeneration of the dorsal root ganglia. If our vectors demonstrate a similar effect in other programs, we may decide or be required to perform additional preclinical studies or to halt or delay further clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. Each of our lead product candidates are expected to be administered by intrathecal injection. While this method of administration has been available for decades, its use for therapies is relatively new, no gene therapy is currently approved for intrathecal administration, and it may be perceived as having greater risk than more common methods of administration, such as intravenous injection. If any such adverse events occur, our clinical trials could be suspended or terminated. If we cannot demonstrate that any adverse events were not caused by the drug or administration process or related procedures, the FDA, EMA or foreign regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

If our product candidates are associated with side effects in clinical trials or have characteristics that are unexpected, we may need to abandon their development or limit development to more narrow

uses in which the side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. The FDA or an IRB may also require that we suspend, discontinue, or limit our clinical trials based on safety information, or that we conduct additional animal or human studies regarding the safety and efficacy of our product candidates which we have not planned or anticipated. Such findings could further result in regulatory authorities failing to provide marketing authorization for our product candidates or limiting the scope of the approved indication, if approved. Many product candidates that initially showed promise in early stage testing have later been found to cause side effects that prevented further development of the product candidate.

Additionally, if one or more of our product candidates receives marketing approval, and we or others identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the labels;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients or other requirements subject to a REMS;
- we could be sued and held liable for harm caused to patients;
- we may not be able to achieve or maintain third-party payor coverage and adequate reimbursement; and
- our reputation and physician or patient acceptance of our products may suffer.

There can be no assurance that we will resolve any issues related to any product-related adverse events to the satisfaction of the FDA or foreign regulatory agency in a timely manner or at all. Moreover, any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations and prospects.

As an organization, we are preparing to conduct our first Phase 1/2 clinical trial, and have never conducted pivotal clinical trials, and may be unable to do so for any product candidates we may develop, including TSHA-101, TSHA-118, TSHA-102, TSHA-103 and TSHA-104.

We will need to successfully complete our ongoing and planned clinical trials, including pivotal clinical trials, in order to obtain FDA approval to market our product candidates. Carrying out later-stage clinical trials and the submission of a successful BLA is a complicated process. As an organization, we are preparing to conduct our first Phase 1/2 clinical trial, have not previously conducted any later stage or pivotal clinical trials, have limited experience in preparing, submitting and prosecuting regulatory filings and have not previously submitted a BLA for any product candidate. In addition, we have had limited interactions with the FDA and cannot be certain how many additional clinical trials of our product candidates will be required or how such trials should be designed. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to BLA submission and approval of any product candidate. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in, our planned clinical trials, could prevent us from or delay us in commercializing our product candidates.

The disorders we seek to treat have low prevalence and it may be difficult to identify and enroll patients with these disorders. If we experience delays or difficulties in the enrollment and/or maintenance of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

Successful and timely completion of clinical trials will require that we enroll a sufficient number of patients. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population and competition for patients with other trials. Genetic diseases generally, and especially the rare diseases for which some of our current product candidates are targeted, have low incidence and prevalence. For example, we estimate global incidence of GM2 gangliosidosis, the target indication for TSHA-101, is approximately 1 in 150,000 live births, and accordingly it may be difficult for us to identify and timely recruit a sufficient number of eligible patients to conduct our clinical trials. Further, any natural history studies that we or our collaborators may conduct may fail to provide us with patients for our clinical trials because patients enrolled in the natural history studies may not be good candidates for our clinical trials, or may choose to not enroll in our clinical trials.

Trials may be subject to delays as a result of patient enrollment taking longer than anticipated or patient withdrawal. We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, EMA or foreign regulatory authorities. We cannot predict how successful we will be at enrolling subjects in future clinical trials. Subject enrollment is affected by other factors including:

- the eligibility criteria for the trial in question;
- the size of the patient population and process for identifying patients;
- the perceived risks and benefits of the product candidate in the trial, including relating to AAV9-based gene therapy approaches and intrathecal delivery systems;
- the availability of competing commercially available therapies and other competing therapeutic candidates' clinical trials;
- the willingness of patients to be enrolled in our clinical trials;
- the efforts to facilitate timely enrollment in clinical trials;
- potential disruptions caused by the COVID-19 pandemic, including difficulties in initiating clinical sites, enrolling and retaining participants, diversion of healthcare resources away from clinical trials, travel or quarantine policies that may be implemented, and other factors;
- the patient referral practices of physicians;
- the ability to monitor patients adequately during and after treatment; and
- the proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we expect to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance.

Furthermore, even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining enrollment of such patients in our clinical trials.

We may seek orphan drug designation for some of our product candidates and we may be unsuccessful, or may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity, for product candidates for which we obtain orphan drug designation.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs or biologics intended to treat relatively small patient populations as orphan drug products. Under the Orphan Drug Act, the FDA may designate a drug or biologic as an orphan drug if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population of 200,000 or more in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States.

In the United States, orphan drug designation entitled a party to financial incentives such as tax advantages and user fee waivers. Opportunities for grant funding toward clinical trial costs may also be available for clinical trials of drugs or biologics for rare diseases, regardless of whether the drugs or biologics are designated for the orphan use. In addition, if a drug or biologic with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a seven year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. If our competitors are able to obtain orphan drug exclusivity prior to us, for products that constitute the “same drug” and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

We have obtained orphan drug designation from the FDA for TSHA-101 for treatment of GM2 gangliosidosis and TSHA-103 for the treatment of SLC6A1-related disorder. In addition, TSHA-118 has received orphan drug designation for the treatment of CLN1 disease from the FDA and EMA. We may seek orphan designation for certain of our other current and future product candidates. However, we may be unsuccessful in obtaining orphan drug designation for these or other product candidates, and may be unable to maintain the benefits associated with orphan drug designation. Even if we obtain orphan drug exclusivity for any of our product candidates, that exclusivity may not effectively protect those product candidates from competition because different drugs can be approved for the same condition, and orphan drug exclusivity does not prevent the FDA from approving the same or a different drug in another indication. Even after an orphan drug is granted orphan exclusivity and approved, the FDA can subsequently approve a later application for the same drug for the same condition before the expiration of the seven-year exclusivity period if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan-drug-exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We have received rare pediatric disease designation for TSHA-101 for the treatment of GM2 gangliosidosis, TSHA-118 for the treatment of CLN1 disease and TSHA-103 for the treatment of SLC6A1 haploinsufficiency, and we have applied for rare pediatric disease designation for TSHA-102 for the treatment of Rett syndrome and TSHA-104 for the treatment of SURF1 deficiency. However, a marketing application for TSHA-101, TSHA-118, TSHA-102, TSHA-103 and TSHA-104, if approved, may not meet the eligibility criteria for a priority review voucher or the rare pediatric disease designation program may sunset before FDA is able consider us for a voucher.

We have received rare pediatric disease designation for TSHA-101 for the treatment of GM2 gangliosidosis (Tay-Sachs Disease and Sandhoff Disease), TSHA-118 for the treatment of CLN1 disease and TSHA-103 for the treatment of SLC6A1 haploinsufficiency, and we have applied for rare pediatric disease designation for TSHA-102 for the treatment of Rett syndrome and TSHA-104 for the treatment of SURF1 deficiency. Designation of a drug or biologic as a product for a rare pediatric disease does not guarantee that a BLA for such drug or biologic will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Under the Federal Food, Drugs, and Cosmetic Act, or FDCA, we will need to request a rare pediatric disease priority review voucher in our original BLA for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and any other candidates for which we submit a marketing application. The FDA may determine that a BLA for TSHA-101, TSHA-118, TSHA-102, TSHA-103 or TSHA-104, if approved, does not meet the eligibility criteria for a priority review voucher, including for the following reasons:

- GM2 gangliosidosis, CLN1 disease, Rett syndrome, SLC6A1 haploinsufficiency or SURF1 deficiency no longer meet the definition of a rare pediatric disease;
- the BLA contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in a BLA;
- the BLA is not deemed eligible for priority review;
- the BLA does not rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population (that is, if the BLA does not contain sufficient clinical data to allow for adequate labeling for use by the full range of affected pediatric patients); or
- the BLA is approved for a different adult indication than the rare pediatric disease for which TSHA-101, TSHA-118, TSHA-102, TSHA-103 or TSHA-104 are designated.

The authority for the FDA to award rare pediatric disease priority review vouchers for drugs that have received rare pediatric disease designation prior to September 30, 2020 currently expires on September 30, 2022. If the BLA for TSHA-101, TSHA-118, TSHA-102, TSHA-103 or TSHA-104 is not approved prior to September 30, 2022 for any reason, regardless of whether it meets the criteria for a rare pediatric disease priority review voucher, it will not be eligible for a priority review voucher. However, it is also possible the authority for FDA to award rare pediatric disease priority review vouchers will be further extended through Federal lawmaking.

We have received fast track designation for TSHA-118 for the treatment of CLN1 disease, and we may seek fast track designation for our other product candidates. Even if received, fast track designation may not actually lead to a faster review or approval process and does not increase the likelihood that our product candidates will receive marketing approval.

We have received fast track designation for TSHA-118 for the treatment of neurocognitive manifestations of the patients with CLN1 disease, and we may seek fast track designation for our other product candidates. If a drug or biologic is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet medical needs for this

condition, the sponsor may apply for FDA fast track designation for a particular indication. There is no assurance that the FDA will grant this status to any of our other proposed product candidates. If granted, fast track designation makes a product eligible for more frequent interactions with FDA to discuss the development plan and clinical trial design, as well as rolling review of the application, which means that the company can submit completed sections of its marketing application for review prior to completion of the entire submission. Marketing applications of products candidates with fast track designation may qualify for priority review under the policies and procedures offered by the FDA, but the fast track designation does not assure any such qualification or ultimate marketing approval by the FDA. The FDA has broad discretion whether or not to grant fast track designation, so even if we believe a particular product candidate is eligible for this designation, there can be no assurance that the FDA would decide to grant it. Even if we do receive fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures, and receiving a fast track designation does not provide any assurance of ultimate FDA approval. In addition, the FDA may withdraw fast track designation at any time if it believes that the designation is no longer supported by data from our clinical development program.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we must focus on development programs and product candidates that we identify for specific indications. As such, we are currently primarily focused on the development of our first five programs: TSHA-101 (GM2 gangliosidosis), TSHA-118 (CLN1 disease), TSHA-102 (Rett syndrome), TSHA-103 (SLC6A1 haploinsufficiency) and TSHA-104 (SURF1 deficiency). As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications for these product candidates that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We plan to conduct and may in the future conduct additional clinical trials for our product candidates outside the United States, and the FDA and similar foreign regulatory authorities may not accept data from such trials.

We plan to conduct a clinical trial in Canada and may in the future choose to conduct additional clinical trials outside the United States, including in Australia, Europe or other foreign jurisdictions. The acceptance of trial data from clinical trials conducted outside the United States by the FDA may be subject to certain conditions. In cases where data from clinical trials conducted outside the United States are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the United States population and United States medical practice; (ii) the trials were performed by clinical investigators of recognized competence and (iii) the data may be considered valid without the need for an on-site inspection by the FDA or, if the FDA considers such an inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. Additionally, the FDA's clinical trial requirements, including sufficient size of patient populations and statistical powering, must be met. Many foreign regulatory bodies have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign

jurisdictions where the trials are conducted. There can be no assurance that the FDA or any similar foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any similar foreign regulatory authority does not accept such data, it would result in the need for additional trials, which would be costly and time-consuming and delay aspects of our business plan, and which may result in our product candidates not receiving approval or clearance for commercialization in the applicable jurisdiction.

We may not be successful in our efforts to build a pipeline of additional product candidates.

Our business model is centered on developing therapies for patients with rare, monogenic central nervous system disorders by establishing focused selection criteria to select, develop and advance product candidates that we believe will have a high probability of technical and regulatory success through development into commercialization. We may not be able to continue to identify and develop new product candidates, including from our next-generation platform technologies, in addition to the pipeline of product candidates that we have established through our collaboration with The University of Texas Southwestern Medical Center, or UT Southwestern. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. For example, they may be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be drugs that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed.

From time to time, we may estimate the timing of the accomplishment of various scientific, clinical, regulatory, manufacturing and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of preclinical studies and clinical trials and the submission of regulatory filings, including IND submissions. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are, and will be, based on a variety of assumptions. The actual timing of these milestones can vary significantly compared to our estimates, in some cases for reasons beyond our control. We may experience numerous unforeseen events during, or as a result of, any future clinical trials that we conduct that could delay or prevent our ability to receive marketing approval or commercialize our product candidates.

Our business and operations may be adversely affected by the evolving and ongoing COVID-19 global pandemic.

Our business and operations may be adversely affected by the effects of the recent and evolving COVID-19 virus, which was declared by the World Health Organization as a global pandemic. The COVID-19 pandemic has resulted in travel and other restrictions in order to reduce the spread of the disease, including public health directives and orders in the United States and the European Union that, among other things and for various periods of time, directed individuals to shelter at their places of residence, directed businesses and governmental agencies to cease non-essential operations at physical locations, prohibited certain non-essential gatherings and events and ordered cessation of non-essential travel. Future remote work policies and similar government orders or other restrictions on the conduct of business operations related to the COVID-19 pandemic may negatively impact productivity and may disrupt our ongoing research and development activities and our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. Further,

such orders also may impact the availability or cost of materials, which would disrupt our supply chain and manufacturing efforts and could affect our ability to conduct ongoing and planned clinical trials and preparatory activities.

Although our planned clinical trials have not been impacted by the COVID-19 pandemic to date, we may experience related disruptions in the future that could severely impact our clinical trials, including:

- delays, difficulties or a suspension in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff;
- interruptions in our ability to manufacture and deliver drug supply for trials;
- diversion of healthcare resources away from the conduct of clinical trials, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- changes in local regulations as part of a response to the COVID-19 outbreak that may require us to change the ways in which our clinical trials are conducted, which may result in unexpected costs, or to discontinue the clinical trials altogether;
- interruption of key clinical trial activities, such as clinical trial site monitoring, and the ability or willingness of subjects to travel to trial sites due to limitations on travel imposed or recommended by federal or state governments, employers and others;
- limitations in employee resources that would otherwise be focused on the conduct of our clinical trials, including because of sickness of employees or their families or the desire of employees to avoid contact with large groups of people;
- delays in necessary interactions with local regulators, ethics committees and other important agencies and contractors due to limitations in employee resources or forced furlough of government employees; and
- refusal of the FDA to accept data from clinical trials in these affected geographies.

The spread of COVID-19, which has caused a broad impact globally, may materially affect us economically. While the potential economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, a widespread pandemic could result in significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the spread of COVID-19 could materially affect our business and the value of our common stock.

The global COVID-19 pandemic continues to rapidly evolve. The extent to which the COVID-19 pandemic impacts our business and operations, including our clinical development and regulatory efforts, will depend on future developments that are highly uncertain and cannot be predicted with confidence at the time of this prospectus, such as the ultimate geographic spread of the disease, the duration of the outbreak, the duration and effect of business disruptions and the short-term effects and ultimate effectiveness of the travel restrictions, quarantines, social distancing requirements and business closures in the United States and other countries to contain and treat the disease. Accordingly, we do not yet know the full extent of potential delays or impacts on our business, our clinical and regulatory activities, healthcare systems or the global economy as a whole. However, these impacts could adversely affect our business, financial condition, results of operations and growth prospects.

In addition, to the extent the ongoing COVID-19 pandemic adversely affects our business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties described in this “Risk Factors” section.

The United Kingdom's withdrawal from the European Union may have a negative effect on global economic conditions, financial markets and our business.

Following the result of a referendum in 2016, the United Kingdom left the European Union on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed to by the United Kingdom and the European Union, the United Kingdom will be subject to a transition period until December 31, 2020, or the Transition Period, during which European Union rules will continue to apply. Negotiations between the United Kingdom and the European Union are expected to continue in relation to the customs and trading relationship between the United Kingdom and the European Union following the expiry of the Transition Period.

Since a significant proportion of the regulatory framework in the United Kingdom is applicable to our business and our product candidates is derived from European Union directives and regulations, Brexit, following the Transition Period, could materially impact the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the United Kingdom or the European Union. For example, as a result of the uncertainty surrounding Brexit, the EMA relocated to Amsterdam from London. Following the Transition Period, the United Kingdom will no longer be covered by the centralized procedures for obtaining European Union-wide marketing and manufacturing authorizations from the EMA and, unless a specific agreement is entered into, a separate process for authorization of drug products will be required in the United Kingdom, the potential process for which is currently unclear. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom or the European Union and limit our ability to generate revenue and achieve and sustain profitability. In addition, we may be required to pay taxes or duties or be subjected to other hurdles in connection with the importation of our product candidates into the European Union, or we may incur expenses in establishing a manufacturing facility in the European Union in order to circumvent such hurdles. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom or the European Union for our product candidates, or incur significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the impacted nations and the United Kingdom. It is also possible that Brexit may negatively affect our ability to attract and retain employees, particularly those from the European Union.

Risks Related to the Manufacturing of our Product Candidates

Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.

The manufacture of gene therapy products is technically complex and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events may delay the availability of material for our clinical studies.

We currently rely on the UT Southwestern Gene Therapy program to manufacture our product candidates. Although we intend to establish our own manufacturing facility to provide clinical and commercial supply of our product candidates, we expect to rely on UT Southwestern for our manufacturing needs for the foreseeable future. To date, UT Southwestern has met our manufacturing requirements and quality standards for our program materials, and we expect that UT Southwestern

will be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial scale demands. We believe that there are alternate sources of supply for our program materials that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure of us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical trials or enforcement action from the FDA, EMA or foreign regulatory authorities. If we or our manufacturers were to fail to comply with the FDA, EMA or other regulatory authority, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our potential future dependence upon others for the manufacture of our product candidates may also adversely affect our future profit margins and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

Biological products are inherently difficult to manufacture. Although we believe that the manufacture of our product candidates may be simplified due to their shared raw materials and other similarities, we cannot be certain that this will be the case and we may be required to develop manufacturing methods that ultimately differ significantly between product candidates, which would require that we invest substantial time and capital to develop suitable manufacturing methods. Our program materials are manufactured using technically complex processes requiring specialized equipment and facilities, highly specific raw materials, cells, and reagents, and other production constraints. Our production process requires a number of highly specific raw materials, cells and reagents with limited suppliers. Even though we aim to have backup supplies of raw materials, cells and reagents whenever possible, we cannot be certain they will be sufficient if our primary sources are unavailable. A shortage of a critical raw material, cell line, or reagent, or a technical issue during manufacturing may lead to delays in clinical development or commercialization plans. We are particularly susceptible to any shortages, delays or our inability to obtain suitable AAV9 raw materials, given that all of our current and planned product candidates require this starting material. Any changes in the manufacturing of components of the raw materials we use could result in unanticipated or unfavorable effects in our manufacturing processes, resulting in delays.

We and our contract manufacturers for AAV9 are subject to significant regulation with respect to manufacturing our products. The third-party manufacturing facilities on which we rely, and any manufacturing facility that we may have in the future, may have limited capacity or fail to meet the applicable stringent regulatory requirements.

We currently have relationships with a limited number of suppliers for the manufacturing of plasmids and viruses, components of our product candidates. However, if we experience slowdowns or problems with our facility or those of our manufacturing partners and are unable to establish or scale our internal manufacturing capabilities, we will need to continue to contract with manufacturers that can produce the preclinical, clinical and commercial supply of our products. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to license such intellectual property rights on reasonable commercial terms or to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for components our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials in the European Union must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We are at an increased risk given that our product candidates have been and for the foreseeable future will be produced on the same manufacturing lines, which could, for example, lead to issues with cross-contamination. We or our contract manufacturers must supply all necessary documentation in support of a BLA or MAA on a timely basis. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted, and they could put a hold on one or more of our clinical trials if the facilities of our contract development and manufacturing organizations, or CDMOs, do not pass such audit or inspections. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, inspect or audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could harm our business. If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be harmed. Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA and/or MAA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully, if approved. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

We depend on third-party suppliers for materials used in the manufacture of our product candidates, and the loss of these third-party suppliers or their inability to supply us with adequate materials could harm our business.

We rely on third-party suppliers for certain materials and components required for the production of our product candidates. Our dependence on these third-party suppliers and the challenges we may face in obtaining adequate supplies of materials involve several risks, including limited control over pricing, availability, and quality and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture gene therapy products. As a small company, our negotiation leverage is limited and we are likely to get lower priority than our competitors that are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements. Any supply interruption in limited or sole sourced raw materials could materially harm our ability to manufacture our product candidates until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of our product candidates, including limiting supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business.

Any contamination or interruption in our manufacturing process, shortages of raw materials or failure of our suppliers of plasmids and viruses to deliver necessary components could result in delays in our clinical development or marketing schedules.

Given the nature of gene therapy manufacturing, there is a risk of contamination. Any contamination could adversely affect our ability to produce product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage. Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could adversely affect our development timelines and our business, financial condition, results of operations and prospects.

Changes in methods of product candidate manufacturing or formulation may result in additional costs or delay.

As product candidates proceed through preclinical studies to late-stage clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to optimize processes and product characteristics. Such changes carry the risk that they will not achieve our intended objectives. Any such changes could cause our product candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or FDA approval. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue. In addition, we may be required to make significant changes to our upstream and downstream processes across our pipeline, which could delay the development of our future product candidates.

Risks Related to the Commercialization of our Product Candidates

Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant revenue and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy, safety and potential advantages compared to alternative treatments;
- our ability to offer our products for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- product labeling or product insert requirements of the FDA or foreign regulatory authorities, including any limitations or warnings contained in a product's approved labeling, including any black box warning or REMS;
- the willingness of the target patient population to try new treatments and of physicians to prescribe these treatments;
- our ability to hire and retain a sales force in the United States;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and any other product candidates, once approved;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products together with other medications.

Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact the development or commercial success of our current and future product candidates.

Our potential therapeutic products involve introducing genetic material into a patient's cells via intrathecal administration. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. For example, in 2003, trials using early versions of murine gamma-retroviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although none of our current product candidates utilize murine gamma-retroviral vectors, our

product candidates use AAV9 viral vectors. Among the risks in any gene therapy product based on viral vectors are the risks of immunogenicity, elevated liver enzymes, and insertional oncogenesis. If any of our vectors demonstrate a similar effect we may decide or be required to halt or delay further clinical development of any product candidates that utilize that vector. Adverse events in our or others' clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical trials or in any clinical trials conducted by other companies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. In addition, for our regulated gene replacement therapy candidates that require that the expression of a therapeutic transgene be tightly regulated, such as TSHA-104, we may inadvertently cause overexpression, which could lead to numerous issues, including safety and toxicity concerns. Furthermore, these regulatory gene replacement therapy candidates require the insertion of miRNA targets into the viral genome, which is a technology that to our knowledge is not present in any approved gene therapy products. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

If we are unable to establish sales, marketing and distribution capabilities for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidate that may receive regulatory approval, we may not be successful in commercializing those product candidates if and when they are approved.

We do not have sales or marketing infrastructure. To achieve commercial success for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidate for which we may obtain marketing approval, we will need to establish a sales and marketing organization. In the future, we expect to build a focused sales and marketing infrastructure to market some of our product candidates in the United States, if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to market our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians in order to educate physicians about our product candidates, once approved;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and are forced to enter into arrangements with, and rely on, third parties to perform these services, our revenue and

our profitability, if any, are likely to be lower than if we had developed such capabilities ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

The affected populations for our other product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.

We currently focus our research and product development on several indications that are orphan diseases. However, our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. These estimates may prove to be incorrect and new studies may further reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our product candidate or patients may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Incidence and prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have used in developing an estimated incidence and prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the incidence and prevalence estimates included in this prospectus should be viewed with caution. Further, the data and statistical information used in this prospectus, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.

Drug development, particularly in the gene therapy field, is highly competitive and subject to rapid and significant technological advancements. As a significant unmet medical need exists in the neurology field, particularly for the treatment of neurodegenerative diseases, neurodevelopmental disorders and genetic epilepsies, there are several large and small pharmaceutical companies focused on delivering therapeutics for the treatment of these diseases. Further, it is likely that additional drugs will become available in the future for the treatment of our target indications.

We believe that the majority of our programs will face limited competition as there are no approved disease-modifying therapies for the treatment of the GM2 gangliosidosis, CLN1 disease, Rett syndrome, SLC6A1 haploinsufficiency disorder, SURF1 deficiency, SLC13A5 disorder, Fragile X syndrome, Angelman syndrome or the other development programs in our pipeline. However, we are aware that our competitors are developing product candidates for the treatment of diseases that our product candidates will target. With respect to TSHA-101, we are aware that Axovant is developing AXO-AAV-GM2 for the treatment of GM2 gangliosidosis, and with respect to TSHA-102, we are aware that Novartis is developing AVXS-201 for the treatment of Rett syndrome. We are also aware that the

Rett Syndrome Research Trust, Amicus Therapeutics and Sarepta Therapeutics have disclosed the existence of discovery-stage gene therapy programs for the treatment of Rett syndrome. With respect to TSHA-118, we are aware that Amicus, in collaboration with Nationwide Children's Hospital, is developing a gene therapy product candidate for CLN1 disease.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, as well as in obtaining regulatory approvals of those product candidates in the United States and in foreign countries. Our current and potential future competitors may also have significantly more experience commercializing drugs, particularly gene therapy and other biological products, that have been approved for marketing. Mergers and acquisitions in the pharmaceutical and biotechnology industries could result in even more resources being concentrated among a small number of our competitors.

We will face competition from other drugs or from other non-drug products currently approved or that will be approved in the future in the neurology field, including for the treatment of diseases and disorders in the therapeutic categories we intend to target. Therefore, our ability to compete successfully will depend largely on our ability to:

- develop and commercialize drugs that are superior to other products in the market;
- demonstrate through our clinical trials that our product candidates are differentiated from existing and future therapies;
- attract qualified scientific, product development and commercial personnel;
- obtain patent or other proprietary protection for our medicines;
- obtain required regulatory approvals;
- obtain coverage and adequate reimbursement from, and negotiate competitive pricing with, third-party payors; and
- successfully collaborate with pharmaceutical companies in the discovery, development and commercialization of new medicines.

The availability of our competitors' products could limit the demand, and the price we are able to charge, for any product candidate we develop. The inability to compete with existing or subsequently introduced drugs would have an adverse impact on our business, financial condition and prospects. In addition, the reimbursement structure of approved gene therapies by other companies could impact the anticipated reimbursement structure of our gene therapies, if approved, and our business, financial condition, results of operations and prospects.

Established pharmaceutical companies may invest heavily to accelerate discovery and development of novel compounds or to in-license novel compounds that could make our product candidates less competitive. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful. Accordingly, our competitors may succeed in obtaining patent protection, discovering, developing, receiving regulatory and marketing approval for, or commercializing, drugs before we do, which would have an adverse impact on our business and results of operations.

Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the ACA, includes a subtitle called the Biologics Price Competition

and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biological products.

There is a risk that any of our product candidates approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain marketing approval for biosimilars referencing our candidates, if approved, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and potential adverse consequences.

The success of our product candidates will depend significantly on coverage and adequate reimbursement or the willingness of patients to pay for these procedures.

We believe our success depends on obtaining and maintaining coverage and adequate reimbursement for our product candidates, including TSHA-101 for the treatment of GM2 gangliosidosis, TSHA-118 for the treatment of CLN1 disease, TSHA-102 for the treatment of Rett syndrome, TSHA-103 for the treatment of SLC6A1 haploinsufficiency and TSHA-104 for the treatment of SURF1 deficiency, and the extent to which patients will be willing to pay out-of-pocket for such products, in the absence of reimbursement for all or part of the cost. In the United States and in other countries, patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. The availability of coverage and adequacy of reimbursement for our products by third-party payors, including government health care programs (e.g., Medicare, Medicaid, TRICARE), managed care providers, private health insurers, health maintenance organizations, and other organizations is essential for most patients to be able to afford medical services and pharmaceutical products such as our product candidates. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided are made on a payor-by-payor basis. One payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage, and adequate reimbursement. The principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS. CMS decides whether and to what extent products will be covered and reimbursed under Medicare and private payors tend to follow CMS to a substantial degree.

Third-party payors determine which products and procedures they will cover and establish reimbursement levels. Even if a third-party payor covers a particular product or procedure, the resulting reimbursement payment rates may not be adequate. Patients who are treated in-office for a medical condition generally rely on third-party payors to reimburse all or part of the costs associated with the procedure, including costs associated with products used during the procedure, and may be unwilling to undergo such procedures in the absence of such coverage and adequate reimbursement. Physicians may be unlikely to offer procedures for such treatment if they are not covered by insurance and may be unlikely to purchase and use our product candidates, if approved, for our stated indications unless coverage is provided and reimbursement is adequate. In addition, for products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs.

Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that a procedure is safe, effective and medically necessary; appropriate for the specific patient; cost-effective; supported by peer-reviewed medical journals; included in clinical practice guidelines; and neither cosmetic, experimental, nor investigational. Further, increasing efforts by third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for newly approved products and, as a result, they may not cover or provide adequate payment for our product candidates. In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA or comparable regulatory approvals. Additionally, we may also need to provide discounts to purchasers, private health plans or government healthcare programs. Our product candidates may nonetheless not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. We expect to experience pricing pressures from third-party payors in connection with the potential sale of any of our product candidates.

Foreign governments also have their own healthcare reimbursement systems, which vary significantly by country and region, and we cannot be sure that coverage and adequate reimbursement will be made available with respect to the treatments in which our products are used under any foreign reimbursement system.

There can be no assurance that TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidate, if approved for sale in the United States or in other countries, will be considered medically reasonable and necessary, that it will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available or that reimbursement policies and practices in the United States and in foreign countries where our products are sold will not adversely affect our ability to sell our product candidates profitably, if they are approved for sale.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or drugs caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or drugs that we may develop;

- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Our business and operations would suffer in the event of computer system failures, cyberattacks or a deficiency in our cybersecurity.

Despite the implementation of security measures, our internal computer systems, and those of third parties on which we rely, are vulnerable to damage from computer viruses, malware, natural disasters, terrorism, war, telecommunication and electrical failures, cyberattacks or cyber-intrusions over the Internet, attachments to emails, persons inside our organization, or persons with access to systems inside our organization. The risk of a security breach or disruption, particularly through cyberattacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur material legal claims and liability and damage to our reputation, and the further development of our product candidates could be delayed.

We are subject to a variety of privacy and data security laws, and our failure to comply with them could harm our business.

We maintain a large quantity of sensitive information, including confidential business and personal information in connection with the conduct of our clinical trials and related to our employees, and we are subject to laws and regulations governing the privacy and security of such information. In the United States, there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, and federal and state consumer protection laws. Each of these constantly evolving laws can be subject to varying interpretations. In May 2018, a new privacy regime, the General Data Protection Regulation or the GDPR, took effect in the European Economic Area, or the EEA. The GDPR governs the collection, use, disclosure, transfer or other processing of personal data of European persons. Among other things, the GDPR imposes requirements regarding the security of personal data and notification of data processing obligations to the competent national data processing authorities, changes the lawful bases on which personal data can be processed, expands the definition of personal data and requires

changes to informed consent practices, as well as more detailed notices for clinical trial subjects and investigators. In addition, the GDPR increases the scrutiny of transfers of personal data from clinical trial sites located in the EEA to the United States and other jurisdictions that the European Commission does not recognize as having "adequate" data protection laws, and imposes substantial fines for breaches and violations (up to the greater of €20 million or 4% of our consolidated annual worldwide gross revenue). The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR.

Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of operations. Furthermore, the laws are not consistent, and compliance in the event of a widespread data breach is costly. In addition, states are constantly adopting new laws or amending existing laws, requiring attention to frequently changing regulatory requirements. For example, California enacted the California Consumer Privacy Act, or the CCPA, on June 28, 2018, and took effect on January 1, 2020, became enforceable by the California Attorney General on July 1, 2020, and has been dubbed the first "GDPR-like" law in the United States. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing and receive detailed information about how their personal information is used by requiring covered companies to provide new disclosures to California consumers (as that term is broadly defined) and provide such consumers new ways to opt-out of certain sales of personal information. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability. While there is currently an exception for protected health information that is subject to HIPAA and clinical trial regulations, as currently written, the CCPA may impact certain of our business activities. Some observers have noted that the CCPA could mark the beginning of a trend toward more stringent privacy legislation in the United States. Other states are beginning to pass similar laws. For example, an amendment to Nevada's privacy laws, which went into effect October 1, 2019, requires us to offer to consumers the right to opt-out of the sale of their personal information.

Risks Related to Our Dependence on Third Parties

We currently rely exclusively on our collaboration with UT Southwestern for our preclinical research and development programs, including for discovering, preclinically developing and conducting all IND-enabling studies for our lead product candidates and our near-term future pipeline. Failure or delay of UT Southwestern to fulfill all or part of its obligations to us under the agreement, a breakdown in collaboration between the parties or a complete or partial loss of this relationship would materially harm our business.

Our collaboration with UT Southwestern is critical to our business. We have entered into a Research, Collaboration and License Agreement, or the UT Southwestern Agreement, with UT Southwestern to discover and develop certain AAV vector-based therapeutics, and the product candidates developed under such collaboration currently represent all of our pipeline and discovery programs. We currently rely exclusively on UT Southwestern for all of our preclinical research and development capabilities, and in particular the UT Southwestern Gene Therapy Program under the direction of Drs. Steven Gray and Berge Minassian. Pursuant to the UT Southwestern Agreement, UT Southwestern is primarily responsible for discovery, preclinical development activities, including all IND-enabling non-clinical studies and research grade manufacturing, and other collaborative activities set forth in the plan for the funded research including leading interactions with FDA and other

regulatory authorities. Although we plan to be the sponsor for each product candidate's IND, UT Southwestern will be the holder of the Health Canada CTA for TSHA-101. Either party has the right in certain circumstances to terminate the collaboration pursuant to the terms of the UT Southwestern Agreement. If UT Southwestern delays or fails to perform its obligations under the UT Southwestern Agreement, disagrees with our interpretation of the terms of the collaboration or our discovery plan or terminates our existing agreement, our pipeline of product candidates would be significantly adversely affected and our prospects will be materially harmed.

The term of the research funding portion of the UT Southwestern Agreement, under which we have the ability to acquire exclusive rights to additional gene therapy products for rare, monogenic central nervous system indications, expires in November 2021, subject to mutual agreement to extend research funding pursuant to sponsored research agreements. If we seek to extend the research portion of our collaboration, we will need to negotiate a new or amended agreement, which may not be available to us on equally favorable terms, if at all. UT Southwestern has also entered into collaborations with third parties, including certain of our competitors, addressing targets and disease indications outside the scope of our collaboration. As a result, UT Southwestern may have competing interests with respect to their priorities and resources. We may have disagreements with UT Southwestern with respect to the interpretation of the UT Southwestern Agreement, use of resources or otherwise that could cause our relationship with UT Southwestern to deteriorate. As a result, UT Southwestern may reduce their focus on, and resources allocated to, our programs, potentially delaying or terminating our ability to advance product candidates through preclinical studies. Additionally, if either of Dr. Gray or Dr. Minassian were to leave UT Southwestern or to otherwise no longer be meaningfully involved with us, our preclinical research and development capabilities may be substantially reduced.

Further, under the UT Southwestern Agreement, UT Southwestern is primarily responsible for prosecuting and maintaining our licensed intellectual property, and it may fail to properly prosecute, maintain or defend such intellectual property. In such event, if we are unable to otherwise maintain or defend such intellectual property, we could face the potential invalidation of the intellectual property or be subjected to litigation or arbitration, any of which would be time-consuming and expensive. To enforce the licensed intellectual property rights under the UT Southwestern Agreement, we will need to coordinate with UT Southwestern, which could slow down or hamper our ability to enforce our licensed intellectual property rights. In such event, we could face increased competition that could materially and adversely affect our business.

We intend to rely on third parties to conduct a significant portion of our existing clinical trials and potential future clinical trials for product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We intend to engage CROs to conduct our planned clinical trials, including our planned Phase 1/2 trials of TSHA-101 and TSHA-118. We expect to continue to rely on third parties, including clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials. Any of these third parties may terminate their engagements with us, some in the event of an uncured material breach and some at any time for convenience. If any of our relationships with these third parties terminate, we may not be able to timely enter into arrangements with alternative third parties or to do so on commercially reasonable terms, if at all. Switching or adding CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects. Further, the performance of our CROs may also be

interrupted by the ongoing COVID-19 pandemic, including due to travel or quarantine policies, heightened exposure of CRO staff who are healthcare providers to COVID-19 or prioritization of resources toward the pandemic.

In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. Consequently, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase substantially and our ability to generate revenue could be delayed significantly.

We rely on these parties for execution of our preclinical studies and clinical trials, and generally do not control their activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CROs or other third parties, including trial sites, fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. In addition, our clinical trials must be conducted with product produced under cGMP conditions. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

In addition, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA. The FDA may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidates.

We also expect to rely on other third parties to store and distribute product supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential revenue.

We may seek collaborations with third parties for the development or commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

We may seek third-party collaborators for the development and commercialization of our product candidates, including for the commercialization of any of our product candidates that are approved for marketing outside the United States. Our likely collaborators for any such arrangements include regional and national pharmaceutical companies and biotechnology companies. If we enter into any additional such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our product candidates would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or drugs, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for any collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate additional collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate revenue.

Risks Related to our Intellectual Property

If we are unable to obtain or protect intellectual property rights related to any of our product candidates, we may not be able to compete effectively in our market.

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates. Our success depends in large part on our ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and product candidates.

As of the date of this prospectus, we in-license one Patent Cooperation Treaty application, 14 pending foreign patent applications, 14 patent applications pending in the United States, of which two are United States utility patent applications, both of which, if issued, are expected to expire in 2037, and 12 are United States provisional patent applications where patent applications claiming priority to these provision patent applications, if issued, are expected to expire between 2040 and 2041. We cannot offer any assurances about which of our patent applications will issue, the breadth of any resulting patent or whether any of the issued patents will be found invalid and unenforceable or will be

threatened by third parties. We cannot offer any assurances that the breadth of our granted patents will be sufficient to stop a competitor from developing and commercializing a product, including a biosimilar product that would be competitive with one or more of our product candidates. Furthermore, any successful challenge to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any of our product candidates. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

The patent prosecution process is expensive and time-consuming. We may not be able to prepare, file and prosecute all necessary or desirable patent applications at a commercially reasonable cost or in a timely manner or in all jurisdictions. It is also possible that we may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Moreover, depending on the terms of any future in-licenses to which we may become a party, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology in-licensed from third parties. Therefore, these patents and patent applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

In addition to the protection provided by our patent estate, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not amenable to patent protection. Although we generally require all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed. Moreover, our competitors may independently develop knowledge, methods and know-how equivalent to our trade secrets. Competitors could purchase our products, if approved, and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, our agreements or security measures may be breached, and we may not have adequate remedies for any breach. Also, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA is considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time, and if we do not obtain protection under the Hatch-Waxman Amendments and similar non-United States legislation for extending the term of patents covering each of our product candidates, our business may be materially harmed.

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. Depending upon the timing, duration and conditions of FDA marketing approval of our product candidates, one or more of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, and similar legislation in the European Union. The Hatch-Waxman Amendments permit a patent term extension of up to five years for a patent covering an approved product as compensation for effective patent term lost during product development and the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent may be extended, and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, the period during which we can enforce our patent rights for that product will be shortened and our competitors may obtain approval to market competing products sooner. As a result, our revenue from applicable products could be reduced and could have a material adverse effect on our business.

If we fail to comply with our obligations in our current and future intellectual property licenses with third parties, we could lose rights that are important to our business.

We are heavily reliant upon licenses to certain patent rights and proprietary technology for the development of our product candidates, in particular the UT Southwestern Agreement and our license agreements with Queen's University at Kingston and Abeona Therapeutics Inc. These license agreements impose diligence, development and commercialization timelines and milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations, our licensors may have the right to terminate our licenses, in which event we might not be able to develop, manufacture or market any product that is covered by the intellectual property we in-license from such licensor and may face other penalties. Such an occurrence would materially adversely affect our business prospects.

Licenses to additional third-party technology and materials that may be required for our development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could have a material adverse effect on our business and financial condition. Although we control the prosecution, maintenance and enforcement of the licensed and sublicensed intellectual property relating to our product candidates, we may require the cooperation of our licensors and any upstream licensor, which may not be forthcoming. Therefore, we cannot be certain that the prosecution, maintenance and enforcement of these patent rights will be in a manner consistent with the best interests of our business. If we or our licensor fail to maintain such patents, or if we or our licensor lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights could be adversely affected. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future. Further, if we fail to comply with our development obligations under our license agreements, we may lose our patent rights with respect to such agreement on a territory-by-territory basis, which would affect our patent rights worldwide.

Termination of our current or any future license agreements would reduce or eliminate our rights under these agreements and may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology. Any of the foregoing could prevent us from commercializing our other product candidates, which could have a material adverse effect on our operating results and overall financial condition.

In addition, intellectual property rights that we in-license in the future may be sublicenses under intellectual property owned by third parties, in some cases through multiple tiers. The actions of our licensors may therefore affect our rights to use our sublicensed intellectual property, even if we are in compliance with all of the obligations under our license agreements. Should our licensors or any of the upstream licensors fail to comply with their obligations under the agreements pursuant to which they obtain the rights that are sublicensed to us, or should such agreements be terminated or amended, our ability to develop and commercialize our product candidates may be materially harmed.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our future patents.

Our ability to obtain patents is highly uncertain because, to date, some legal principles remain unresolved, and there has not been a consistent policy regarding the breadth or interpretation of claims allowed in patents in the United States. Furthermore, the specific content of patents and patent applications that are necessary to support and interpret patent claims is highly uncertain due to the complex nature of the relevant legal, scientific, and factual issues. Changes in either patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection.

For example, on September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act included a number of significant changes to United States patent law. These included provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The United States Patent and Trademark Office, or USPTO, has developed new and untested regulations and procedures to govern the full implementation of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, became effective in March 2013. The Leahy-Smith Act has also introduced procedures making it easier for third parties to challenge issued patents, as well as to intervene in the prosecution of patent applications. Finally, the Leahy-Smith Act contained new statutory provisions that require the USPTO to issue new regulations for their implementation, and it may take the courts years to interpret the provisions of the new statute. It is too early to tell what, if any, impact the Leahy-Smith Act will have on the operation of our business and the protection and enforcement of our intellectual property. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our future patents. Further, the United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the United States Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have owned or licensed or that we might obtain in the future. An inability to obtain, enforce, and defend patents covering our proprietary technologies would materially and adversely affect our business prospects and financial condition.

Similarly, changes in patent laws and regulations in other countries or jurisdictions, changes in the governmental bodies that enforce them or changes in how the relevant governmental authority

enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we may obtain in the future. Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. For example, if the issuance in a given country of a patent covering an invention is not followed by the issuance in other countries of patents covering the same invention, or if any judicial interpretation of the validity, enforceability or scope of the claims or the written description or enablement, in a patent issued in one country is not similar to the interpretation given to the corresponding patent issued in another country, our ability to protect our intellectual property in those countries may be limited. Changes in either patent laws or in interpretations of patent laws in the United States and other countries may materially diminish the value of our intellectual property or narrow the scope of our patent protection.

We may be involved in lawsuits to protect or enforce our patents, which could be expensive, time consuming and unsuccessful.

Competitors may infringe the patents for which we have applied. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. If we initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the United States, counterclaims alleging invalidity and/or unenforceability are common, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. In an infringement proceeding, a court may decide that the patent claims we are asserting are invalid and/or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patent claims do not cover the technology in question. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, inter partes review and equivalent proceedings in foreign jurisdictions (for example, opposition proceedings). Such proceedings could result in revocation of or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel, and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could have a material adverse impact on our business.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patent applications. An unfavorable outcome could require us to cease using the related technology or force us to take a license under the patent rights of the prevailing party, if available. Furthermore, our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of

the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may be unsuccessful in licensing or acquiring intellectual property from third parties that may be required to develop and commercialize our product candidates.

A third party may hold intellectual property, including patent rights that are important or necessary to the development and commercialization of our product candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our product candidates, in which case we would be required to acquire or obtain a license to such intellectual property from these third parties, and we may be unable to do so on commercially reasonable terms or at all. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate, which could have a material adverse effect on our business.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain.

As our current and future product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. We cannot provide any assurance that our current and future product candidates do not infringe other parties' patents or other proprietary rights, and competitors or other parties may assert that we infringe their proprietary rights in any event. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our current and future product candidates, including interference or derivation proceedings before the USPTO. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could have a negative impact on our ability to commercialize TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates. In order to successfully challenge the validity of any such United States patent in federal court, we would need to overcome a presumption of validity. As this burden is high and requires us to present clear and convincing evidence as to the invalidity of any such United States patent claim, there is no assurance that a court of competent jurisdiction would agree with us and invalidate the claims of any such United States patent. Moreover, given the vast number of patents in our field of technology, we cannot be certain that we do not infringe existing patents or that we will not infringe patents that may be granted in the future.

While we may decide to initiate proceedings to challenge the validity of these or other patents in the future, we may be unsuccessful, and courts or patent offices in the United States and abroad could uphold the validity of any such patent. Furthermore, because patent applications can take many years to issue and may be confidential for 18 months or more after filing, and because pending patent claims can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use or sale of our product candidates. Regardless of when filed, we may fail to identify relevant third-party patents or patent applications, or we may incorrectly conclude that a third-party patent is invalid or not infringed by our product

candidates or activities. If a patent holder believes that one of our product candidates infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant drug revenue and against whom our own patent portfolio may thus have no deterrent effect. If a patent infringement suit were threatened or brought against us, we could be forced to stop or delay research, development, manufacturing or sales of the drug or product candidate that is the subject of the actual or threatened suit.

We are aware of issued patent or patents issued to REGENXBIO Inc., or REGENX, that claim AAV vectors that have an AAV9 capsid serotype. If we commercialize any of our product candidates prior to the expiry of those patents in 2026 without a license, the patent owner could bring an action claiming infringement. In August 2020, we received a letter from REGENX that stated REGENX is the owner of or has exclusive licenses to various issued patents or patent applications regarding the use of AAV9 vectors and offered to discuss licensing the applicable patents from them. We intend to review their letter in detail and to take appropriate actions based on our review, which may include, if we deem it appropriate, speaking with REGENX regarding their letter. If we are found to infringe a third party's valid intellectual property rights, we could be required to obtain a license from such third party to continue commercializing our product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. If we fail to obtain a required license, we may be unable to effectively market product candidates based on our technology, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. Alternatively, we may need to redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. Under certain circumstances, we could be forced, including by court orders, to cease commercializing our product candidates. In addition, in any such proceeding or litigation, we could be found liable for substantial monetary damages, potentially including treble damages and attorneys' fees, if we are found to have willfully infringed the patent at issue. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business.

The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties.

We employ individuals who were previously employed at other biotechnology or biopharmaceutical companies. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants, or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. We may also be subject to claims that former employers or other third parties have an ownership interest in our future patents. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees.

We may be subject to claims challenging the inventorship or ownership of our future patents and other intellectual property.

We may also be subject to claims that former employees, collaborators, or other third parties have an ownership interest in our patent applications, our future patents, or other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Although it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own, and we cannot be certain that our agreements with such parties will be upheld in the face of a potential challenge, or that they will not be breached, for which we may not have an adequate remedy. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

If we rely on third parties to manufacture or commercialize our product candidates, or if we collaborate with additional third parties for the development of such product candidates, we must, at times, share trade secrets with them. We may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor’s discovery of our trade secrets or other unauthorized use or disclosure could have an adverse effect on our business and results of operations.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets. Despite our efforts to protect our trade secrets, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Moreover, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our confidential information or proprietary technology and processes. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees, contractors and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets as a result. Moreover, if confidential information that is licensed or disclosed to us by our partners, collaborators, or others is inadvertently disclosed or subject

to a breach or violation, we may be exposed to liability to the owner of that confidential information. Enforcing a claim that a third party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing to protect trade secrets.

We may enjoy only limited geographical protection with respect to certain patents and we may not be able to protect our intellectual property rights throughout the world.

Filing and prosecuting patent applications and defending patents covering our product candidates in all countries throughout the world would be prohibitively expensive. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as that in the United States or Europe. These products may compete with our product candidates, and our future patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

In addition, we may decide to abandon national and regional patent applications before they are granted. The examination of each national or regional patent application is an independent proceeding. As a result, patent applications in the same family may issue as patents in some jurisdictions, such as in the United States, but may issue as patents with claims of different scope or may even be refused in other jurisdictions. It is also quite common that depending on the country, the scope of patent protection may vary for the same product candidate or technology.

While we intend to protect our intellectual property rights in our expected significant markets, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our product candidates. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate, which may have an adverse effect on our ability to successfully commercialize our product candidates in all of our expected significant foreign markets. If we encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition from others in those jurisdictions.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws or rules and regulations in the United States and Europe and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property rights, which could make it difficult for us to stop the infringement of our future patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our future patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing as patents, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Some countries also have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors. In those countries, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment, and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our patents and/or applications and any patent rights we may obtain in the future. Furthermore, the USPTO and various non-United States government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse of a patent or patent application can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patents or patent applications, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market, which could have a material adverse effect on our business.

Any trademarks we have obtained or may obtain may be infringed or otherwise violated, or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish our product candidates, if approved for marketing, from the drugs of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe or otherwise violate our trademarks and we may not have adequate resources to enforce our trademarks. Any of the foregoing events may have a material adverse effect on our business.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make products that are similar to or otherwise competitive with our product candidates but that are not covered by the claims of our current or future patents;
- an in-license necessary for the manufacture, use, sale, offer for sale or importation of one or more of our product candidates may be terminated by the licensor;
- we or future collaborators might not have been the first to make the inventions covered by our issued or future issued patents or our pending patent applications;
- we or future collaborators might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or in-license may be held invalid or unenforceable as a result of legal challenges by our competitors;

- issued patents that we own or in-license may not provide coverage for all aspects of our product candidates in all countries;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Should any of these events occur, they could significantly harm our business, results of operations and prospects.

Risks Related to Legal and Regulatory Compliance Matters

Our relationships with customers, healthcare providers, including physicians, and third-party payors are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

Healthcare providers, including physicians, and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors subject us to various federal and state fraud and abuse laws and other healthcare laws, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal false claims laws and the law commonly referred to as the Physician Payments Sunshine Act and regulations promulgated under such laws. These laws will impact, among other things, our clinical research, proposed sales, marketing and educational programs, and other interactions with healthcare professionals. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct or may conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, individuals or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind in return for, or to induce, either the referral of an individual, or the purchase, lease, order or arrangement for or recommendation of the purchase, lease, order or arrangement for any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. The term “remuneration” has been broadly interpreted to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. A person does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the ACA signed into law in 2010, provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act;
- the federal civil and criminal false claims laws, including, without limitation, the federal False Claims Act, which can be enforced by private citizens through civil whistleblower or qui tam actions, and civil monetary penalty laws which prohibit, among other things, individuals or

entities from knowingly presenting, or causing to be presented, claims for payment or approval from the federal government, including Medicare, Medicaid and other government payors, that are false or fraudulent or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government. A claim includes “any request or demand” for money or property presented to the United States federal government. Several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies’ marketing of products for unapproved, and thus non-reimbursable, uses;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes which prohibit, among other things, a person from knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information on health plans, healthcare clearinghouses and certain healthcare providers, known as “covered entities”, and their respective HIPAA “business associates”, which are independent contractors that perform certain services for or on behalf of covered entities involving the use or disclosure of individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys’ fees and costs associated with pursuing federal civil actions;
- the federal transparency laws, including the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, medical devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the State Children’s Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to: (i) payments or other “transfers of value’’ made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and (ii) ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made, as well as ownership and investment interests held, during the previous year to certain non-physician providers such as physician assistants and nurse practitioners; and
- analogous state and foreign laws and regulations; state laws that require manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures or drug pricing; state laws that require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or that otherwise restrict payments that may be made to healthcare providers; state and local laws that require the registration of pharmaceutical sales representatives; and state and foreign laws that govern the privacy and security of health information in some circumstances, many of

which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in federal and state funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, diminished profits and future earnings, reputational harm and the curtailment or restructuring of our operations, any of which could harm our business.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a healthcare company may run afoul of one or more of the requirements.

Even if we obtain regulatory approval for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates, they will remain subject to ongoing regulatory oversight.

Even if we obtain any regulatory approval for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates, such product candidates, once approved, will be subject to ongoing regulatory requirements applicable to manufacturing, labeling, packaging, storage, advertising, promoting, sampling, record-keeping and submitting of safety and other post-market information, among other things. Any regulatory approvals that we receive for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates may also be subject to a risk evaluation and mitigation strategy, limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or requirements that we conduct potentially costly post-marketing testing, including Phase 4 trials and surveillance to monitor the quality, safety and efficacy of the drug. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval. We will further be required to immediately report any serious and unexpected adverse events and certain quality or production problems with our products to regulatory authorities along with other periodic reports.

Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance. We will also have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drug products are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we will not be allowed to promote our products for indications or uses for which they do not have approval, commonly known as off-label promotion. The holder of an approved BLA must submit new or supplemental applications and obtain prior approval for certain changes to the approved product, product labeling, or

manufacturing process. A company that is found to have improperly promoted off-label uses of their products may be subject to significant civil, criminal and administrative penalties.

In addition, drug manufacturers are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or if a regulatory authority disagrees with the promotion, marketing or labeling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or us, including requesting a recall or requiring withdrawal of the drug from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates, a regulatory authority may:

- issue an untitled letter or warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;
- refuse to permit the import or export of product candidates; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates and harm our business, financial condition, results of operations and prospects.

Even if we obtain FDA or EMA approval any of our product candidates in the United States or European Union, we may never obtain approval for or commercialize any of them in any other jurisdiction, which would limit our ability to realize their full market potential.

In order to market any products in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy.

Approval by the FDA in the United States or the EMA in the European Union does not ensure approval by regulatory authorities in other countries or jurisdictions. However, the failure to obtain approval in one jurisdiction may negatively impact our ability to obtain approval elsewhere. In addition, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country.

Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and increased costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. We do not have any product candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any product we develop will be unrealized.

Healthcare legislative or regulatory reform measures may have a negative impact on our business and results of operations.

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of product candidates, restrict or regulate post-approval activities, and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in March 2010, the ACA was passed, which substantially changed the way healthcare is financed by both the government and private insurers, and significantly impacts the United States pharmaceutical industry. The ACA, among other things: (i) established an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs; (ii) expanded the entities eligible for discounts under the 340B drug pricing program; (iii) increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively, and capped the total rebate amount for innovator drugs at 100% of the Average Manufacturer Price, or AMP; (iv) expanded the eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new eligibility categories for individuals with income at or below 133% (as calculated, it constitutes 138%) of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability; (v) addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for certain drugs and biologics that are inhaled, infused, instilled, implanted or injected; (vi) introduced a new Medicare Part D coverage gap discount program in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D (increased from 50%, effective January 1, 2019, pursuant to the Bipartisan Budget Act of 2018); (vii) created a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and (viii) established the Center for Medicare and Medicaid Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug.

There remain judicial and Congressional challenges to certain aspects of the ACA, as well as efforts by the Trump administration to repeal or replace certain aspects of the ACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act of 2017, or Tax Act, includes a provision that repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate." In addition, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. The Bipartisan Budget Act of 2018, or the BBA, among other things, amended the ACA, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." In December 2018, CMS published a new final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On April 27, 2020, the United States Supreme Court reversed a Federal Circuit decision that previously upheld Congress' denial of $12 billion in "risk corridor" funding. On December 14, 2018, a Texas United States District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the United States Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case, and has allotted one hour for oral arguments, which are expected to occur in the fall. It is unclear how such litigation and other efforts to repeal and replace the ACA will impact the ACA and our business.

Other legislative changes have been proposed and adopted since the ACA was enacted. These changes include aggregate reductions to Medicare payments to providers of 2% per fiscal year pursuant to the Budget Control Act of 2011, which began in 2013, and due to subsequent legislative amendments to the statute, including the BBA, will remain in effect through 2030, unless additional Congressional action is taken. The Coronavirus Aid, Relief and Economic Security Act, or the CARES Act, which was signed into law in March 2020 and is designed to provide financial support and resources to individuals and businesses affected by the COVID-19 pandemic, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. The American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding, which could have an adverse effect on customers for our product candidates, if approved, and, accordingly, our financial operations.

Additionally, there has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. At the federal level, the Trump administration's budget proposal for fiscal year 2021 includes a $135 billion allowance (over a period of time) to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs. On March 10, 2020, the Trump administration sent "principles" for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy

expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses, and place limits on pharmaceutical price increases. Further, the Trump administration previously released a plan to lower drug prices and reduce out of pocket costs of drugs that contained proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The HHS has solicited feedback on some of these measures and has implemented others under its existing authority. On July 24, 2020, President Trump announced four executive orders related to prescription drug pricing that attempt to implement several of the Administration's proposals, including a policy that would tie Medicare Part B drug prices to international drug prices; one that directs HHS to finalize the Canadian drug importation proposed rule previously issued by HHS and makes other changes allowing for personal importation of drugs from Canada; one that directs HHS to finalize the rulemaking process on modifying the anti-kickback law safe harbors for plans, pharmacies, and pharmaceutical benefit managers; and one that reduces costs of insulin and epipens to patients of federally qualified health centers. The probability of success of these newly announced policies and their impact on the U.S. prescription drug marketplace is unknown. While some of these and other measures may require additional authorization to become effective, Congress and the Trump administration have indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that these and other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs. It is also possible that additional governmental action is taken to address the COVID-19 pandemic.

In addition, FDA regulations and guidance may be revised or reinterpreted by the FDA in ways that may significantly affect our business and our products. The Trump administration has also taken several executive actions, including the issuance of a number of Executive Orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. It is difficult to predict how these requirements will be interpreted and implemented and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose restrictions on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. Any new regulations or guidance, or revisions or reinterpretations of existing regulations or guidance, may impose additional costs or lengthen FDA review times for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future product candidates. We cannot determine how changes in regulations, statutes, policies, or interpretations when and if issued, enacted or adopted, may affect our business in the future. Such changes could, among other things, require:

- additional clinical trials to be conducted prior to obtaining approval;
- changes to manufacturing methods;
- recalls, replacements, or discontinuance of one or more of our products; and
- additional recordkeeping.

Such changes would likely require substantial time and impose significant costs, or could reduce the potential commercial value of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or other product candidates, and could materially harm our business and our financial results. In addition, delays in receipt of or failure to receive regulatory clearances or approvals for any other products would harm our business, financial condition, and results of operations.

Our business activities will be subject to the Foreign Corrupt Practices Act, or FCPA, and similar anti-bribery and anti-corruption laws.

As we expand our business activities outside of the United States, including our clinical trial efforts, we will be subject to the FCPA and similar anti-bribery or anti-corruption laws, regulations or rules of other countries in which we operate. The FCPA generally prohibits offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a non-United States government official in order to influence official action, or otherwise obtain or retain business. The FCPA also requires public companies to make and keep books and records that accurately and fairly reflect the transactions of the corporation and to devise and maintain an adequate system of internal accounting controls. Our business is heavily regulated and therefore involves significant interaction with public officials, including officials of non-United States governments. Additionally, in many other countries, the healthcare providers who prescribe pharmaceuticals are employed by their government, and the purchasers of pharmaceuticals are government entities; therefore, our dealings with these prescribers and purchasers will be subject to regulation under the FCPA. Recently the SEC and Department of Justice have increased their FCPA enforcement activities with respect to biotechnology and pharmaceutical companies. There is no certainty that all of our employees, agents, suppliers, manufacturers, contractors, or collaborators, or those of our affiliates, will comply with all applicable laws and regulations, particularly given the high level of complexity of these laws. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers, or our employees, the closing down of facilities, including those of our suppliers and manufacturers, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries as well as difficulties in manufacturing or continuing to develop our products, and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, and our business, prospects, operating results, and financial condition.

Disruptions at the FDA, the SEC and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs or biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including most recently from December 22, 2018 to January 25, 2019, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and

other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Separately, in response to the COVID-19 pandemic, on March 10, 2020 the FDA announced its intention to postpone most inspections of foreign manufacturing facilities and products while local, national and international conditions warrant. The FDA has developed a rating system to assist in determining when and where it is safest to conduct prioritized domestic inspections. Regulatory authorities outside the U.S. may adopt similar restrictions or other policy measures in response to the COVID-19 pandemic and may experience delays in their regulatory activities.

Risks Related to Employee Matters and Managing our Growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, development, clinical, financial and business development expertise of our executive officers, particularly RA Session II, our President and Chief Executive Officer. Each of our executive officers may currently terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives or employees.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our product pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We currently have a limited number of employees, and we rely on PBM Capital Group, LLC to provide various administrative and management services.

We currently rely on the support and administrative services provided by PBM Capital Group, LLC, which, together with its affiliate PBM TGT Holdings, LLC, is a holder of more than 5% of our capital stock, pursuant to our services agreement with PBM Capital Group, LLC. We do not expect personnel and support staff that provide services to us under these services agreements will have as their primary responsibility the management and administration of our business or act exclusively for us. As a result, such individuals will not allocate all of their time and resources to us. See “Certain Relationships and Related Party Transactions—Our Relationship with PBM Capital Group, LLC.”

If PBM Capital Group, LLC fails to perform their obligations in accordance with the terms of the services agreements, it could be difficult for us to operate our business. Any failure by PBM Capital

Group, LLC to effectively manage administrative or other services that they provide to us could harm our business, financial condition and results of operations. In addition, the termination of our relationship with PBM Capital Group, LLC and any delay in appointing or finding a suitable replacement provider (if one exists) could make it difficult for us to operate our business.

Additionally, over time we will need to transition from receiving the services that PBM Capital Group, LLC is currently providing to performing such activities internally. If we do not have adequate financial resources or personnel and systems in place at the time that we assume responsibilities for such services, we may not be successful in effectively or efficiently transitioning these services from PBM Capital Group, LLC, which could disrupt our business and have a material adverse effect on our financial condition and results of operations. Even if we are able to successfully transition these services, they may be more expensive or less efficient than the services we are receiving from PBM Capital Group, LLC during the transition period.

We expect to expand our clinical development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of August 31, 2020, we had ten employees. As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of clinical product development, regulatory affairs and, if any of our product candidates receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators, principal investigators, CROs, suppliers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct that violates FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of business conduct and ethics, but it is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights,

those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations.

Risks Related to This Offering, Ownership of our Common Stock and our Status as a Public Company

An active trading market for our common stock may not develop and you may not be able to resell your shares of our common stock at or above the initial offering price, if at all.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock was determined through negotiations with the underwriters and may not be indicative of the price at which our common stock will trade after the closing of this offering. Although our common stock has been approved for listing on The Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop or is not sustained, it may be difficult for you to sell shares you purchased in this offering at an attractive price or at all.

The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could incur substantial losses.

Our stock price may be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- the reporting of unfavorable preclinical results;
- the commencement, enrollment or results of our clinical trials of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- any delay in our regulatory filings for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidate we may develop, and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- adverse results from, delays in or termination of clinical trials;
- adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- unanticipated serious safety concerns related to the use of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidate;
- changes in financial estimates by us or by any equity research analysts who might cover our stock;
- conditions or trends in our industry;
- changes in the market valuations of similar companies;

- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- our relationships with our collaborators;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- investors' general perception of our company and our business;
- recruitment or departure of key personnel;
- overall performance of the equity markets;
- trading volume of our common stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- changes in the structure of healthcare payment systems;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

The stock market in general, and the Nasdaq Global Market and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies, including very recently in connection with the ongoing COVID-19 pandemic, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects or developments relating to the ongoing COVID-19 pandemic, may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this section, could have a significant and material adverse impact on the market price of our common stock.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources from our business.

If you purchase shares of our common stock in this offering, you will suffer immediate dilution of your investment.

The initial public offering price of our common stock is substantially higher than the net tangible book value (deficit) per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our pro forma as adjusted net tangible book value per share after this offering. Based on the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this

prospectus, you will experience immediate dilution of $ per share, representing the difference between our pro forma as adjusted net tangible book value per share after this offering and the assumed initial public offering price.

In addition, as of September 2, 2020, we had outstanding stock options to purchase an aggregate of 15,000 shares of common stock at an exercise price of $16.23 per share. To the extent these outstanding options are exercised, there will be further dilution to investors in this offering.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

Upon the closing of this offering, we will have outstanding shares of common stock, after giving effect to the automatic conversion of our outstanding convertible preferred stock into 15,647,048 shares of our common stock, and assuming no exercise of outstanding options to purchase shares of our convertible preferred stock. Of these shares, the shares sold in this offering will be freely tradable upon the closing of this offering and the remaining shares of common stock will be available for sale in the public market beginning 180 days after the date of this prospectus following the expiration of lock-up agreements between some of our stockholders and the underwriters. Goldman Sachs & Co. LLC, Morgan Stanley & Co. LLC and Jefferies LLC may release these stockholders from their lock-up agreements with the underwriters at any time and without notice, which would allow for earlier sales of shares in the public market.

In addition, following the closing of this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act of 1933, as amended, or the Securities Act, registering the issuance of shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under these registration statements on Form S-8 will be available for sale in the public market subject to vesting arrangements and exercise of options, the lock-up agreements described above and the restrictions of Rule 144 in the case of our affiliates.

Additionally, after this offering, the holders of an aggregate of 25,647,048 shares of our common stock, or their transferees, will have rights, subject to some conditions, to require us to file one or more

registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.

There are provisions in our certificate of incorporation and bylaws to be in effect upon the closing of this offering that may make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change of control was considered favorable by you and other stockholders. For example, our board of directors will have the authority to issue up to shares of preferred stock. The board of directors can fix the price, rights, preferences, privileges, and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change of control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

Our charter documents will also contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors will be elected each year;
- stockholders will not be entitled to remove directors other than by a 66 2/3% vote and only for cause;
- stockholders will not be permitted to take actions by written consent;
- stockholders cannot call a special meeting of stockholders; and
- stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change of control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent new investors from influencing significant corporate decisions.

Our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates beneficially own over 80% of our outstanding common stock prior to this offering and will continue to own a majority of our common stock following this offering. As a result, these persons, acting together, would be able to significantly influence all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current market

price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders.

We are an "emerging growth company" and a "smaller reporting company" and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an emerging growth company. We will remain an emerging growth company until December 31, 2025 or, if earlier, (i) the last day of the fiscal year in which we have total annual gross revenue of at least $1.07 billion, (ii) the date on which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, or (iii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Even after we no longer qualify as an emerging growth company, we may, under certain circumstances, still qualify as a "smaller reporting company," which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

After the closing of this offering, we will be subject to the reporting requirements of the Securities Exchange Act of 1934, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting.

Commencing with our fiscal year ending December 31, 2021, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for that

year, as required by Section 404 of the Sarbanes-Oxley Act. This will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to this offering, we have never been required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the Securities and Exchange Commission or other regulatory authorities.

We will have broad discretion in the use of proceeds from this offering and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.

We will have broad discretion over the use of proceeds from this offering. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment. We expect to use the net proceeds to us from this offering, together with our existing cash and cash equivalents, to . In addition, we may use a portion of the proceeds from this offering to pursue our strategy to in-license or acquire additional product candidates. Our failure to apply the net proceeds from this offering effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds. You will not have the opportunity to influence our decisions on how to use our net proceeds from this offering.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

We are subject to taxation in more than one tax jurisdiction. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each such jurisdiction. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors, including passage of newly enacted tax legislation, changes in the mix of our profitability from jurisdiction to jurisdiction, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes and changes in existing tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

We might not be able to utilize a significant portion of our net operating loss carryforwards.

We expect to generate significant federal and state net operating loss, or NOL, carryforwards in the future. These NOL carryforwards could expire unused and be unavailable to offset future income

tax liabilities. Under the Tax Cuts and Jobs, or the Tax Act, as modified by the CARES Act, federal NOLs incurred in taxable years beginning after December 31, 2017 and in future taxable years may be carried forward indefinitely, but the deductibility of such federal NOLs incurred in the taxable year beginning after December 31, 2020 is limited. It is uncertain how various states will respond to the Tax Act and CARES Act. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. The completion of this offering, together with private placements and other transactions that have occurred since our inception, may trigger such an ownership change pursuant to Section 382. We have not yet completed a Section 382 analysis. We may experience ownership changes as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our NOL carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gains and you may never receive a return on your investment.

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

We will incur increased costs and demands upon management as a result of being a public company.

As a public company listed in the United States, we will incur significant additional legal, accounting and other costs, which we anticipate will be between $1.0 million and $2.0 million annually. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and the Nasdaq Stock Market, may increase legal and financial compliance costs and make some activities more time-consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware and the federal district courts of the United States of America will be the exclusive forums for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising under the Delaware General Corporation Law, our amended and restated certificate of incorporation, or our amended and restated bylaws;
- any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate or our amended and restated bylaws;
- any claim or cause of action as to which the Delaware General Corporation Law confers jurisdiction on the Court of Chancery of the state of Delaware; and
- any action asserting a claim against us that is governed by the internal-affairs doctrine.

This provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation further provides that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive-forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. The forward-looking statements are contained principally in the sections titled “Prospectus Summary,” “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” “Business” and elsewhere in this prospectus. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “estimate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions intended to identify statements about the future. These statements speak only as of the date of this prospectus and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements include, without limitation, statements about the following:

- the timing, progress and results of our preclinical studies and clinical trials of our product candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing of our planned IND and CTA submissions, initiation of clinical trials and timing of expected clinical results for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and our other future product candidates;
- the timing of any submission of filings for regulatory approval of, and our ability to obtain and maintain regulatory approvals for, our current and future product candidates;
- the outbreak of the novel strain of coronavirus disease, COVID-19, which could adversely impact our business, including our preclinical studies and clinical trials;
- our ability to identify patients with the diseases treated by our product candidates, and to enroll patients in trials;
- our expectations regarding the size of the patient populations, market acceptance and opportunity for and clinical utility of our product candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our expectations regarding the scope of any approved indication for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 or any other product candidate;
- our ability to successfully commercialize our product candidates;
- our ability to leverage our platform, including our next-generation technologies, to identify and develop future product candidates;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our need for or ability to obtain additional funding before we can expect to generate any revenue from product sales;
- our ability to establish or maintain collaborations or strategic relationships;
- our ability to identify, recruit and retain key personnel;
- our reliance upon intellectual property licensed from third parties and our ability to obtain such licenses on commercially reasonable terms or at all;

- our ability to protect and enforce our intellectual property position for our product candidates, and the scope of such protection;
- our financial performance;
- our expected use of proceeds from this offering;
- our competitive position and the development of and projections relating to our competitors or our industry;
- our estimates regarding future revenue, expenses and needs for additional financing;
- the impact of laws and regulations; and
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act.

The foregoing list of risks is not exhaustive. Other sections of this prospectus may include additional factors that could harm our business and financial performance. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this prospectus will prove to be accurate. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. You should, however, review the factors and risks and other information we describe in the reports we will file from time to time with the SEC after the date of this prospectus.

Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. Although we believe that we have a reasonable basis for each forward-looking statement contained in this prospectus, the events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. You should refer to the ‘‘Risk Factors’’ section of this prospectus for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

MARKET AND INDUSTRY DATA

We obtained the industry, statistical and market data in this prospectus from our own internal estimates and research as well as from industry and general publications and research, surveys and studies conducted by third parties. All of the market data used in this prospectus involve a number of assumptions and limitations, and the sources of such data cannot guarantee the accuracy or completeness of such information. While we are not aware of any misstatements regarding the third-party information and we believe that each of these studies and publications is reliable, the industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of important factors, including those described in the section titled “Risk Factors.” These and other factors could cause results to differ materially from those expressed in the estimates made by third parties and by us.

USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of shares of our common stock in this offering will be approximately $ million (or approximately $ million if the underwriters exercise in full their option to purchase up to additional shares), assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) the net proceeds to us from this offering by approximately $ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each increase (decrease) of 1.0 million in the number of shares we are offering would increase (decrease) the net proceeds to us from this offering, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, by approximately $ million, assuming the assumed initial public offering price stays the same. We do not expect that a change in the offering price or the number of shares by these amounts would have a material effect on our intended uses of the net proceeds from this offering, although it may impact the amount of time prior to which we may need to seek additional capital.

As of June 30, 2020, we had a cash balance of $11.2 million. In July and August 2020, we received $107.1 million of aggregate net proceeds from an additional closing of our Series A convertible preferred stock financing and the issuance and sale of our Series B convertible preferred stock. We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately $ million to ;
- approximately $ million to ; and
- the remainder for working capital and other general corporate purposes.

We believe that the anticipated net proceeds of this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital requirements into . Based on our current operational plans and assumptions, we expect our cash and cash equivalents, together with the net proceeds from this offering, will be sufficient to . We have based these estimates on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we expect.

We may also use a portion of the remaining net proceeds to in-license, acquire or invest in complementary businesses, technologies, products or assets, although we have no current agreements, commitments or understandings to do so.

This expected use of net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials, as well as any collaborations that we may enter into with third parties for our drug candidates, and any unforeseen cash needs. As a result, our management will have broad discretion in the application of the net proceeds from this offering, and investors will be relying on the judgment of our management regarding the application of those net proceeds. The timing and amount of our actual expenditures will be based on many factors, including cash flows from operations and the anticipated growth of our business. Pending these uses, we plan to invest these net proceeds in short-term, interest bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the United States.

DIVIDEND POLICY

We have never declared or paid, and do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant.

CAPITALIZATION

The following table sets forth our cash and cash equivalents, and our capitalization as of June 30, 2020:

- on an actual basis;
- on a pro forma basis to give effect to: (i) the receipt of $107.1 million in aggregate net proceeds from the issuance and sale of Series A and Series B convertible preferred stock in July and August 2020 and the related settlement of the convertible preferred stock tranche liability of $17.2 million; (ii) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 15,647,048 shares of our common stock, including the conversion of the convertible preferred stock issued in July and August 2020, as if such conversion had occurred on June 30, 2020; and (iii) the filing and effectiveness of our amended and restated certificate of incorporation upon the closing of this offering; and
- on a pro forma as adjusted basis to give effect to: (i) the pro forma adjustments described above; and (ii) our issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and estimated offering expenses payable by us. This pro forma as adjusted information is illustrative only and will depend on the actual initial public offering price and other terms of this offering determined at pricing.

Our capitalization following the closing of this offering will depend on the actual initial public offering price and other terms of this offering determined at pricing. You should read this information in conjunction with our financial statements and the related notes appearing at the end of this prospectus, the sections of this prospectus titled “Selected Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and other financial information contained in this prospectus.

	As of June 30, 2020		
	Actual	Pro Forma	Pro Forma As Adjusted
	(in thousands, except share data)		
Cash and cash equivalents	$ 11,200	$118,252	$
Preferred stock tranche liability	$ 17,176	$ —	$
Series A convertible preferred stock, $0.00001 par value; 10,000,000 shares authorized, 6,200,000 issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	18,014	—	
Series B convertible preferred stock, $0.00001 par value; no shares authorized, issued and outstanding, actual, pro forma and pro forma as adjusted	—	—	
Stockholders’ (deficit) equity:			
Preferred stock, $0.00001 par value; no shares authorized, issued or outstanding, actual; shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	
Common stock, $0.00001 par value; 23,000,000 shares authorized, 10,000,000 shares issued and outstanding, actual; shares authorized, 25,647,048 shares issued and outstanding, pro forma; shares authorized, shares issued and outstanding, pro forma as adjusted	—	—	
Additional paid-in capital	980	143,222	
Accumulated deficit	(27,766)	(27,766)	
Total stockholders’ (deficit) equity	(26,786)	115,456	
Total capitalization	$ 8,404	$115,456	$

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders’ equity and total capitalization by $ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1.0 million shares in the number of shares offered by us at the assumed initial public offering price per share of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of additional paid-in capital, total stockholders’ equity and total capitalization by $ million.

The number of shares of our common stock outstanding in the table above excludes:

- 15,000 shares of our common stock issuable upon the exercise of options under the Existing Plan, granted subsequent to June 30, 2020, at an exercise price of $16.23 per share;
- 705,882 shares of our restricted common stock awarded under the Existing Plan subsequent to June 30, 2020;
- 2,615,935 shares of common stock issuable upon the vesting of restricted stock units awarded under the Existing Plan subsequent to June 30, 2020;
- 192,595 shares of our common stock reserved for future issuance under the Existing Plan, which shares will cease to be available for issuance at the time the New Plan becomes effective and will be added to, and become available for issuance under, the New Plan;
- shares of our common stock reserved for future issuance under the New Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the New Plan; and
- shares of our common stock reserved for future issuance under the ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of June 30, 2020, we had a historical net tangible book value (deficit) of $(26.8) million, or $(2.68) per share of common stock. Our historical net tangible book value (deficit) per share represents total tangible assets less total liabilities, including our convertible preferred stock, divided by the number of shares of our common stock outstanding as of June 30, 2020.

Our pro forma net tangible book value as of June 30, 2020 was $115.5 million, or $4.50 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to (i) the receipt of $107.1 million in net proceeds from the issuance and sale of Series A and Series B convertible preferred stock in July and August 2020 and the related settlement of the convertible preferred stock tranche liability and (ii) the conversion of all outstanding shares of our convertible preferred stock, including the conversion of the convertible preferred stock issued in July and August 2020, into an aggregate of 15,647,048 shares of our common stock, as if such conversion had occurred on June 30, 2020. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of June 30, 2020, after giving effect to the pro forma adjustments described above.

After giving further effect to the sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of June 30, 2020 would have been $ million, or $ per share. This amount represents an immediate increase in pro forma net tangible book value of $ per share to our existing stockholders and immediate dilution of $ per share to new investors in this offering. We determine dilution by subtracting the pro forma as adjusted net tangible book value per share after this offering from the amount of cash that a new investor paid for a share of common stock in this offering.

The following table illustrates this dilution:

Assumed initial public offering price per share		$
Historical net tangible book value (deficit) per share as of June 30, 2020	$(2.68)	
Pro forma increase in net tangible book value (deficit) per share attributable to the pro forma transactions described above	7.18	
Pro forma net tangible book value per share as of June 30, 2020	4.50	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors participating in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to new investors participating in this offering		$

The dilution information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted net tangible book value per share after this offering by approximately $, and

dilution in pro forma net tangible book value per share to new investors by approximately $, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1.0 million shares in the number of shares we are offering would increase the pro forma as adjusted net tangible book value per share after this offering by $ and decrease the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions. A decrease of 1.0 million shares in the number of shares we are offering would decrease the pro forma as adjusted net tangible book value per share after this offering by $ and increase the dilution per share to new investors participating in this offering by $, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions.

If the underwriters exercise their option to purchase additional shares of our common stock in full, the pro forma as adjusted net tangible book value after this offering would be $ per share, the increase in pro forma net tangible book value per share would be $ and the dilution per share to new investors would be $ per share, in each case assuming an initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus.

The following table summarizes, as of June 30, 2020, on the pro forma as adjusted basis described above, the differences between the number of shares purchased from us, the total consideration paid to us in cash and the average price per share that existing stockholders and new investors paid for such shares. The calculation below is based on an assumed initial public offering price of $ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

	Total Shares		Total Consideration		Weighted-Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	25,647,048	%	$125,999,896	%	$ 4.91
New investors					$
Total		100%	$	100%	

The foregoing tables and calculations are based on the number of shares of our common stock outstanding as of June 30, 2020, after giving effect to the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 15,647,048 shares of common stock, including the conversion of the convertible preferred stock issued in July and August 2020, and excludes:

- 15,000 shares of our common stock issuable upon the exercise of options under the Existing Plan, granted subsequent to June 30, 2020, at an exercise price of $16.23 per share;
- 705,882 shares of our restricted common stock awarded under the Existing Plan subsequent to June 30, 2020;
- 2,615,935 shares of common stock issuable upon the vesting of restricted stock units awarded under the Existing Plan subsequent to June 30, 2020;
- 192,595 shares of our common stock reserved for future issuance under the Existing Plan, which shares will cease to be available for issuance at the time the New Plan becomes effective and will be added to, and become available for issuance under, the New Plan;

- shares of our common stock reserved for future issuance under the New Plan, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the New Plan; and
- shares of our common stock reserved for future issuance under the ESPP, which will become effective in connection with this offering, as well as any automatic increases in the number of shares of common stock reserved for future issuance under the ESPP.

To the extent that stock options are exercised, new stock options are issued under our equity incentive plan or we issue additional shares of common stock in the future, there will be further dilution to investors participating in this offering. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

SELECTED FINANCIAL DATA

The following tables set forth our selected statement of operations data for the period from September 20, 2019 (the date of our inception) through December 31, 2019 and the selected balance sheet data as of December 31, 2019. We have derived the statement of operations data for the period from September 20, 2019 (the date of our inception) through December 31, 2019 and the balance sheet data as of December 31, 2019 from our audited financial statements appearing elsewhere in this prospectus. The statement of operations data for the six months ended June 30, 2020 and the balance sheet data as of June 30, 2020 have been derived from our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as the audited financial statements. In the opinion of management, the unaudited data reflects all adjustments, consisting only of normal recurring adjustments, considered necessary for a fair presentation of the financial information in those statements.

You should read the following selected financial data together with our financial statements and the related notes thereto included elsewhere in this prospectus and the section of this prospectus titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” The selected financial data in this section are not intended to replace our financial statements and are qualified in their entirety by our financial statements and related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected in the future.

	Period from September 20, 2019 (date of inception) through December 31, 2019	Six Months Ended June 30, 2020
Statement of Operations Data:		
(in thousands, except share and per share data)		
Operating expenses:		
Research and development	$ 987	$ 8,576
General and administrative	128	1,018
Total operating expenses	1,115	9,594
Loss from operations	(1,115)	(9,594)
Other expense:		
Change in fair value of preferred stock tranche liability	—	(17,030)
Interest expense	—	(27)
Total other expense	—	(17,057)
Net loss	$ (1,115)	$ (26,651)
Net loss per common share, basic and diluted	$ (0.13)	$ (2.67)
Weighted average common shares outstanding, basic and diluted	8,834,951	10,000,000
Pro forma net loss per common share, basic and diluted (unaudited) (1)		$ (1.91)
Weighted average shares outstanding used in computing pro forma net loss per share (unaudited)(1)		13,924,176

(1) See Note 6 to our unaudited condensed consolidated financial statements included elsewhere in this prospectus for an explanation of the method used to calculate historical and pro forma net loss per share and historical and pro forma weighted average common shares outstanding.

	As of December 31, 2019	As of June 30, 2020
Balance Sheet Data:		
(in thousands)		
Cash and cash equivalents	$ —	$ 11,200
Working capital deficit(1)	(135)	(8,791)
Total assets	15	11,318
Preferred stock tranche liability	—	17,176
Convertible preferred stock	—	18,014
Accumulated deficit	(1,115)	(27,766)
Total stockholders' deficit	(135)	(26,786)

(1) We define working capital deficit as current assets less current liabilities. See our financial statements appearing elsewhere in this prospectus.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the "Selected Financial Data" section of this prospectus and our financial statements and related notes, appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations. We were founded in partnership with The University of Texas Southwestern Medical Center, or UT Southwestern, to develop and commercialize transformative gene therapy treatments. Together with UT Southwestern, we are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates, with exclusive options to acquire four additional development programs at no cost. By combining our management team's proven experience in gene therapy drug development and commercialization with UT Southwestern's world-class gene therapy capabilities, we believe we have created a powerful engine to develop transformative therapies to dramatically improve patients' lives. We expect to initiate a Phase 1/2 clinical trial of TSHA-101 for the treatment of GM2 gangliosidosis, under a Clinical Trial Application, in Canada by the end of 2020. In addition, we plan to submit investigational new drug applications, or INDs, for four programs to the U.S. Food and Drug Administration, or the FDA, by the end of 2021: TSHA-101, TSHA-102 (Rett syndrome), TSHA-103 (SLC6A1 haploinsufficiency) and TSHA-104 (SURF1 deficiency). We are also developing TSHA-118 (formerly ABO-202) for the treatment of CLN1 disease (or infantile Batten disease) and intend to initiate a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. In addition to our product pipeline candidates, we are building a platform of next-generation technologies to optimize key components of our AAV-based gene therapies, including redosing, transgene regulation and capsid development.

We have a limited operating history. Since our inception, our operations have focused on organizing and staffing our company, business planning, raising capital and entering into collaboration agreements for conducting preclinical research and development activities for our product candidates. All of our lead product candidates are still in the preclinical testing stage. We do not have any product candidates approved for sale and have not generated any revenue from product sales. We have funded our operations through the sale of equity, raising an aggregate of $126.0 million of gross proceeds from the sale of shares of our convertible preferred stock through August 2020.

Since our inception, we have incurred significant operating losses. Our net losses were $1.1 million for the period from September 20, 2019 (date of inception) through December 31, 2019, and $26.7 million for the six months ended June 30, 2020. As of June 30, 2020, we had an accumulated deficit of $27.8 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- continue to advance the preclinical and clinical development of our product candidates and preclinical and discovery programs;

- conduct our planned clinical trial of TSHA-101, as well as initiate and complete additional clinical trials of TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and future product candidates;
- seek regulatory approval for any product candidates that successfully complete clinical trials;
- continue to develop our gene therapy product candidate pipeline and next-generation platforms;
- scale up our clinical and regulatory capabilities;
- manufacture current good manufacturing practices, or cGMP, material for clinical trials or potential commercial sales;
- establish and validate a commercial-scale cGMP manufacturing facility;
- establish a commercialization infrastructure and scale up internal and external manufacturing and distribution capabilities to commercialize any product candidates for which we may obtain regulatory approval;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, manufacturing quality control, regulatory, manufacturing and scientific and administrative personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- incur additional legal, accounting and other expenses in operating as a public company.

License Agreements

Research, Collaboration and License Agreement with The University of Texas Southwestern Medical Center

In November 2019, we entered into the UT Southwestern Agreement with The Board of Regents of the University of Texas System on behalf of UT Southwestern, as amended in April 2020.

In connection with the UT Southwestern Agreement, we obtained an exclusive, worldwide, royalty-free license under certain patent rights of UT Southwestern and a non-exclusive, worldwide, royalty-free license under certain know-how of UT Southwestern, in each case to make, have made, use, sell, offer for sale and import licensed products for use in certain specified indications. Additionally, we obtained a non-exclusive, worldwide, royalty-free license under certain patents and know-how of UT Southwestern for use in all human uses, with a right of first refusal to obtain an exclusive license under certain of such patent rights and an option to negotiate an exclusive license under other of such patent rights. We are required to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product.

In connection with the entry into the UT Southwestern Agreement, we issued to UT Southwestern 2,000,000 shares of our common stock. We do not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement, other than costs related to the maintenance of patents.

License Agreement with Queen's University at Kingston

In February 2020, we entered into a license agreement, or the Queen's University Agreement, with Queen's University at Kingston, or Queen's University. In connection with the Queen's University Agreement, we obtained an exclusive, perpetual, worldwide, royalty-bearing license, with the right to grant sublicenses, under certain patent rights and know-how of Queen's University, including certain improvements to the foregoing, to make, have made, use, offer for sale, sell and import licensed products and otherwise exploit such patents and know-how for use in certain specified indications. We also obtained an exclusive right of first negotiation to license certain next generation technology and improvements of Queen's University that do not constitute an already-licensed improvement to the licensed technology.

In connection with the Queen's University Agreement, we paid Queen's University a one-time fee of $3.0 million as an upfront fee and approximately $220,000 to reimburse Queen's University for certain plasmid production costs. We are obligated to pay Queen's University up to $10.0 million in the aggregate upon achievement of certain regulatory milestones and up to $10.0 million in the aggregate upon achievement of certain commercial milestones, a low single digit royalty on net sales of licensed products, subject to certain customary reductions, and a percentage of non-royalty sublicensing revenue ranging in the low double digits. Royalties are payable on a licensed product-by-licensed product basis and country-by-country basis until expiration of the last valid claim of a licensed patent covering such licensed product in such country and the expiration of any regulatory exclusivity for such licensed product in such country. Additionally, we are obligated to pay Queen's University a low double-digit portion of any amounts received by us in connection with the sale of a priority review voucher related to a licensed product, not to exceed a low eight-figure amount.

We are also obligated under the terms of a separate research grant agreement between us and Queen's University to pay up to a total of $3.8 million to fund certain manufacturing expenses.

License Agreement with Abeona Therapeutics Inc.

In August 2020, we entered into a license agreement, or the Abeona Agreement, with Abeona. In connection with the Abeona Agreement, we obtained an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses under certain patents, know-how and materials originally developed by University of North Carolina at Chapel Hill and Abeona to research, develop, manufacture, have manufactured, use, and commercialize licensed products for gene therapy for the prevention, treatment, or diagnosis of CLN1 Disease (or infantile Batten disease) in humans.

In connection with the license grant, we will pay Abeona a one-time upfront license fee of $3.0 million. We are obligated to pay Abeona up to $26.0 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed product and high single-digit royalties on net sales of licensed products. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the latest of the expiration or revocation or complete rejection of the last licensed patent covering such licensed product in the country where the licensed product is sold, the loss of market exclusivity in such country where the product is sold, or, if no licensed product exists in such country and no market exclusivity exists in such country, ten years from first commercial sale of such licensed product in such country. In addition, concurrent with the Abeona Agreement we entered into a purchase and reimbursement agreement with Abeona, pursuant to which we will purchase specified inventory from Abeona for total consideration of $4.0 million.

The Abeona Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last royalty term of a licensed product. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. We may terminate the agreement for convenience upon specified prior written notice to Abeona.

Components of Results of Operations

Revenue

To date, we have not recognized any revenue from any sources, including from product sales, and we do not expect to generate any revenue from the sale of products in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, or license agreements with third parties, we may generate revenue in the future from product sales. However, there can be no assurance as to when we will generate such revenue, if at all.

Operating Expenses

Research and Development Expenses

Research and development expenses primarily consist of preclinical development of our product candidates and discovery efforts, including conducting preclinical studies, manufacturing development efforts, preparing for clinical trials and activities related to regulatory filings for our product candidates. Research and development expenses are recognized as incurred and payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods or services are received. Costs incurred in obtaining technology licenses through asset acquisitions are charged to research and development expense if the licensed technology has not reached technological feasibility and has no alternative future use. Research and development expenses include or could include:

- employee-related expenses, including salaries, bonuses, benefits, stock-based compensation, other related costs for those employees involved in research and development efforts;
- external research and development expenses incurred under agreements with contract research organizations, or CROs, investigative sites and consultants to conduct our preclinical studies;
- costs related to manufacturing material for our preclinical studies and clinical trials, including fees paid to contract manufacturing organizations, or CMOs;
- laboratory supplies and research materials;
- costs related to compliance with regulatory requirements; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent, maintenance of facilities, insurance and equipment.

Research and development activities are central to our business model. We do not currently track our research and development expenses on a program-by-program basis as such costs are deployed across multiple projects under development. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We plan to substantially increase our research and development expenses for the foreseeable future as we continue the development of our product candidates and manufacturing processes and conduct discovery and research activities for our preclinical programs. We cannot determine with certainty the timing of initiation, the duration or the completion costs of current or future preclinical studies and clinical trials of our product candidates due to the inherently unpredictable nature of preclinical and clinical development. Clinical and preclinical development timelines, the probability of success and development costs can differ materially from expectations. We anticipate that we will make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the results of ongoing and future preclinical studies and clinical trials, regulatory developments and our ongoing assessments as to each product candidate’s commercial potential. We will need to raise

substantial additional capital in the future. Our clinical development costs are expected to increase significantly as we commence clinical trials. Our future expenses may vary significantly each period based on factors such as:

- expenses incurred to conduct preclinical studies required to advance our product candidates into clinical development;
- per patient trial costs, including based on the number of doses that patients received;
- the number of patients who enroll in each trial;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the length of time required to enroll eligible patients;
- the drop-out or discontinuation rates of patients;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the phase of development of the product candidate;
- third-party contractors failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards requiring that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks; and
- the efficacy and safety profile of our product candidates.

General and Administrative Expenses

General and administrative expenses consist or will consist principally of salaries and related costs for personnel in executive and administrative functions, including stock-based compensation, travel expenses and recruiting expenses. Other general and administrative expenses include professional fees for legal, accounting and tax-related services and insurance costs. We began paying salaries and related costs for personnel during the quarter ended June 30, 2020 and granted equity awards to employees and directors during the quarter ending September 30, 2020.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our expanded infrastructure, as well as the initiation and continuation of our preclinical studies and clinical trials for our product candidates. We also anticipate that our general and administrative expenses will increase as a result of payments for accounting, audit, legal, consulting services, as well as costs associated with maintaining compliance with Nasdaq listing rules and SEC requirements, director and officer liability insurance, investor and public relations activities and other expenses associated with operating as a public company. We anticipate the additional costs for these services will increase our general and administrative expenses by between $1.0 million and $2.0 million on an annual basis.

Results of Operations

Results of Operations for the Six Months Ended June 30, 2020

The following table summarizes our results of operations for the six months ended June 30, 2020 (in thousands):

	For the Six Months Ended June 30, 2020
Operating expenses:	
Research and development	$ 8,576
General and administrative	1,018
Total operating expenses	9,594
Loss from operations	(9,594)
Other expense:	
Change in fair value of preferred stock tranche liability	(17,030)
Interest expense	(27)
Total other expense	(17,057)
Net loss	**$ (26,651)**

Research and Development Expenses

Research and development expenses were $8.6 million for the six months ended June 30, 2020, and were primarily attributable to the upfront payment pursuant to the Queen's University Agreement for $3.0 million in March 2020, $3.8 million of chemistry, manufacturing and control costs for clinical trial material, $1.5 million of research expenses and the remainder in research and development personnel and consulting costs. We expensed the initial cost of the license acquired pursuant to the Queen's University Agreement within research and development expenses as it does not have an alternative future use.

General and Administrative Expenses

General and administrative expenses were $1.0 million for the six months ended June 30, 2020 and were primarily attributable to $0.5 million of compensation and benefits to new hires, $0.3 million in consulting fees and $0.2 million of legal expenses related to general corporate matters.

Other Expense

Change in Fair Value of Preferred Stock Tranche Liability

We determined that our obligation to issue, and the investors' right to purchase, additional shares of Series A convertible preferred stock pursuant to the milestone closings represented a freestanding financial instrument, or the tranche liability. The tranche liability was initially recorded at fair value. We concluded that the tranche liability met the definition of a freestanding financial instrument, as it was legally detachable and separately exercisable from the initial closing of the Series A convertible preferred stock.

On June 30, 2020, ahead of the anticipated closing of the Series B preferred stock financing at a purchase price of $17.00 per share on July 2, 2020, certain investors elected to exercise in full their options to purchase their pro-rata portion of the milestone shares prior to our achievement of the

clinical milestones and purchased 200,000 shares of Series A convertible preferred stock. We remeasured the fair value of the entire tranche liability at June 30, 2020, and recognized a non-cash expense of approximately $17.0 million.

Results of Operations from September 20, 2019 (date of inception) through December 31, 2019

The following table summarizes our results of operations for the period from September 20, 2019 (date of inception) through December 31, 2019:

	Period from September 20, 2019 (date of inception) through December 31, 2019 (in thousands)
Operating expenses:	
Research and development	$ 987
General and administrative	128
Total operating expenses	1,115
Net loss	$ (1,115)

Research and Development Expenses

Research and development expenses were $1.0 million for the period from September 20, 2019 (date of inception) through December 31, 2019, and were primarily attributable to the issuance of 2,000,000 shares to UT Southwestern as consideration for the license rights granted under the UT Southwestern Agreement. We expensed the initial cost of the license within research and development expenses as it does not have an alternative future use, and measured the cost of the license based on the grant date fair value of the aggregate shares of common stock issued to UT Southwestern of $980,000, or $0.49 per share. We anticipate our research and development expenses will continue to increase as we advance development of our programs.

General and Administrative Expenses

General and administrative expenses were $0.1 million for the period from September 20, 2019 (date of inception) through December 31, 2019, and were primarily attributable to legal and formation costs. We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our expanded operations as well as from increases in costs related to our public company compliance efforts.

Liquidity and Capital Resources

Overview

Since our inception, we have not generated any revenue and have incurred significant operating losses. As of June 30, 2020, we had cash and cash equivalents of $11.2 million. We have funded our operations through sales of convertible preferred stock, raising an aggregate of $126.0 million of gross proceeds from the sale of shares of our convertible preferred stock through August 2020. Specifically, between March and July 2020, we closed on the sale of an aggregate of 10,000,000 shares of Series A convertible preferred stock for gross proceeds of $30.0 million. In July and August 2020, we closed on the sale of an aggregate of 5,647,048 shares of Series B convertible preferred stock for gross proceeds of $96.0 million.

Funding Requirements

To date, we have not generated any revenues from the commercial sale of approved drug products, and we do not expect to generate substantial revenue for at least the next few years. If we fail to complete the development of our product candidates in a timely manner or fail to obtain their regulatory approval, our ability to generate future revenue will be compromised. We do not know when, or if, we will generate any revenue from our product candidates, and we do not expect to generate significant revenue unless and until we obtain regulatory approval of, and commercialize, our product candidates. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, initiate clinical trials of and seek marketing approval for our product candidates. In addition, if we obtain approval for any of our product candidates, we expect to incur significant commercialization expenses related to sales, marketing, manufacturing and distribution. Furthermore, following the completion of this offering, we expect to incur additional costs associated with operating as a public company. We anticipate that we will need substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital requirements into . We intend to devote the majority of the net proceeds from this offering to the clinical and preclinical development of our product candidates. We have based this estimate on assumptions that may prove to be imprecise, and we could utilize our available capital resources sooner than we expect.

Because of the numerous risks and uncertainties associated with research, development and commercialization of biological products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, progress, costs and results of discovery, preclinical development, laboratory testing and clinical trials for TSHA-101, TSHA-118, TSHA-102, TSHA-103, TSHA-104 and future product candidates;
- the extent to which we develop, in-license or acquire other product candidates and technologies in our gene therapy product candidate pipeline;
- the costs and timing of process development and manufacturing scale-up activities associated with our product candidates and other programs as we advance them through preclinical and clinical development;
- the number and development requirements of product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- our headcount growth and associated costs as we expand our research and development capabilities and establish a commercial infrastructure;
- the costs of establishing and maintaining our own commercial-scale cGMP manufacturing facility;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales, and distribution, for any of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;

- the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available in the near term, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the terms of these equity securities or this debt may restrict our ability to operate. Any future debt financing and equity financing, if available, may involve agreements that include covenants limiting and restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, entering into profit-sharing or other arrangements or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

We are continuing to assess the effect that the COVID-19 pandemic may have on our business and operations. The extent to which COVID-19 may impact our business and operations will depend on future developments that are highly uncertain and cannot be predicted with confidence, such as the ultimate geographic spread of the disease, the duration of the outbreak, the duration and effect of business disruptions and the short-term effects and ultimate effectiveness of the travel restrictions, quarantines, social distancing requirements and business closures in the United States and other countries to contain and treat the disease. While the potential economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, a widespread pandemic could result in significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the spread of COVID-19 could materially affect our business and the value of our common stock.

Cash Flows

Prior to the issuance and sale of our Series A convertible preferred stock in March 2020, we had no cash. Outstanding accrued expenses of $0.2 million as of December 31, 2019 were paid in 2020 following the receipt of the proceeds from the issuance and sale of our Series A convertible preferred stock.

The following table shows a summary of our cash flows for the six months ended June 30, 2020 (in thousands):

	Six Months Ended June 30, 2020
Net cash used in operating activities	$ (4,165)
Net cash used in investing activities	(3,000)
Net cash provided by financing activities	18,365
Net change in cash and cash equivalents	$ 11,200

Operating Activities

During the six months ended June 30, 2020, operating activities used $4.2 million of cash. Net cash used in operating activities for the six months ended June 30, 2020 was primarily attributable to a $26.7 million net loss and offset by $20.0 million of non-cash items, primarily driven by a change in fair value of our preferred stock tranche of $17.0 million liability related to the issuance of Series A convertible preferred stock and the upfront payment to acquire the license rights pursuant to the Queen's University Agreement for $3.0 million, which was recorded as a component of research and development expenses. The $26.7 million net loss was also partially offset by a $2.5 million increase in the cash provided by operating assets and liabilities, primarily resulting from an increase in accounts payable and accrued expenses.

Investing Activities

During the six months ended June 30, 2020, investing activities used $3.0 million of cash attributable to the upfront payment to acquire the license rights pursuant to the Queen's University Agreement of $3.0 million.

Financing Activities

During the six months ended June 30, 2020, financing activities provided $18.4 million of cash, which was primarily attributable to the issuance of 6,200,000 shares of our Series A convertible preferred stock in exchange for gross proceeds of $18.6 million, net of the payment of issuance costs of approximately $0.3 million. In January 2020, we also entered into two secured promissory notes with a related party, our President and Chief Executive Officer, RA Session II, for an aggregate of $1.67 million. During March 2020, we repaid $1.65 million of the notes. The remaining balance of approximately $28,000 was repaid in July 2020.

Contractual Obligations and Other Commitments

As of December 31, 2019, we did not have any commitments or contractual obligations. In addition, we enter into agreements in the normal course of business with CROs, CMOs and other vendors for research and development services for operating purposes, which are generally cancelable upon written notice. These payments are therefore not included in our contractual obligations herein.

We have not included milestone or royalty payments or other contractual payment obligations as the timing and amount of such obligations are unknown or uncertain, and are contingent upon the initiation and successful completion of future activities. See Notes 4 and 10 to our unaudited condensed consolidated financial statements included elsewhere in this prospectus.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Critical Accounting Policies and Significant Judgments and Estimates

This management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. In

accordance with U.S. GAAP, we evaluate our estimates and judgments on an ongoing basis, including those related to accrued expenses, the preferred stock tranche liability and stock-based compensation. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

We define our critical accounting policies as those accounting principles that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our significant accounting policies are more fully described in Note 2 to our audited financial statements and our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus, we believe the following are the critical accounting policies used in the preparation of our financial statements that require significant estimates and judgments.

Research and Development Expenses

We have entered into research and development contracts with research institutions and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected on the balance sheet as prepaid or accrued expenses. We record accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, we analyze progress of the studies, including the phase or completion of events, invoices received and contracted costs.

Research and development costs primarily consist of laboratory costs and other supplies, and the cost to acquire third-party licenses.

Costs incurred in obtaining technology licenses through asset acquisitions are charged to research and development expense if the licensed technology has not reached technological feasibility and has no alternative future use.

Stock-Based Compensation

For the six months ended June 30, 2020 and the period from September 20, 2019 (date of inception) through December 31, 2019, we did not incur any stock-based compensation expense related to equity awards granted to any employee, director or consultant. We began granting equity awards to employees and directors in the quarter ending September 30, 2020.

On July 1, 2020, we granted options to purchase an aggregate of 2,658,822 shares of common stock, all of which were subsequently cancelled, and a restricted stock award with respect to 705,882 shares of restricted common stock under our 2020 Equity Incentive Plan. We estimated that the fair value of such restricted stock was $5.75 per share. On September 2, 2020, we granted options to purchase an aggregate of 15,000 shares with an exercise price of $16.23 per share and restricted stock unit awards with respect to 2,615,935 shares of common stock. We estimated that the fair value of such restricted stock units was $16.23 per share.

We measure and recognize compensation expense for all options, restricted common stock awards and restricted stock units based on the estimated fair value of the award on the grant date. For our grants of restricted common stock awards and restricted stock units, the grant date fair value is based on the fair value of our common stock on the date of grant. We use the Black-Scholes option-pricing model to estimate the fair value of option awards. The fair value of these awards is recognized

as expense over the requisite service period, which is generally the vesting period of the respective award, on a straight-line basis when the only condition to vesting is continued service. We account for forfeitures as they occur. For awards where vesting is subject to a market or performance condition, expense recognition would be based on the derived service period. Expense for awards with performance conditions would be estimated and adjusted on a quarterly basis based upon our assessment of the probability that the performance condition will be met.

The determination of the grant date fair value of options using an option-pricing model is affected principally by our estimated fair value of shares of our common stock and requires management to make a number of other assumptions, including the expected life of the option, the volatility of the underlying shares, the risk-free interest rate and expected dividends. The assumptions used in our Black-Scholes option-pricing model represent management's best estimates at the time of grant. These estimates are complex and involve a number of variables, uncertainties and assumptions and the application of management's judgment, as they are inherently subjective. If any assumptions change, our stock-based compensation expense could be materially different in the future.

These assumptions are estimated as follows:

- *Fair Value of Common Stock*. As our common stock has not historically been publicly traded, we estimate the fair value of our common stock. See "—Fair Value of Common Stock."
- *Expected Term*. The expected term represents the period that our options are expected to be outstanding. For options that are considered "plain vanilla", we calculate the expected term using the simplified method based on the average of each option's vesting term and the contractual period during which the option can be exercised, which is typically 10 years following the date of grant.
- *Expected Volatility*. The expected volatility is based on the historical share volatility of several of our comparable publicly traded companies over a period of time equal to the expected term of the options, as we do not have any trading history to use the volatility of our own common stock.
- *Risk-Free Interest Rate*. The risk-free interest rate is based on the yields of U.S. Treasury securities with maturities appropriate for the term of the award.
- *Expected Dividend Yield*. We have not paid dividends on our common stock nor do we expect to pay dividends in the foreseeable future.

Fair Value of Common Stock

Beginning in July 2020 with the approval by our board of directors of our 2020 Equity Incentive Plan, the fair values of the shares of common stock underlying our options were estimated on each grant date by our board of directors. In order to determine the fair value, our board of directors considered, among other things, contemporaneous valuations of our common stock prepared by unrelated third-party valuation firms in accordance with the guidance provided by the American Institute of Certified Public Accountants Audit and Accounting Practice Aid Series: Valuation of Privately-Held-Company Equity Securities Issued as Compensation, or the Practice Aid. Given the absence of a public trading market of our capital stock, our board of directors will exercise reasonable judgment and consider a number of objective and subjective factors to determine the best estimate of the fair value of our common and preferred stock, including:

- contemporaneous third-party valuations of our common stock;
- the prices, rights, preferences and privileges of our convertible preferred stock relative to our common stock;

- our business, financial condition and results of operations, including related industry trends affecting our operations;
- the likelihood of achieving a liquidity event, such as an initial public offering or sale of our company, given prevailing market conditions;
- the lack of marketability of our common stock;
- the market performance of comparable publicly traded companies; and
- U.S. and global economic and capital market conditions and outlook.

The contemporaneous valuations prepared as of November 19, 2019, March 4, 2020, July 1, 2020 and August 15, 2020 resulted in a valuation of our common stock of $0.49, $0.52, $5.75 and $16.23 per share, respectively, as of those dates.

Common Stock Valuation Methodology

In determining the estimated fair value of common stock, our board of directors considered the subjective factors discussed above in conjunction with the most recent valuations of our common stock that were prepared by an independent third-party.

In estimating the fair market value of our common stock, our board of directors first determined the equity value of our business using accepted valuation methods. For valuations as of July 1, 2020 and November 19, 2019, we used the option-pricing model, or OPM, under which shares are valued by creating a series of call options with exercise prices based on the liquidation preferences and conversion terms of each equity class. The estimated fair values of the preferred and common stock are inferred by analyzing these options. Specifically, we use the OPM backsolve method to estimate the fair value of our common stock, which derives the implied equity value for one type of equity security from a contemporaneous transaction involving another type of security, such as the shares of our Series A and Series B convertible preferred stock. We used the OPM backsolve method because we were at an early stage of development and future liquidity events were difficult to forecast. We applied a discount for lack of marketability to account for a lack of access to an active public market.

For the valuation conducted as of August 15, 2020, we used a hybrid equity valuation and allocation model to determine our total equity value and resulting common stock per share value. The methodology aligns with the “Hybrid” method as described in the Practice Aid that incorporates weighted outcomes akin to the Probability Weighted Expected Return Method. Specifically, we considered the possibility of a near term low-priced, mid-priced and high-priced IPO and a scenario where no IPO takes place. This method is generally considered appropriate to use when there are several distinct scenarios to be considered.

Following the closing of this offering, the fair value of our common stock will be the closing price of our common stock on the Nasdaq Global Market as reported on the date of the grant.

Preferred Stock Tranche Liability

We determined that our obligation to issue, and the investors’ right to purchase, additional shares of Series A convertible preferred stock pursuant to the milestone closings represent a freestanding financial instrument, or the tranche liability. The tranche liability was initially recorded at fair value. The proceeds from the sale of the convertible preferred stock are first allocated to the fair value of the tranche liability, with the remaining proceeds from the sale of the convertible preferred stock allocated to the Series A convertible preferred stock. The tranche liability is remeasured at each reporting period and upon the exercise or expiration of the obligation, with gains and losses arising from subsequent

changes in its fair value recognized as a component of other expense in the condensed consolidated statement of operations. At the time of the exercise or expiration of the tranche liability, any remaining value of the tranche liability is reclassified to convertible preferred stock on the condensed consolidated balance sheet.

We estimated the fair value of the tranche liability using a Monte Carlo simulation at the initial issuance date. As of March 4, 2020, the simulations occurred based on our implied aggregate equity value derived from the Series A convertible preferred stock offering price of $3.00 per share, along with, in part, the following subjective assumptions: risk-free rate of 0.59%, an expected volatility of 80%, the expected term to a liquidity event of one year and a 60% probability of achieving the clinical milestones and timing thereof. Subsequently, we estimated the fair value of the tranche liability using a backsolve approach at June 30, 2020, which was calculated based on our aggregate equity value derived from the Series B convertible preferred stock offering price of $17.00 per share. The subsequent remeasurement also considered, in part, a risk-free rate of 0.17%, an expected volatility of 80% and the expected term to a liquidity event of 0.5 years.

Income Taxes

Income taxes are recorded in accordance with ASC 740, Income Taxes, or ASC 740, which provides for deferred taxes using an asset and liability approach. We recognize deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse, and net operating loss, or NOL, carryforwards and research and development tax credit carryforwards. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. We have recorded a full valuation allowance to reduce our net deferred income tax assets to zero. In the event we were to determine that we would be able to realize some or all of our deferred income tax assets in the future, an adjustment to the deferred income tax asset valuation allowance would increase income in the period such determination was made.

Recent Accounting Pronouncements

See Note 2 to our audited financial statements and our unaudited condensed consolidated financial statements appearing elsewhere in this prospectus for a description of recent accounting pronouncements applicable to our financial statements.

Qualitative and Quantitative Disclosures About Market Risk

Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because of our investments, including cash equivalents, which may be in the form of a money market fund.

We occasionally contract with vendors globally. We may be subject to fluctuations in foreign currency rates in connection with certain of these agreements. Transactions denominated in currencies other than the United States dollar are recorded based on exchange rates at the time such transactions arise. We have not engaged in the hedging of our foreign currency transactions to date. As of June 30, 2020, substantially all of our total liabilities were denominated in the United States dollar. We therefore believe that the risk of a significant impact on our loss from operations from foreign currency fluctuations is not substantial.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations for the six months ended June 30, 2020 and the period from September 20, 2019 (date of inception) through December 31, 2019.

Emerging Growth Company and Smaller Reporting Company Status

In April 2012, the JOBS Act was enacted. Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We elected the extended transition period for complying with new or revised accounting standards, which delays the adoption of these accounting standards until they would apply to private companies.

In addition, as an emerging growth company, we may take advantage of specified reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

- being permitted to present only two years of audited financial statements in addition to any required unaudited interim financial statements, with correspondingly reduced disclosure in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations";
- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements;
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements; and
- an exemption from compliance with the requirements of the Public Company Accounting Oversight Board regarding the communication of critical audit matters in the auditor's report on financial statements.

We may take advantage of these provisions until we no longer qualify as an emerging growth company. We will cease to qualify as an emerging growth company on the date that is the earliest of: (i) the December 31, 2025, (ii) the last day of the fiscal year in which we have more than $1.07 billion in total annual gross revenues, (iii) the date on which we are deemed to be a "large accelerated filer" under the rules of the SEC, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, or (iv) the date on which we have issued more than $1.0 billion of non-convertible debt over the prior three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. We have taken advantage of certain reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different than you might obtain from other public companies in which you hold equity interests.

We are also a “smaller reporting company,” meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

BUSINESS

Overview

We are a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations. We were founded in partnership with The University of Texas Southwestern Medical Center, or UT Southwestern, to develop and commercialize transformative gene therapy treatments. Together with UT Southwestern, we are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates, with exclusive options to acquire four additional development programs at no cost. By combining our management team's proven experience in gene therapy drug development and commercialization with UT Southwestern's world-class gene therapy research capabilities, we believe we have created a powerful engine to develop transformative therapies to dramatically improve patients' lives. We expect to initiate a Phase 1/2 clinical trial of TSHA-101 for the treatment of GM2 gangliosidosis, under a Clinical Trial Application, or CTA, in Canada by the end of 2020. In addition, we plan to submit investigational new drug applications, or INDs, for four programs to the U.S. Food and Drug Administration, or the FDA, by the end of 2021: TSHA-101, TSHA-102 (Rett syndrome), TSHA-103 (SLC6A1 haploinsufficiency) and TSHA-104 (SURF1 deficiency). We are also developing TSHA-118 for the treatment of CLN1 disease (or infantile Batten disease) and intend to initiate a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. In addition to our product pipeline candidates, we are building a platform of next-generation technologies to optimize key components of our AAV-based gene therapies, including redosing, transgene regulation and capsid development.

Our pipeline consists of AAV9-based gene therapies intended to be delivered using the intrathecal route of administration. Our manufacturing process utilizes suspension HEK293 cell culture that is highly scalable from the preclinical stages of development through commercialization. We believe this combination of AAV9, intrathecal delivery and suspension cell culture will accelerate our product development timelines while enhancing our probability of successfully developing and commercializing safe, efficacious therapies for patients.

We use an adeno-associated virus serotype 9, or AAV9, capsid, to deliver therapeutic genes engineered to replace a mutated gene, enhance the expression of a silenced gene or decrease the expression of a gene, depending on the underlying biology of the specific disease. In preclinical studies, the AAV9 capsid has been observed to have significantly higher transduction efficiency in cells of the central nervous system, or CNS, in comparison to AAV serotypes used in other gene therapy programs. In third-party clinical trials, AAV9 has been shown to be well tolerated, and in 2019, Zolgensma was approved as the first systemic gene therapy utilizing AAV9 for the treatment of spinal muscular atrophy, or SMA, Type 1, a severe neurodegenerative disease.

We use intrathecal administration, which involves direct delivery of our gene therapies to the cerebrospinal fluid, or CSF, to facilitate optimal biodistribution and cell transduction within the CNS. Because the CNS is immune-privileged, intrathecal gene therapy may be administered even in the presence of pre-existing antibodies to AAV. We believe that intrathecal delivery of AAV9-based gene therapies provides the highest likelihood of achieving transformative efficacy for patients suffering from severe, life-threatening neurological diseases.

Our flexible manufacturing processes allow us to produce our gene therapy product candidates efficiently at scale. Through our partnership with UT Southwestern, we have access to a Good Manufacturing Practice-, or GMP-, compliant manufacturing suite that utilizes a suspension HEK293 process to produce AAV9. We believe this capacity will be sufficient to meet the clinical demand for our full pipeline of product candidates. We also intend to establish our own commercial-scale, GMP-

compliant manufacturing facility to meet demand in the event that our product candidates receive marketing approval.

Our portfolio of gene therapy candidates targets broad neurological indications across three distinct therapeutic categories, which together have the potential to address over 500,000 patients in the United States and the European Union: neurodegenerative diseases, neurodevelopmental disorders and genetic epilepsies. Neurodegenerative diseases refer to conditions characterized by the progressive degeneration of the structures and functions of the CNS. Our neurodegenerative product candidates include TSHA-101 for the treatment of GM2 gangliosidosis, a family of severe neurodegenerative diseases that includes Tay-Sachs disease and Sandhoff disease, TSHA-118 for the treatment of CLN1, a progressive, fatal neurodegenerative disease with early childhood onset, and TSHA-104 for the treatment of SURF1 deficiency, a fatal, early-onset neurodegenerative disease. Neurodevelopmental disorders are a group of conditions with onset during the time when the brain is developing and are a reflection of disabilities associated primarily with the functioning of the neurological system and brain. One of our neurodevelopmental product candidates, TSHA-102, is in development for the treatment of Rett syndrome, which is one of the most common genetic causes of severe intellectual disability. Genetic epilepsies refer to disorders with recurrent seizures associated with abnormal development of the brain. One of our genetic epilepsy product candidates, TSHA-103, is in development for the treatment of SLC6A1 haploinsufficiency disorder, which is one of the most common monogenic causes of epilepsy. We expect to initiate a Phase 1/2 clinical trial of TSHA-101 by the end of 2020 under a CTA in Canada, and we plan to submit INDs to the FDA by the end of 2021 for each of TSHA-101, TSHA-102, TSHA-103 and TSHA-104. We intend to initiate a Phase 1/2 clinical trial of TSHA-118 under a currently open IND.

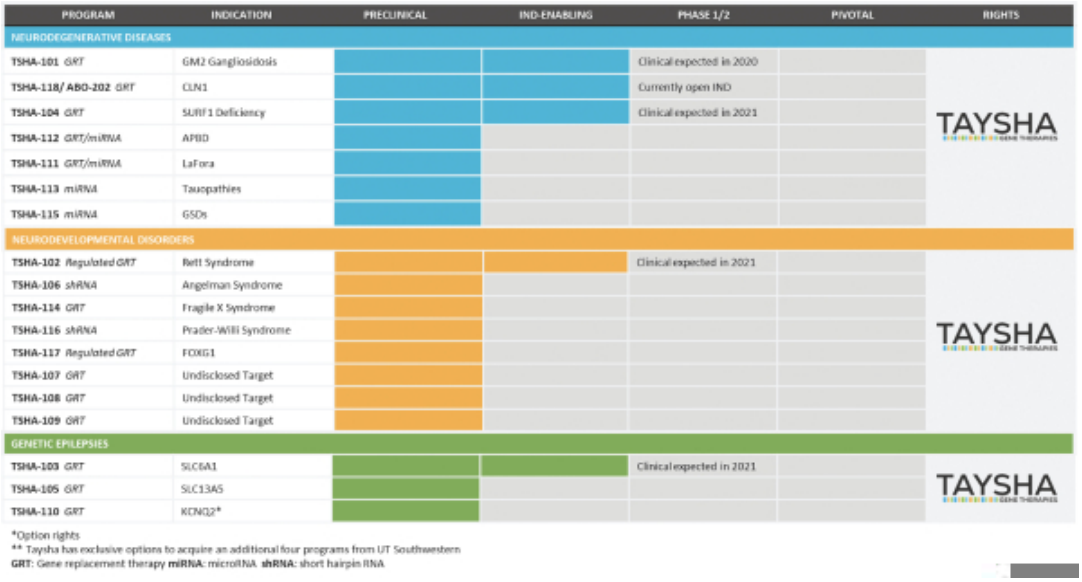
We have established an exclusive, differentiated partnership with UT Southwestern, one of the premier academic medical centers in the United States. We hold an exclusive, worldwide royalty-free license from UT Southwestern to discover, develop and commercialize gene therapies for our pipeline. Within the framework of our partnership, UT Southwestern will conduct discovery and preclinical research, lead IND-enabling studies, manufacture GMP vectors for use in preclinical studies and clinical trials and execute natural history studies to support the development of our product candidates. We are responsible for all clinical development, regulatory filings, strategy, commercial manufacturing and commercialization of approved product candidates. UT Southwestern has developed a state-of-the-art GMP viral vector manufacturing facility with the capacity to support the development of our product candidates from the discovery stage through early clinical development. We believe these factors differentiate our partnership with UT Southwestern from traditional collaborations between industry and academia and will enable us to advance our development programs with speed and scale. Our collaboration with UT Southwestern is led by Dr. Steven Gray, an expert in the development of AAV-based gene therapies for CNS disorders, and Dr. Berge Minassian, an expert in the diagnosis, management and treatment of rare pediatric neurological disorders. Drs. Gray and Minassian are also our scientific founders and currently serve as our Chief Scientific Advisor and Chief Medical Advisor, respectively.

We believe that we have established a unique position in advancing the development of gene therapies. Our scientific founders, Drs. Gray and Minassian, have extensive experience in developing gene therapies and conducting clinical trials for complex CNS diseases. Our management team has significant experience in discovering, developing, manufacturing and commercializing gene therapies. The members of our leadership team have specialized expertise developed at companies including Audentes Therapeutics, AveXis, BioMarin, PTC Therapeutics, Rocket Pharmaceuticals, and Sanofi-Genzyme. Our board of directors played an integral role in the formation of our company and is comprised of Sean Nolan, the chairman of our board of directors and former Chief Executive Officer of AveXis, Phillip B. Donenberg, the former Chief Financial Officer of AveXis, Paul Manning of PBM Capital, Sukumar Nagendran, M.D., the former Chief Medical Officer of AveXis, and RA Session II, our

President, Chief Executive Officer and Founder. Since our inception, we have raised an aggregate of approximately $126.0 million of gross proceeds from the sale of preferred stock, including from leading institutional and life science investors such as PBM Capital, certain funds managed by Fidelity Management & Research Company LLC, Nolan Capital, GV (formerly Google Ventures), Invus, Casdin Capital, Franklin Templeton, Octagon Capital, Perceptive Advisors LLC, Sands Capital, ArrowMark Partners, Venrock Healthcare Capital Partners and other mutual fund and institutional investors.

Our Pipeline

We are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates for monogenic diseases of the CNS in both rare and large patient populations, with exclusive options to acquire four additional development programs at no cost. Our portfolio of gene therapy candidates targets broad neurological indications across three distinct therapeutic categories: neurodegenerative diseases, neurodevelopmental disorders and genetic epilepsies. Our current pipeline, including the stage of development of each of our product candidates, is represented in the table below.



TSHA-101, a neurodegenerative disease product candidate, is being developed for the treatment of GM2 gangliosidosis, including Tay-Sachs disease and Sandhoff disease. GM2 gangliosidosis refers to a group of lysosomal storage disorders resulting from a deficiency in the ß-hexosaminidase A enzyme, leading to an accumulation of GM2 ganglioside in lysosomes and ultimately neuronal cell death and neurodegeneration. We are developing TSHA-101 as a bicistronic *HEXB*P2A-*HEXA* transgene packaged into an AAV9 vector under the control of a CAG promoter. We plan to initiate a Phase 1/2 clinical trial of TSHA-101 by the end of 2020 under a CTA in Canada, and we plan to submit an IND to the FDA by the end of 2021.

TSHA-118, a neurodegenerative disease product candidate, is being developed for the treatment of CLN1 disease (or infantile Batten disease), a lysosomal storage disorder that is a progressive, fatal disease with early childhood onset. TSHA-118 is a self-complementary AAV9 viral vector that expresses human codon-optimized *CLN1* complementary deoxyribonucleic acid under control of the

chicken ß-actin hybrid promoter. Preclinical studies evaluating safety and biodistribution have been conducted, and we plan to conduct a Phase 1/2 clinical trial of TSHA-118 under a currently open IND.

TSHA-102, a neurodevelopmental disorder product candidate, is being developed for the treatment of Rett syndrome, one of the most common genetic causes of severe intellectual disability, characterized by rapid developmental regression and in many cases caused by heterozygous loss of function mutations in *MECP2*, a gene essential for neuronal and synaptic function in the brain. We designed TSHA-102 to prevent gene overexpression-related toxicity by inserting microRNA, or miRNA, targets into the 3' untranslated region of viral genomes. This overexpression of *MECP2* is seen in the clinic in patients with a condition known as Rett duplication syndrome, where elevated levels of *MECP2* result in a clinical phenotype similar to Rett syndrome both in terms of symptoms and severity. TSHA-102 is constructed from a neuronal specific promoter, MeP426, coupled with the *miniMECP2* transgene, a truncated version of *MECP2*, and miRNA-Responsive Auto-Regulatory Element, or miRARE, our novel miRNA target panel, packaged in self-complementary AAV9. We plan to submit an IND for TSHA-102 to the FDA by the end of 2021.

TSHA-103, a genetic epilepsy product candidate, is being developed for the treatment of SLC6A1 haploinsufficiency disorder, one of the most common monogenic causes of epilepsy characterized by myoclonic atonic seizures, autism spectrum disorder and intellectual disability. TSHA-103 is constructed from a codon-optimized version of the human *SLC6A1* gene packaged within a self-complementary AAV9 viral vector under the control of a JeT promoter. We plan to submit an IND for TSHA-103 to the FDA by the end of 2021.

TSHA-104, a neurodegenerative disease product candidate, is being developed for the treatment of Surfeit locus 1, or SURF1, deficiency, a fatal, early-onset neurodegenerative disease. TSHA-104 is constructed from a codon-optimized version of the human *SURF1* gene packaged within a self-complementary AAV9 viral vector under the control of a modified version of the chicken ß-actin, or CBA, promoter CBA hybrid intron, or CBh. We plan to submit an IND for TSHA-104 to the FDA by the end of 2021.

Our Strategic Partnership with The University of Texas Southwestern Medical Center

We have established a differentiated partnership with UT Southwestern, one of the premier academic medical centers in the United States with a focus on integrating pioneering biomedical research with exceptional clinical care and education. Collectively, UT Southwestern faculty members have received six Nobel Prizes, and the faculty includes 24 members of the National Academy of Sciences, 16 members of the National Academy of Medicine and 13 Howard Hughes Medical Institute Investigators. The faculty of more than 2,700 is responsible for groundbreaking medical advances and is committed to translating science-driven research quickly to new clinical treatments.

The UT Southwestern Gene Therapy Program is led by Steven Gray, Ph.D., Director of the Viral Vector Core and Associate Professor in the Department of Pediatrics, and Berge Minassian, M.D., Division Chief of Child Neurology.

Dr. Gray serves as our Chief Scientific Advisor. His core expertise is in AAV-based gene therapy vector engineering and optimizing approaches to deliver therapeutic transgene to the CNS. His research also includes the design and execution of preclinical studies to apply these approaches toward the development of treatments for neurological diseases including giant axonal neuropathy, or GAN, Krabbe disease, Batten disease, Tay-Sachs disease, Sandhoff disease and Rett syndrome. He is the lead investigator on the GAN gene therapy project, which was the first clinical development program to deliver AAV9 through intrathecal administration. Dr. Gray has published over 50 peer-

reviewed papers in journals such as *New England Journal of Medicine*, *Molecular Therapy*, *Nature Biotechnology*, *Gene Therapy* and *The Proceedings of the National Academy of Sciences*. His research has been funded by the National Institute for Neurological Disorders and Stroke, as well as numerous large and small research foundations and patient advocacy organizations. In 2019, Dr. Gray was the recipient of the American Society for Gene and Cell Therapy Outstanding New Investigator Award. He earned his Ph.D. in molecular biology from Vanderbilt University and a B.S. with honors from Auburn University. He performed his postdoctoral fellowship focusing on gene therapy in the laboratory of world-renowned gene therapy expert Jude Samulski at the University of North Carolina, Chapel Hill.

Dr. Minassian serves as our Chief Medical Advisor. He is a Professor in the Departments of Pediatrics, Neurology and Neurotherapeutics and Neuroscience at UT Southwestern. He is the Division Chief of Child Neurology and serves on the faculty of the Children's Medical Center Research Institute at UT Southwestern. Dr. Minassian is a pediatric neurologist whose clinical specialties are epilepsy, neurodegenerative diseases and neurodevelopmental conditions. In 2004, Dr. Minassian described a new *MECP2* isoform, *MECP2B* and its encoded protein MECP2B. Dr. Minassian is also credited with discovering the *EPM2A and EPM2B* genes that cause Lafora disease. He has published more than 120 scholarly articles and authored or contributed to 10 books, and his many professional honors include the Jacob's Ladder 2014 Norman Saunders International Research Prize for Outstanding Scientist, the American Academy of Neurology 2007 Dreifuss-Penry Epilepsy Award, the Canadian Pediatric Society 2008 Sanofi Pasteur Research Award and the American Epilepsy Society 1996 Young Investigator Award. Dr. Minassian is a Fellow (Neurology) of the Royal College of Physicians and Surgeons of Canada.

Our partnership with UT Southwestern is differentiated from traditional collaborations between industry and academia due to our access to UT Southwestern's faculty, manufacturing facility and integrated research and clinical care approach, which, together, we believe will enable us to advance our development programs with speed and scale. Under the terms of our collaboration and license agreement with UT Southwestern, we have received an exclusive, worldwide royalty-free license to discover, develop and commercialize gene therapies for our pipeline.

Through our partnership, we are able to leverage the collective expertise of UT Southwestern researchers, clinicians and investigators with decades of experience in conducting cutting-edge research and providing clinical care, including in the neurodegenerative disease, neurodevelopmental disorder and genetic epilepsy therapeutic categories. Drs. Gray and Minassian expect significant growth in the number of researchers, clinicians, scientists and experts in gene therapy process development and manufacturing affiliated with the Gene Therapy Program over the next five years.

UT Southwestern's state-of-the art, GMP viral vector manufacturing facility consists of a full process development laboratory and 500 liter GMP suite with the capacity to support multiple preclinical and early clinical development efforts in parallel.

UT Southwestern is home a major pediatric neurology residency program. Dr. Minassian has spent more than two decades diagnosing patients with rare, often fatal disorders of the CNS. Dr. Minassian is focused on transforming the practice of clinical neurology through a commitment to integrating patient care and teaching, with the goal of fostering the development of new disease-modifying therapies. This unique integration of research and clinical care provides us with key insights into our patients' disease and its progression, symptoms and impact on quality of life. We plan to use these insights to design and refine our preclinical studies and clinical trials, understand incidence, prevalence and molecular epidemiology of the diseases and disorders we intend to treat, select the appropriate clinical endpoints for our efficacy studies and identify and characterize serum and imaging biomarkers. We also expect that UT Southwestern will serve as a source of enrollment for our clinical

trials, which is critically important for the less prevalent diseases in our pipeline. We collaborate with UT Southwestern to foster relationships with patient advocacy organizations and research foundations, including the National Tay-Sachs & Allied Diseases Association, Cure and Action for Tay-Sachs, SLC6A1 Connect, TESS Research Foundation, Cure Surf1 Foundation, Rett Syndrome Research Trust and International FOXG1 Foundation.

Our Strategy

We are building a patient-centric business with the goal of developing AAV-based gene therapies for the treatment of monogenic diseases of the CNS in both rare and large patient populations. We are focused on executing the following elements of our strategy:

- **Build a sustainable gene therapy company.** Our goal is to build a gene therapy company with a sustainable pipeline of product candidates and a consistent stream of new commercial product launches. To that end, we are focused on rapidly advancing our current pipeline of AAV9-based gene therapies while actively developing our next-generation platforms to discover and develop additional product candidates.
- **Advance our lead product candidates through clinical trials to commercialization.** Our product portfolio currently consists of 18 gene therapy product candidates targeting a diverse set of rare and prevalent CNS indications, with exclusive options to acquire four additional development programs from UT Southwestern at no cost. We intend to develop, seek regulatory approval and commercialize each product candidate in our portfolio. We expect to initiate a Phase 1/2 clinical trial of TSHA-101, a neurodegenerative product candidate, by the end of 2020, and we plan to submit INDs to the FDA for four programs by the end of 2021: TSHA-101, TSHA-102, TSHA-103 and TSHA-104. We intend to initiate a Phase 1/2 clinical trial of TSHA-118 under a currently open IND. If our clinical trials are successful, we plan to discuss expedited regulatory approval strategies with regulatory authorities.
- **Leverage our relationship with UT Southwestern.** We are anchored by a differentiated strategic partnership with UT Southwestern that allows us to access a highly experienced team of researchers and clinicians with deep experience in the underlying biology and treatment of monogenic CNS disorders and the patient populations that they treat. We believe that our partnership with UT Southwestern provides us with a significant advantage to rapidly discover, develop and commercialize novel gene therapies.
- **Utilize scalable manufacturing technologies.** A critical component of the development of any complex biological therapy, including gene therapies, is the ability to manufacture the therapy efficiently at scale. Through our partnership with UT Southwestern, we have access to a flexible, scalable and well-characterized GMP manufacturing suite that utilizes a suspension HEK293 process to produce AAV9, which we believe will enable us to produce material suitable for clinical trials in a cost and time-efficient manner. We believe the capacity offered by UT Southwestern's manufacturing facility will be sufficient to meet the clinical demand for our full pipeline of product candidates. Because we currently utilize the AAV9 capsid across our product portfolio, our product candidates differ primarily by their therapeutic transgene, and we believe that minimal changes to our optimized upstream and downstream processes are expected to be required to manufacture each product candidate. In addition, we intend to establish our own commercial scale GMP-compliant manufacturing facility to meet commercial demand if our product candidates receive marketing approval.
- **Develop our next-generation platform technologies.** In addition to our pipeline of AAV9-based gene therapies, we are actively developing three distinct platform technologies to enable the discovery, development, and rapid translation of new gene therapies: a proprietary technology to allow redosing of AAV-based gene therapies, transgene regulation (miRARE) and novel capsid development.

- **Evaluate strategic opportunities to accelerate development timelines and maximize the value of our product candidate pipeline.** We are evaluating opportunities to maximize the value of our product candidate pipeline, including through a joint venture or other structure in China. We believe these structures may provide us with the opportunity to leverage the financial and other resources of partners to advance the development of our product candidate pipeline in a key geography such as China.

Our Approach

Our approach to rapidly developing and commercializing gene therapies centers on the use of AAV9 delivered directly to the CSF using intrathecal administration. We manufacture our therapies using a scalable, HEK293 suspension-based process. We believe the combination of AAV9 manufactured in suspension and delivered intrathecally will allow us to rapidly advance the development candidates in our product pipeline.

Background on AAV9, Intrathecal Administration and Manufacturing

Since its discovery more than 50 years ago, AAV has been one of the most well studied vectors for the delivery of gene therapies, having been studied in over 250 clinical trials. We utilize AAV9, an AAV serotype with a unique ability to cross the blood-brain barrier and transduce cells of the CNS. AAV9 has been widely characterized across numerous preclinical studies and more than 15 ongoing or completed clinical trials and has a well-characterized biodistribution, safety, tolerability and efficacy profile. In 2019, the FDA approved Zolgensma, the first systemic gene therapy that utilizes AAV9, for the treatment of SMA Type 1 in infants.

Intrathecal administration refers to the injection of a therapy directly into the CSF. The procedure is routinely performed in an outpatient setting and is generally well tolerated. We intend to administer our product candidates intrathecally, as we believe that intrathecal administration confers several advantages for the delivery of gene therapies to the CNS. In comparison to intravenous administration, intrathecal administration allows for a lower dose of the therapy, as the vector is confined to the CNS with limited uptake into off-target tissues. Because the CNS is immune-privileged, intrathecal gene therapy may be administered even in the presence of pre-existing antibodies to AAV. Finally, intrathecally delivered gene therapies have limited exposure to peripheral organs, which enables a higher concentration of vector to be delivered to the diseased tissue of interest. A growing body of literature supports the safety and relevance of lumbar intrathecal injection to deliver AAV9 to the CNS and achieve favorable biodistribution and transgene expression profiles. In comparison to other AAV serotypes, AAV9 administration through lumbar intrathecal injection has been shown to result in superior transduction of multiple cells within the CNS.

We believe that intrathecally delivered AAV9-based gene therapies are more likely to achieve clinical and regulatory success than alternative delivery approaches. A third party is conducting a clinical trial of 32 patients to evaluate the efficacy, safety and tolerability of a one-time intrathecal administration of AAV9 delivering a copy of the *SMN1* gene for the treatment of SMA Type 2. As of December 2019, a similar tolerability and adverse event profile had been observed in the patients receiving intrathecal delivery as compared to patients receiving intravenous delivery. The same third party has dosed over 600 patients intrathecally or intravenously with this AAV9 therapy and has observed that it has been well tolerated, with durability of up to five years post-dosing. In a third-party clinical trial of patients with CLN6 Batten disease, 12 infant and pediatric patients were dosed with AAV9 delivering *CLN6* via intrathecal administration. In this trial, the AAV9 product candidate was well tolerated, with no drug-related adverse events observed. The same third party is currently conducting a clinical trial of an AAV9 product candidate delivering *CLN3* via intrathecal administration. Four patients have been dosed to date, with no serious adverse events observed.

We use a scalable production process for our product candidates using a suspension cell culture process in which mammalian HEK293 cells are transiently transfected with plasmid DNA. Our production process, including all process development, product characterization, analytical capabilities and purification techniques, is designed to efficiently scale to support our clinical and commercial development needs. The utilization of the AAV9 capsid across our product portfolio allows us to manufacture each product with minimal changes expected to be required to our optimized upstream and downstream process, since each of our product candidates differ primarily by their therapeutic transgene.

Our Therapeutic Strategy

We design our product candidates based on the underlying biology of the disease target and the characteristics that we believe will result in maximum therapeutic benefit for patients.

Gene replacement therapies. To treat diseases or disorders caused by a missing gene or limited expression of a gene due to loss-of-function mutations, we design our product candidates to replace the gene of interest. In general, these product candidates are comprised of a codon-optimized DNA transgene that encodes the wild type gene of interest, coupled with a promoter selected to ensure expression in the cell or tissue-type of interest.

Regulated gene replacement therapies. In a number of disorders, including Rett syndrome and FOXG1 syndrome, the expression of a therapeutic transgene needs to be regulated. In these disorders, high doses of transgene-expressing vectors may be harmful, while low doses may avoid toxicity but be sub-therapeutic. For disorders that require replacement of dose-sensitive genes, we have combined high-throughput miRNA profiling and genome mining to create miRARE, our novel miRNA target panel. This approach is designed to enable our product candidates to maintain safe transgene expression levels in the brain. Importantly, this built-in regulation system is fully endogenous, and therefore does not require any additional exogenous drug application.

Vectorized miRNA gene therapies. In certain diseases within our pipeline, including Lafora disease, adult polyglucosan body disease and tauopathies, the goal of our product candidates is to silence the expression of genes that are involved in or considered to be the root cause of disease onset and progression. To accomplish this, we design transgenes that express miRNA, which are small, non-coding sequences of RNA that result in silencing of gene expression.

Vectorized shRNA gene therapies. In certain diseases such as Prader-Willi syndrome and Angelman syndrome, the goal of our product candidates is to activate a constitutively silenced gene to generate a therapeutic effect under control of the endogenous promoters of the cell. We utilize transgenes that express short-hairpin RNA, or shRNA, which, upon binding to the target of interest, are designed to reactivate a silenced gene.

We design our preclinical studies to be highly translational into clinical trials, including to replicate the timing of dosing at points when the disease model has advanced and the phenotype is more pronounced, include relevant immunosuppressive regimes that are the standard of care for the disease target or commonly administered with gene therapies and use animal models that are reflective of the severity of the human disease. Our goal is to evaluate the safety, efficacy and biodistribution of our product candidates and generate dose-response data that inform our selection of the optimal doses for clinical translation. We believe that our stringent approach to evaluating efficacy in preclinical studies will translate into clinical trials and may be predictive of the clinical effect of our product candidates.

Our Next-Generation Platform Technologies

In addition to our pipeline of AAV9 product candidates, we are building a suite of platforms to develop next-generation technologies that can optimize key components of an AAV-based gene therapy.

Novel Route of Administration to Allow Redosing

We are advancing a novel AAV dosing platform with the potential to facilitate redosing by administering AAV-based gene therapies directly to the vagus nerve. In preclinical studies in adult rats, we observed that AAV9 delivery to the vagus nerve resulted in efficient targeting of the vagal neurons. In preclinical studies in dogs, AAV delivery to the vagus nerve was well tolerated at all doses. Post-mortem analysis showed that vagal nerve fibers and neurons were microscopically normal.

We believe that direct administration of our AAV9 therapies to the vagus nerve could be useful to treat the peripheral and autonomic manifestations of the CNS diseases in our pipeline. We plan to further evaluate the safety and feasibility of this approach in non-human primates, or NHPs.

Regulated Transgene Expression Using miRARE

In a number of disorders, including Rett syndrome and FOXG1 syndrome, the expression of a therapeutic transgene needs to be regulated. In these disorders, high doses of transgene-expressing vectors may be harmful, while low doses may avoid toxicity but be sub-therapeutic. For disorders that require replacement of dose-sensitive genes, we have combined high-throughput miRNA profiling and genome mining to create miRARE, our novel miRNA target panel. This approach is designed to enable our product candidates to maintain safe transgene expression levels in the brain. Importantly, this built-in regulation system is fully endogenous, and therefore does not require any additional exogenous drug application.

Novel Capsid Identification

We are developing a novel AAV capsid platform that utilizes machine learning, capsid shuffling and directed evolution to improve targeted delivery. Our approach allows us to identify capsids with improved properties in mice and NHPs in parallel to maximize their translational relevance. We are utilizing single-molecule, real-time, or SMRT, sequencing analysis for high throughput characterization of these capsids.

We believe that our approach will allow us to rapidly identify new capsids to drive new product candidates for CNS disorders with novel biodistribution and transduction profiles into our development pipeline.

Our Product Pipeline

Neurodegenerative Diseases

Our neurodegenerative disease programs target diseases that are characterized by the progressive degeneration of the structures and functions of the CNS. Degeneration and death of neuronal cells causes symptoms ranging from cognitive decline, functional impairment and, ultimately, death. Globally, neurodegenerative diseases represent an immense unmet medical need and disease management is complicated by a lack of effective symptomatic and disease-modifying therapies. Progressive neurodegeneration is a hallmark of numerous severe diseases each characterized by distinct etiology. Common neurodegenerative diseases include Alzheimer’s disease, Parkinson’s disease, amyotrophic lateral sclerosis, SMA and GM2 gangliosidosis.

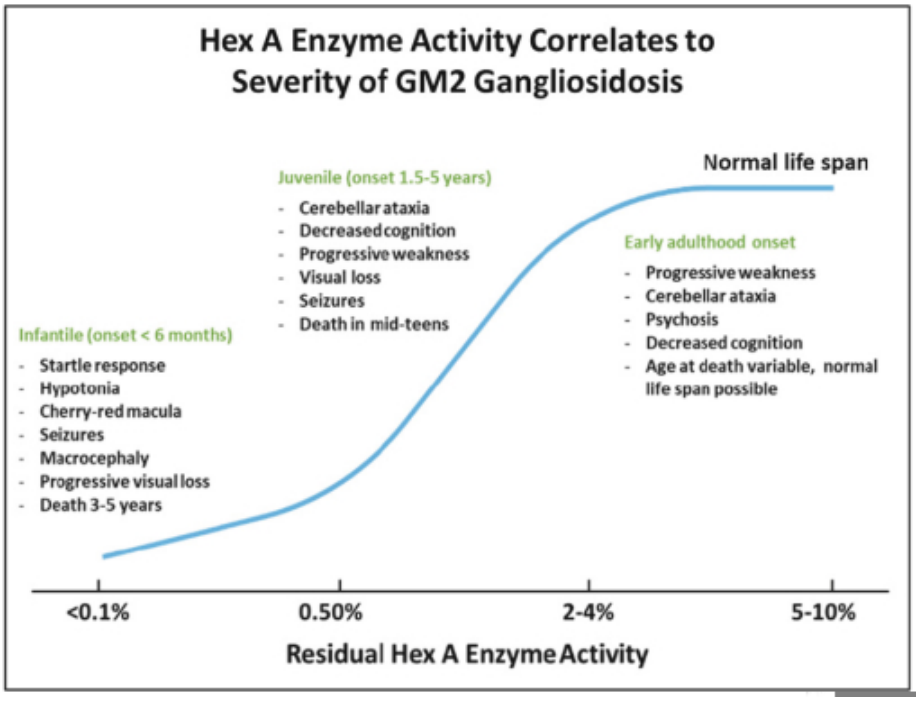
We are developing TSHA-101, a neurodegenerative product candidate, for the treatment of GM2 gangliosidosis, and we expect to initiate a Phase 1/2 trial by the end of 2020. We are also developing TSHA-118 for the treatment of CLN1 disease (or infantile Batten disease), for which we intend to conduct a Phase 1/2 clinical trial under a currently open IND, and TSHA-104 for the treatment of SURF1 deficiency. We are developing additional product candidates for the treatment of both prevalent neurodegenerative diseases, including Alzheimer's disease, Parkinson's disease and other tauopathies, and rare diseases such as adult polyglucosan body disease and Lafora disease.

TSHA-101 for the Treatment of GM2 Gangliosidosis

Overview of GM2 Gangliosidosis

GM2 gangliosidosis, including Tay-Sachs disease and Sandhoff disease, refers to a group of lysosomal storage disorders caused by accumulation of the GM2 ganglioside in the lysosomes of cells within the CNS. Gangliosides are lipid components of cell membranes particularly abundant in the plasma membranes of neurons. Accumulation of GM2 ganglioside is caused by a deficiency in the ß-hexosaminidase A, or Hex A, enzyme, which is responsible for hydrolysis, or breakdown, of the GM2 ganglioside. This accumulation results in lysosomal rupture, leading to a poorly understood inflammatory cascade that leads to neuronal cell death and neurodegeneration. The global incidence of GM2 gangliosidosis is approximately one per 150,000 live births. Approximately 80% to 85% of patients are diagnosed with an infantile form of GM2 gangliosidosis, with the remainder diagnosed with a juvenile or early-adulthood form of the disease. There are no approved therapies for the treatment of GM2 gangliosidosis, and care is generally palliative. Children diagnosed with infantile GM2 gangliosidosis appear normal at birth but experience rapid neurodegeneration, culminating in death before the age of four, and patients with juvenile GM2 gangliosidosis rarely survive beyond their mid-teens.

The Hex A enzyme is a heterodimer composed of two subunits: ß-hexosaminidase a (encoded in humans by the *HEXA* gene) and ß-hexosaminidase ß (encoded in humans by the *HEXB* gene). GM2 gangliosidosis caused by a mutation of the *HEXA* gene is termed Tay-Sachs disease, while Sandhoff disease is caused by a mutation of the *HEXB* gene. Tay-Sachs disease and Sandhoff disease result in clinically indistinguishable phenotypes for which there is no effective treatment. As illustrated in the graphic below, in infantile GM2 gangliosidosis, its most common and severe form, the disease is characterized by a lack of Hex A enzyme activity, while juvenile GM2 gangliosidosis is characterized by Hex A enzyme activity that is 0.5% to less than 2% of normal activity. Adult-onset GM2 gangliosidosis patients have Hex A enzyme activity levels typically in the range of 2% to 4% of normal Hex A activity and may live a normal lifespan. We believe that the "critical threshold" for normal hydrolysis of GM2 ganglioside is estimated to be 5% to 10% of normal Hex A activity.



We believe that successful gene therapy to treat Tay-Sachs disease or Sandhoff disease requires expression of the a and ß subunits in a 1:1 ratio to ensure that Hex A expression confers a therapeutic benefit. An imbalanced expression of either subunit could result in the formation of a dysfunctional homodimer, or identical proteins, which would limit the efficacy of the therapy. Several therapeutic approaches utilize single vectors encoding either the a or ß subunit, while other approaches have utilized multiple vectors carrying the *HEXA* and *HEXB* genes separately. However, these approaches either fail to deliver the Hex A subunits in the appropriate ratio or require the simultaneous transduction of cells to achieve efficacy.

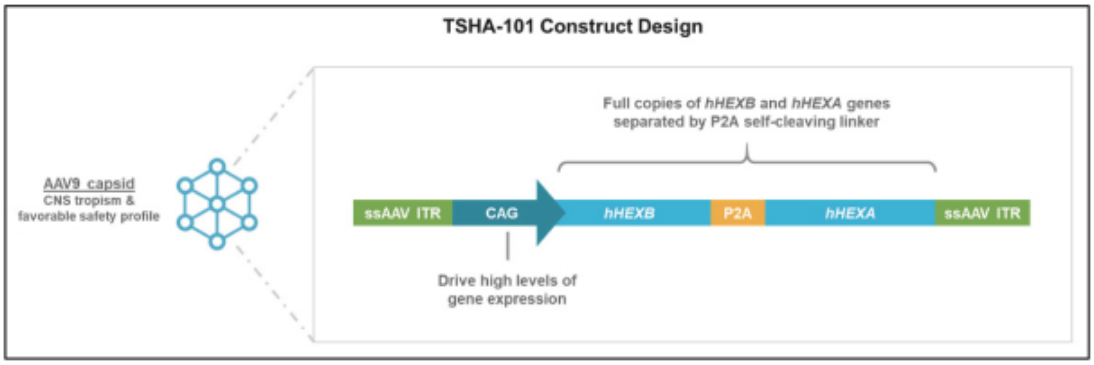
Similar to other lysosomal enzymes, Hex A is ubiquitously expressed and therefore concerns related to off-target effects or overexpression are limited. In addition, Hex A is secreted from transduced cells and can be taken up by neighboring cells to correct their phenotype, making it possible to cure these diseases without the need to transduce every cell, a process referred to as cross-correction. Studies suggest that restoring Hex A enzyme levels to approximately 10% of normal may result in complete phenotypic absence of the disease.

Our Solution: TSHA-101

We are developing TSHA-101, a neurodegenerative product candidate, for the treatment of GM2 gangliosidosis. TSHA-101 is a bicistronic, or dual loci of transcription, *HEXB*P2A- *HEXA* transgene packaged into an AAV9 vector under the control of the CAG promoter. We have designed TSHA-101 to link the human *HEXA* and *HEXB* genes, utilizing a cleavable peptide linker, to ensure that the expression of each the subunit occurs simultaneously at the appropriate 1:1 ratio. This approach is designed to maximize the expression of Hex A enzyme while minimizing the required therapeutic dosage.

Because GM2 gangliosidosis is clinically well defined, we believe we can leverage that knowledge to develop TSHA-101 with a higher probability of clinical and regulatory success. If approved, we believe that TSHA-101 could have a transformational impact on these severely underserved patients and their families. As TSHA-101 is designed to secrete the Hex A enzyme from transduced cells, uptake of the enzyme by neighboring cells via cross-correction has the potential to result in therapeutic benefit independent of their transduction status. In addition, we believe Hex A enzyme activity in the serum and CSF can serve as a potential biomarker to detect and help verify treatment effects on GM2 gangliosidosis during the early stages of clinical development.

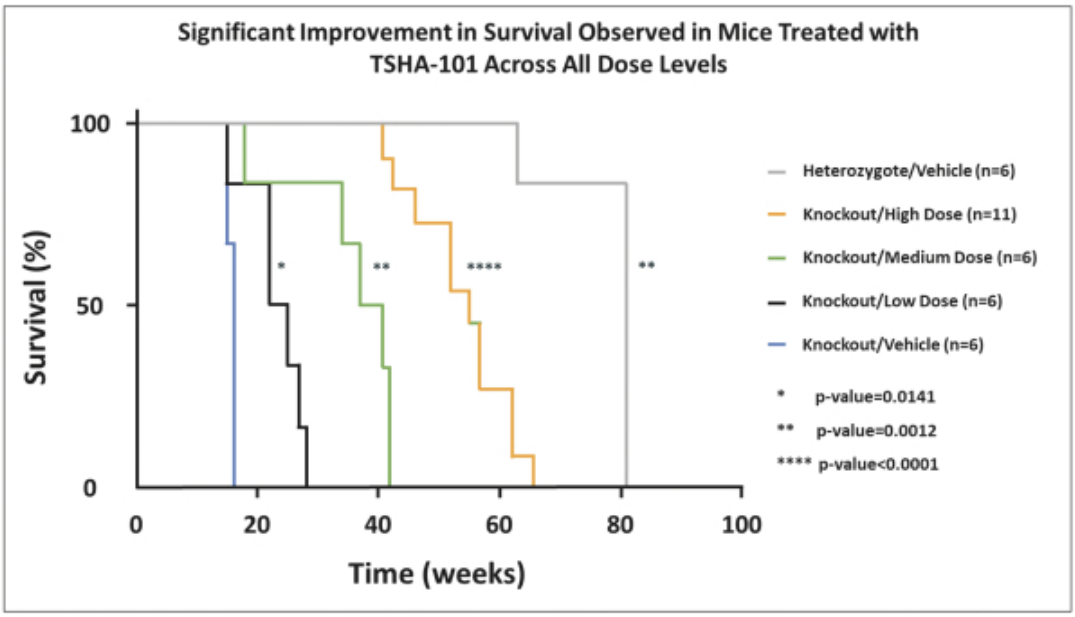
We have received orphan drug designation and rare pediatric disease designation from the FDA for TSHA-101 for the treatment of GM2 gangliosidosis.



Preclinical Studies

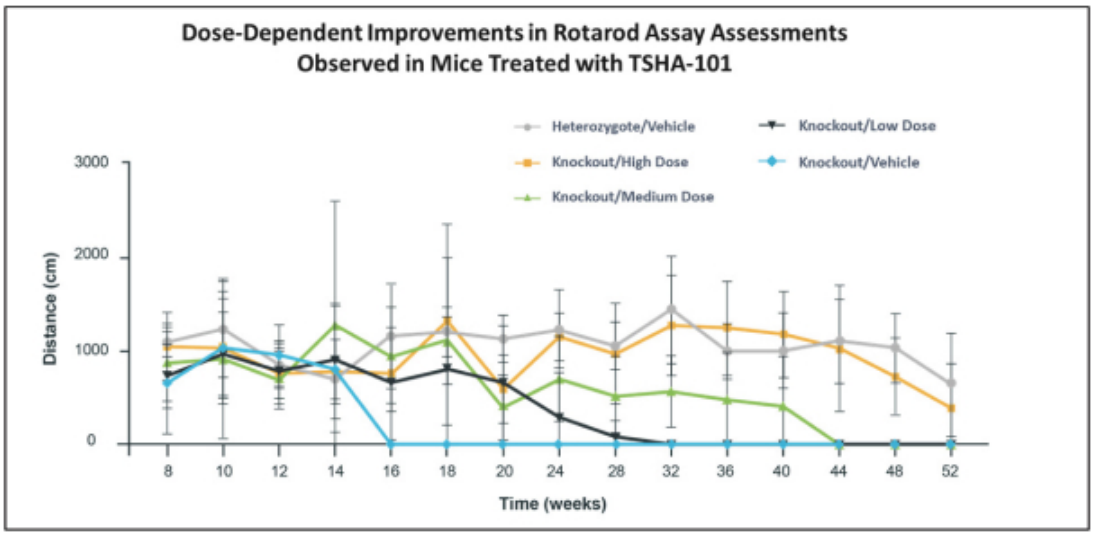
In preclinical studies, we observed evidence of improvements in behavioral assays and GM2 ganglioside accumulation across all dose levels of TSHA-101, which we believe supports continued development of TSHA-101. In these studies, TSHA-101 was administered at three dose levels via intrathecal lumbar puncture to a mouse model of Sandhoff disease, selected for its ability to recapitulate the severity of the human disease: a high dose of 2.5 x 10^{11} vg/mouse, a medium dose of 1.25 x 10^{11} vg/mouse and a low dose of 0.625 x 10^{11} vg/mouse. Mice were dosed at six weeks of age and subjected to a battery of behavioral tests when they reached eight weeks of age. At 16 weeks, a cohort of mice were euthanized, at which time GM2 ganglioside accumulation, Hex A enzyme levels and vector biodistribution were evaluated. An additional cohort was followed to a humane long-term endpoint defined as 15% body weight loss or an absent righting reflex.

Across all dose levels, we observed a significant improvement in survival in mice treated with TSHA-101 as compared to mice treated with vehicle alone, as illustrated below. A clear dose-response relationship was observed, with the high dose, medium dose and low dose of TSHA-101 shown to increase survival by 3.4-, 2.3- and 1.5-fold relative to vehicle control, respectively.

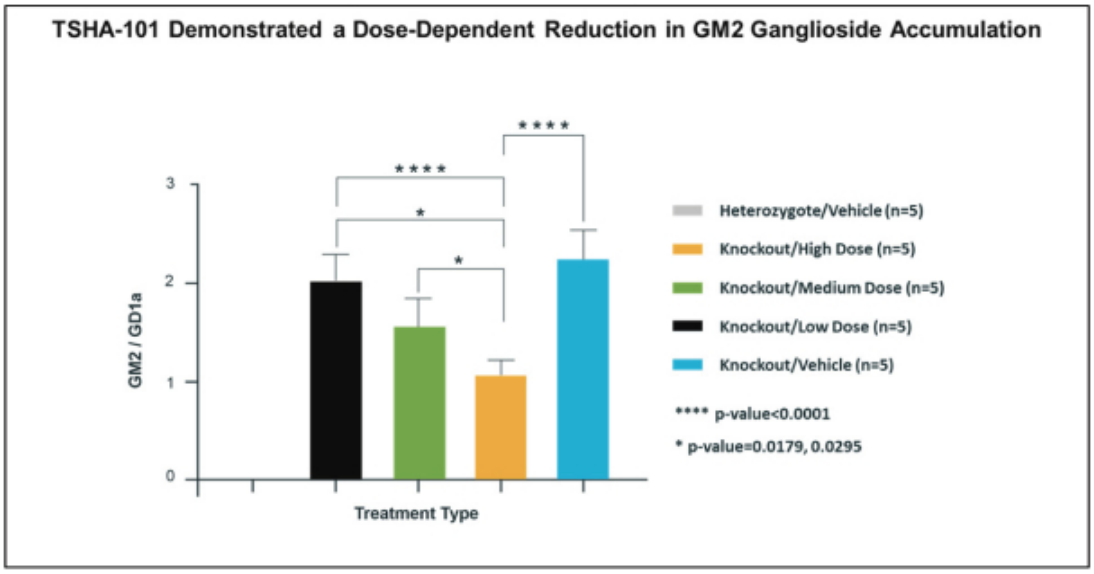


A result is considered to be statistically significant when the probability of the result occurring by random chance, rather than from the efficacy of the treatment, is sufficiently low. The conventional method for determining the statistical significance of a result is known as the "p-value," which represents the probability that random chance caused the result (e.g., a p-value = 0.01 means that there is a 1% probability that the difference between the control group and the treatment group is purely due to random chance). Generally, a p-value less than 0.05 is considered statistically significant.

We observed similar dose-dependent responses in our behavioral assessments of mice in the open field test and rotarod assays, both of which are commonly used tests to evaluate motor coordination in mice, as illustrated below.



At 16 weeks, we performed a GM2 ganglioside assay on the middle section of the brain of mice in all treatment groups. We observed a dose-dependent decrease in ganglioside accumulation, as illustrated below, suggesting a restoration of Hex A enzyme activity.



In preclinical studies designed to evaluate safety, no adverse findings or evidence of toxicity attributable to TSHA-101 were observed. To characterize the safety profile of TSHA-101, wild type mice were intrathecally administered a low dose of TSHA-101 of 2.5 x 10^{11} vg/mouse or a high dose of 5 x 10^{11} vg/mouse. The effect of immunosuppressants were also investigated. Mice received daily administration of prednisone and rapamycin, two immunosuppressants commonly used in combination with gene therapies.

At five weeks of age, a cohort of nine mice were euthanized as baseline controls. These mice did not receive gene therapy or immunosuppression. The experimental mice began receiving daily dosages of either 24 µg prednisone and 100 µg rapamycin or a vehicle gavage that continued for the remainder of the study. At six weeks, the experimental mice were administered either a vehicle treatment or TSHA-101 at either the low dose or high dose. Mice continued daily gavaging until their pre-assigned endpoint. Five male and five female mice from the first, second and third cohorts were euthanized at one week, four weeks and three months post-gene therapy injections, respectively. All mice from the fourth cohort were euthanized at four weeks post-gene therapy administration.

Complete blood cell count and biochemistry analyses were performed on whole blood and serum samples. Enzyme-linked immunospot assays were performed on the mice euthanized at four weeks and three months post-injection to assess the immune response of the mice. The biodistribution of the virus was also determined through quantitative polymerase chain reaction.

Clinical Development

We plan to submit a CTA to Health Canada for TSHA-101 and initiate an open-label, single center Phase 1/2 clinical trial in patients with a confirmed diagnosis of GM2 gangliosidosis by the end of 2020. As currently planned, patients will be evaluated over one year, followed by a longer-term extension period to monitor ongoing safety, developmental progression, select efficacy measures and

biomarkers, consistent with other pediatric gene therapy trials. An independent data safety monitoring board will review data from each patient on an ongoing basis to ensure that stopping criteria are not met and monitor the well-being of the patients in the trial. Patients will receive TSHA-101 in a total dose of $5x10^{14}$ vg (modified dependent on age) via intrathecal administration. We expect that the trial will measure safety as the primary endpoint, with key secondary endpoints including measures of efficacy and biomarkers. Efficacy will be evaluated by motor function scales, prevention of developmental regression, control of seizure activity and prevention of loss of milestones, as measured by validated scales. Additional measures of efficacy are expected to include plasma and CSF Hex A enzyme activity, GM2 ganglioside reduction and magnetic resonance imaging. We also intend to measure patient quality of life and caretaker burden using appropriate and accepted scales and observer-reported outcomes. Following preliminary results from our Phase 1/2 clinical trial, we plan to submit an IND to the FDA by the end of 2021 to commence a clinical trial for TSHA-101 in the United States, which we believe could serve as a pivotal trial to support registration subject to discussions with the FDA.

TSHA-118 (Formerly ABO-202) for the Treatment of CLN1 Disease

Overview of CLN1 Disease

CLN1 disease (or infantile Batten disease), a lysosomal storage disorder, is a progressive, fatal neurodegenerative disease with early childhood onset that has an estimated incidence of approximately 1 in 138,000 live births worldwide. CLN1 disease is caused by loss-of-function mutations in the *CLN1* gene that encodes the enzyme palmitoyl-protein thioesterase-1, or PPT1, a small glycoprotein involved in the degradation of certain lipid-modified proteins. Loss of function mutations in the *CLN1* gene causes accumulation of these lipid-modified proteins in cells, eventually leading to aggregation, neuronal cellular dysfunction and, ultimately neuronal cell death.

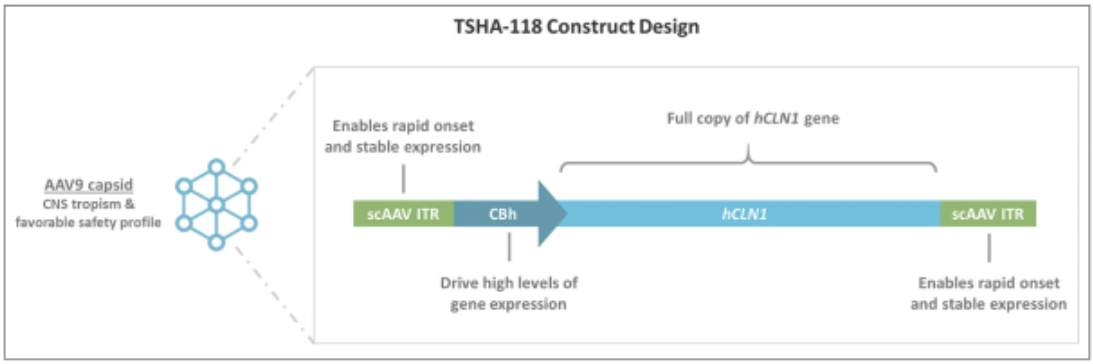
In the infantile-onset form of CLN1 disease, clinical symptoms appear between six to 24 months and include rapid deterioration of speech and motor function, refractory epilepsy, ataxia and visual failure. Infantile-onset CLN1 patients are typically poorly responsive by five years of age and remain noncommunicative until their death, which usually occurs by seven years of age. Late-infantile-onset CLN1 disease begins between two to four years of age with initial visual and cognitive decline followed by the development of ataxia and myoclonus, or quick, involuntary muscle jerks. Juvenile-onset CLN1 disease patients present between the ages of five to ten years old, with vision loss as a first symptom followed by cognitive decline, seizures and motor decline. Approximately 60% of the children diagnosed with CLN1 disease in the United States present with early-onset infantile forms, with the remaining 40% experiencing later-onset childhood forms.

All currently available therapeutic approaches for patients with CLN1 disease are targeted towards the treatment of symptoms, and no disease-modifying therapies have been approved. Gene therapy has shown promise in correcting forms of neuronal ceroid lipofuscinoses, or NCL, diseases that involve mutations in soluble enzymes, in part, due to cross-correction of neighboring non-transduced cells.

Our Solution: TSHA-118

We believe that the introduction of a functional *CLN1* gene using an AAV9 vector delivered intrathecally to the CNS exposure offers the potential of a disease-modifying therapeutic approach for this disease. TSHA-118 is a self-complementary AAV9 viral vector that expresses human codon-optimized *CLN1* complementary deoxyribonucleic acid under control of the chicken ß-actin hybrid promoter. We acquired exclusive worldwide rights to TSHA-118 (formerly ABO-202) in August 2020 pursuant to a license agreement with Abeona Therapeutics Inc., or Abeona.

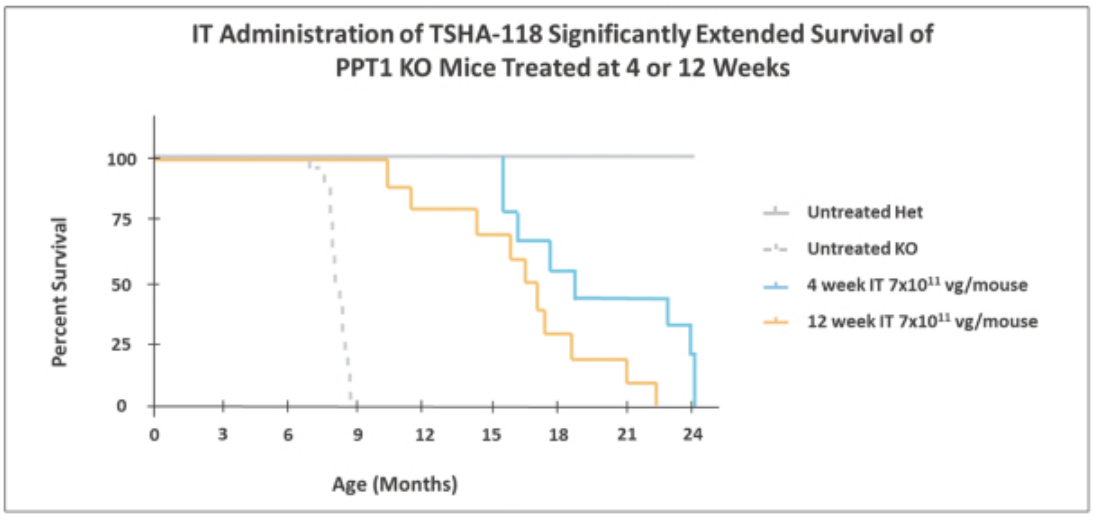
TSHA-118 has been granted orphan drug designation, rare pediatric disease designation and fast track designation from the FDA and orphan drug designation from the EMA for the treatment of CLN1 disease.



Preclinical Studies

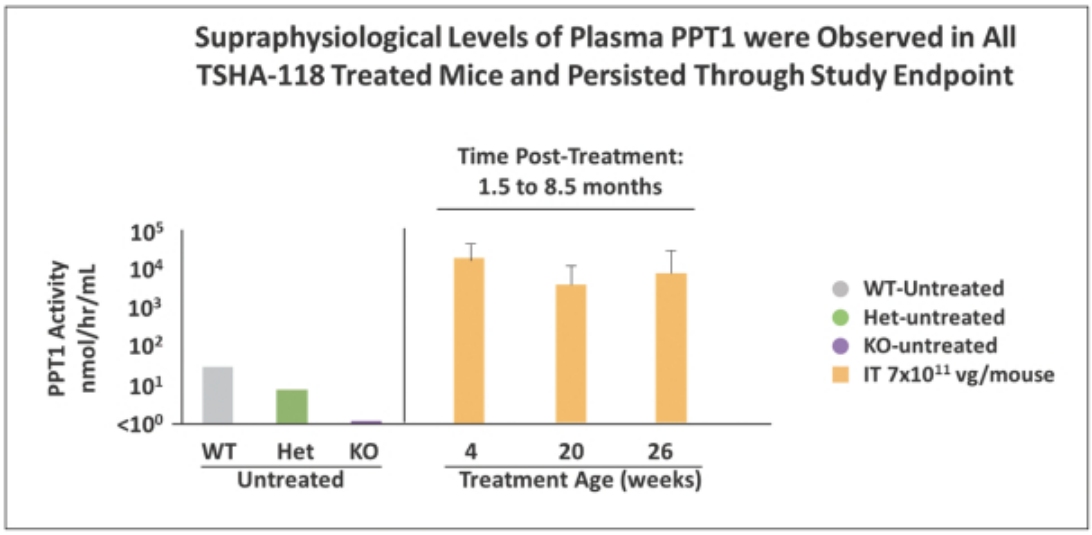
In third-party preclinical studies, evidence of improvements in behavioral outcomes, survival and restoration of PPT1 enzymatic activity was observed, which we believe supports continued development of TSHA-118. In these studies, TSHA-118 was administered at a dose of 7.0 x 10^{11} vg/mouse via intrathecal lumbar puncture to a mouse model of CLN1 disease, selected for its ability to recapitulate the severity of the human disease. The results from this study showed that intrathecal treatment with TSHA-118 significantly extended survival of CLN1 knockout mice, with enhanced survival and behavioral outcomes correlating with treatment at younger ages and higher doses.

As illustrated in the figure below, mice treated with TSHA-118 at four weeks or twelve weeks of age had a mean survival of 18.7 or 16.7 months, respectively, compared to approximately 8 months survival for untreated CLN1 knockout mice.



PPT1 enzyme activity in serum was measured at selected timepoints following TSHA-118 delivery by intrathecal administration at four, twenty or twenty-six weeks of age. Serum was collected either at four months post-treatment or at the humane endpoint.

As shown in the figure below, heterozygous mice had roughly 30% of normal serum PPT1 activity compared to wild-type mice. In contrast, treatment of CLN1 knockout mice with TSHA-118 resulted in supraphysiological levels of active PPT1 in the serum in comparison to wild-type and heterozygous mice.



Clinical Development

We plan to conduct a clinical proof-of-concept, dose-escalation Phase 1/2 trial in the United States and European Union, subject to feedback from the FDA and the EMA. The trial is expected to enroll approximately 12 patients, including patients with classic infantile onset and late infantile onset forms of CLN1 disease. We will conduct the trial in the United States under a currently open IND that Abeona is transferring to us. Patients will be enrolled in three cohorts of four patients each; within each cohort, three patients will be randomized to receive TSHA-118, and one patient will be randomized to receive delayed treatment as a concurrent control. We anticipate that the dose for the first cohort will be 5 x 10^{14} total vg administered intrathecally, with an escalation in doses in the second and third cohorts to be decided based on PPT1 enzyme levels in CSF and serum, clinical improvement and safety outcomes in the first cohort, as discussed with and reviewed by an independent data safety monitoring board. The data safety monitoring board will also review the safety information from the first patient dosed in each cohort before the second participant within that cohort can be dosed and will be involved in overall safety review and safety management in the trial.

We expect that the primary endpoints in the trial will include: safety and tolerability; developmental milestones; PPT1 enzyme activity in the serum and CSF; change in clinical progression as measured by the Unified Batten Disease Rating Scale; changes in a number of other developmental scales, including the Vineland Adaptive Behavior Scales-Third Edition, Bayley Scales of Infant and Toddler Development; seizure frequency, type and medication; ophthalmological assessment; quality of life and caregiver burden; and changes in MRI, including volumetric changes of the brain.

Additional Neurodegenerative Programs

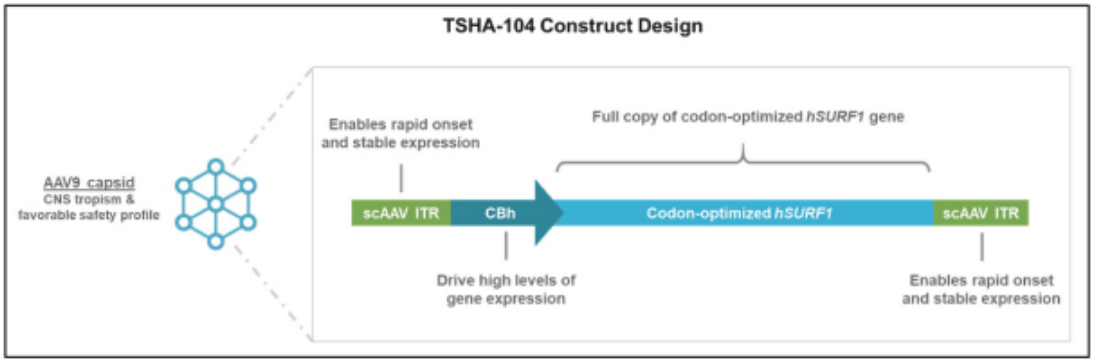
TSHA-104 for SURF1 Deficiency

We are developing TSHA-104, a neurodegenerative product candidate, for the treatment of SURF1 deficiency. The *SURF1* gene encodes the SURF1 protein, which plays a critical role in

mitochondrial translation and is involved in the assembly of the cytochrome c oxidase complex. Mutations in *SURF1* lead to SURF1 deficiency, a recessively inherited mitochondrial disease, and are the most frequent cause of Leigh syndrome, a rapidly progressive neurological condition characterized by the degeneration of the CNS. To date over 100 *SURF1* mutations, including non-sense, frame shift and missense variants have been described in literature. The incidence of SURF1 deficiency is estimated to be approximately 1 in 100,000 live births.

SURF1 deficiency can lead to difficulty swallowing in infancy, with subsequent failure to thrive. Severely diseased muscle tone leading to respiratory failure, movement disorders and balance abnormalities are common. According to the literature, only a few patients have been reported to survive beyond 10 years of age. In the majority of SURF1-deficient patients, serum lactate is elevated, and elevated levels of serum lactate have been reported in the CSF as well, indicative of mitochondrial dysfunction. We are pursuing a gene replacement strategy with the goal of restoring mitochondrial function in patients with SURF1 deficiency caused by loss-of-function mutations.

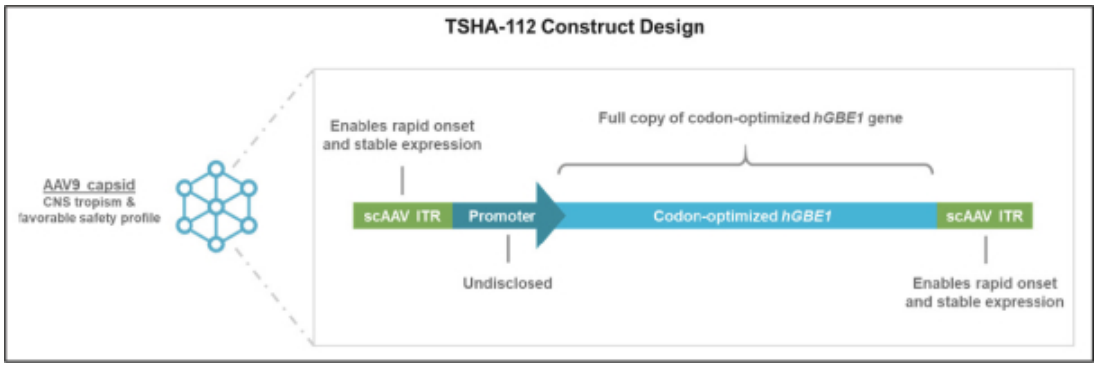
We are constructing TSHA-104 from a codon-optimized version of the human *SURF1* gene packaged within a self-complementary AAV9 viral vector under the control of a CBh promoter. We plan to submit an IND for TSHA-104 to the FDA by the end of 2021.



TSHA-112 for Adult Polyglucosan Body Disease

We are developing TSHA-112 for the treatment of adult polyglucosan body disease, or APBD. APBD is caused by reduced glycogen branching enzyme, or GBE1, activity. GBE1 is responsible for the creation of branches during glycogen synthesis and reduced GBE1 activity results in elongated glycogen changes that form poorly soluble aggregates (polyglucosan bodies) in the liver, muscle and CNS. Symptoms of APBD include sensory loss in the legs, progressive muscle weakness, gait disturbances, urinary difficulties and mild cognitive impairment. APBD is a late onset, prime of life disease with an age of onset between 40 to 50 years. The prevalence of APBD is unknown, but estimates range from 2,700 to 12,000 patients in the United States.

We are developing TSHA-112 as a gene replacement therapy to deliver wild type GBE1 packaged within a self-complementary AAV9 vector.

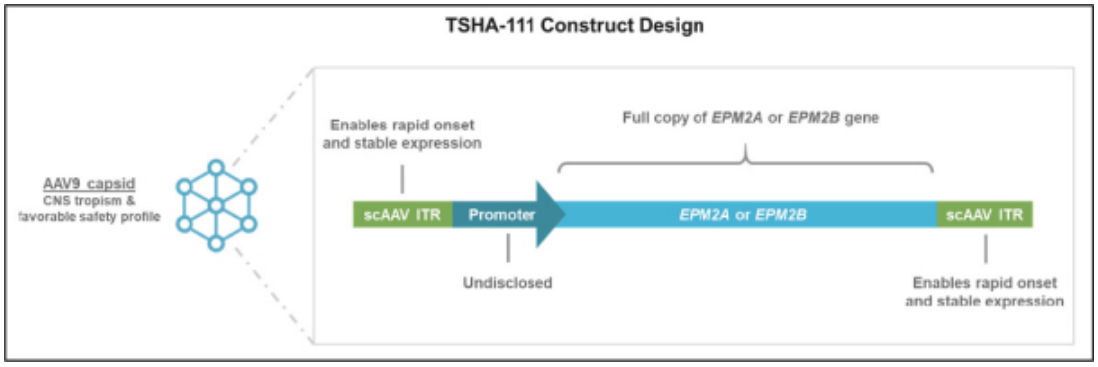


TSHA-111 for Lafora Disease

We are developing TSHA-111 for the treatment of Lafora disease, a fatal glycogen storage disorder. Lafora disease starts in healthy adolescents, usually with myoclonus, or involuntary muscle jerks, that rapidly evolve into progressive dementia, refractory epilepsy, cerebellar ataxia and respiratory failure, and generally results in death within about a decade. The epidemiology of Lafora disease is unknown although estimates suggests a prevalence of approximately 1 in 250,000 individuals worldwide. Currently, there is no treatment available for Lafora disease.

Lafora disease is caused by loss of function mutations in *EPM2A* or *EPM2B* genes, which encode the glycogen phosphatase laforin or the E3 ubiquitin ligase malin, respectively. Both laforin and malin are involved in the formation of regulating glycogen metabolism and the absence of either protein results in poorly branched cytoplasmic inclusions, known as Lafora bodies. Studies indicate that these inclusions are the primary of driver and neurodegeneration and other brain abnormalities associated with Lafora disease.

We are developing TSHA-111 as a gene replacement therapy that packages wild type laforin or malin in a self-complementary AAV9 vector for single-dose intrathecal administration.

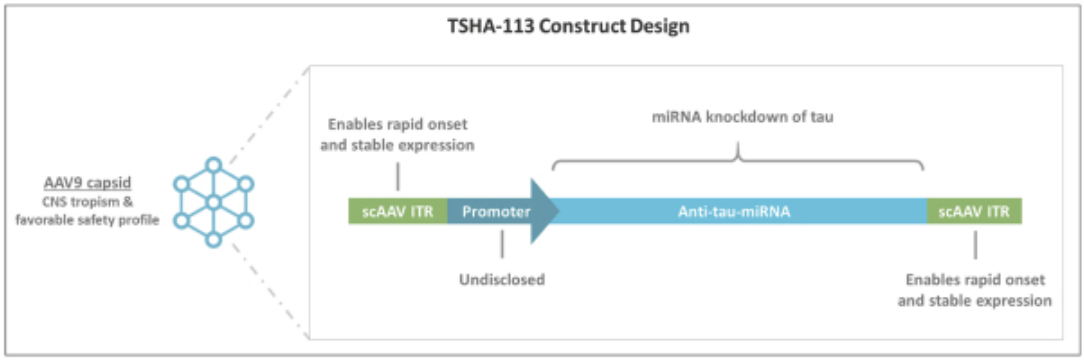


TSHA-113 for Tauopathies

We are developing TSHA-113 for the treatment of tauopathies. Tau accumulation predicts neurodegeneration in Alzheimer's disease, and the propagation of tau aggregates is thought to mediate the progression of several neurodegenerative diseases, including progressive supranuclear palsy, corticobasal degeneration, behavioral variant frontotemporal degeneration, chronic traumatic encephalopathy, frontotemporal dementia and parkinsonism linked to chromosome 17.

As a result, multiple strategies are currently being tested to reduce tau and ameliorate the effects of these diseases. Preclinical studies testing tau anti-sense oligonucleotides, or ASOs, in the PS19 tauopathy mouse model prevented neuronal loss and showed a reversal of pathological tau deposition and seeding. This treatment in being tested in clinical trials. While promising, ASOs only reduced tau protein levels by approximately 50% in mice, and they required repeated, life-long intrathecal administration to reach this maximum effect.

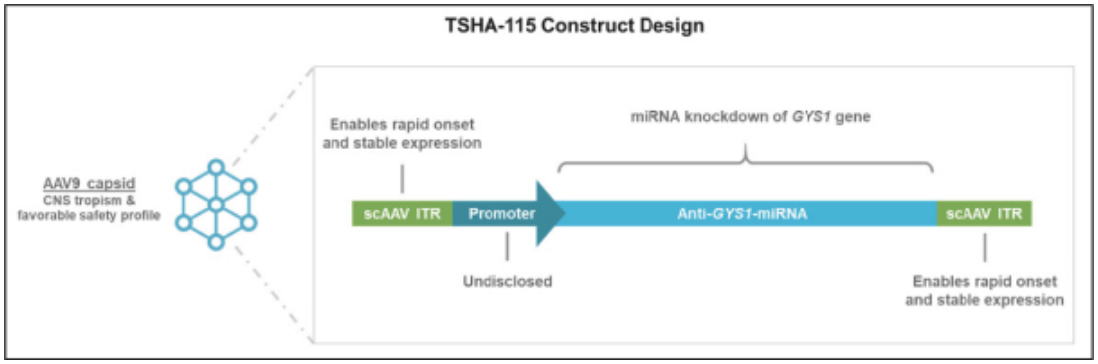
We are developing TSHA-113 to utilize AAV-mediated gene silencing to deliver life-long reduction of tau protein levels in neurons following administration of a single dose. We are developing tau-specific miRNA shuttles that have been designed to target mRNA for all six isoforms of tau found in the human brain and/or mouse brain. Our preliminary data in cells has shown that our tau miRNA selectively reduced some human and mouse tau expression *in vitro* and we have packaged our miRNA shuttles in AAV9 capsids for further evaluation in mouse models of human tauopathies.



TSHA-115 for Glycogen Storage Diseases

We are developing TSHA-115 for the treatment of glycogen storage diseases. TSHA-115 is designed to utilize a miRNA approach to knockdown GYS1, thereby inhibiting glycogen synthase in the brain. We believe this approach will provide therapeutic utility in not only Lafora Disease or APBD, but potentially other glycogen storage diseases as well.

Our preliminary data show that RNA interference-mediated silencing of GYS1 messenger RNA, or mRNA, provides therapeutic benefit in low dose mouse models by decreasing abnormal glycogen formation in the brain.



Neurodevelopmental Disorders

Neurodevelopmental disorders are a group of conditions whose age of onset occurs during the time when the brain is developing, typically between infancy and adolescence. Neurodevelopmental disorders can manifest as difficulties with language, speech and communication, motor skills, behavior, memory, learning and other neurological functions. The range of developmental deficits varies from very specific limitations of learning or control of executive functions to severe global impairments of social skills, intelligence and motor functioning.

The treatment of neurodevelopmental disorders is tailored towards symptom management and administered by an interdisciplinary team of neurologists, clinical psychologists, cognitive therapists, speech and language experts, behavioral therapists, and other specialists. Pharmacological interventions include but are not limited to typical and atypical antipsychotics, antiepileptic drugs, antidepressants and anti-anxiety medications.

We are developing TSHA-102, a neurodevelopmental product candidate, for the treatment of Rett syndrome. We are also developing product candidates for the treatment of other neurodevelopmental disorders, including Angelman syndrome, Fragile X syndrome, Prader-Willi syndrome and FOXG1 syndrome.

TSHA-102 for the Treatment of Rett Syndrome

Overview of Rett Syndrome

Rett syndrome is one of the most common genetic causes of severe intellectual disability worldwide with an incidence rate of one in every 10,000 live female births. After a period of normal development, girls with Rett syndrome experience rapid developmental regression characterized by a loss of speech and motor control. Cognitive and intellectual disabilities are common, as is dysfunction of the autonomic nervous system resulting in breathing, cardiovascular, and gastrointestinal abnormalities. Approximately 95% of Rett syndrome patients are females carrying heterozygous loss-of-function mutations in *MECP2*, a gene encoding methyl CpG-binding protein that is essential for neuronal and synaptic function in the brain.

Currently, there is no cure for Rett syndrome and available treatments for Rett syndrome are mainly to address symptoms. The primary focus of symptomatic therapy is ensuring that patients have access to the appropriate care specialists along with access to occupational therapy, speech therapy and physiotherapy. Sodium valproate, lamotrigine, levetiracetam, carbamazepine and clobazam are commonly prescribed to control seizures. There is a sudden death rate of 26% in Rett syndrome and patients mainly perish due to cardiac complications, respiratory infection and respiratory failure.

In February 2007, Sir Adrian Bird, Ph.D., a Professor of Genetics at the University of Edinburgh, published a seminal paper demonstrating the reversibility of symptoms associated with Rett syndrome in mice. This work suggested for the first time that neurological damage caused by the absence of *MECP2* may be reversible in children and adults with Rett syndrome. To demonstrate the ability to reverse the onset of Rett syndrome, researchers in the Bird lab generated mouse models in which *MECP2* expression was conditionally silenced, which resulted in neurological and behavioral deficits that mirrored those seen in patients diagnosed with Rett syndrome. Upon reactivation of the *MECP2* gene, behavioral deficits were restored, and electrophysiological function was also returned to levels seen in wild type mice. These findings represented a landmark moment for the field and provided evidence that it may be possible to not only halt the symptoms of Rett syndrome, but to potentially reverse the course of the disease.

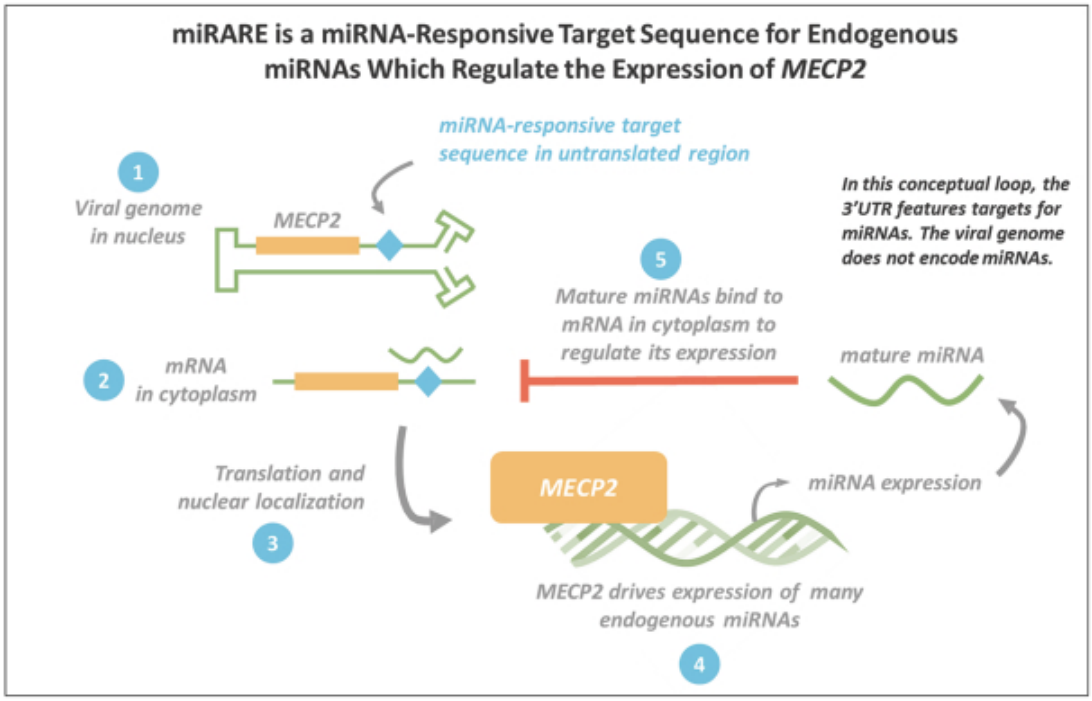
Several peer-reviewed publications have subsequently explored the safety and efficacy of AAV-based gene therapy for the treatment of Rett syndrome, including in preclinical studies that

showed that delivery of *MECP2* and, more recently, *miniMECP2*, extended the survival of *MECP2* knockout mice, a commonly used mouse model that recapitulates the symptoms of Rett syndrome. However, AAV delivery of *MECP2* or *miniMECP2* resulted in dose-dependent toxicity in wild type and *MECP2* knock out mice. Given the underlying biology of the disease, there is consensus among researchers that toxicity in these models is most likely linked to unregulated expression of *MECP2* or *miniMECP2*. This overexpression of *MECP2* is seen in the clinic in patients with a condition known as Rett duplication syndrome, where elevated levels of *MECP2* result in a clinical phenotype similar to Rett syndrome both in terms of symptoms and severity.

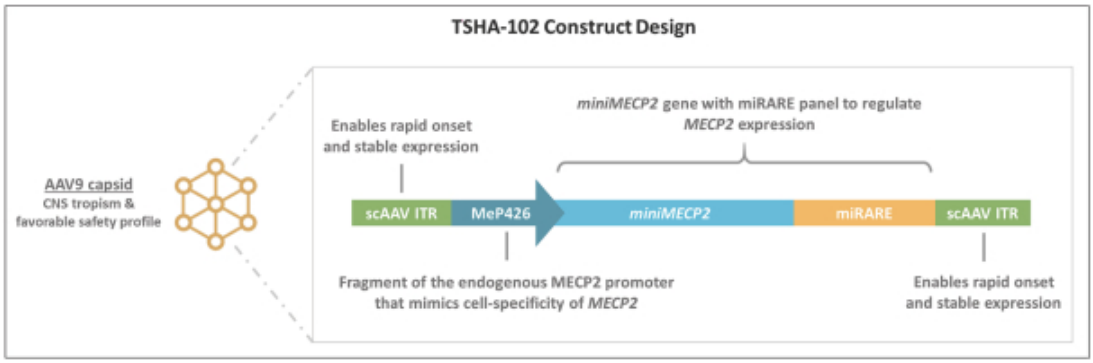
Our Solution: TSHA-102

We are developing TSHA-102, a neurodevelopmental product candidate, for the treatment of Rett syndrome. In order to develop an effective therapy for Rett syndrome, *MECP2* expression needs to be titrated to correct the *MECP2* deficiency while avoiding side effects associated with toxic overexpression.

MECP2 is a transcription factor within the cell that regulates many other genes involved in neurodevelopmental function. Levels of *MECP2* are regulated within the cell endogenously by a number of different miRNAs. We utilized this natural regulatory phenomenon to develop miRARE, our miRNA target panel. miRARE binds those endogenous miRNA species whose expression levels are upregulated as a result of exogenous *MECP2* protein expression. When miRARE is incorporated into the 3' untranslated region of our expression cassette, it functions as a mechanism to permit therapeutic expression of *MECP2* while buffering against toxic overexpression, as shown in the figure below.

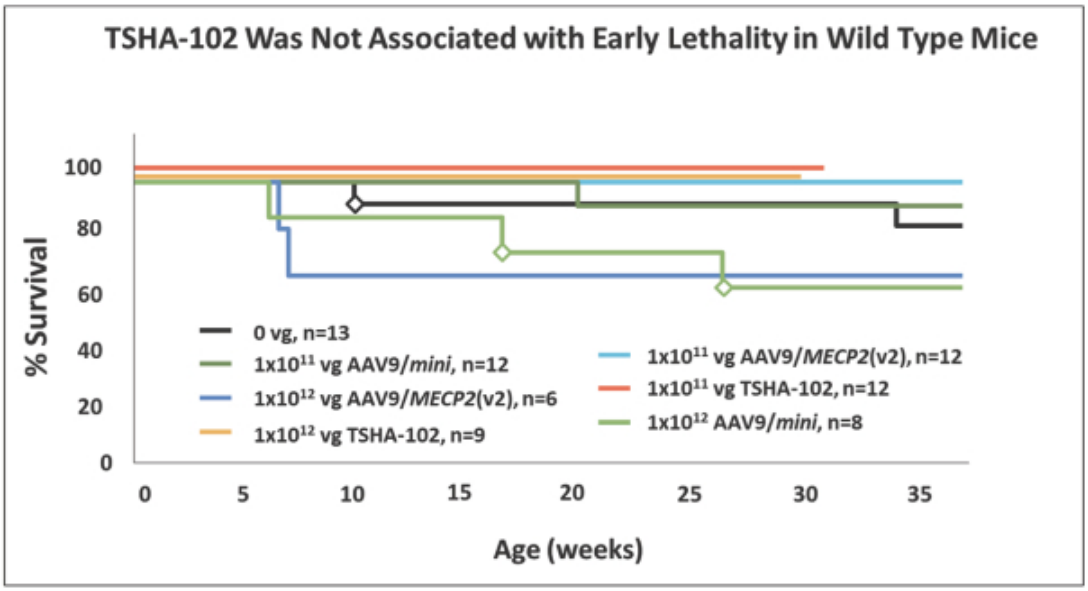


TSHA-102 is constructed from a neuronal specific promoter, MeP426, coupled with the *miniMECP2* transgene and our miRARE panel packaged in self-complementary AAV9.



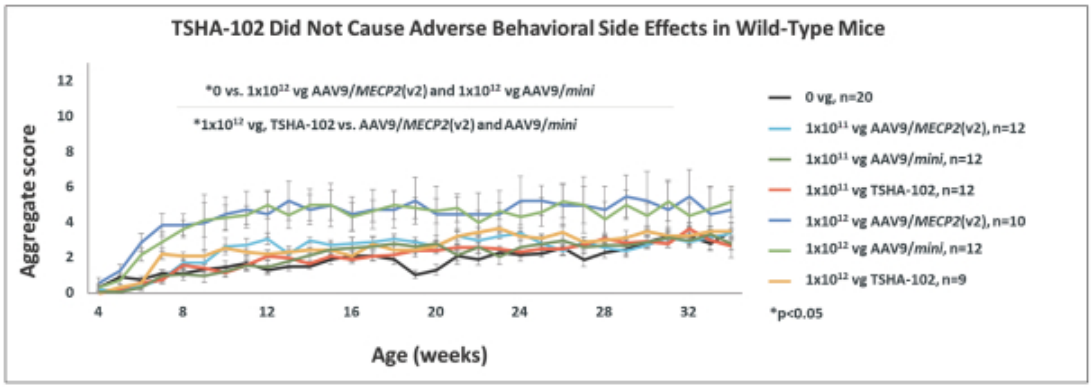
Preclinical Studies

In preclinical studies, we observed that TSHA-102 had a favorable tolerability profile and increased survival in a wild type mice model. In these studies, wild type mice were treated with low doses of 1 x 10^{11} vg or high doses of 1 x 10^{12} doses of either unregulated AAV9/*MECP2*, unregulated AAV9/*miniMECP2* or TSHA-102 at four to five weeks of age. Mice treated with the high dose of unregulated constructs experienced early lethality, while the low dose unregulated constructs were similar to vehicle. Mice treated with TSHA-102 had no deaths associated with treatment, as shown in the figure below.

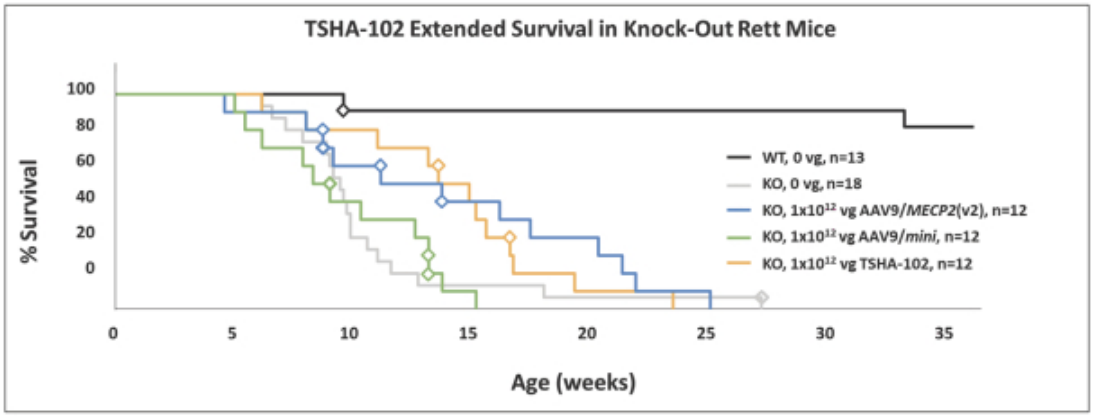


MECP2 dose-dependent side effects, leading to an increase in the aggregate score of a well-recognized, validated Rett behavioral assessment tool used in mice, were observed in the cohorts treated with unregulated AAV9/*MECP2* or AAV9/*miniMECP2*, as shown in the figure below. This increase in aggregate score is a reflection of overexpression of *MECP2* causing Rett-like symptoms in

these mice, as is seen in Rett duplication syndrome. In contrast, TSHA-102 was well tolerated and aggregate scores were similar to those of mice treated with vehicle only. This aggregate scale includes six sub-scales for abnormal mobility, abnormal gait, hindlimb clasping, tremors, abnormal breathing and abnormal general appearance. Sub-scales were each assessed on a scale of 0 to 12, with 12 being the maximum aggregate score attainable. Scorers were blind to treatment and genotype, and lower scores indicate better tolerability.



In *MECP2* knockout mice, unregulated AAV9/*miniMECP2* failed to extend survival at either dose tested. In contrast, TSHA-102 dosed at 1x10^{12} vg/mouse extended survival by 56%. Importantly, although there is a strong trend toward increased survival in mice treated with AAV9/*MECP2*, studies in wild type mice showed unacceptable toxicity with this vector design at this dose.



We observed TSHA-102 to be well tolerated, with no behavioral abnormalities or other side effects associated with *MECP2* overexpression. We evaluated the safety of TSHA-102 in wild type mice following intrathecal administration. While AAV9/*MECP2* and AAV9/*miniMECP2* caused dose-responsive reductions in weight, weight reduction was not observed in mice treated with TSHA-102. In addition, no tail lesions were observed among mice treated with TSHA-102. In contrast, lesions were observed in 8% to 17% of wild type mice treated with unregulated AAV9/*miniMECP2* and 8% of mice treated with unregulated AAV9/*MECP2*.

Clinical Development

Following the completion of Good Laboratory Practice, or GLP,-toxicology studies in NHPs and the completion of additional preclinical studies, we intend to submit an IND to the FDA by the end of 2021. In clinical trials, we intend to evaluate safety, tolerability and efficacy utilizing multiple clinical scales and biomarkers. Breathing disorders are reported in over 66% of patients with Rett syndrome, often leading to sleep disorders. Patients hyperventilate, hold their breath and have apneic episodes, leading to poor sleep quality. Accordingly, we plan to measure both sleep quality and breathing in our clinical trials. In addition, epilepsy is reported in over 60% of patients with Rett syndrome and likely will serve as a secondary efficacy endpoint in our trials.

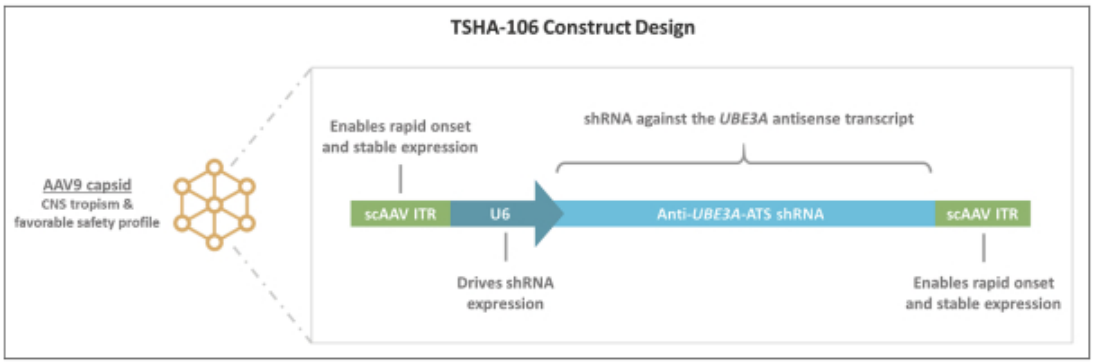
Additional Neurodevelopmental Programs

TSHA-106 for Angelman Syndrome

We are developing TSHA-106 for the treatment of Angelman syndrome, a neurodevelopmental disorder caused by a maternal deficiency of the *UBE3A* gene. Angelman syndrome is characterized by profound developmental delay, ataxia and gait disturbance, sleep disorder, seizures, heightened anxiety and aggression and severe speech impairments. Angelman syndrome affects approximately one per 12,000 to 20,000 patients worldwide.

Angelman syndrome is an imprinting disorder in which the maternal gene is deficient and the paternal copy of *UBE3A* is intact but silenced by a long non-coding RNA, *UBE3A* antisense transcript, or *UBE3A*-ATS. Delivery of an ASO targeting UBE3A-ATS showed promising results in ameliorating Angelman syndrome symptoms in a transgenic mouse model.

We are developing TSHA-106 to target the *UBE3A*-ATS transcript through shRNA knock-down with an AAV-based strategy in order to achieve broad distribution of the shRNA expression cassette across the entire CNS following a single intrathecal dose.



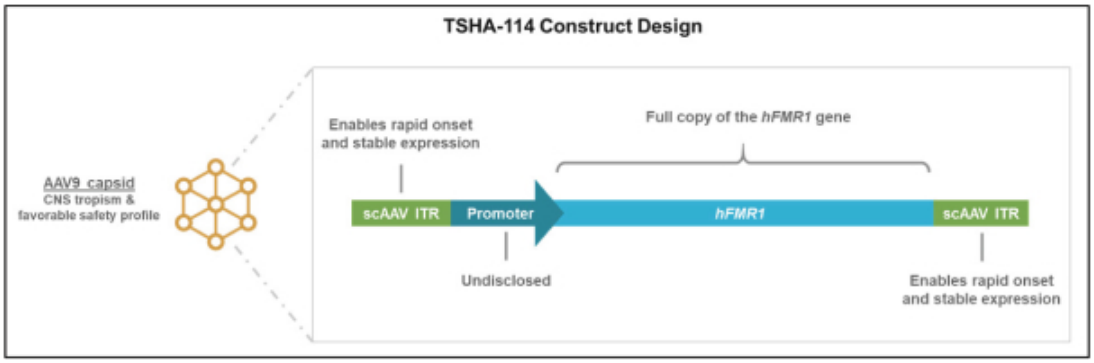
TSHA-114 for Fragile X Syndrome

We are developing TSHA-114 for the treatment of Fragile X syndrome, the most common single gene cause of autism and cognitive impairment, affecting about one in 6,000 individuals worldwide. Fragile X syndrome is diagnosed around three years of age and characterized by anxiety, aggression, hyperactivity, attention deficits and sleep and communication disruption.

Fragile X syndrome is caused by a pathological expansion of a CGG triplet repeat in the 5' untranslated region of the *FMR1* gene. Expansion of the triplet above the normal 5–55 repeats to 200

or more causes hypermethylation of the gene promoter, and shutdown of transcription and translation of the encoded protein, fragile X mental retardation protein, or FMRP. The expanded repeat also induces formation of RNA: DNA heteroduplexes that induces epigenetic gene silencing. Although most patients with Fragile X syndrome do not express FMRP, some individuals with the full mutation produce low amounts of the protein (less than 10% of normal levels). FMRP expression in unaffected persons varies greatly from person to person. Current pharmacotherapeutic treatments for Fragile X syndrome are solely directed towards symptom relief.

We are developing TSHA-114 using truncated promoters that mimic the natural developmental expression of FMRP. In addition, we have selected to express a human FMRP isoform to ensure that FMRP expression is appropriately localized to the cytosol, axon, nerve terminals, and nucleus in the proper ratios.

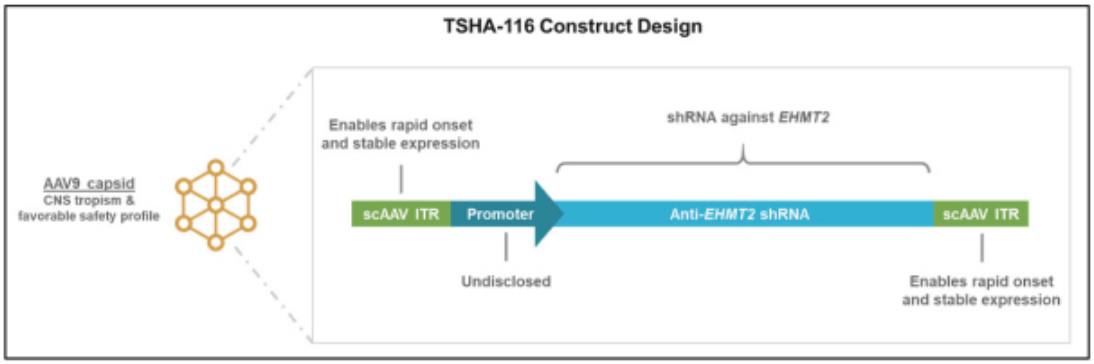


TSHA-116 for Prader-Willi Syndrome

We are developing TSHA-116 for the treatment of Prader-Willi syndrome, a genetic disorder caused by paternal loss-of-function of genes along 15q11-q13 chromosomal region, or *PWS* genes, due to an imprinting defect. The prevalence of Prader-Willi syndrome is estimated to be approximately one per 10,000 to 30,000 persons. During infancy, patients with Prader-Willi syndrome have feeding difficulties, poor growth and weak muscle tone. As the patient begins to age, their appetite becomes insatiable, resulting in obesity and type 2 diabetes. In addition, patients typically have mild to moderate intellectual disabilities, behavioral issues and sleep abnormalities.

In patients, with Prader-Willi syndrome, the maternal genes are intact but silenced by epigenetic regulation, or the modulation of gene expression. Epigenetic regulation is accomplished by the euchromatic histone lysine N-methyltransferase-2, or *EHMT2*, gene.

We are developing TSHA-116 to restore gene function through reactivation of the silenced maternal genes. To accomplish this, we are designing vectors to target *EHMT2* through shRNA knock-down and reduced levels of *EHMT2*, which would in turn activate the silenced *PWS* gene.

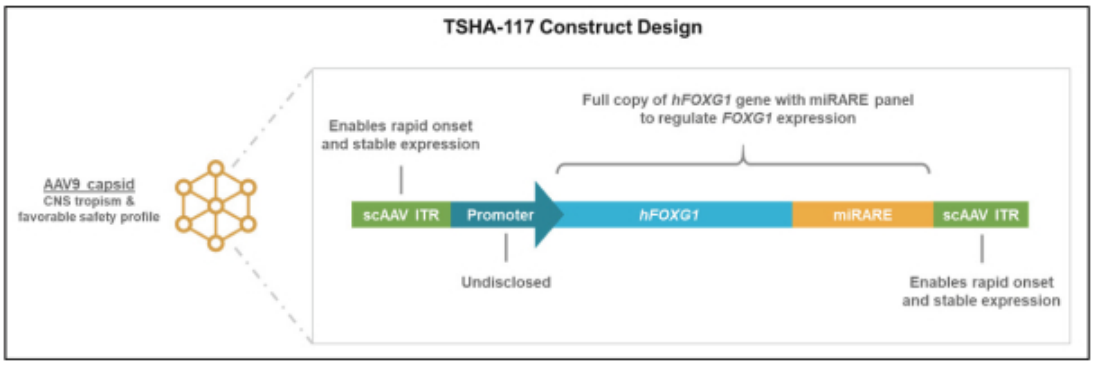


TSHA-117 for FOXG1 Syndrome

We are developing TSHA-117 for the treatment of FOXG1 syndrome. FOXG1 syndrome is a neurodevelopmental disorder caused by pathogenic mutations in the *FOXG1* gene. The symptoms of FOXG1 syndrome include severe developmental and intellectual disabilities, growth restriction with microcephaly, epilepsy and hyperkinetic-dyskinetic movement disorder. Approximately 700 patients with FOXG1 syndrome have been identified worldwide to date and the incidence rate is estimated to be one per 30,000 live births. The number of identified patients is expected to steadily increase as more children are tested for Autism Spectrum Disorder and other genetic disorders. Currently, there are no specific therapies for FOXG1 syndrome and medical management of the disease is largely focused on symptomatic relief.

The literature suggests that dosage of the *FOXG1* gene needs to be regulated to avoid off-target expression or overexpression of *FOXG1*.

We are developing TSHA-117 using our miRARE panel to regulate *FOXG1* expression levels.



Genetic Epilepsies

Our genetic epilepsy category targets disorders with recurrent seizures associated with abnormal development of the brain. In children with early-onset epilepsy, the detrimental effects of uncontrolled

seizures are often associated with, and may be responsible for, the neurodevelopmental disabilities that often appear subsequent to the onset of seizures. A number of childhood-onset epilepsies are caused by mutations in a single gene and represent a compelling target for gene therapy. The goal of our product candidates is to target the underlying cause of the disease to simultaneously control seizures and treat any associated developmental comorbidities.

We are developing TSHA-103, a genetic epilepsy product candidate, for the treatment of SLC6A1 haploinsufficiency disorder. We are also developing product candidates for the treatment of other disorders, including SLC13A5 disorder and KCNQ2-related disorders.

TSHA-103 for SLC6A1 Haploinsufficiency Disorder

Overview of SLC6A1 Haploinsufficiency Disorder

SLC6A1 haploinsufficiency disorder is caused by loss-of-function mutations in the *SLC6A1* gene. Loss of-function mutations in the *SLC6A1* gene have been identified as one of the most common monogenic causes of epilepsy with myoclonic atonic seizures, or brief and abrupt seizures followed by loss of muscle strength, as well as autism spectrum disorder and intellectual disability.

Patients diagnosed with SLC6A1 haploinsufficiency disorder typically present with developmental delay, varying degrees of intellectual disability, seizures and abnormal EEG characterized by generalized spike-wave discharges. Most patients are refractory to pharmacological seizure control although a portion of patients become seizure free during the course of disease progression. Importantly, seizure control is not associated with improved cognitive outcomes, which highlights the complexity of the disease as well as the need for novel therapies directed at its underlying pathology.

Approximately 81% of patients with SLC6A1 haploinsufficiency disorder have epilepsy, with typical absence seizures, which are abrupt and followed by lack of awareness, being the predominant form observed. In addition, 91% of individuals exhibit developmental delays, with more than 80% characterized as mild or moderate intellectual disability. Ataxia, or tremors, is present in approximately 29% of individuals, while autism or autistic features are observed in approximately 24% of individuals diagnosed with SLC6A1 haploinsufficiency disorder.

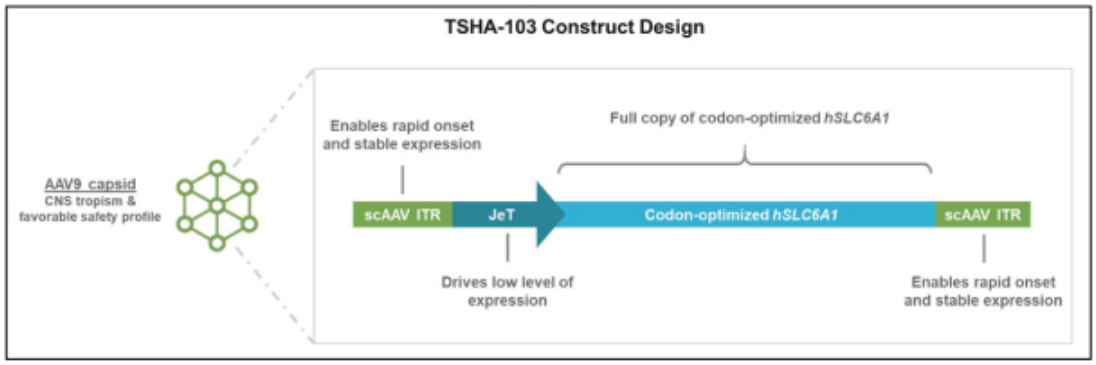
The *SLC6A1* gene encodes the gamma-aminobutyric acid, or GABA, transporter 1, or GAT1. GAT1 is a voltage-dependent transporter responsible for the reuptake of GABA, a non-protein amino acid that is well characterized for its role as a major inhibitory neurotransmitter within the mammalian CNS. GAT1 plays a critical role in the reuptake of GABA from neuronal synapses and extracellular spaces and as a result, a critical role in balancing neuronal excitations. When GABA transport is disrupted, brain development is negatively impacted resulting in deficits in attention and cognition as well as seizures.

The exact incidence and prevalence of SLC6A1 haploinsufficiency disorder is unknown. According to recently published data, the incidence of SLC6A1 haploinsufficiency disorder is approximately 1 in 36,000 live births. We believe that SLC6A1 haploinsufficiency disorder is underdiagnosed as the underlying biology was only recently elucidated and the gene had not been not part of commercially available genetic epilepsy screening panels. Clinician education and expanded use of genetic screening panels that include SLC6A1 will likely lead to increased identification of individuals with these mutations.

Our Solution: TSHA-103

We are developing TSHA-103, a genetic epilepsy product candidate, for the treatment of SLC6A1 haploinsufficiency disorder. TSHA-103 is a gene replacement therapy constructed from a codon-

optimized version of the human *SLC6A1* gene packaged within a self-complementary AAV9 viral vector under the control of a JeT promoter. We are currently conducting preclinical studies of TSHA-103 and plan to submit an IND for TSHA-103 to the FDA by the end of 2021.



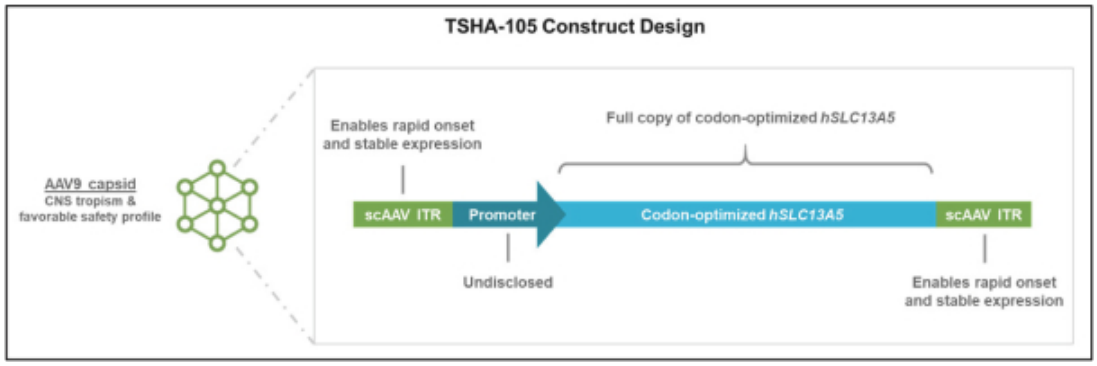
Additional Genetic Epilepsy Programs

TSHA-105 for SLC13A5

We are developing TSHA-105 for the treatment of SLC13A5 deficiency, a rare autosomal recessive epileptic encephalopathy characterized by the onset of seizures within the first few days of life. Affected children have impairments in gross motor function and speech production with relative preservation of fine motor skills and receptive speech. SLC13A5 deficiency is caused by bi-allelic loss-of function mutations in the *SLC13A5* gene, which codes for a sodium dependent citrate transporter, or NaCT, that is largely expressed in the brain and liver. To date, all tested mutations result in no or a greatly reduced amount of the citrate in the cells.

Diminished NaCT function leads to loss of neuronal uptake of citrate and other metabolites such as succinate that are critical to brain energy metabolism and function. Currently, there are no approved therapies for SLC13A5 deficiency, and treatment is largely to address symptoms.

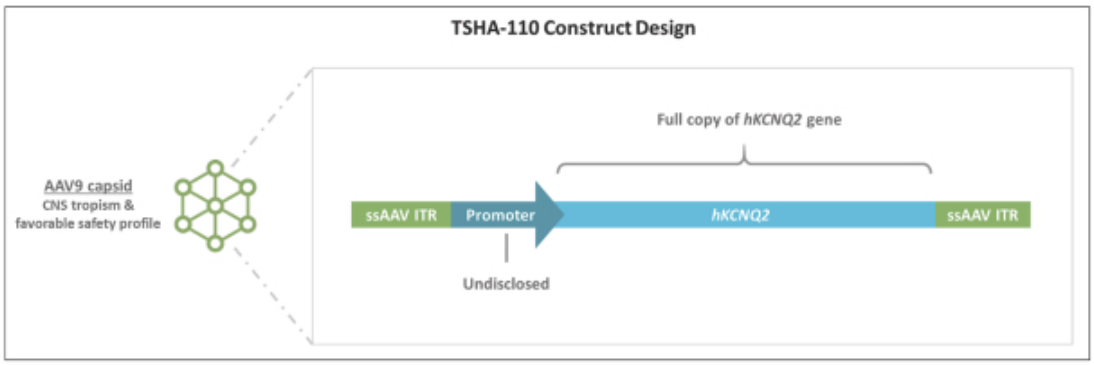
We are developing TSHA-105 as a gene replacement therapy for SLC13A5 deficiency. TSHA-105 is constructed from a codon-optimized human *SLC13A5* gene packaged in a self-complementary AAV9 capsid.



TSHA-110 for KNCQ2 Developmental & Epileptic Encephalopathy

We have an exclusive option from UT Southwestern to develop TSHA-110 for the treatment of *KCNQ2* Developmental and Epileptic Encephalopathy, or KCNQ2. Patients with KCNQ2 typically present with seizures in the first week of life. Seizures appear as tonic, or with stiffening of the body, often associated with jerking and changes in breathing or heart rate. These seizures often resolve within months to years but children have some degree of developmental impairment involving one or more domains (motor, social, language, cognition). There is wide variability in the symptoms of patients with a KCNQ2 diagnosis. Some have very limited, or no noticeable seizure activity and the developmental impairment can range from mild to severe, depending on a number of different factors. Some children may also have autistic features or other comorbidities. The incidence of KCNQ2 is approximately 1 in 35,000 live births.

We plan to exercise the option to acquire rights to TSHA-110 from UT Southwestern, following which we expect to develop TSHA-110 as a *KCNQ2* transgene packaged within an AAV9 vector.

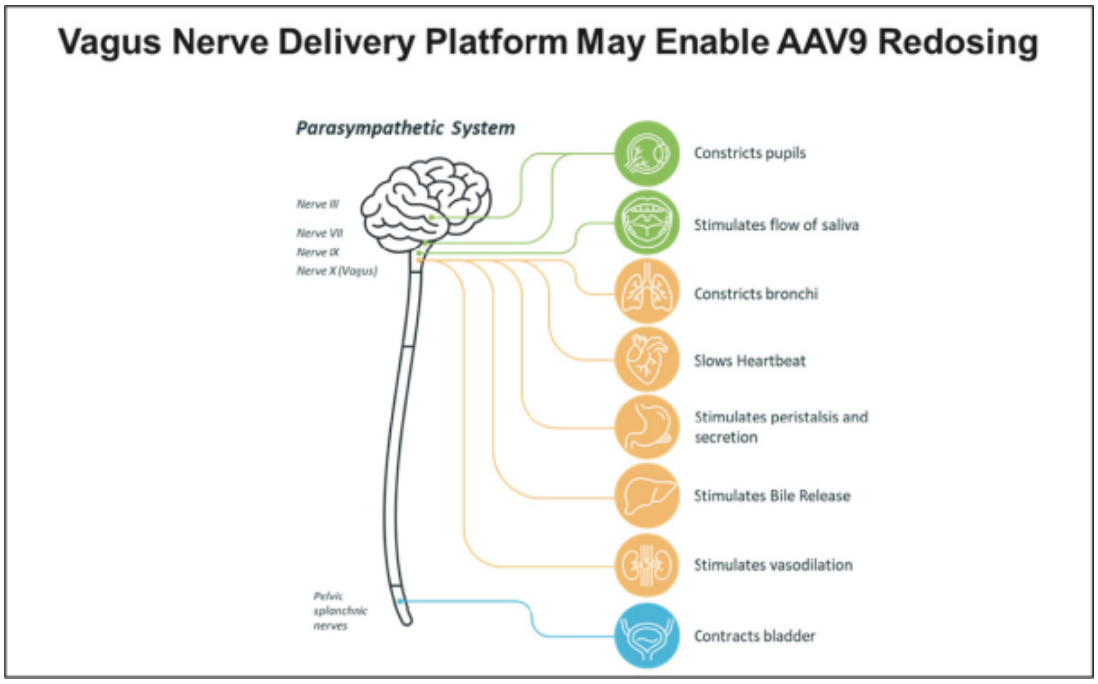


Our Next-Generation Platform Technologies

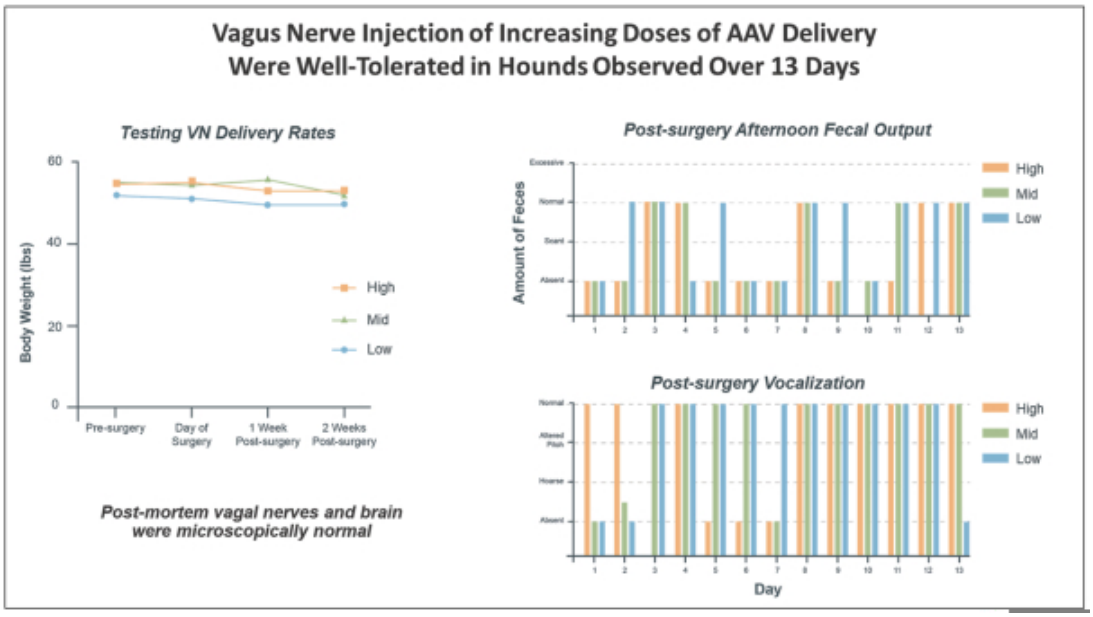
In addition to our AAV9 candidates, we are building a suite of platforms to develop next-generation technologies that can optimize key components of an AAV-based gene therapy as part of our strategy to develop a sustainable pipeline of product candidates and a consistent stream of new commercial product launches.

Novel Route of Administration to Allow Redosing

We are advancing a novel AAV dosing platform with the potential to facilitate redosing by administering AAV-based gene therapies directly to the vagus nerve. The development of AAV-neutralizing antibodies after administration of an initial dose has historically presented a challenge in the ability to redose patients with gene therapies. In preclinical studies in adult rats, we observed that AAV9 delivery to the vagus nerve resulted in efficient targeting of the vagal neurons in the autonomic nervous system, or the ANS. We subsequently performed experiments to assess the feasibility of targeting genes to ANS neurons in rats already treated with an intrathecal injection of AAV9. Rats were immunized with AAV9 by an intrathecal injection of AAV9/GAN and then received a direct vagus nerve injection with AAV9/GFP either four or fourteen weeks after the intrathecal injection. AAV9-immunized rats had efficient green fluorescent protein, or GFP, transduction in vagal nerve fibers and neurons. There was no evidence of neuroinflammation or significant chronic inflammatory infiltrates.



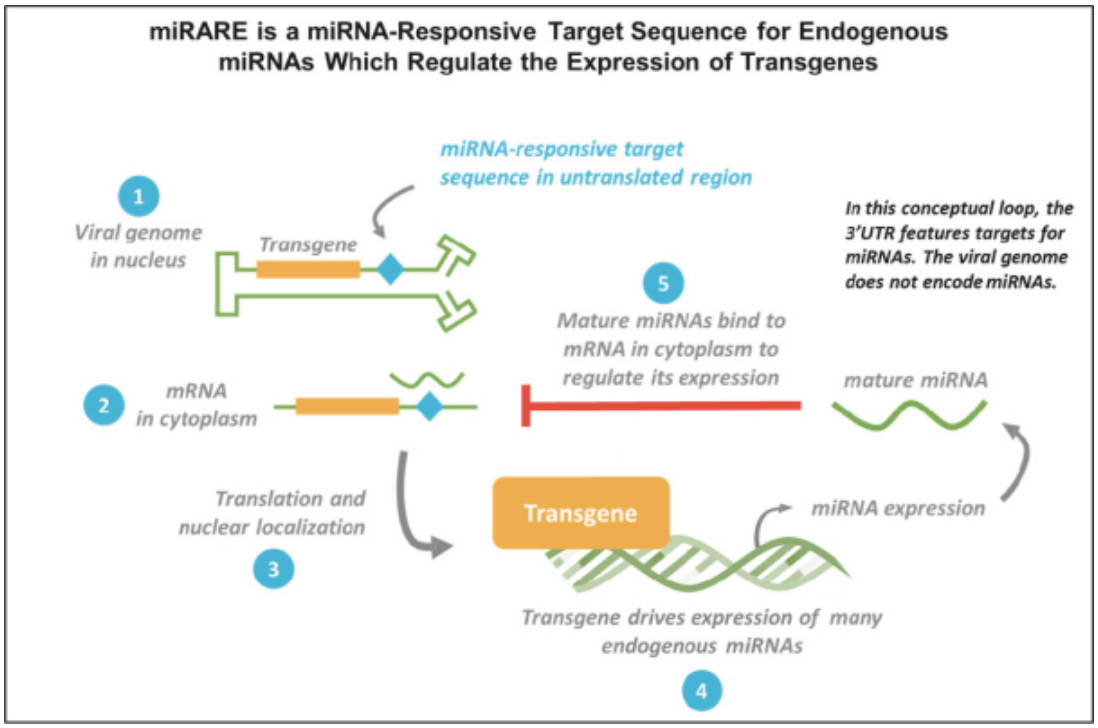
In preclinical studies in dogs, adult hounds received a direct vagus nerve injection of increasing volumes of vehicle and were allowed to recover for two weeks. AAV delivery to the vagus nerve was well tolerated at all doses. Vocalization, weight, eating and fecal output were monitored pre- and post-surgery, and post-mortem analysis showed that vagal nerve fibers and neurons were microscopically normal.



We plan to further evaluate the safety and feasibility of this approach in NHPs.

Regulated Transgene Expression Using miRARE

In a number of disorders, including Rett syndrome and FOXG1 syndrome, the expression of a therapeutic transgene needs to be regulated. In these disorders, high doses of transgene-expressing vectors may be harmful, while low doses may avoid toxicity but be sub-therapeutic. For disorders that require replacement of dose-sensitive genes, we have combined high-throughput microRNA, or miRNA, profiling and genome mining to create miRNA-Responsive Auto-Regulatory Element, or miRARE, our novel miRNA target panel. This approach is designed to enable our product candidates to maintain safe transgene expression levels in the brain. Importantly, this built-in regulation system is fully endogenous, and therefore does not require any additional exogenous drug application. Instead, the miRARE system utilizes endogenous transgene-responsive miRNA to downregulate transgene expression in the event that overexpression occurs. While our initial intent is to utilize our miRARE panel to develop gene replacement therapies for Rett syndrome and FOXG1 syndrome, we believe the system may be applicable to a number of other dose-sensitive genes.

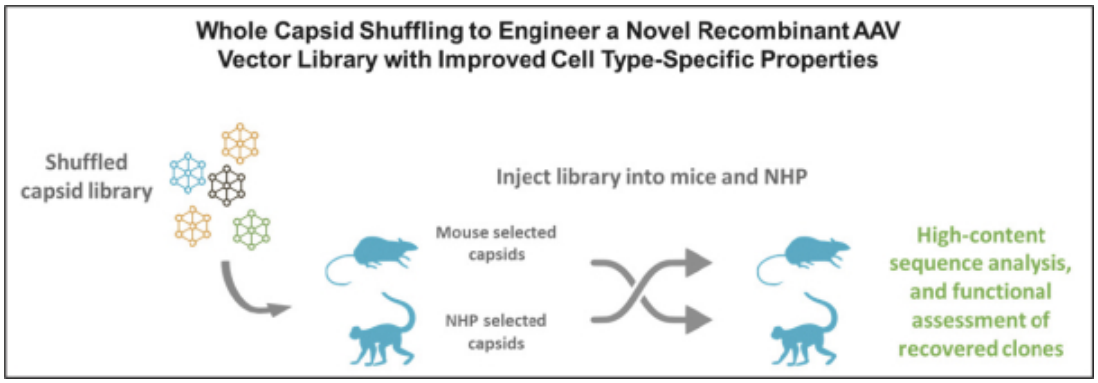


Novel Capsid Identification

Successful clinical translation of gene therapies depends upon efficient transgene delivery and expression across the entire CNS. Directed evolution is a powerful and proven method to develop novel AAV vector capsids that exhibit properties distinct from naturally occurring serotypes. However, to date, most novel capsids have been derived in rodents or in vitro models whose properties may or may not translate to other species, in particular primates.

We have developed a new approach that utilizes AAV whole capsid shuffling and directed evolution, combined with machine learning techniques, to develop novel AAV capsids in mice and non-human primates in parallel. We are utilizing SMRT sequencing analysis for a high throughput evaluation of recovered whole AAV capsid genes. SMRT sequencing has the advantage of sequencing

the entire capsid gene in a single read with high accuracy, enabling sequencing the entire shuffled capsid rather than only an insert region or barcode.



We believe that our approach will allow us to rapidly identify new capsids for the treatment of CNS disorders and drive new product candidates with novel biodistribution and transduction profiles into our development pipeline.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is also true for the development and commercialization of treatments for neurodegenerative diseases, neurodevelopmental disorders and genetic epilepsies, and broadly across gene therapies. While we believe that our focus, strength of team, expertise in gene therapy, scientific knowledge and intellectual property provide us with competitive advantages, we face competition from several different sources, including large and small biopharmaceutical companies, academic research institutions, government agencies and public and private research institutions. Not only must we compete with other companies that are focused on gene transfer technology, but any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and product marketing than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The majority of our programs face limited competition as there are no approved disease-modifying therapies for the treatment of the GM2 gangliosidosis, CLN1 disease, Rett syndrome, SLC6A1 haploinsufficiency disorder, SURF1 deficiency, SLC13A5 disorder, Fragile X syndrome, Angelman syndrome or the other development programs in our pipeline.

Axovant is developing AXO-AAV-GM2 for the treatment of GM2 gangliosidosis. AXO-AAV-GM2 delivers two AAVrh8 vectors, each encoding either *HEXA* or *HEXB* genes, directly to the CNS with the intention of producing functioning ß-hexosaminidase.

Amicus, in collaboration with Nationwide Children's Hospital, is developing a gene therapy candidate for CLN1 disease that is currently in discovery stage.

Novartis, through its acquisition of AveXis, is developing a product candidate for the treatment of Rett syndrome, AVXS-201, a preclinical AAV9 capsid vector that carries AAV ITR sequences and a minimal *MECP2* promoter that is designed to be active in the CNS in an unregulated manner. In addition, the Rett Syndrome Research Trust, Amicus Therapeutics and Sarepta have disclosed the existence of discovery-stage gene therapy programs although the development status of these programs is unclear.

Manufacturing

Through our collaboration with the UT Southwestern Gene Therapy Program, we have access to a new, state-of-the-art AAV production facility which includes approximately 2,000 square feet of laboratory space for process development and research-grade AAV vector production, as well as a 1,000 square foot ISO7 cleanroom facility dedicated solely for cGMP AAV vector manufacture.

This facility is outfitted with new equipment purchased in 2018 and 2019 for research-grade and GMP-grade AAV vector production. The process development lab is equipped with two Sartorius Biostat B Controller Stations with four 10 liter glass bioreactors and four 2 liter glass bioreactors, a Sartorius Biostat STR 50 liter bioreactor with controller and a Sartorius AMBR 250 process development bioreactor. Our strategic partnership with UT Southwestern provides us with access to this facility to allow for process development for development programs.

The UT Southwestern GMP facility is equipped with a Sartorius Biostat STR 50 liter bioreactor with controller, a Sartorius Biostat STR 500 liter bioreactor with controller, Sartoflow Smart tangential flow filtration unit, a Sartorius FlexAct UD, two bioprocessing workstations and an automated dispenser to support our AAV9 manufacturing needs for early clinical trials. We believe this capacity will be sufficient to meet the clinical demand for our full pipeline of product candidates.

In addition to our manufacturing capabilities provided by UT Southwestern, we are currently engaged with an external consulting firm to aid us with the development of a comprehensive facility build strategy including pipeline and capacity planning assessment, site selection assessment, benchmarking, quality systems and programs, and other strategic efforts related to the design and build out of a new facility to support commercial scale manufacturing of our pipeline programs.

License Agreements

Research, Collaboration and License Agreement with The University of Texas Southwestern Medical Center

In November 2019, we entered into a research, collaboration and license agreement, or the UT Southwestern Agreement, with The Board of Regents of the University of Texas System on behalf of UT Southwestern, as amended in April 2020. Under the UT Southwestern Agreement, UT Southwestern is primarily responsible for preclinical development activities with respect to licensed products for use in certain specified indications (up to IND-enabling studies), and we are responsible for all subsequent clinical development and commercialization activities with respect to licensed products. UT Southwestern will conduct such preclinical activities for a two-year period under mutually agreed upon sponsored research agreements that are funded by us. During the initial research phase, we have the right to expand the scope of specified indications under the UT Southwestern Agreement. We currently have 15 product candidates pursuant to the UT Southwestern Agreement, option rights with respect to KCNQ2 and options for an additional four indications. The research program activities will be overseen by a joint steering committee.

In connection with the UT Southwestern Agreement, we obtained an exclusive, worldwide, royalty-free license under certain patent rights of UT Southwestern and a non-exclusive, worldwide, royalty-free license under certain know-how of UT Southwestern, in each case to make, have made, use, sell, offer for sale and import licensed products for use in certain specified indications. Additionally, we obtained a non-exclusive, worldwide, royalty-free license under certain patents and know-how of UT Southwestern for use in all human uses, with a right of first refusal to obtain an exclusive license under certain of such patent rights and an option to negotiate an exclusive license under other of such patent rights at no cost. We are required to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product.

In connection with the license grant, we issued to UT Southwestern 2,000,000 shares of our common stock. We do not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement, other than costs related to the maintenance of patents.

The UT Southwestern Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last valid claim of a licensed patent in such country for such licensed product. After the initial research term, we may terminate the agreement, on an indication-by-indication and licensed product-by-licensed product basis, at any time upon specified written notice to UT Southwestern. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party.

License Agreement with Queen's University at Kingston

In February 2020, we entered into a license agreement, or the Queen's University Agreement, with Queen's University at Kingston, or Queen's University. In connection with the Queen's University Agreement, we obtained an exclusive, perpetual, worldwide, royalty-bearing license, with the right to grant sublicenses through multiple tiers, under certain patent rights and know-how of Queen's University, including certain improvements to such patent rights and know-how, to develop products in any field which use one or more valid claims of the patents licensed under the Queen's University Agreement, or the Licensed Patents, or the technology, information and intellectual property related to the patents licensed under the Queen's University Agreement, or the Licensed Technology and together with the Licensed Patents, the Licensed Products, and to make, have made, use, sell, offer for sale, import and export Licensed Products and otherwise exploit such patents and know-how for use in certain specified indications. We also obtained an exclusive right of first negotiation to license certain next-generation technology and improvements of Queen's University that do not constitute an already-licensed improvement to the Licensed Technology.

We are required to use commercially reasonable efforts to exploit the Licensed Technology in countries where it is commercially reasonable to develop Licensed Products, including by meeting certain diligence milestones within certain specified periods of time. In addition, we have final decision-making authority on the filing, prosecution or maintenance of Licensed Patents.

In connection with the Queen's University Agreement, we paid Queen's University a one-time fee of $3 million as an upfront fee and $221,300 to reimburse Queen's University for certain plasmid production costs. We are obligated to pay Queen's University up to $10.0 million in the aggregate upon achievement of certain regulatory milestones, up to $10.0 million in the aggregate upon achievement of commercial milestones, a low single digit royalty on net sales of Licensed Products, subject to certain customary reductions, and a percentage of non-royalty sublicensing revenue ranging in the low double digits. Royalties are payable on a Licensed Product-by-Licensed Product basis and country-by-country basis until expiration of the last valid claim of a Licensed Patent covering such Licensed Product in such country and the expiration of any regulatory exclusivity for such Licensed Product in such country. Additionally, we are obligated to pay Queen's University a low double-digit portion of any amounts

received by us in connection with the sale of a priority review voucher related to a Licensed Product, not to exceed a low eight-figure amount.

The Queen's University Agreement expires upon the expiration of the last royalty term of a Licensed Product. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. We may elect to terminate the agreement upon specified prior written notice to Queen's University.

We are also obligated under the terms of a separate research grant agreement to reimburse Queen's University for certain clinical manufacturing production costs of approximately $3.8 million.

License Agreement with Abeona Therapeutics Inc.

In August 2020, we entered into a license agreement, or the Abeona Agreement, with Abeona Therapeutics Inc., or Abeona. In connection with the Abeona Agreement, we obtained an exclusive, worldwide, royalty-bearing license, with the right to grant sublicenses under certain patents, know-how and materials originally developed by University of North Carolina at Chapel Hill and Abeona to research, develop, manufacture, have manufactured, use, and commercialize licensed products for gene therapy for the prevention, treatment, or diagnosis of CLN1 disease in humans.

Subject to certain obligations of Abeona, we are required to use commercially reasonable efforts to develop at least one licensed product and commercialize at least one licensed product in the United States.

In connection with the license grant, we will pay Abeona a one-time upfront license fee of $3.0 million. We are obligated to pay Abeona up to $26.0 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed product and high single-digit royalties on net sales of licensed products. Royalties are payable on a licensed product-by-licensed product and country-by-country basis until the latest of the expiration or revocation or complete rejection of the last licensed patent covering such licensed product in the country where the licensed product is sold, the loss of market exclusivity in such country where the product is sold, or, if no licensed product exists in such country and no market exclusivity exists in such country, ten years from first commercial sale of such licensed product in such country. In addition, concurrent with the Abeona Agreement we entered into a purchase and reimbursement agreement with Abeona, pursuant to which we will purchase specified inventory from Abeona for total consideration of $4.0 million.

The Abeona Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last royalty term of a licensed product. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party. We may terminate the agreement for convenience upon specified prior written notice to Abeona.

Intellectual Property

We actively seek to protect our proprietary technology, inventions, and other intellectual property that is commercially important to the development of our business by a variety of means, for example seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also may rely on trade secrets and know-how relating to our proprietary technology platform, on continuing technological innovation and on in-licensing opportunities to develop, strengthen and maintain the strength of our position in the field of gene therapy that may be important for the development of our business. We also intend to seek patent protection or rely upon trade secret rights to protect other technologies that may be used to discover and validate targets, and that may be

used to manufacture and develop novel gene therapy products. We are a party to license agreements that give us rights to use specific technologies in our gene therapy products and in manufacturing our products. Additional regulatory protection may also be afforded through data exclusivity, market exclusivity and patent term extensions where available.

As of the date of this prospectus, we in-license one Patent Cooperation Treaty application, 14 pending foreign patent applications, 14 patent applications pending in the United States, of which two are United States utility patent applications, both of which, if issued, are expected to expire in 2037, and 12 are United States provisional patent applications, where patent applications claiming priority to these provisional patent applications, if issued, are expected to expire between 2040 and 2041. Our policy is to file patent applications to protect technology, inventions and improvements to inventions that may be commercially important to the development of our business.

We in-license one United States provisional patent application that relates to our TSHA-103 SLCA61 program, where a patent application claiming priority to this provisional patent application, if issued, is expected to expire in 2040, without taking into account any possible patent term adjustment. We in-license one United States provisional patent application that relates to our TSHA-104 SURF1 program, where a patent application claiming priority to this provisional patent application, if issued, is expected to expire in 2040, without taking into account any possible patent term adjustment. Patent applications directed to our most advanced product candidates are summarized below:

TSHA-101

We in-license a United States utility patent application directed to a bicistronic *HEXB*P2A-*HEXA* transgene packaged into an AAV vector, and methods of using that vector to treat GM2 gangliosidosis, such as Tay-Sachs disease or Sandhoff disease, which, if issued, is expected to expire in 2037, without taking into account any possible patent term adjustment. This application has no foreign counterparts.

TSHA-118

We in-license certain patent rights directed to a palmitoyl-protein thioesterase 1 transgene packaged into an AAV vector, and methods of using that vector to treat CLN1 disease (also known as infantile Batten disease). Specifically, pursuant to our license agreement with Abeona we have in-licensed one PCT patent application assigned to Abeona. Patent applications based on this application, if issued, are expected to expire in 2040, without taking into account any possible patent term adjustment. In addition, pursuant to the Abeona agreement, we have sublicensed 15 pending patent applications worldwide assigned to the University of North Carolina at Chapel Hill. These patent applications, if issued, are expected to expire in 2037.

TSHA-102

We in-license four United States provisional patent applications directed to a minigene encoding *MECP2* packaged into an AAV vector, and methods of using that vector to treat Rett syndrome. Patent applications claiming priority to these provisional patent applications, if issued, are expected to expire between 2040 and 2041, without taking into account any possible patent term adjustment.

We also rely on trade secrets, know-how, continuing technological innovation and confidential information to develop and maintain our proprietary position and protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements and invention assignment agreements with our employees, consultants, scientific advisors, contractors and others who may have access to proprietary information, under which they are bound to assign to us

inventions made during the term of their employment or term of service. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Government Regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of biologics such as those we are developing.

Biological products are subject to regulation under the Food, Drug, and Cosmetic Act, or FDCA, and the Public Health Service Act, or PHSA, and other federal, state, local and foreign statutes and regulations. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

U.S. Biologics Regulation

The process required by the FDA before biological product candidates may be marketed in the United States generally involves the following:

- completion of extensive preclinical laboratory tests and animal studies performed in accordance with applicable regulations, including the GLP regulations;
- submission to the FDA of an IND, which must become effective before clinical trials may begin;
- approval by an independent institutional review board, or IRB, or ethics committee at each clinical site before the trial is commenced;
- performance of adequate and well-controlled human clinical trials to establish the safety, purity and potency of the proposed biological product candidate for its intended purpose;
- preparation of and submission to the FDA of a biologics license application, or BLA, after completion of all pivotal clinical trials;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with current Good Manufacturing Practice requirements, or cGMPs, and to assure that the facilities, methods and controls are adequate to preserve the biological product's continued safety, purity and potency, and of selected clinical investigation sites to assess compliance with the FDA's good clinical practices, or GCPs;
- satisfactory completion of an FDA Advisory Committee review, if applicable; and
- FDA review and approval, or licensure, of a BLA to permit commercial marketing of the product for particular indications for use in the United States.

Preclinical and Clinical Development

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug

product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

In addition to the submission of an IND to the FDA before initiation of a clinical trial in the United States, certain human clinical trials involving recombinant or synthetic nucleic acid molecules are subject to oversight at the local level as set forth in the NIH Guidelines. Specifically, under the NIH Guidelines, supervision of human gene transfer trials includes evaluation and assessment by an Institutional Biosafety Committee, or IBC, a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing preclinical studies and clinical trials and clinical study results to public registries.

For purposes of BLA approval of a product candidate, human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1*. For gene therapies, the investigational product is initially introduced into patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.

- *Phase 2*. The investigational product is administered to a limited patient population to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks.
- *Phase 3*. The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

When these phases overlap or are combined, the trials may be referred to as Phase 1/2 or Phase 2/3.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and potency. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical study investigators. The FDA or the sponsor or its data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research patients or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product candidate has been associated with unexpected serious harm to patients. There are also requirements governing the reporting of ongoing clinical trials and completed clinical trial results to public registries. Sponsors of clinical trials of FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information, which is publicly available at www.clinicaltrials.gov.

BLA Submission and Review

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, nonclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. The submission of a BLA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies. The FDA has sixty days from the applicant's submission of a BLA to either issue a refusal to file letter or accept the BLA for filing, indicating that it is sufficiently complete to permit substantive review.

Once a BLA has been accepted for filing, the FDA's goal is to review standard applications within ten months after it accepts the application for filing, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review

process is often significantly extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent for its intended use, and whether the facility in which it is manufactured, processed, packed or held meets standards designed to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may convene an advisory committee to provide clinical insight on application review questions. Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be manufactured, the FDA may issue an approval letter or a Complete Response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response letter will describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing the Complete Response letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification, which may include the potential requirement for additional clinical studies. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy, or REMS, to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies.

Expedited Development and Review Programs

The FDA offers a number of expedited development and review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor

of a fast track product has opportunities for frequent interactions with the review team during product development and, once a BLA is submitted, the product may be eligible for priority review. A fast track product may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA.

A product intended to treat a serious or life-threatening disease or condition may also be eligible for breakthrough therapy designation to expedite its development and review. A product can receive breakthrough therapy designation if preliminary clinical evidence indicates that the product, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product, including involvement of senior managers.

An RMAT is defined as cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells that lead to a durable modification of cells or tissues may meet the definition of a Regenerative Medicine Therapy. The RMAT program is intended to facilitate efficient development and expedite review of RMATs, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A new drug application or a BLA for an RMAT may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A Regenerative Medicine Therapy that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a fast track designation and/or breakthrough therapy designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide a significant improvement in the treatment, diagnosis or prevention of a serious disease or condition. For original BLAs, priority review designation means the FDA's goal is to take action on the marketing application within six months of the 60-day filing date.

Additionally, products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Fast track designation, breakthrough therapy designation, RMAT designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or 200,000 or more than individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if the holder of the orphan drug exclusivity cannot assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Rare Pediatric Disease Designation and Priority Review Vouchers

Under the FDCA, as amended, the FDA incentivizes the development of drugs and biologics that meet the definition of a “rare pediatric disease,” defined to mean a serious or life-threatening disease in which the serious of life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects 200,000 or more in the United States and for which there is no reasonable expectation that the cost of developing and making in the United States a drug for such disease or condition will be received from sales in the United States of such drug. The sponsor of a product candidate for a rare pediatric disease may be eligible for a voucher that can be used to obtain a priority review for a subsequent human drug or biologic application after the date of approval of the rare pediatric disease drug product, referred to as a priority review voucher, or PRV. A sponsor may request rare pediatric disease designation from the FDA prior to the submission of its NDA or BLA. A rare pediatric disease designation does not guarantee that a sponsor will receive a PRV upon approval of its NDA or BLA. Moreover, a sponsor who chooses not to submit a rare pediatric disease designation request may nonetheless receive a PRV upon approval of their marketing application if they request such a voucher in their original

marketing application and meet all of the eligibility criteria. If a PRV is received, it may be sold or transferred an unlimited number of times. Congress has extended the PRV program until September 30, 2020, with the potential for PRVs to be granted until 2022.

Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning or untitled letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. However,

companies may share truthful and not misleading information that is otherwise consistent with a product's FDA approved labeling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Biosimilars and Reference Product Exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-approved reference biological product. To date, a number of biosimilars have been licensed under the BPCIA, and numerous biosimilars have been approved in Europe. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. Complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and impact of the BPCIA is subject to significant uncertainty.

Foreign Regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety, and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable foreign regulatory authorities before we can commence clinical trials or marketing of the product in foreign countries and jurisdictions. Although many of the issues discussed above with respect to the United States apply similarly in the context of the European Union, the approval process varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Other Healthcare Laws and Compliance Requirements

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation: the U.S. federal Anti-Kickback Statute, the civil False Claims Act, HIPAA and similar foreign, federal and state fraud and abuse, transparency and privacy laws.

The U.S. federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value, including stock options. The U.S. federal Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and others on the other hand. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, but they are drawn narrowly, and practices that involve remuneration, such as consulting agreements, that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the U.S. federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, a claim including items or services resulting from a violation of the U.S. federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act.

Civil and criminal false claims laws, and civil monetary penalty laws, including the civil False Claims Act, which can be enforced through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment to the federal government, including federal healthcare programs, that are false or fraudulent. For example, the civil False Claims Act prohibits any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. A claim includes “any request or demand” for money or property presented

to the U.S. government. Several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA created additional federal civil and criminal liability for, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing, or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statute or specific intent to violate it. In addition, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, impose certain requirements on HIPAA covered entities, which include certain healthcare providers, healthcare clearing houses and health plans, and individuals and entities that provide services on their behalf that involve individually identifiable health information, known as business associates, relating to the privacy, security and transmission of individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.

The U.S. federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to annually report to CMS information related to payments or other transfers of value made to physicians (currently defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Effective January 1, 2022, these reporting obligations will extend to include transfers of value made to certain non-physician providers such as physician assistants and nurse practitioners.

We are also subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers, or that apply regardless of payor, state laws which require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, state and local laws which require pharmaceutical companies to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, state laws which require the reporting of information related to drug pricing, state and local laws requiring the registration of pharmaceutical sales representatives, and state and foreign laws governing the privacy and security of health information which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, significant civil, criminal and administrative penalties, imprisonment damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of our product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor.

Nonetheless, product candidates may not be considered medically necessary or cost effective. A decision by a third-party payor not to cover any product candidates we may develop could reduce physician utilization of such product candidates once approved and have a material adverse effect on our sales, results of operations and financial condition Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

Nonetheless, product candidates may not be considered medically necessary or cost effective. A decision by a third-party payor not to cover any product candidates we may develop could reduce physician utilization of such product candidates once approved and have a material adverse effect on our sales, results of operations and financial condition.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third-party not to cover a product could reduce physician usage and patient demand for the product.

Outside the United States, ensuring adequate coverage and payment for any biological candidates we may develop will face challenges. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us to conduct a clinical trial that compares the cost effectiveness of any product candidates we may develop to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts. In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a

reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on healthcare costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic, and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states, and parallel trade (arbitrage between low-priced and high-priced member states), can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

Healthcare Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded healthcare programs, and increased governmental control of drug pricing.

The ACA, which was enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal healthcare programs. Since its enactment, there have been judicial, Congressional and executive branch challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, it has enacted laws that modify certain provisions of the ACA such as removing penalties, which started on January 1, 2019, for not complying with ACA's individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. On December 14, 2018, a U.S. District Court Judge in Texas ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit ruled that the individual mandate was unconstitutional and

remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case. It is unclear when such oral arguments are to be held and when a decision is expected to be made. It is also unclear how such litigation and other efforts to repeal and replace the ACA will impact the ACA and our business.

Other legislative changes have been proposed and adopted since the ACA was enacted, including aggregate reductions of Medicare payments to providers of 2% per fiscal year and reduced payments to several types of Medicare providers. These reductions went into effect in April 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2030 unless additional action is taken by Congress. However, pursuant to the Coronavirus Aid, Relief and Economic Security Act, or CARES Act, these Medicare sequester reductions will be suspended from May 1, 2020 through December 31, 2020 due to the COVID-19 pandemic.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. At the federal level, the Trump administration's budget proposal for fiscal year 2021 contains includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients and increase patient access to lower-cost generic and biosimilar drugs. On March 10, 2020, the Trump administration sent "principles" for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses and place limits on pharmaceutical price increases. On May 11, 2018, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. HHS has solicited feedback on certain of these measures and implemented others under its existing authority. For example, in May 2019, CMS issued a final rule to allow Medicare Advantage plans the option to use step therapy for Part B drugs beginning January 1, 2020. This final rule codified CMS's policy change that was effective January 1, 2019. On July 24, 2020, President Trump signed four executive orders aimed at lowering drug prices. The executive orders direct the Secretary of the U.S. Department of Health and Human Services, or HHS, to: (1) eliminate protection under an Anti-Kickback Statute safe harbor for certain retrospective price reductions provided by drug manufacturers to sponsors of Medicare Part D plans or pharmacy benefit managers that are not applied at the point-of-sale; (2) allow the importation of certain drugs from other countries through individual waivers, permit the re-importation of insulin products, and prioritize finalization of FDA's December 2019 proposed rule to permit the importation of drugs from Canada; (3) ensure that payment by the Medicare program for certain Medicare Part B drugs is not higher than the payment by other comparable countries (depending on whether pharmaceutical manufacturers agree to other measures); and (4) allow certain low income individuals receiving insulin and epinephrine purchased by a Federally Qualified Health Center, or FQHC, as part of the 340B drug program to purchase those drugs at the discounted price paid by the FQHC. It is unclear if, when, and to what extent these executive orders may be implemented. On August 6, 2020, President Trump signed an additional executive order directing U.S. government agencies to encourage the domestic procurement of Essential Medicines, Medical Countermeasures, and Critical Inputs, which include among other things, active pharmaceutical ingredients and drugs intended for use in the diagnosis, cure, mitigation, treatment, or prevention of COVID-19. FDA shall release a full list of Essential Medicines, Medical Countermeasures, and Critical Inputs affected by this order by November 5, 2020. The regulatory and market implications of these

executive orders are unknown at this time, but legislation, regulations or policies allowing the reimportation of drugs, if enacted and implemented, could decrease the price we receive for any products that we may develop and commercialize and could adversely affect our future revenues and prospects for profitability.

Although a number of these and other proposed measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Additional state and federal healthcare reform measures may be adopted in the future. Further, it is possible that additional governmental action is taken in response to the COVID-19 pandemic. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Facilities

As part of our strategic partnership with UT Southwestern, we are provided with office space at UT Southwestern for administrative activities that we believe is adequate for our current needs. We believe that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

Employees

As of August 31, 2020, we had ten full-time employees. None of our employees are represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

Legal Proceedings

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently subject to any material legal proceedings.

MANAGEMENT

Executive Officers and Directors

The following table provides information regarding our current executive officers and directors, including their ages as of August 31, 2020:

Name	Age	Position(s)
Executive Officers		
RA Session II(3)	41	President, Chief Executive Officer and Director
Kamran Alam	42	Chief Financial Officer
Suyash Prasad, M.B.B.S., F.F.P.M.	50	Chief Medical Officer and Head of Research and Development
Non-Employee Directors		
Sean P. Nolan(1)(2)(3)	52	Chairman of the Board of Directors
Phillip B. Donenberg(1)(2)	60	Director
Paul B. Manning(3)	64	Director
Sukumar Nagendran, M.D.(1)(2)	54	Director

(1) Member of the audit committee.
(2) Member of the compensation committee.
(3) Member of the nominating and corporate governance committee.

Executive Officers

RA Session II has served as our President and Chief Executive Officer and a member of our board of directors since our founding in September 2019. In addition to serving as our President and Chief Executive Officer, Mr. Session currently serves as Entrepreneur-in-Residence of the University of Texas Southwestern Medical Center. He has served on the board of directors of Chardan Healthcare Acquisition 2 Corp. since April 2020. Mr. Session previously served as Chief Business Officer of the gene therapy subsidiaries of BridgeBio Pharma, Inc., a biopharmaceutical company, from January 2019 to April 2020; Senior Vice President, Corporate Strategy and Project Management of AveXis, Inc., a gene therapy company, from March 2017 to May 2018; and in various roles for PTC Therapeutics, Inc., a biopharmaceutical company, from June 2013 to March 2017, most recently as the Vice President of Commercial Development. Mr. Session has also served as an advisor to multiple biotechnology companies, including Alcyone Lifesciences, Inc. from January 2019 to December 2019, 4D Molecular Therapeutics, Inc. from August 2018 to December 2019 and Celenex from June 2018 to September 2018. Mr. Session earned a B.S.B.A. in finance from the University of North Carolina at Charlotte, an M.S.F. in finance from Texas A&M University-Commerce and an M.B.A. from Texas A&M University-Commerce. Our board of directors believes that Mr. Session is qualified to serve as a director based on his role as our Chief Executive Officer and his extensive management experience in the biotechnology industry.

Kamran Alam has served as our Chief Financial Officer since August 2020. Mr. Alam previously served as Senior Vice President, Finance and Principal Financial Officer of Rocket Pharmaceuticals, Inc., a biopharmaceutical company, from October 2019 to July 2020 and as Vice President, Finance at AveXis, Inc., a gene therapy company, from April 2016 to October 2019. From 2013 to April 2016, he held positions of increasing responsibility at Aptinyx Inc., a biopharmaceutical company, where at the time of his departure he was a Senior Director, Finance and Accounting. Mr. Alam is a Certified Public Accountant and earned a B.B.A. from the Ross School of Business at University of Michigan and an M.B.A. in finance from the Kelley School of Business at Indiana University.

Suyash Prasad, M.B.B.S., F.F.P.M., has served as our Chief Medical Officer and Head of Research and Development since June 2020. Dr. Prasad has served as principal of Suyash Prasad Consulting LLC, a consulting firm, since October 2019. He previously served as Senior Vice President and Chief Medical Officer of Audentes Therapeutics, Inc., a gene therapy company, from 2014 to June 2019. Dr. Prasad earned a medical degree from the University of Newcastle-upon-Tyne, United Kingdom, a master's degree (with distinction) in translational science from Kings College, London, United Kingdom, and a diploma in pharmaceutical medicine from the Faculty of Pharmaceutical Medicine of the Royal Colleges of Physicians of the United Kingdom. Dr. Prasad is a United Kingdom board-certified physician and is a member of the Royal College of Physicians and the Royal College of Pediatrics and Child Health and is a Fellow of the Faculty of Pharmaceutical Medicine of the Royal College of Physicians of the United Kingdom.

Non-Employee Directors

Sean P. Nolan has served as the Chairman of our board of directors since March 2020. He has served as the President of Nolan Capital, LLC, an investment fund, since October 2019. Mr. Nolan most recently served as President, Chief Executive Officer and a member of the board of directors of AveXis, Inc., a gene therapy company, from June 2015 to May 2018 until its acquisition by Novartis International AG. Mr. Nolan has served on the board of directors of Ventas, Inc., a healthcare real estate investment trust company, since July 2019 and previously served on the board of directors of Neoleukin Therapeutics, Inc., a biopharmaceutical company, from February 2015 to June 2020. Mr. Nolan earned a B.S. in biology from John Carroll University. Our board of directors believes that Mr. Nolan is qualified to serve as a director based upon his more than 29 years of broad leadership and management experience in the biopharmaceutical industry.

Phillip B. Donenberg has served as a member of our board of directors since August 2020. Mr. Donenberg has served on the board of directors and as chairman of the audit committee of AVROBIO, Inc., a gene therapy company, since June 2018. Previously, Mr. Donenberg served as Chief Financial Officer and Senior Vice President of Assertio Therapeutics, Inc., a pharmaceutical company, from July 2018 to November 2018. He served as Senior Vice President and Chief Financial Officer of AveXis, Inc., a gene therapy company, from October 2017 to June 2018 and as Vice President, Corporate Controller from September 2016 to October 2017. Mr. Donenberg served as Chief Financial Officer of RestorGenex Corporation from 2014 until its merger with Diffusion Pharmaceuticals LLC, a pharmaceutical company, in January 2016, and served as the merged company's consultant Chief Financial Officer until September 2016. Mr. Donenberg earned a B.S. in accountancy from the University of Illinois Champaign-Urbana College of Business and is a Certified Public Accountant. Our board of directors believes that Mr. Donenberg is qualified to serve as a director based on his financial expertise and his experience as a director and executive of companies in the biotechnology and pharmaceutical industries.

Paul B. Manning has served as a member of our board of directors since March 2020. Mr. Manning currently serves as the Chief Executive Officer of PBM Capital Group, LLC, a private equity investment firm in the business of investing in healthcare and life-science related companies, which he founded in 2010. Mr. Manning currently serves as Chairman of the board of directors of Verrica Pharmaceuticals Inc., a biopharmaceutical company. Additionally, he previously served on the boards of directors of Dova Pharmaceuticals, Inc., a biopharmaceutical company, from September 2016 to November 2019 and AveXis, Inc., a gene therapy company, from April 2014 to May 2018. Mr. Manning earned a B.S. in microbiology from the University of Massachusetts. Our board of directors believes that Mr. Manning is qualified to serve as a director based upon his more than 30 years of managerial and operational experience in the healthcare industry and as an investor in healthcare-related companies.

Sukumar Nagendran, M.D., has served as a member of our board of directors since July 2020. Dr. Nagendran has served on the board of directors of Solid Biosciences Inc., a life sciences company, since September 2018 and currently serves as an advisor to Encoded Therapeutics, Inc., a biotechnology company. He previously served on the board of directors of Health Sciences Acquisition Corp., a special purpose acquisition company, from March 2019 to December 2019 prior to its merger with Immunovant, Inc. Dr. Nagendran most recently served as Senior Vice President and Chief Medical Officer of AveXis, Inc., a gene therapy company, from September 2015 to May 2018, and previously served as Vice President, Head of Medical Affairs of U.S. and International Business for Quest Diagnostics Inc., a clinical laboratory, from March 2013 to September 2015. Dr. Nagendran earned a B.A. from Rutgers University and an M.D. from the University of Medicine and Dentistry of New Jersey and trained in Internal Medicine at Mayo Clinic in Rochester, Minnesota. Our board of directors believes that Dr. Nagendran is qualified to serve as a director based upon his more than 30 years of experience with gene therapy development and clinical development strategy.

Board Composition

Our business and affairs are managed under the direction of our board of directors, which currently consists of five members. Our directors were elected to, and currently serve on, the board pursuant to a voting agreement among us and all of our stockholders and voting rights granted by our current amended and restated certificate of incorporation. The voting agreement will terminate upon the closing of this offering, after which there will be no further contractual obligations regarding the election of our directors.

In accordance with our amended and restated certificate of incorporation that will be in effect upon the closing of this offering, our board of directors will be divided into three classes which will serve staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Our directors will be divided among the three classes as follows:

- Class I, which will consist of and , and their terms will expire at our first annual meeting of stockholders to be held after the closing of this offering;
- Class II, which will consist of and , and their terms will expire at our second annual meeting of stockholders to be held after the closing of this offering; and
- Class III, which will consist of and , and their terms will expire at our third annual meeting of stockholders to be held after the closing of this offering.

Our amended and restated bylaws, which will become effective upon the closing of this offering, will provide that the authorized number of directors may be changed only by resolution approved by a majority of our board of directors. The division of our board of directors into three classes with staggered three-year terms may delay or prevent a change of our management or a change of control.

Director Independence

Applicable Nasdaq rules, or the Nasdaq Listing Rules, require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the Nasdaq Listing Rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act of 1934, as amended, or the Exchange Act. The Nasdaq independence definition

includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees, that neither the director nor any of his family members has engaged in various types of business dealings with us and that the director is not associated with the holders of more than 5% of our common stock. In addition, under applicable Nasdaq rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Our board of directors has determined that all of our directors other than , representing of our directors, are "independent directors" as defined under applicable Nasdaq rules. In making such determination, our board of directors considered the current and prior relationships that each such director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his independence, including the beneficial ownership of our capital stock by each director and the transactions described in the section titled "Certain Relationships and Related Party Transactions."

There are no family relationships among any of our directors or executive officers.

Role of the Board in Risk Oversight

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure. Following the completion of this offering, we intend for our audit committee to have the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee will also monitor compliance with legal and regulatory requirements.

Board Committees

Our board of directors has established an audit committee, compensation committee and a nominating and corporate governance committee, each of which operate pursuant to a committee charter. Our board of directors may establish other committees to facilitate the management of our business. The composition and functions of each committee are described below.

Audit Committee

Upon the completion of this offering, our audit committee will consist of Messrs. Donenberg and Nolan and Dr. Nagendran, with Mr. Donenberg serving as chair of the audit committee. Our board of directors has determined that each of these individuals meets the independence requirements of Rule 10A-3 under the Securities Exchange Act of 1934, or the Exchange Act, and the applicable listing standards of Nasdaq. Each member of our audit committee can read and understand fundamental financial statements in accordance with Nasdaq audit committee requirements. Our board of directors has also determined that Mr. Donenberg qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the Nasdaq Listing Rules. In arriving at these determinations, the board has examined each audit committee member's scope of experience and the nature of their prior and/or current employment.

The functions of this committee include, among other things:

- helping our board of directors oversee our corporate accounting and financial reporting processes;
- managing the selection, engagement, qualifications, independence and performance of a qualified firm to serve as the independent registered public accounting firm to audit our financial statements;
- discussing the scope and results of the audit with the independent registered public accounting firm, and reviewing, with management and the independent accountants, our interim and year-end operating results;
- developing procedures for employees to submit concerns anonymously about questionable accounting or audit matters;
- reviewing related person transactions;
- obtaining and reviewing a report by the independent registered public accounting firm at least annually that describes our internal quality control procedures, any material issues with such procedures and any steps taken to deal with such issues when required by applicable law; and
- approving or, as permitted, pre-approving, audit and permissible non-audit services to be performed by the independent registered public accounting firm.

We believe that the composition and functioning of our audit committee will comply with all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation Committee

Upon the completion of this offering, our compensation committee will consist of Messrs. Donenberg and Nolan and Dr. Nagendran, with Dr. Nagendran serving as chair of the compensation committee. Each of these individuals is a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act and is an “outside director,” as defined pursuant to Section 162(m) of the Internal Revenue Code of 1986, as amended, or the Code. Our board of directors has determined that each of these individuals is ‘‘independent’’ as defined under the applicable listing standards of Nasdaq, including the standards specific to members of a compensation committee. The functions of this committee include, among other things:

- reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;
- making recommendations to the full board of directors regarding the compensation and other terms of employment of our executive officers;
- reviewing and making recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;
- evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;

- reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our non-employee board members;
- establishing policies with respect to votes by our stockholders to approve executive compensation to the extent required by Section 14A of the Exchange Act and, if applicable, determining our recommendations regarding the frequency of advisory votes on executive compensation;
- reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;
- administering our equity incentive plans;
- establishing policies with respect to equity compensation arrangements;
- reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;
- reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- reviewing with management and approving our disclosures under the caption “Compensation Discussion and Analysis” in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;
- preparing the report that the SEC requires in our annual proxy statement; and
- reviewing and evaluating on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee will comply with all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Nominating and Corporate Governance Committee

Upon the completion of this offering, our nominating and corporate governance committee will consist of Messrs. Manning, Nolan and Session, with Mr. Manning serving as chair of the nominating and corporate governance committee. We expect that within one year of our listing on Nasdaq, Mr. Session will resign from the nominating and corporate governance committee and will be replaced by an independent director; at that point, all members of the nominating and corporate governance committee will be independent. The functions of this committee include, among other things:

- identifying, reviewing and evaluating candidates to serve on our board of directors;
- determining the minimum qualifications for service on our board of directors;
- evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- evaluating, nominating and recommending individuals for membership on our board of directors;
- evaluating nominations by stockholders of candidates for election to our board of directors;
- considering and assessing the independence of members of our board of directors;
- developing a set of corporate governance policies and principles and recommending to our board of directors any changes to such policies and principles;

- reviewing and making recommendations to the board of directors with respect to management succession planning;
- considering questions of possible conflicts of interest of directors as such questions arise; and
- reviewing and evaluating on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee will comply with all applicable SEC and Nasdaq rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Compensation Committee Interlocks and Insider Participation

None of our directors who serve as a member of our compensation committee is, or has at any time during the past year been, one of our officers or employees. None of our executive officers currently serves, or in the past year has served, as a member of the board of directors or compensation committee of any other entity that has one or more executive officers serving on our board of directors or compensation committee.

Code of Business Conduct and Ethics

Effective upon the closing of this offering, we will adopt a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors. This includes our principal executive officer, principal financial officer and principal accounting officer or controller, or persons performing similar functions. Following the closing of this offering, the full text of the Code of Conduct will be available on our website at *www.tayshagtx.com*. We intend to post on our website all disclosures that are required by law or the listing standards of the Nasdaq Stock Market concerning any amendments to, or waivers from, any provision of the Code of Conduct. Information contained on, or that can be accessed through, our website is not incorporated by reference into this prospectus. We have included our website in this prospectus solely as an inactive textual reference.

Non-Employee Director Compensation

During the period from September 20, 2019 (the date of our inception) through December 31, 2019, our board of directors did not consist of any non-employee directors. Mr. Session, our President and Chief Executive Officer, was the sole member of our board of directors during this period but did not receive any additional compensation for his service as a director. Accordingly, the director compensation table required by SEC rules has been omitted.

We intend to adopt a non-employee director compensation policy, pursuant to which our non-employee directors will be eligible to receive compensation for service on our board of directors and committees of our board of directors, to be effective following the completion of this offering.

EXECUTIVE COMPENSATION

Our only named executive officer for the period from September 20, 2019 (the date of our inception) through December 31, 2019 was RA Session II, our President and Chief Executive Officer. Due to our limited operating history as described in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations," we did not have an executive compensation program, nor did we pay any employee compensation or issue any stock-based compensation to any employee, director or consultant, during the period from September 20, 2019 (the date of our inception) through December 31, 2019. We refer to Mr. Session elsewhere in this prospectus as our "named executive officer" for the period from September 20, 2019 (the date of our inception) through December 31, 2019.

Summary Compensation Table

We did not pay any employee compensation or issue any stock-based compensation to Mr. Session during the period from September 20, 2019 (the date of our inception) through December 31, 2019. Accordingly, the Summary Compensation Table required by SEC rules for the period from September 20, 2019 (the date of our inception) through December 31, 2019 has been omitted.

Outstanding Equity Awards at December 31, 2019

As of December 31, 2019, Mr. Session did not hold any outstanding equity awards, nor did we grant, cancel or modify any equity awards during the period from September 20, 2019 (the date of our inception) through December 31, 2019. Accordingly, the table of outstanding equity awards required by SEC rules has been omitted.

Employment Agreement with Mr. Session and Potential Payments and Benefits Upon Termination or Change in Control

We entered into an executive employment agreement with Mr. Session in April 2020. Mr. Session's employment with us is at will and may be terminated at any time by us or by him. The executive employment agreement provides for an initial annual base salary of $450,000, which is subject to adjustment at the discretion of our board of directors, and an annual bonus equal to 50% of Mr. Session's annual base salary based upon the achievement of individual and company performance goals as determined by our board of directors. The executive employment agreement further provides for the grant of restricted stock equal to 3% of our common stock outstanding at the time of grant, and such restricted shares were granted on July 1, 2020.

If we terminate Mr. Session's employment without "cause," or if Mr. Session terminates his employment for "good reason" (each, as defined in the executive employment agreement), he will be entitled to continued payment of his base salary for 12 months and his then-outstanding equity awards will vest in full. Such severance and acceleration benefits are conditioned upon Mr. Session's execution of and compliance with an effective and irrevocable general release, compliance with certain non-competition and non-solicitation obligations, resignation from all positions with us and return of all our property. The executive employment agreement further provides that if we undergo a "change in control" (as defined in the executive employment agreement) or if Mr. Session dies or becomes permanently disabled (as determined reasonably by his physician), Mr. Session's then-outstanding equity awards will vest in full.

Equity Incentive Plans

2020 Stock Incentive Plan

We expect that, prior to the effectiveness of the registration statement for this offering, our board of directors will adopt and our stockholders will approve our 2020 Stock Incentive Plan, or the New

Plan. The New Plan will become effective immediately upon the execution of the underwriting agreement for this offering, at which point no further grants will be made under our Existing Plan, as described in "—Equity Incentive Plans—2020 Equity Incentive Plan." No awards have been granted and no shares of our common stock have been issued under our New Plan. Our New Plan will provide for the grant of stock options qualifying as incentive stock options, or ISOs, within the meaning of Section 422 of the Internal Revenue Code of 1986, as amended, or the Code, to our employees and for the grant of nonstatutory stock options, or NSOs, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance stock awards and other forms of stock compensation to our employees, consultants and directors. Our New Plan will also provide for the grant of performance cash awards to our employees, consultants and directors.

Authorized Shares. The number of shares of our common stock initially reserved for issuance under our New Plan will be shares of our common stock, which is the sum of (i) new shares of our common stock, (ii) the number of shares remaining available for issuance under our Existing Plan when the New Plan becomes effective and (iii) the number of shares of our common stock subject to outstanding awards under our Existing Plan when the New Plan becomes effective that thereafter expire or are forfeited, canceled, withheld to satisfy tax withholding or to purchase or exercise an award, repurchased by us or are otherwise terminated. The number of shares of our common stock reserved for issuance under our New Plan will automatically increase on January 1 of each year, for a period of ten years, from January 1, 2021 continuing through January 1, 2030, by % of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by our board of directors. The maximum number of shares that may be issued pursuant to the exercise of ISOs under the New Plan is .

Shares issued under our New Plan may be authorized but unissued or reacquired shares of our common stock. Shares subject to stock awards granted under our New Plan that expire or terminate without being exercised in full, or that are paid out in cash rather than in shares, will not reduce the number of shares available for issuance under our New Plan. Additionally, shares issued pursuant to stock awards under our New Plan that we repurchase or that are forfeited, as well as shares reacquired by us as consideration for the exercise or purchase price of a stock award or to satisfy tax withholding obligations related to a stock award, will become available for future grant under our New Plan.

Administration. Our board of directors, or a duly authorized committee thereof, has the authority to administer our New Plan. Our board of directors has delegated its authority to administer our New Plan to our compensation committee under the terms of the compensation committee's charter. Our board of directors may also delegate to one or more of our officers the authority to (i) designate employees other than officers to receive specified stock awards and (ii) determine the number of shares of our common stock to be subject to such stock awards. Subject to the terms of our New Plan, the administrator has the authority to determine the terms of awards, including recipients, the exercise price or strike price of stock awards, if any, the number of shares subject to each stock award, the fair market value of a share of our common stock, the vesting schedule applicable to the awards, together with any vesting acceleration, the form of consideration, if any, payable upon exercise or settlement of the stock award and the terms and conditions of the award agreements for use under our New Plan.

The administrator has the power to modify outstanding awards under our New Plan. Subject to the terms of our New Plan, the administrator has the authority to reprice any outstanding option or stock award, cancel and re-grant any outstanding option or stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

Limitation on Grants to Non-Employee Directors. The maximum number of shares of our common stock subject to awards granted under our New Plan or otherwise during a single calendar

year to any of our non-employee directors, taken together with any cash fees paid by us to such non-employee director during the calendar year for serving on our board, will not exceed $ in total value (the value of any such stock awards to be based on their grant date fair market value for financial reporting purposes), or, with respect to the calendar year in which a non-employee director is first appointed or elected to our board, $.

Corporate Transactions. Our New Plan provides that in the event of a specified corporate transaction, including without limitation a consolidation, merger or similar transaction involving our company, the sale or other disposition of all or substantially all of the assets of our company or the consolidated assets of our company and our subsidiaries, or a sale or disposition of more than 50% of the outstanding capital stock of our company, the administrator will determine how to treat each outstanding stock award. The administrator may:

- arrange for the assumption, continuation or substitution of a stock award by a successor corporation;
- arrange for the assignment of any reacquisition or repurchase rights held by us to a successor corporation;
- accelerate the vesting of the stock award and provide for its termination prior to the effective time of the corporate transaction;
- arrange for the lapse, in whole or in part, of any reacquisition or repurchase right held by us;
- cancel the stock award prior to the transaction in exchange for such cash consideration, if any, that the administrator in its discretion determines to be appropriate; or
- make a payment in a form determined by the administrator equal to the excess of the value of the property the participant would have received upon exercise of the stock award immediately prior to the transaction over the exercise price payable in connection with the stock award.

The administrator is not obligated to treat all stock awards or portions of stock awards, even those that are of the same type, in the same manner. The administrator may take different actions with respect to the vested and unvested portions of a stock award.

Change in Control. The administrator may provide, in an individual award agreement or in any other written agreement between us and the participant, that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change in control (as defined in the New Plan). In the absence of such a provision, no such acceleration of the stock award will occur.

Amendment or Termination. Our board has the authority to amend, suspend, or terminate our New Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent. No ISOs may be granted after the tenth anniversary of the date our board of directors adopts our New Plan.

2020 Equity Incentive Plan

Our board of directors adopted the 2020 Equity Incentive Plan, or the Existing Plan, in July 2020, and our stockholders approved the Existing Plan in July 2020.

Stock Awards. The Existing Plan provides for the grant of options to purchase shares of our common stock intended to qualify as "incentive stock options" under Section 422 of the U.S. Internal Revenue Code of 1986, as amended, or ISOs, options that do not so qualify, or NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards and other stock awards, or collectively, stock awards. ISOs may be granted only to our employees and the employees of any

parent corporation or subsidiary corporation. All other awards may be granted to our employees, non-employee directors and consultants and the employees and consultants of our affiliates. We have granted stock options and restricted stock awards under the Existing Plan. As of September 2, 2020, we had outstanding options to purchase 15,000 shares of our common stock with a weighted-average exercise price of $16.23 per share, 2,615,935 shares of common stock issuable upon the vesting of restricted stock units and restricted stock awards with respect to 705,882 shares of our common were outstanding, and 192,595 shares of our common stock remained available for future awards under the Existing Plan.

Share Reserve. Subject to certain capitalization adjustments, the aggregate number of shares of our common stock that has been reserved for issuance pursuant to stock awards under the Existing Plan is 3,529,412 shares.

If a stock award granted under the Existing Plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the Existing Plan. In addition, the following types of shares of our common stock under the Existing Plan may become available for the grant of new stock awards under the Existing Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Upon closing of this offering, any shares that would otherwise be returned to the Existing Plan will instead be added to the shares of common stock available for issuance under the New Plan.

Administration. Our board of directors, or a duly authorized committee thereof, has the authority to administer the Existing Plan. Our board of directors may also delegate to one or more of our officers the authority to (i) designate employees (other than other officers) to be recipients of certain stock awards, and (ii) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the Existing Plan, the plan administrator determines the award recipients, dates of grant, the numbers and types of stock awards to be granted and the applicable fair market value and the provisions of the stock awards, including the period of their exercisability, the vesting schedule applicable to a stock award and any repurchase rights that may apply.

The plan administrator has the authority to modify outstanding awards, including reducing the exercise, purchase or strike price of any outstanding stock award, canceling any outstanding stock award in exchange for new stock awards, cash or other consideration or taking any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

Stock Options. ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the Existing Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the Existing Plan, up to a maximum of ten years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that the exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally

exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (i) cash, check, bank draft, electronic funds transfer or money order, (ii) a broker-assisted cashless exercise, (iii) the tender of shares of our common stock previously owned by the optionholder, (iv) a net exercise of the option if it is an NSO, (v) deferred payment or a similar arrangement with the optionholder and (vi) other legal consideration approved by the plan administrator.

Tax Limitations on Incentive Stock Options. The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed $100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (i) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant and (ii) the term of the ISO does not exceed five years from the date of grant.

Incentive Stock Option Limit. The maximum number of shares of our common stock that may be issued upon the exercise of ISOs under the Existing Plan is 10,588,236.

Restricted Stock Awards. Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (i) cash, check, bank draft or money order, (ii) services rendered to us or our affiliates or (iii) any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be determined by the plan administrator. A restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock awards that have not vested may be forfeited or repurchased by us upon the participant's cessation of continuous service for any reason.

Restricted Stock Unit Awards. Restricted stock unit awards are granted under restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration that may be acceptable to our board of directors and permissible under applicable law. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the administrator or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock unit awards that have not vested will be forfeited once the participant's continuous service ends for any reason.

Changes to Capital Structure. In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to (i) the class and maximum number of shares reserved for issuance under the Existing Plan, (ii) the class and maximum number of shares that may be issued upon the exercise of ISOs and (iii) the class and number of shares and price per share of stock subject to outstanding stock awards.

Corporate Transactions. The Existing Plan provides that in the event of certain specified significant corporate transactions, unless otherwise provided in an award agreement or other written agreement between us and the award holder, the plan administrator may take one or more of the following actions with respect to each stock award, contingent upon the closing or completion of the transaction: (i) arrange for the assumption, continuation or substitution of the stock award by a successor corporation, (ii) arrange for the assignment of any reacquisition or repurchase rights held by us in respect of our common stock issued pursuant to the stock award to a successor corporation, (iii) accelerate the vesting, in whole or in part, of the stock award and provide for its termination if not exercised prior to the effective time of the transaction, (iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by us with respect to the stock award, (v) cancel or arrange for the cancellation of the stock award, to the extent not vested or not exercised prior to the effective time of the transaction, in exchange for a cash payment, or no payment, as determined by the plan administrator or (vi) make a payment, in the form determined by the plan administrator, equal to the excess, if any, of the value of the property the holder would have received upon exercise of the stock award immediately prior to the effective time of the transaction over any exercise price payable by the holder (which payment may be delayed to the same extent that payment of consideration to the holders of our common stock in connection with the transaction is delayed as a result of any escrow, holdback, earnout or other contingencies). The plan administrator is not obligated to treat all stock awards or portions thereof in the same manner, and the plan administrator may take different actions with respect to the vested and unvested portions of a stock award.

Under the Existing Plan, a significant corporate transaction is generally the consummation of (i) a sale or other disposition of all or substantially all of our consolidated assets, (ii) a sale or other disposition of more than 50% of our outstanding securities, (iii) a merger, consolidation or similar transaction following which we are not the surviving corporation or (iv) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

Change in Control. In the event of a change in control, awards granted under the Existing Plan will not receive additional acceleration of vesting and exercisability, although this treatment may be provided for in a stock award agreement or other written agreement between us or any of our affiliates and the holder. Under the Existing Plan, a change in control is generally (i) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (ii) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity or (iii) a consummated sale, lease, exclusive license or other disposition of all or substantially all of our consolidated assets other than to an entity more than 50% of the combined voting power of which is owned by our stockholders.

Transferability. A participant generally may not transfer stock awards under the Existing Plan other than by will, the laws of descent and distribution or as otherwise provided under the Existing Plan.

Amendment and Termination. Our board of directors has the authority to amend, suspend or terminate the Existing Plan, provided that, with certain exceptions, such action does not impair the existing rights of any participant without such participant's written consent. Certain material amendments also require the approval of our stockholders. Unless terminated sooner by our board of directors, the Existing Plan will automatically terminate on July 1, 2030. No stock awards may be granted under the Existing Plan while it is suspended or terminated. Our board of directors has determined not to make any further awards under the Existing Plan following the closing of this offering.

2020 Employee Stock Purchase Plan

We expect that our board of directors will adopt and our stockholders will approve prior to the closing of this offering our 2020 Employee Stock Purchase Plan, or ESPP. The ESPP will become effective immediately upon the execution of the underwriting agreement for this offering. The purpose of the ESPP is to secure the services of new employees, to retain the services of existing employees and to provide incentives for such individuals to exert maximum efforts toward our success. The ESPP is intended to qualify as an "employee stock purchase plan" within the meaning of Section 423 of the Code.

Share Reserve. Following this offering, the ESPP will authorize the issuance of shares of our common stock pursuant to purchase rights granted to our employees or to employees of any of our designated affiliates. The 2020 ESPP will initially provide participating employees with the opportunity to purchase up to an aggregate of shares of our common stock. The number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year, from January 1, 2021 through January 1, 2030, by the lesser of (i) % of the total number of shares of our common stock outstanding on December 31 of the preceding calendar year, and (ii) shares; provided, that prior to the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). If purchase rights granted under the ESPP terminate without having been exercised, the shares of our common stock not purchased under such purchase rights will again become available for issuance under the ESPP.

Administration. Our board of directors intends to delegate concurrent authority to administer the ESPP to our compensation committee. The ESPP is implemented through a series of offerings under which eligible employees are granted purchase rights to purchase shares of our common stock on specified dates during such offerings. Under the ESPP, we may specify offerings with durations of not more than 27 months, and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for employees participating in the offering. An offering under the ESPP may be terminated under certain circumstances.

Payroll Deductions. Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the ESPP and may contribute, normally through payroll deductions, up to 15% of their earnings (as defined in the ESPP) for the purchase of our common stock under the ESPP. Unless otherwise determined by our board of directors, common stock will be purchased for the accounts of employees participating in the ESPP at a price per share equal to the lower of (i) 85% of the fair market value of a share of our common stock on the first trading date of an offering or (ii) 85% of the fair market value of a share of our common stock on the date of purchase.

Limitations. Employees may have to satisfy one or more of the following service requirements before participating in the ESPP, as determined by our board of directors, including: (i) being customarily employed for more than 20 hours per week; (ii) being customarily employed for more than five months per calendar year; or (iii) continuous employment with us or one of our affiliates for a period of time (not to exceed two years). No employee may purchase shares under the ESPP at a rate in excess of $25,000 worth of our common stock based on the fair market value per share of our common stock at the beginning of an offering for each year such a purchase right is outstanding. Finally, no employee will be eligible for the grant of any purchase rights under the ESPP if immediately after such rights are granted, such employee has voting power over 5% or more of our outstanding capital stock measured by vote or value pursuant to Section 424(d) of the Code.

Changes to Capital Structure. In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or similar transaction, the board of directors will make appropriate adjustments to (i) the number of shares reserved under the ESPP, (ii) the maximum number of shares by which the share reserve may increase automatically each year, (iii) the number of shares and purchase price applicable to all outstanding offerings and purchase rights and (iv) the number of shares that are subject to purchase limits under ongoing offerings.

Corporate Transactions. In the event of certain significant corporate transactions, including (i) a sale of all or substantially all of our assets, (ii) the sale or disposition of more than 50% of our outstanding securities, (iii) the consummation of a merger or consolidation where we do not survive the transactions and (iv) the consummation of a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue or substitute for such purchase rights, then the participants' accumulated payroll contributions will be used to purchase shares of our common stock within ten business days prior to such corporate transaction, and such purchase rights will terminate immediately.

Amendments or Termination. Our board of directors has the authority to amend or terminate our ESPP, provided that except in certain circumstances such amendment or termination may not materially impair any outstanding purchase rights without the holder's consent. We will obtain stockholder approval of any amendment to our ESPP, as required by applicable law or listing requirements.

Limitations on Liability and Indemnification Matters

Upon the closing of this offering, our amended and restated certificate of incorporation will contain provisions that limit the liability of our current and former directors for monetary damages to the fullest extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to the corporation or its stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the Delaware General Corporation Law; or
- any transaction from which the director derived an improper personal benefit.

These limitations of liability do not apply to liabilities arising under federal securities laws and does not affect the availability of equitable remedies such as injunctive relief or rescission.

Our amended and restated certificate of incorporation and our amended and restated bylaws that will be in effect upon the closing of this offering will provide that we are required to indemnify our directors to the fullest extent permitted by Delaware law. Our amended and restated bylaws will also provide that, upon satisfaction of certain conditions, we are required to advance expenses incurred by

a director in advance of the final disposition of any action or proceeding, and permit us to secure insurance on behalf of any officer, director, employee or other agent for any liability arising out of his or her actions in that capacity regardless of whether we would otherwise be permitted to indemnify him or her under the provisions of Delaware law. Our amended and restated bylaws will also provide our board of directors with discretion to indemnify our officers and employees when determined appropriate by the board.

We have entered into indemnification agreements with each of our directors and expect to enter into indemnification agreements with each of our executive officers prior to the closing of this offering. With certain exceptions, these agreements provide for indemnification for related expenses including, among other things, attorneys' fees, judgments, fines and settlement amounts incurred by any of these individuals in any action or proceeding. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and executive officers. We also maintain customary directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against our directors for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions. At present, there is no pending litigation or proceeding involving any of our directors, officers or employees for which indemnification is sought and we are not aware of any threatened litigation that may result in claims for indemnification.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted for our directors, executive officers or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

Rule 10b5-1 Sales Plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from them. The director or officer may amend a Rule 10b5-1 plan in some circumstances and may terminate a plan at any time. Our directors and executive officers also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material nonpublic information subject to compliance with the terms of our insider trading policy. Prior to 180 days after the date of this offering, subject to early termination, the sale of any shares under such plans would be prohibited by the lock-up agreement that the director or officer has entered into with the underwriters.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a description of transactions since our inception in September 2019 to which we have been a participant in which the amount involved exceeded or will exceed $120,000, and in which any of our directors, executive officers or holders of more than 5% of our voting securities, or any members of their immediate family, had or will have a direct or indirect material interest, other than compensation arrangements which are described under “Executive Compensation.”

Our Relationship with UT Southwestern

In November 2019, we entered into the UT Southwestern Agreement with UT Southwestern, a beneficial owner of more than 5% of our capital stock. Claire Aldridge, Ph.D., a former member of our board of directors, is the Associate Vice President of Commercialization and Business Development at UT Southwestern. In accordance with the terms of, and as consideration for, the UT Southwestern Agreement, we issued 2,000,000 shares of our common stock to UT Southwestern in November 2019. We do not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement.

We are also obligated to provide research and development funding pursuant to certain sponsored research agreements entered into beginning in April 2020 in connection with the UT Southwestern Agreement. We had not paid any amounts to UT Southwestern pursuant to these sponsored research agreements as of June 30, 2020. See “Business—Research, Collaboration and License Agreement with the University of Texas Southwestern Medical Center” for additional information.

Agreements with RA Session II

Guarantee and Security Agreement

In December 2019, Mr. Session, our President and Chief Executive Officer and a member of our board of directors, entered into a guarantee and security agreement by and among Queen’s University, our company and himself, pursuant to which Mr. Session has personally guaranteed payments due by us to Queen’s University in the event that we fail to fund our obligations under a research grant agreement by and between Queen’s University and us.

Loan Agreement

In January 2020, Mr. Session loaned us the principal amount of approximately $1.7 million with interest accruing at a rate of 10% per annum, and we granted Mr. Session a first priority security interest in certain of our assets as collateral for the loan. We repaid Mr. Session an aggregate of approximately $1.7 million, including interest, in payments made in March 2020 and July 2020, and Mr. Session released his security interest in the collateral.

Private Placements of Our Securities

Series A Convertible Preferred Stock Financing

In March 2020, we entered into a preferred stock purchase agreement with certain investors, including beneficial owners of greater than 5% of our capital stock and affiliates of members of our board of directors, pursuant to which we issued and sold to such investors an aggregate of 6,000,000 shares of our Series A convertible preferred stock at a purchase price of $3.00 per share for aggregate gross proceeds of $18.0 million.

Under the agreement, such investors were required to purchase up to an aggregate of 4,000,000 additional shares of our Series A convertible preferred stock upon our achievement of certain

milestones. Moreover, such investors had the right, in their sole discretion, to purchase any or all of such additional shares whether or not the we achieved the specified milestones. In June and July 2020, such investors exercised in full their option to purchase these additional shares prior to our achievement of such milestones, and we issued and sold to such investors an aggregate of 4,000,000 shares of Series A convertible preferred stock at a purchase price of $3.00 per share for aggregate gross proceeds of $12.0 million.

The table below sets forth the aggregate number of shares of Series A convertible preferred stock issued to our related parties in this financing:

Name	Series A Convertible Preferred Stock (#)	Aggregate Purchase Price ($)
PBM TGT Holdings, LLC(1)	8,166,667	24,500,001.00
Nolan Capital, LLC(2)	1,000,000	3,000,000.00

(1) Paul B. Manning, a member of our board of directors, is the Chief Executive Officer of PBM Capital Group, LLC and has sole voting and investment power with respect to the shares held by PBM TGT Holdings, LLC. Entities affiliated with PBM Capital Group, LLC collectively hold more than 5% of our capital stock prior to this offering.
(2) Sean Nolan, the Chairman of our board of directors, is the President of Nolan Capital, LLC, and, as a result, may be deemed to share voting and investment power with respect to the shares held by Nolan Capital, LLC.

Series B Convertible Preferred Stock Financing

In July 2020, we entered into a preferred stock purchase agreement with certain investors, including beneficial owners of greater than 5% of our capital stock, members of our board of directors and affiliates of members of our board of directors, pursuant to which we issued and sold to such investors an aggregate of 5,647,048 shares of our Series B convertible preferred stock at a purchase price of $17.00 per share for aggregate gross proceeds of $96.0 million. The financing closed in July and August 2020.

The table below sets forth the aggregate number of shares of Series B convertible preferred stock issued to our related parties in this financing:

Name	Series B Convertible Preferred Stock (#)	Aggregate Purchase Price ($)
Entities affiliated with FMR, LLC(1)	1,705,882	28,999,994.00
PBM Capital Group, LLC(2)	117,647	1,999,999.00
Sukumar Nagendran, M.D.	17,647	299,999.00
Suyash Prasad, M.B.B.S., F.F.P.M	3,529	59,993.00
Nolan Capital, LLC(3)	1,470	24,990.00

(1) Entities affiliated with FMR, LLC collectively hold more than 5% of our capital stock prior to this offering.
(2) Paul B. Manning, a member of our board of directors, is the Chief Executive Officer of PBM Capital Group, LLC, and has sole voting and investment power with respect to the shares held by PBM Capital Group, LLC. Entities affiliated with PBM Capital Group, LLC collectively hold more than 5% of our capital stock prior to this offering. The shares acquired by PBM Capital Group, LLC were subsequently sold or otherwise distributed to third parties.
(3) Sean Nolan, the Chairman of our board of directors, is the President of Nolan Capital, LLC, and, as a result, may be deemed to share voting and investment power with respect to the shares held by Nolan Capital, LLC.

Investors' Rights, Voting and Right of First Refusal Agreements

In connection with the sales of convertible preferred stock described above, we entered into an amended and restated investors' rights agreement, an amended and restated voting agreement and an amended and restated right of first refusal and co-sale agreement containing registration rights, information rights, voting rights and rights of first refusal, among other things, with the holders of our convertible preferred stock, including PBM TGT Holdings, LLC, Nolan Capital, LLC and the entities affiliated with FMR, LLC and certain other stockholders. These agreements will terminate upon the closing of this offering, except for the registration rights granted under our amended and restated investors' rights agreement, as more fully described in the section of this prospectus titled "Description of Capital Stock—Registration Rights."

Our Relationship with PBM Capital Group, LLC

In March 2020, we entered into a services agreement with PBM Capital Group, LLC, or the PBM Services Agreement. Under the PBM Services Agreement, PBM Capital Group, LLC provides accounting and other administrative and management services related to payroll administration, human resources, bookkeeping, preparation of financial statements and tax returns, accounts payable and receivable, and other similar functions for a fee of $2,500 per month.

Employment Arrangements

We have entered into employment agreements or offer letter agreements with certain of our executive officers. For more information regarding our employment agreement with Mr. Session, our President and Chief Executive Officer, see "Executive Compensation—Employment Agreement with Mr. Session and Potential Payments and Benefits Upon Termination or Change in Control."

Indemnification Agreements

Our amended and restated certificate of incorporation that will be in effect upon the closing of this offering will contain provisions limiting the liability of directors, and our amended and restated bylaws will provide that we will indemnify each of our directors to the fullest extent permitted under Delaware law. Our amended and restated certificate of incorporation and amended and restated bylaws will also provide our board of directors with discretion to indemnify our officers and employees when determined appropriate by the board.

In addition, we have entered into indemnification agreements with each of our directors, and we expect to enter into indemnification agreements with each of our executive officers prior to the closing of this offering. For more information regarding these agreements, see "Executive Compensation—Limitations on Liability and Indemnification Matters."

Related Person Transaction Policy

Prior to this offering, we have not had a formal policy regarding approval of transactions with related parties. In connection with this offering, we have adopted a related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions, which policy will become effective immediately upon the execution of the underwriting agreement for this offering. For purposes of our policy only, a related person transaction will be a transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we and any related person are, were or will be participants in which the

amount involved exceeds $120,000. Transactions involving compensation for services provided to us as an employee or director will not be covered by this policy. A related person will be any executive officer, director or beneficial owner of more than 5% of any class of our voting securities, including any of their immediate family members and any entity owned or controlled by such persons.

Under the policy, if a transaction has been identified as a related person transaction, including any transaction that was not a related person transaction when originally consummated or any transaction that was not initially identified as a related person transaction prior to consummation, our management must present information regarding the related person transaction to our audit committee, or, if audit committee approval would be inappropriate, to another independent body of our board of directors, for review, consideration and approval or ratification. The presentation must include a description of, among other things, the material facts, the interests, direct and indirect, of the related persons, the benefits to us of the transaction and whether the transaction is on terms that are comparable to the terms available to or from, as the case may be, an unrelated third party or to or from employees generally. Under the policy, we will collect information that we deem reasonably necessary from each director, executive officer and, to the extent feasible, significant stockholder to enable us to identify any existing or potential related-person transactions and to effectuate the terms of the policy. In addition, under our Code of Conduct that we expect to adopt prior to the closing of this offering, our employees and directors will have an affirmative responsibility to disclose any transaction or relationship that reasonably could be expected to give rise to a conflict of interest. In considering related person transactions, our audit committee, or other independent body of our board of directors, will take into account the relevant available facts and circumstances including:

- the risks, costs and benefits to us;
- the impact on a director's independence in the event that the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

The policy will require that, in determining whether to approve, ratify or reject a related person transaction, our audit committee, or other independent body of our board of directors, must consider, in light of known circumstances, whether the transaction is in, or is not inconsistent with, our best interests and those of our stockholders, as our audit committee, or other independent body of our board of directors, determines in the good faith exercise of its discretion.

All of the transactions described in this section were entered into prior to the adoption of this policy. Although we have not had a written policy for the review and approval of transactions with related persons, our board of directors has historically reviewed and approved any transaction where a director or officer had a financial interest, including the transactions described above. Prior to approving such a transaction, the material facts as to a director's or officer's relationship or interest in the agreement or transaction were disclosed to our board of directors. Our board of directors took this information into account when evaluating the transaction and in determining whether such transaction was fair to us and in the best interest of all our stockholders.

PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding beneficial ownership of our capital stock by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- our named executive officer; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Under these rules, beneficial ownership includes any shares of common stock as to which the individual or entity has sole or shared voting power or investment power. Applicable percentage ownership is based on 26,352,930 shares of common stock outstanding as of August 15, 2020, after giving effect to the conversion of all of our convertible preferred stock. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options held by such person that are currently exercisable or will become exercisable within 60 days of August 15, 2020 are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person.

Unless noted otherwise, the address of all listed stockholders is c/o Taysha Gene Therapies, Inc., 2280 Inwood Road, Dallas, Texas 75235.

Except as indicated by the footnotes below, we believe, based on information furnished to us, that each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

Name of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
Greater than 5% stockholders			
RA Session II(1)	8,655,882	32.9%	%
Entities affiliated with PBM Capital Group, LLC(2)	8,284,314	31.4	
UT Southwestern(3)	2,000,000	7.6	
Entities affiliated with FMR LLC(4)	1,705,882	6.5	
Named Executive Officer and Directors			
RA Session II(1)	8,655,882	32.9	
Paul B. Manning(2)	8,284,314	31.4	
Sean P. Nolan(5)	1,001,470	3.8	
Sukumar Nagendran, M.D(6)	17,647	*	
Phillip B. Donenberg	—	—	
All current executive officers and directors as a group (7 persons)(7)	17,962,842	68.2	

* Represents beneficial ownership of less than one percent.
(1) Consists of 8,655,882 shares of common stock.
(2) Consists of (a) 8,166,667 shares of common stock issuable upon conversion of Series A convertible preferred stock held by PBM TGT Holdings, LLC and (b) 117,647 shares of common stock issuable upon conversion of Series B convertible preferred stock held by PBM Capital

Group, LLC. PBM TGT Holdings, LLC is majority owned by PBM Capital Investments II, LLC, which is managed by PBM Capital Group, LLC. Paul B. Manning, a member of our board of directors, is the Chief Executive Officer of PBM Capital Group, LLC, and has sole voting and investment power with respect to the shares held by PBM TGT Holdings, LLC and PBM Capital Group, LLC. The business address for each person and entity named in this footnote is 200 Garrett Street, Suite S, Charlottesville, Virginia 22902

(3) Consists of 2,000,000 shares of common stock held by The Board of Regents of the University of Texas System on behalf of UT Southwestern. The business address for The Board of Regents of the University of Texas System is 5323 Harry Hines Boulevard, Dallas, Texas 75390-9094.

(4) Consists of (a) 78,554 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund, (b) 330,967 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund, (c) 294,548 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Fidelity Growth Company Commingled Pool, (d) 31,225 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company K6 Fund, (e) 734,388 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Fidelity Select Portfolios: Biotechnology Portfolio and (f) 236,200 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund. These accounts are managed by direct or indirect subsidiaries of FMR LLC. Abigail P. Johnson is a director, the chairman, the chief executive officer and the president of FMR LLC. Members of the Johnson family, including Abigail P. Johnson, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders of FMR LLC have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. Neither FMR LLC nor Abigail P. Johnson has the sole power to vote or direct the voting of the shares owned directly by the various investment companies registered under the Investment Company Act of 1940, or the Fidelity Funds, advised by Fidelity Management & Research Company, a wholly owned subsidiary of FMR LLC, which power resides with the Fidelity Funds' Boards of Trustees. Fidelity Management & Research Company carries out the voting of the shares under written guidelines established by the Fidelity Funds' Boards of Trustees. The business address for each person and entity named in this footnote is 245 Summer Street, Boston, Massachusetts 02110.

(5) Consists of (a) 1,000,000 shares of common stock issuable upon conversion of Series A convertible preferred stock held by Nolan Capital, LLC and (b) 1,470 shares of common stock issuable upon conversion of Series B convertible preferred stock held by Nolan Capital, LLC. Mr. Nolan is the President of Nolan Capital, LLC, and as such, Mr. Nolan has sole voting and investment power with respect to the shares held by Nolan Capital, LLC. The business address for each person and entity named in this footnote is 8 The Green, Ste. R, Dover, Delaware 19901.

(6) Consists of 17,647 shares of common stock issuable upon conversion of Series B convertible preferred stock.

(7) Consists of (a) 8,655,882 shares of common stock; (b) 9,166,667 shares of common stock issuable upon conversion of Series A convertible preferred stock; and (c) 140,293 shares of common stock issuable upon conversion of Series B convertible preferred stock.

DESCRIPTION OF CAPITAL STOCK

The following description of our capital stock, certain provisions of our amended and restated certificate of incorporation and amended and restated bylaws, as each will be in effect following the completion of this offering, and certain provisions of Delaware law are summaries. You should also refer to the amended and restated certificate of incorporation and the amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is part.

General

Upon the completion of this offering, our amended and restated certificate of incorporation will authorize us to issue up to shares of common stock, $0.00001 par value per share, and shares of preferred stock, $0.00001 par value per share, all of which shares of preferred stock will be undesignated. Our board of directors may establish the rights and preferences of the preferred stock from time to time.

As of June 30, 2020, we had outstanding 10,000,000 shares of common stock, held by three stockholders of record. As of June 30, 2020, after giving effect to the conversion of all of the outstanding shares of our convertible preferred stock, including 3,800,000 shares of our Series A convertible preferred stock and 5,647,048 shares of our Series B convertible preferred stock issued subsequent to June 30, 2020, into 15,647,048 shares of common stock, and the sales and distributions of shares of Series B convertible preferred stock by PBM Capital Group, LLC to third parties discussed in "—Certain Relationships and Related Party Transactions," there would have been 25,647,048 shares of common stock issued and outstanding, held by 108 stockholders of record.

Common Stock

Voting Rights

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. The affirmative vote of holders of at least $66^{2}/_{3}\%$ of the voting power of all of the then-outstanding shares of capital stock, voting as a single class, will be required to amend certain provisions of our amended and restated certificate of incorporation, including provisions relating to amending our amended and restated bylaws, the classified board, the size of our board, removal of directors, director liability, vacancies on our board, special meetings, stockholder notices, actions by written consent and exclusive forum.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of common stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and Preferences

Holders of common stock have no preemptive, conversion or subscription rights and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the right of the holders of shares of any series of preferred stock that we may designate in the future.

Preferred Stock

As of June 30, 2020, there were no shares of preferred stock or Series B convertible preferred stock outstanding and 6,200,000 shares of Series A convertible preferred stock outstanding. We issued 3,800,000 additional shares of Series A convertible preferred stock in July 2020 and 5,647,048 shares of Series B convertible preferred stock in July and August 2020. All currently outstanding shares of convertible preferred stock will be converted into an aggregate of 15,647,048 shares of common stock upon the closing of this offering.

Following the closing of this offering, our board of directors will have the authority under our amended and restated certificate of incorporation, without further action by our stockholders, to issue up to shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our common stock. The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of us and may adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock. It is not possible to state the actual effect of the issuance of any shares of preferred stock on the rights of holders of common stock until the board of directors determines the specific rights attached to that preferred stock.

We have no present plans to issue any shares of preferred stock following completion of this offering.

Options

As of June 30, 2020, there were no options to purchase shares of common stock outstanding. For additional information regarding the terms of our 2020 Equity Incentive Plan, see “Executive Compensation—Equity Incentive Plans.” Subsequent to June 30, 2020, we granted options under our 2020 Equity Incentive Plan to purchase an aggregate of 15,000 shares of common stock, at an exercise price of $16.23 per share, to certain of our directors.

Restricted Stock Awards

As of June 30, 2020, there were no restricted stock awards with respect to our common stock outstanding. For additional information regarding the terms of our 2020 Equity Incentive Plan, see “Executive Compensation—Equity Incentive Plans.” Subsequent to June 30, 2020, we granted restricted stock awards with respect to 705,882 shares of common stock to an employee.

Restricted Stock Units

As of June 30, 2020, there were no restricted stock units outstanding. For additional information regarding the terms of our 2020 Equity Incentive Plan, see “Executive Compensation—Equity Incentive Plans.” Subsequent to June 30, 2020, we granted restricted stock units with respect to 2,615,935 shares of common stock to employees.

Registration Rights

We, the holders of our existing convertible preferred stock and certain holders of our existing common stock have entered into an amended and restated investors' rights agreement. The registration rights provisions of this agreement provide those holders with demand, piggyback and Form S-3 registration rights with respect to the shares of common stock currently held by them and issuable to them upon conversion of our convertible preferred stock in connection with our initial public offering. These shares are collectively referred to herein as registrable securities. An aggregate of 25,647,048 shares of common stock will be entitled to the registration rights described below.

Demand Registration Rights

At any time beginning 180 days following the effective date of the registration statement of which this prospectus is a part, the holders of a majority of registrable securities then outstanding have the right to demand that we file a registration statement covering registrable securities then outstanding having an aggregate offering price in excess of $10.0 million, net of certain selling expenses. These registration rights are subject to specified conditions and limitations, including the right of the underwriters, if any, to limit the number of shares included in any such registration under specified circumstances. Upon such a request, we are required to effect the registration as soon as practicable, but in any event no later than 60 days after the receipt of such request.

Piggyback Registration Rights

If we propose to register any of our securities under the Securities Act either for our own account or for the account of other stockholders, the holders of registrable securities will each be entitled to notice of the registration and will be entitled to include their shares of common stock in the registration statement. These piggyback registration rights are subject to specified conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under specified circumstances.

Registration on Form S-3

At any time after we become eligible to file a registration statement on Form S-3, the holders of a majority of registrable securities then outstanding will be entitled to request to have such shares registered by us on a Form S-3 registration statement. These Form S-3 registration rights are subject to other specified conditions and limitations, including the condition that the anticipated aggregate offering price, net of certain selling expenses, is at least $5.0 million. Upon receipt of this request, the holders of registrable securities will each be entitled to participate in this registration.

Expenses of Registration

We are required to pay all expenses, including fees and expenses of one counsel to represent the selling stockholders (up to $50,000 total), relating to any demand, piggyback or Form S-3 registration, other than underwriting discounts and commissions, stock transfer taxes and any additional fees of counsel for the selling stockholders, subject to specified conditions and limitations. We are not required to pay registration expenses if a demand registration request is withdrawn at the request of a majority of holders of registrable securities to be registered, unless holders of a majority of the registrable securities agree to forfeit their right to one demand registration.

The amended and restated investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the applicable registration statement attributable to us, and the selling stockholders are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them, subject to certain limitations.

Termination of Registration Rights

The registration rights granted under the investors' rights agreement will terminate with respect to any particular stockholder upon the earlier of (a) the closing of a deemed liquidation event, as defined in our certificate of incorporation, (b) the third anniversary of the closing of this offering and (c) with respect to each stockholder, at such time such stockholder is able to sell all of its shares pursuant to Rule 144 or another similar exemption under the Securities Act during a three-month period without registration.

Anti-Takeover Provisions

Section 203 of the Delaware General Corporation Law

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66⅔% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines a "business combination" to include the following:

- any merger or consolidation involving the corporation or any direct or indirect majority-owned subsidiary of the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder (in one transaction or a series of transactions);
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation or by any direct or indirect majority-owned subsidiary of the corporation of any stock of the corporation or of such subsidiary to the interested stockholder;
- any transaction involving the corporation or any direct or indirect majority-owned subsidiary of the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an "interested stockholder" as an entity or person who, together with the person's affiliates and associates, beneficially owns, or within three years prior to the time of

determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws

Our amended and restated certificate of incorporation to be in effect upon the completion of this offering, or our restated certificate, will provide for our board of directors to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Because our stockholders do not have cumulative voting rights, stockholders holding a majority of the shares of common stock outstanding will be able to elect all of our directors. Our restated certificate and our amended and restated bylaws to be effective upon the completion of this offering, or our restated bylaws, will also provide that directors may be removed by the stockholders only for cause upon the vote of 66$^{2}/_{3}$% or more of our outstanding common stock. Furthermore, the authorized number of directors may be changed only by resolution of the board of directors, and vacancies and newly created directorships on the board of directors may, except as otherwise required by law or determined by the board, only be filled by a majority vote of the directors then serving on the board, even though less than a quorum.

Under our restated certificate of incorporation and amended and restated bylaws our stockholders will not have cumulative voting rights. Because of this, the holders of a majority of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose.

Our restated certificate and restated bylaws will also provide that all stockholder actions must be effected at a duly called meeting of stockholders and will eliminate the right of stockholders to act by written consent without a meeting. Our restated bylaws will also provide that only our Chairman of the board, Chief Executive Officer or the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors may call a special meeting of stockholders.

Our restated bylaws will also provide that stockholders seeking to present proposals before a meeting of stockholders to nominate candidates for election as directors at a meeting of stockholders must provide timely advance notice in writing, and will specify requirements as to the form and content of a stockholder's notice.

Our restated certificate and restated bylaws will provide that the stockholders cannot amend many of the provisions described above except by a vote of 66$^{2}/_{3}$% or more of our outstanding common stock.

As described in "—Preferred Stock" above, our restated certificate will give our board of directors the authority, without further action by our stockholders, to issue up to shares of preferred stock in one or more series, with any rights, preferences and privileges as they may designate, including the right to approve an acquisition or other change in control.

The combination of these provisions will make it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Since our board of directors has the power to retain and discharge our officers, these provisions could also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate

takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

Choice of Forum

Our amended and restated certificate of incorporation will provide that the Court of Chancery of the state of Delaware will be the exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate, or our amended and restated bylaws;
- any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate or our amended and restated bylaws;
- any claim or cause of action as to which the Delaware General Corporation Law confers jurisdiction on the Court of Chancery of the state of Delaware; or
- any action asserting a claim against us that is governed by the internal affairs doctrine.

The provision would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our amended and restated certificate of incorporation will also provide that unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act.

While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions

These exclusive forum provisions may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive-forum provision in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

Our amended and restated certificate of incorporation will further provide that the federal district courts of the United States of America will be the exclusive forum for resolving any complaint asserting

a cause of action arising under the Securities Act, subject to and contingent upon a final adjudication in the State of Delaware of the enforceability of such exclusive forum provision.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is . The transfer agent's address is .

Listing

We have applied to list our common stock on the Nasdaq Global Market under the trading symbol "TSHA."

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, no public market existed for our common stock. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of June 30, 2020, upon the closing of this offering and assuming no exercise of the underwriters' option to purchase additional shares, shares of common stock will be outstanding, assuming no outstanding options are exercised. All of the shares of common stock sold in this offering will be freely tradable without restrictions or further registration under the Securities Act, except for any shares sold to our "affiliates," as that term is defined under Rule 144 under the Securities Act. The remaining shares of common stock held by existing stockholders are "restricted securities," as that term is defined in Rule 144 under the Securities Act. Restricted securities may be sold in the public market only if registered or if their resale qualifies for exemption from registration described below under Rule 144 promulgated under the Securities Act or another available exemption.

As a result of the lock-up agreements described below and the provisions of Rules 144 and 701 under the Securities Act, the shares of common stock that will be deemed restricted securities after this offering will be available for sale in the public market as follows:

- none of the existing shares will be eligible for immediate sale upon the completion of this offering; and
- shares will be eligible for sale in the public market upon expiration of lock-up agreements 180 days after the date of this prospectus, subject in certain circumstances to the volume, manner of sale and other limitations under Rule 144 and Rule 701 under the Securities Act, which are summarized below.

Rule 144

In general, non-affiliate persons who have beneficially owned restricted shares of our common stock for at least six months, and any affiliate of the company who owns either restricted or unrestricted shares of our common stock, are entitled to sell their securities without registration with the SEC under an exemption from registration provided by Rule 144 under the Securities Act.

Non-Affiliates

Any person who is not deemed to have been one of our affiliates at the time of, or at any time during the three months preceding, a sale may sell an unlimited number of restricted securities under Rule 144 if:

- the restricted securities have been held for at least six months, including the holding period of any prior owner other than one of our affiliates (subject to certain exceptions);
- we have been subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale; and
- we are current in our Exchange Act reporting at the time of sale.

Any person who is not deemed to have been an affiliate of ours at the time of, or at any time during the three months preceding, a sale and has held the restricted securities for at least one year, including the holding period of any prior owner other than one of our affiliates, will be entitled to sell an unlimited number of restricted securities without regard to the length of time we have been subject to Exchange Act periodic reporting or whether we are current in our Exchange Act reporting. Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Affiliates

Persons seeking to sell restricted securities who are our affiliates at the time of, or any time during the three months preceding, a sale, would be subject to the restrictions described above. They are also subject to additional restrictions, by which such person would be required to comply with the manner of sale and notice provisions of Rule 144 and would be entitled to sell within any three-month period only that number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately shares immediately after the completion of this offering based on the number of shares outstanding as of June 30, 2020; or
- the average weekly trading volume of our common stock on the stock exchange on which our shares are listed during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Additionally, persons who are our affiliates at the time of, or any time during the three months preceding, a sale may sell unrestricted securities under the requirements of Rule 144 described above, without regard to the six-month holding period of Rule 144, which does not apply to sales of unrestricted securities.

Rule 701

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and in the section titled “Underwriting” and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

Form S-8 Registration Statements

We intend to file one or more registration statements on Form S-8 under the Securities Act to register all shares of common stock subject to outstanding stock options and common stock issued or issuable under our equity plans. We expect to file the registration statement covering shares offered pursuant to our stock plans as soon as practicable after the closing of this offering, permitting the resale of such shares by non-affiliates in the public market without restriction under the Securities Act and the sale by affiliates in the public market, subject to compliance with the resale provisions of Rule 144 and expiration or release from the terms of the lock-up agreements described above.

Lock-Up Agreements

We, our executive officers and directors and substantially all of the holders of our common stock outstanding on the date of this prospectus have entered into lock-up agreements with the underwriters

or otherwise agreed, subject to certain exceptions, that we and they will not, directly or indirectly, offer, sell, contract to sell, pledge, grant any option to purchase, make any short sale, or otherwise dispose of or hedge any of our shares of common stock, any options or warrants to purchase shares of our common stock, or any securities convertible into, or exchangeable for or that represent the right to receive shares of our common stock, without the prior written consent of Goldman Sachs & Co. LLC, Morgan Stanley & Co. LLC and Jefferies LLC for a period of 180 days from the date of this prospectus.

In addition to the restrictions contained in the lock-up agreements described above, we have entered into an agreement with the holders of our convertible preferred stock that contains market stand-off provisions imposing restrictions on the ability of such security holders to sell or otherwise transfer or dispose of any registrable securities for a period of 180 days following the date of this prospectus.

Registration Rights

Upon the closing of this offering, the holders of 25,647,048 shares of our common stock, including common stock issuable upon the conversion of our convertible preferred stock, or their transferees, will be entitled to specified rights with respect to the registration of their registrable shares under the Securities Act, subject to certain limitations and the expiration, waiver or termination of the lock-up agreements. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act immediately upon effectiveness of the registration. See “Description of Capital Stock—Registration Rights” for additional information.

MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following summary describes the material U.S. federal income tax consequences to Non-U.S. Holders (as defined below) of the acquisition, ownership and disposition of our common stock acquired in this offering. This summary is not a complete analysis of all potential U.S. federal income tax consequences relating thereto, and does not address Non-U.S. or U.S. state and local consequences that may be relevant to Non-U.S. Holders in light of their particular circumstances, nor does it address U.S. federal tax consequences (such as gift and estate taxes) other than income taxes. Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Code, such as financial institutions, insurance companies, tax-exempt organizations, broker-dealers and traders in securities, U.S. expatriates, "controlled foreign corporations," "passive foreign investment companies," corporations that accumulate earnings to avoid U.S. federal income tax, corporations organized outside of the United States, any state thereof and the District of Columbia that are nonetheless treated as U.S. taxpayers for U.S. federal income tax purposes, persons that hold our common stock as part of a "straddle," "hedge," "conversion transaction," "synthetic security" or integrated investment or other risk reduction strategy, persons who acquire our common stock through the exercise of an option or otherwise as compensation, persons subject to the alternative minimum tax or federal Medicare contribution tax on net investment income, persons subject to special tax accounting rules under Section 451(b) of the Code, persons that hold more than 5% of our outstanding common stock, directly or indirectly during the applicable testing period (except to the extent specifically set forth below), "qualified foreign pension funds" as defined in Section 897(l)(2) of the Code and entities all of the interests of which are held by one or more qualified foreign pension funds, partnerships and other pass-through entities or arrangements, and investors in such pass-through entities or arrangements. Non-U.S. Holders are urged to consult their tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and Treasury Regulations, rulings and judicial decisions thereunder as of the date hereof, and such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income tax consequences different from those discussed below. We have not requested and will not request a ruling from the U.S. Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions. This discussion assumes that the Non-U.S. Holder holds our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment).

If an entity or arrangement treated as a partnership for U.S. federal income tax purposes holds our common stock, the tax treatment of a partner in the partnership (or entity or arrangement treated as partnership for U.S. federal income tax purposes) will depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Accordingly, partnerships holding our common stock and the partners in such partnerships should consult their tax advisors regarding the U.S. federal income tax consequences to them.

THIS DISCUSSION IS FOR INFORMATIONAL PURPOSES ONLY AND IS NOT TAX ADVICE. PERSONS CONSIDERING THE PURCHASE OF OUR COMMON STOCK PURSUANT TO THIS OFFERING SHOULD CONSULT THEIR TAX ADVISORS CONCERNING THE U.S. FEDERAL INCOME, ESTATE AND OTHER TAX CONSEQUENCES OF ACQUIRING, OWNING AND DISPOSING OF OUR COMMON STOCK IN LIGHT OF THEIR PARTICULAR SITUATIONS AS WELL AS ANY CONSEQUENCES ARISING UNDER THE LAWS OF ANY OTHER TAXING JURISDICTION, INCLUDING ANY STATE, LOCAL OR NON-U.S. TAX CONSEQUENCES.

For the purposes of this discussion, the term "Non-U.S. Holder" means, for U.S. federal income tax purposes, a beneficial owner of common stock that is not a partnership (or other entity or

arrangement treated as a partnership for U.S. federal income tax purposes regardless of its place of organization or formation) and is not, for U.S. federal income tax purposes, any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation or other entity treated as a corporation for U.S. federal income tax purposes created or organized in or under the laws of the United States, any state thereof or the District of Columbia;
- an estate the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust if it (i) is subject to the primary supervision of a court within the United States and one or more U.S. persons have the authority to control all substantial decisions of the trust or (ii) has a valid election in effect under applicable Treasury Regulations to be treated as a U.S. person.

Distributions

As discussed under “Dividend Policy” above, we do not anticipate to declare or pay any cash dividends to holders of our common stock in the foreseeable future. Distributions, if any, made on our common stock to a Non-U.S. Holder to the extent made out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles) generally will constitute dividends for U.S. federal income tax purposes. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and first be applied against and reduce a Non-U.S. Holder’s adjusted tax basis in its common stock, but not below zero. Any excess will be treated as capital gain and will be treated as described below under “—Sale or Other Taxable Disposition.”

Subject to the discussion below on effectively connected income, dividends paid to a Non-U.S. Holder will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends (or such lower rate specified by an applicable income tax treaty, provided the Non-U.S. Holder furnishes a properly executed IRS Form W-8BEN (in the case of individuals) or W-8BEN-E (in the case of entities), or other appropriate form, certifying the Non-U.S. Holder’s entitlement to benefits under such income tax treaty). This certification must be provided to us or our paying agent prior to the payment of dividends and must be updated periodically. In the case of a Non-U.S. Holder that is an entity, Treasury Regulations and the relevant tax treaty provide rules to determine whether, for purposes of determining the applicability of a tax treaty, dividends will be treated as paid to the entity or to those holding an interest in that entity. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the Non-U.S. Holder’s behalf, the Non-U.S. Holder will be required to provide appropriate documentation to such agent. The Non-U.S. Holder’s agent will then be required to provide certification to us or our paying agent, either directly or through other intermediaries. If a Non-U.S. Holder is eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty and does not timely file the required certification, the Non-U.S. Holder may be able to obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS. Non-U.S. Holders should consult their tax advisors regarding their entitlement to benefits under any applicable income tax treaties.

If dividends paid to a Non-U.S. Holder are effectively connected with the Non-U.S. Holder’s conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the Non-U.S. Holder maintains a permanent establishment in the United States to which such dividends are attributable), the Non-U.S. Holder will be exempt from the U.S. federal withholding tax described above if a properly executed IRS Form W-8ECI, stating that the dividends are effectively connected with the Non-U.S. Holder’s conduct of a trade or business within the United States, is furnished to us (or, if stock is held through a financial institution or other agent, to such agent).

In general, such effectively connected dividends will be subject to U.S. federal income tax, on a net-income basis at the regular rates applicable to U.S. residents. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional "branch profits tax," which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable income tax treaty) on the corporate Non-U.S. Holder's effectively connected earnings and profits, subject to certain adjustments. Non-U.S. Holders should consult their tax advisors regarding any applicable income tax treaties that may provide for different rules.

Sale or Other Taxable Disposition

Subject to the discussions below regarding backup withholding and foreign accounts, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other disposition of our common stock unless:

- the gain is effectively connected with a trade or business of such Non-U.S. Holder in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment or fixed base that such holder maintains in the United States);
- the Non-U.S. Holder is a nonresident alien individual and is treated, for U.S. federal income tax purposes, as present in the United States for a period or periods aggregating to 183 or more days in the taxable year of the disposition and certain other conditions are met; or
- our common stock constitutes a United States real property interest," or USRPI, by reason of our status as a "United States real property holding corporation," or USRPHC, within the meaning of Section 897(c)(2) of the Code, for U.S. federal income tax purposes

In general, we would be a USRPHC if the fair market value of our U.S. real property interests comprise at least 50% of the sum of the fair market value of our worldwide real property assets and our other assets which are used or held for use in a trade or business. We believe that we have not been and we are not, and do not anticipate becoming, a USRPHC. Even if we are treated as a USRPHC, in general, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (i) such Non-U.S. Holder owned, directly, indirectly and constructively, no more than 5% of our common stock at all times within the shorter of (x) the five-year period preceding such disposition or (y) such Non-U.S. Holder's holding period and (ii) our common stock is regularly traded on an established securities market. There can be no assurance that our common stock will continue to qualify as regularly traded on an established securities market. If we are determined to be a USRPHC and the foregoing exception does not apply, then a Non-U.S. Holder will be taxed on a disposition of our common stock, generally, in the manner as gain that is effectively connected with the conduct of a U.S. trade or business (subject to the provisions under an applicable income tax treaty), except that the branch profits tax generally will not apply.

A Non-U.S. Holder described in the first bullet point above generally will be subject to U.S. federal income tax on a net income basis at the regular rates, and corporate Non-U.S. Holders described in the first bullet point above may be subject to the additional branch profits tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty.

Gain described in second bullet point above will be subject to U.S. federal income tax at a flat 30% rate or such lower rate as may be specified by an applicable income tax treaty, which gain may be offset by certain U.S.-source capital losses (even though the Non-U.S. Holder is not considered a resident of the United States), provided that the Non-U.S. Holder has timely filed U.S. federal income tax returns with respect to such losses.

Non-U.S. Holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

Information Reporting Requirements and Backup Withholding

Generally, we must report information to the IRS with respect to any dividends we pay on our common stock (even if the payments are exempt from withholding), including the amount of any such dividends, the name and address of the recipient and the amount, if any, of tax withheld. A similar report is sent to the Non-U.S. Holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities of the country in which the Non-U.S. Holder's resides or is established.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding (currently at a rate of 24%). U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN, IRS Form W-8BEN-E or IRS Form W-ECI (as applicable), or otherwise establishes an exemption. Notwithstanding the foregoing, backup withholding may apply if the payer has actual knowledge, or reason to know, that the holder is a U.S. person who is not an exempt recipient.

U.S. information reporting and backup withholding requirements generally will apply to the proceeds of a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or foreign, except that information reporting and such requirements may be avoided if the Non-U.S. Holder provides a properly executed IRS Form W-8BEN or IRS Form W-8BEN-E or otherwise meets documentary evidence requirements for establishing non-U.S. person status or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. Information reporting and backup withholding requirements may, however, apply to a payment of disposition proceeds if the broker has actual knowledge, or reason to know, that the holder is, in fact, a U.S. person. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be credited against the tax liability of persons subject to backup withholding, provided that the required information is timely furnished to the IRS.

Foreign Accounts

Sections 1471 through 1474 of the Code (commonly referred to as FATCA) impose a U.S. federal withholding tax of 30% on certain payments, including dividends paid on, and (subject to the proposed Treasury Regulations discussed below) the gross proceeds of a disposition of, our common stock paid to a non-U.S. financial institution (as specifically defined by applicable rules) unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding U.S. account holders of such institution (which includes certain equity holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). FATCA also generally imposes a U.S. federal withholding tax of 30% on certain payments, including dividends paid on, and (subject to the proposed Treasury Regulations discussed below) the gross proceeds of a disposition of, our common stock to a non-financial non-U.S. entity unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding substantial direct and indirect U.S. owners of the entity. An intergovernmental agreement between the United States and an applicable foreign country may modify those requirements. The withholding tax described above will not apply if the non-U.S. financial institution or non-financial non-U.S. entity otherwise qualifies for an exemption from the rules.

The withholding provisions described above currently apply to payments of dividends, and, subject to the proposed Treasury Regulations described below, to payments of gross proceeds from a sale or other disposition of common stock.

The U.S. Treasury Department released proposed Treasury Regulations which, if finalized in their present form, would eliminate the U.S. federal withholding tax of 30% applicable to the gross proceeds of a disposition of our common stock. In its preamble to such proposed Treasury Regulations, the U.S. Treasury Department stated that taxpayers, including withholding agents, may generally rely on the proposed Treasury Regulations until final Treasury Regulations are issued. Non-U.S. Holders are encouraged to consult with their tax advisors regarding the possible implications of FATCA on their investment in our common stock.

EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY RECENT OR PROPOSED CHANGE IN APPLICABLE LAW.

UNDERWRITING

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. Goldman Sachs & Co. LLC, Morgan Stanley & Co. LLC and Jefferies LLC are the representatives of the underwriters.

Underwriters	Number of Shares
Goldman Sachs & Co. LLC	
Morgan Stanley & Co. LLC	
Jefferies LLC	
Chardan Capital Markets, LLC	
Total	

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until this option is exercised. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters have an option to buy up to an additional shares from us to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise that option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above.

The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters by us. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase up to additional shares from us.

	No Exercise	Full Exercise
Per Share	$	$
Total	$	$

Shares sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover of this prospectus. Any shares sold by the underwriters to securities dealers may be sold at a discount of up to $ per share from the initial public offering price. After the initial offering of the shares, the representatives may change the offering price and the other selling terms. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make internet distributions on the same basis as other allocations.

We and our officers, directors, and holders of substantially all of our common stock and securities convertible into or exchangeable for our common stock have agreed or will agree with the underwriters, subject to certain exceptions, not to dispose of or hedge any of our or their common stock or securities

convertible into or exchangeable for shares of common stock during the period from the date of this prospectus continuing through the date 180 days after the date of this prospectus, except with the prior written consent of the representatives. See “Shares Eligible for Future Sale” for a discussion of certain transfer restrictions.

The restrictions described in the immediately preceding paragraph do not apply to certain transfers, dispositions or transactions, including:

(i) as a *bona fide* gift or gifts or as a charitable contribution; provided that the donee or donees thereof agree to be bound in writing by these restrictions; and provided further, that that any such transfer shall not involve a disposition for value;

(ii) to any trust for the direct or indirect benefit of the holder or of any member of the immediate family of the holder, or if the holder is a trust, to a trustor, trustee (or co-trustee) or beneficiary of the trust or to the estate of the beneficiary of such trust; provided that the transferee agrees to be bound in writing by these restrictions; and provided further, that any such transfer shall not involve a disposition for value;

(iii) in connection with the sale of any of the holder’s shares acquired (a) in this offering (other than any of our directed shares acquired by an officer or director of ours) or (b) in open market transactions after completion of this offering;

(iv) if the holder is a corporation, partnership, limited liability company, trust or other business entity, (a) to any corporation, partnership limited liability company or other business entity, all of the beneficial ownership interests of which, in each such case, are held by the holder, (b) to another corporation, partnership, limited liability company, trust or other business entity that is an affiliate (as defined in Rule 405 promulgated under the Securities Act of the holder or the immediate family of the holder, or to any investment fund or other entity controlling, controlled by, managing or managed by or under common control with the holder or affiliates or immediate family of the holder (including, for the avoidance of doubt, if the holder is a partnership, to its general partner or a successor partnership or fund, or any other funds managed by such partnership), or (c) as part of a distribution, transfer or disposition without consideration by the holder to its stockholders, partners, members, beneficiaries or other equity holders; provided, however, that in the case of any transfer or disposition contemplated by this clause (iv), it shall be a condition to the transfer or disposition that the transferee agrees to be bound in writing by these restrictions;

(v) by surrender or forfeiture of shares of common stock or other securities of ours to us to satisfy tax withholding obligations upon exercise or vesting or the exercise price upon a cashless net exercise, in each case, of stock options, restricted stock, other equity awards, warrants or other right to acquire common stock as described in this prospectus; provided that any filing under Section 16 of the Exchange Act shall indicate in the footnotes thereto that (A) the filing relates to the applicable circumstances described in this clause and (B) no securities were sold by the holder, and no other public announcement shall be required or shall be made voluntarily in connection with such transfer;

(vi) by will or intestacy, provided that any filing made under Exchange Act shall include a footnote noting the circumstances described in this clause; and provided further, that that any such transfer shall not involve a disposition for value;

(vii) to any immediate family member of the holder, provided that such family member agrees to be bound in writing by these restrictions; and provided further, that that any such transfer shall not involve a disposition for value;

(viii) to us pursuant to an agreement under which we have (a) the option to repurchase such shares upon termination of the holder’s employment or other service relationship with us

pursuant to contractual agreements with us as in effect as of the date of this prospectus, and provided further, that any filing made under Exchange Act shall include a footnote noting the circumstances described in this clause;

(ix) by operation of law or pursuant to a court order or settlement agreement related to the distribution of assets in connection with the dissolution of a marriage, domestic partnership or civil union, provided that such transferee or distributee agrees to be bound in writing by these restrictions; and provided further, that any required filing under Section 16 of the Exchange Act shall indicate in the footnotes thereto that (A) the filing relates to the circumstances described in this clause and (B) that no securities were sold by the holder, and no other public announcement shall be made voluntarily by the holder in connection with such transfer or disposition;

(x) pursuant to a *bona fide* third party tender offer, merger, consolidation or other similar transaction made to all holders of common stock, the result of which is that any "person" of greater than 50% of the total voting power of our voting share capital after the closing of this offering and approved by our board of directors; provided that in the event that the tender offer, merger, consolidation or other such transaction is not completed, the holder's shares shall remain subject to these restrictions;

(xi) with the prior written consent of the representatives of the underwriters on behalf of the underwriters; and

(xii) in connection with the conversion of outstanding shares of our convertible preferred stock into common stock as described in this prospectus, or any reclassification or conversion of the common stock; provided that any common stock received upon such conversion or reclassification will be subject to the restrictions set forth in this paragraph; and provided further, that any required filing under Section 16 of the Exchange Act shall indicate in the footnotes thereto that the filing relates to the circumstances described in this clause;

provided, that, in the case of clauses (i), (ii), (iii), (iv) and (vii) above, no filing under Section 16 of the Exchange Act reporting a reduction in beneficial ownership of the holder's shares shall be required or shall be voluntarily made during the restricted period.

Additionally, the holder may establish a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of the holder's shares, provided that such plan does not provide for any transfers of common stock during the restricted period and no filing under the Exchange Act nor any other public filing or disclosure of such trading plan shall be made during the restricted period.

Prior to the offering, there has been no public market for the shares. The initial public offering price will be negotiated among us and the representatives. Among the factors to be considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, will be our historical performance, estimates of our business potential and earnings prospects, an assessment of our management and the consideration of the above factors in relation to market valuation of companies in related businesses.

We have applied to list our common stock on the Nasdaq Global Market under the symbol "TSHA".

In connection with the offering, the underwriters may purchase and sell shares of our common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A "covered

short position" is a short position that is not greater than the amount of additional shares for which the underwriters' option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. "Naked" short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of our common stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on the Nasdaq Global Market, in the over-the-counter market or otherwise.

We estimate that our share of the total expenses of the offering, excluding underwriting discounts and commissions, will be approximately $ million. We have agreed to reimburse the underwriters for certain of their expenses in an amount up to $30,000.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively traded securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities and/or instruments of the issuer (directly, as collateral securing other obligations or otherwise) and/or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such assets, securities or instruments and may at any time

hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

Certain affiliates of Chardan Capital Markets, LLC purchased 333,333 shares of our Series A convertible preferred stock in our Series A convertible preferred stock financing. Those shares of Series A convertible preferred stock will convert into 333,333 shares of common stock immediately prior to and in connection with the completion of this offering.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions related to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

European Economic Area and United Kingdom

In relation to each Member State of the European Economic Area and the United Kingdom, or each a Relevant State, no common shares, or the Shares, have been offered or will be offered pursuant to the offering to the public in that Relevant State prior to the publication of a prospectus in relation to the Shares which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation), except that offers of Shares may be made to the public in that Relevant State at any time under the following exemptions under the Prospectus Regulation:

(a) to any legal entity which is a qualified investor as defined under the Prospectus Regulation;

(b) to fewer than 150 natural or legal persons (other than qualified investors as defined under the Prospectus Regulation); or

(c) in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of Shares shall require us or any representative to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation and each person who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the representatives and us that it is a “qualified investor” as defined in the Prospectus Regulation.

In the case of any shares being offered to a financial intermediary as that term is used in Article 5 of the Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a nondiscretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Member State to qualified investors as so defined or in circumstances in which the prior consent of the representatives has been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an “offer to the public” in relation to any Shares in any Relevant State means the communication in any form and by any means of sufficient

information on the terms of the offer and any Shares to be offered so as to enable an investor to decide to purchase or subscribe for any Shares, and the expression “Prospectus Regulation” means Regulation (EU) 2017/1129.

United Kingdom

Each Underwriter has represented and agreed that:

(a) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000, as amended, or the FSMA) received by it in connection with the issue or sale of the shares in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(b) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares in, from or otherwise involving the United Kingdom.

Canada

The securities may be sold in Canada only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions, and Ongoing Registrant Obligations. Any resale of the securities must be made in accordance with an exemption form, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser’s province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser’s province or territory of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Hong Kong

The shares may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32 of the Laws of Hong Kong), or Companies (Winding Up and Miscellaneous Provisions) Ordinance, or which do not constitute an invitation to the public within the meaning of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong), or Securities and Futures Ordinance, or (ii) to “professional investors” as defined in the Securities and Futures Ordinance and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a “prospectus” as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance, and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to “professional investors” in Hong Kong as defined in the Securities and Futures Ordinance and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor (as defined under Section 4A of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA) under Section 274 of the SFA, (ii) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to conditions set forth in the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor, the securities (as defined in Section 239(1) of the SFA) of that corporation shall not be transferable for 6 months after that corporation has acquired the shares under Section 275 of the SFA except: (i) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (ii) where such transfer arises from an offer in that corporation's securities pursuant to Section 275(1A) of the SFA, (iii) where no consideration is or will be given for the transfer, (iv) where the transfer is by operation of law, (v) as specified in Section 276(7) of the SFA, or (vi) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore, or Regulation 32.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is a trust (where the trustee is not an accredited investor (as defined in Section 4A of the SFA)) whose sole purpose is to hold investments and each beneficiary of the trust is an accredited investor, the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferable for 6 months after that trust has acquired the shares under Section 275 of the SFA except: (i) to an institutional investor under Section 274 of the SFA or to a relevant person (as defined in Section 275(2) of the SFA), (ii) where such transfer arises from an offer that is made on terms that such rights or interest are acquired at a consideration of not less than S$200,000 (or its equivalent in a foreign currency) for each transaction (whether such amount is to be paid for in cash or by exchange of securities or other assets), (iii) where no consideration is or will be given for the transfer, (iv) where the transfer is by operation of law, (v) as specified in Section 276(7) of the SFA, or (vi) as specified in Regulation 32.

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Act of Japan (Act No. 25 of 1948, as amended), or the FIEA. The securities may not be offered or sold, directly or indirectly, in Japan or to or for the benefit of any resident of Japan (including any person resident in Japan or any corporation or other entity organized under the laws of Japan) or to others for reoffering or resale, directly or indirectly, in Japan or to or for the benefit of any resident of Japan, except pursuant to an exemption from the registration requirements of the FIEA and otherwise in compliance with any relevant laws and regulations of Japan.

LEGAL MATTERS

The validity of the shares of common stock offered hereby will be passed upon for us by Cooley LLP, New York, New York. Certain legal matters will be passed upon for the underwriters by Goodwin Procter LLP, Boston, Massachusetts. As of the date of this prospectus, GC&H Investments, LLC, an entity consisting of current and former partners and associates of Cooley LLP, beneficially holds an aggregate of 26,469 shares of our common stock on an as-converted basis.

EXPERTS

The financial statements as of December 31, 2019 and for the period from September 20, 2019 (date of inception) through December 31, 2019, included in this prospectus have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report appearing herein. Such financial statements have been so included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to our company and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the internet at the SEC's website at *www.sec.gov*. Upon completion of this offering, we will be subject to the information reporting requirements of the Exchange Act, and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available at *www.sec.gov*.

We also maintain a website at *www.tayshagtx.com*, at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of, and is not incorporated into, this prospectus. We have included our website in this prospectus solely as an inactive textual reference.

Taysha Gene Therapies, Inc.
Index to Financial Statements as of December 31, 2019 and for the period from September 20, 2019 (date of inception) through December 31, 2019

Taysha Gene Therapies, Inc.
Index to Unaudited Condensed Consolidated Financial Statements as of and for the Six Months Ended June 30, 2020

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Taysha Gene Therapies, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheet of Taysha Gene Therapies, Inc. (the "Company") as of December 31, 2019, the related statements of operations, stockholders' deficit, and cash flows for the period from September 20, 2019 (date of inception) through December 31, 2019, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2019, and the results of its operations and its cash flows for the period from September 20, 2019 (date of inception) through December 31, 2019, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audit, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audit included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Dallas, Texas
July 31, 2020

We have served as the Company's auditor since 2020.

Taysha Gene Therapies, Inc.
Balance Sheet
(in thousands, except share and per share data)

	December 31, 2019
ASSETS	
Current assets:	
Deferred offering costs	$ 15
Total assets	**$ 15**
LIABILITIES AND STOCKHOLDERS' DEFICIT	
Current liabilities:	
Accrued expenses	$ 150
Total liabilities	150
Commitments and contingencies - Note 8	
Stockholders' deficit	
Common stock, $0.00001 par value per share; 10,000,000 shares authorized, issued and outstanding as of December 31, 2019	—
Additional paid-in capital	980
Accumulated deficit	(1,115)
Total stockholders' deficit	(135)
Total liabilities and stockholders' deficit	**$ 15**

The accompanying notes are an integral part of these financial statements.

Taysha Gene Therapies, Inc.
Statement of Operations
(in thousands, except share and per share data)

	For the period from September 20, 2019 (date of inception) to December 31, 2019
Operating expenses:	
Research and development	$ 987
General and administrative	128
Total operating expenses	1,115
Net loss	**$ (1,115)**
Net loss per common share, basic and diluted	$ (0.13)
Weighted average common shares outstanding, basic and diluted	8,834,951

The accompanying notes are an integral part of these financial statements.

Taysha Gene Therapies, Inc.
Statement of Stockholders' Deficit
(in thousands, except share data)

	Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount			
Balance as of September 20, 2019 (date of inception)	—	$ —	$ —	$ —	$ —
Issuance of Founders Shares	8,000,000	—	—	—	—
Issuance of common stock for UT Southwestern license	2,000,000	—	980	—	980
Net loss	—	—	—	(1,115)	(1,115)
Balance as of December 31, 2019	**10,000,000**	**$ —**	**$ 980**	**$ (1,115)**	**$ (135)**

The accompanying notes are an integral part of these financial statements.

Taysha Gene Therapies, Inc.
Statement of Cash Flows
(in thousands)

	For the period from September 20, 2019 (date of inception) to December 31, 2019
Cash flows from operating activities	
Net loss	$ (1,115)
Adjustments to reconcile net loss to net cash used in operating activities:	
Noncash research and development license expense	980
Changes in operating assets and liabilities:	
Accrued expenses	135
Net cash used in operating activities	—
Net cash used in investing activities	—
Net cash used in financing activities	—
Net decrease in cash and cash equivalents	—
Cash at the beginning of the period	—
Cash at the end of the period	**$ —**
Supplemental disclosure of noncash financing activities:	
Deferred offering costs not yet paid	$ 15

The accompanying notes are an integral part of these financial statements.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

Note 1—Organization and Description of Business Operations

Taysha Gene Therapies, Inc. (the "Company" or "Taysha") was originally formed under the laws of the State of Texas on September 20, 2019 ("Inception"). Taysha converted to a Delaware corporation on February 13, 2020, which had no impact to the Company's par value or issued and authorized capital structure.

Taysha is a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system ("CNS") in both rare and large patient populations.

Liquidity and Capital Resources

The Company has incurred operating losses since Inception, and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of December 31, 2019, the Company had an accumulated deficit of $1.1 million.

Between March and July 2020, the Company closed on the sale of an aggregate of 10,000,000 shares of Series A convertible preferred stock for gross proceeds of $30.0 million. In July 2020, the Company closed on the sale of an aggregate of 5,623,520 shares of Series B convertible preferred stock for gross proceeds of approximately $95.6 million.

Due to the sale of its Series A and Series B convertible preferred stock, management believes that its existing financial resources are sufficient to continue operating activities at least one year past the issuance date of these financial statements. Future capital requirements will depend on many factors, including the timing and extent of spending on research and development and the market acceptance of the Company's products. The Company will need to obtain additional financing in order to complete clinical studies and launch and commercialize any product candidates for which it receives regulatory approval. There can be no assurance that such financing will be available or will be at terms acceptable to the Company.

Note 2—Significant Accounting Policies

Basis of Presentation

The Company's financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position for the period presented.

Emerging Growth Company

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board ("FASB"), or other standard setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the impact of recently issued standards that are not yet effective will not have a material impact on the Company's financial statements upon adoption. Under the Jumpstart Our Business Startups Act of 2012, as amended, the Company meets the definition of an emerging growth company, and has elected the extended transition period for complying with new or revised accounting standards, which delays the adoption of these accounting standards until they would apply to private companies.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. The most significant estimates and assumptions in the Company's financial statements relate to the determination of the fair value of the common stock, estimating preclinical manufacturing accruals and accrued or prepaid research and development expenses, and the valuation allowance of deferred tax assets. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

Risks and Uncertainties

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, dependence on key personnel, protection of proprietary technology, compliance with government regulations, and ability to transition from preclinical manufacturing to commercial production of products.

The Company's product candidates require approvals from the U.S. Food and Drug Administration and comparable foreign regulatory agencies prior to commercial sales in their respective jurisdictions. There can be no assurance that any product candidates will receive the necessary approvals. If the Company was denied approval, approval was delayed or the Company was unable to maintain approval for any product candidate, it could have a materially adverse impact on the Company.

Segments

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business as a single operating segment, which is the business of developing AAV-based gene therapies for the treatment of rare monogenic diseases of the CNS.

Deferred Offering Costs

The Company capitalizes certain legal, professional, accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' deficit as a reduction of additional paid-in capital generated as a result of the equity financing. Should the in-process equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the statement of operations. As of December 31, 2019, $15,000 of deferred offering costs were capitalized on the balance sheet related to the Company's Series A convertible preferred stock financing that closed in March 2020 (see Note 9).

Taysha Gene Therapies, Inc.
Notes to Financial Statements

Research and Development

The Company has entered into research and development contracts with research institutions and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected on the balance sheet as prepaid or accrued expenses. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs.

Research and development costs primarily consist of laboratory costs and other supplies, and the cost to acquire licenses.

Costs incurred in obtaining technology licenses through asset acquisitions are charged to research and development expense if the licensed technology has not reached technological feasibility and has no alternative future use.

Income Taxes

Income taxes are recorded in accordance with ASC 740, *Income Taxes* (“ASC 740”), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse, and net operating loss (“NOL”) carryforwards and research and development tax credit (“R&D Credit”) carryforwards. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has recorded a full valuation allowance to reduce its net deferred income tax assets to zero. In the event the Company were to determine that it would be able to realize some or all its deferred income tax assets in the future, an adjustment to the deferred income tax asset valuation allowance would increase income in the period such determination was made.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit would more likely than not be realized assuming examination by the taxing authority. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. At December 31, 2019, the Company had no liability for income tax associated with uncertain tax positions. The Company would recognize any corresponding interest and penalties associated with its income tax positions in income tax expense. There was no income tax interest or penalties incurred in 2019 since Inception.

Comprehensive Loss

Comprehensive loss is equal to net loss as presented in the accompanying statement of operations, as the Company did not have any other comprehensive income or loss for the period presented.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding during the period. Diluted net loss per share is the same as basic net loss per share, since the effects of potentially dilutive securities are antidilutive given the Company has reported a net loss in 2019 since Inception. There were no potentially dilutive securities outstanding at any point during 2019.

Recently Adopted Accounting Pronouncements

In May 2014, the FASB issued ASC 606, *Revenue from Contracts with Customers* ("ASC 606"). This guidance applies to any entity that either enters into contracts with customers to transfer goods or services or enters into contracts for the transfer of nonfinancial assets unless those contracts are within the scope of other standards. The core principle of this guidance is that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. This guidance supersedes existing revenue recognition guidance, including most industry-specific guidance, as well as certain related guidance on accounting for contract costs. The Company early adopted ASC 606 upon its Inception. As the Company does not have any contracts with customers, the adoption of this guidance did not have any impact on the Company's financial statements.

In June 2018, the FASB issued Accounting Standards Update ("ASU") No. 2018-07, *Compensation – Stock Compensation* (Topic 718) ("ASU 2018-07"). This update is intended to reduce cost and complexity and to improve financial reporting for share-based payments issued to non-employees (for example, service providers, external legal counsel, suppliers, etc.). The ASU expands the scope of Topic 718, *Compensation—Stock Compensation,* which currently only includes share-based payments issued to employees, to also include share-based payments issued to non-employees for goods and services. Consequently, the accounting for share-based payments to non-employees and employees will be substantially aligned. The Company early adopted ASU 2018-07 upon its Inception. The adoption of this ASU did not have a material effect on the Company's financial statements at Inception. The Company applied this accounting pronouncement to the issuance of shares to the Board of Regents of the University of Texas System (see Note 3).

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*, as amended, with guidance regarding the accounting for and disclosure of leases. This update requires lessees to recognize the liabilities related to all leases, including operating leases, with a term greater than 12 months on the balance sheet. This update also requires lessees and lessors to disclose key information about their leasing transactions. This guidance will become effective for the Company for annual reporting periods beginning after December 15, 2021 and interim periods within fiscal years beginning after December 15, 2022. The Company is currently evaluating the impact of this standard on its financial statements.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes* ("ASU 2019-12"), which is intended to simplify various aspects related to accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in ASC 740 and also clarifies and amends existing guidance to improve consistent application. This guidance is effective for fiscal years, and interim periods within those fiscal years,

beginning after December 15, 2020, with early adoption permitted. The Company is currently evaluating the impact of this standard on its financial statements.

Note 3—Research, Collaboration and License Agreements

On November 19, 2019, the Company entered into a research, collaboration and license agreement ("UT Southwestern Agreement") with the Board of Regents of the University of Texas System ("UT System") on behalf of The University of Southwestern Medical Center ("UT Southwestern"). Under the UT Southwestern Agreement, UT Southwestern is primarily responsible for preclinical development activities with respect to licensed products for use in certain specified indications (up to IND-enabling studies), and the Company is responsible for all subsequent clinical development and commercialization activities with respect to the licensed products. UT Southwestern will conduct such preclinical activities for a two-year period under mutually agreed upon sponsored research agreements that were entered into beginning in April 2020. During the initial research phase, the Company has the right to expand the scope of specified indications under the UT Southwestern Agreement.

In connection with the UT Southwestern Agreement, the Company obtained an exclusive, worldwide, royalty-free license under certain patent rights of UT Southwestern and a non-exclusive, worldwide, royalty-free license under certain know-how of UT Southwestern, in each case to make, have made, use, sell, offer for sale and import licensed products for use in certain specified indications. Additionally, the Company obtained a non-exclusive, worldwide, royalty-free license under certain patents and know-how of UT Southwestern for use in all human uses, with a right of first refusal to obtain an exclusive license under certain of such patent rights and an option to negotiate an exclusive license under other of such patent rights. The Company is required to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product.

The UT Southwestern Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last valid claim of a licensed patent in such country for such licensed product. After the initial research term, the Company may terminate the agreement, on an indication-by-indication and licensed product-by-licensed product basis, at any time upon specified written notice to UT Southwestern. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party.

As partial consideration for the license rights granted under the UT Southwestern Agreement, the Company issued 2,000,000 shares of its common stock, or 20% of its then outstanding fully-diluted common stock, to UT Southwestern. As additional consideration, UT Southwestern was entitled to receive additional shares if their holdings fell below 10% on a fully-diluted basis before or as a result of the completion of a qualified financing. In March 2020, following the initial closing of the Series A convertible preferred stock agreement, which met the definition of such qualified financing, the anti-dilution feature expired and no additional shares were issued. The Company does not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement other than costs related to maintenance of patents. In 2019, total pass-through costs related to the UT Southwestern Agreement totaled approximately $16,000.

The Company also has the right of first refusal for any shares that UT Southwestern wishes to sell that expires upon an initial public offering or change of control of the Company.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

The fair value of the shares issued was determined to be $980,000 which was recorded as an increase to common stock and additional paid-in capital and a corresponding research and development expense during the period from Inception through December 31, 2019. The cost to acquire this license is recorded within research and development expense in the statement of operations because the license relates to specific preclinical research and development activities that do not have an alternative future use. As the acquisition of the license was settled through the issuance of shares of the Company's common stock, this transaction fell within the scope of ASC Topic 718 since equity was issued in exchange for goods (the license). Specifically, the Company recorded the cost of the license as a non-employee share based payment, measured at the grant date fair value of the common stock of $0.49 per share. The common shares were equity-classified. The anti-dilution provision was concluded to represent a performance condition tied to a future liquidity event, which was not considered probable of occurring at December 31, 2019 since it was deemed outside of the Company's control.

Since there is an absence of a public trading market for the Company's common stock, the valuation of the common stock issued to UT System was performed using methodologies, approaches, and assumptions consistent with the American Institute of Certified Public Accountants Audit and Accounting Practice Aid Series: Valuation of Privately-Held-Company Equity Securities Issued as Compensation. The estimated fair value of the Company's common stock was determined with the assistance of a third-party specialist using an Option-Pricing Method ("OPM"). Under the OPM, the Company used a backsolve method so that the enterprise value equaled the contemplated value of the Company determined by the Series A convertible preferred stock financing transaction which initially closed on March 4, 2020 (see Note 9), and giving consideration to any value-generating events that may have occurred between March 4, 2020 and the date the common shares were issued to UT System on November 19, 2019. A discount for lack of marketability ("DLOM") of 20% was applied to derive the valuation price. The DLOM used was derived from the then-current estimates of the time to a liquidity event made by the Company's board of directors, with input from the Company's senior management. The estimates are based, in part, on subjective assumptions.

Note 4—Research Grant Agreement

In late December 2019, the Company entered into a research grant agreement ("RGA") with Queen's University at Kingston ("Queen's"), for certain research and development activities related to the generation of AAV9 vector. The Company committed to fund $3.8 million under the RGA with Queen's. The Company agreed to issue Queen's a promise-to-pay note whereby any amounts paid directly by Queen's for the manufacture of the vector for use in the funded research activities, to the extent such amounts have not already been funded by the Company to Queen's, become a loan obligation for the Company (the "Note"), subject to an interest rate of 6%. Any amounts outstanding under the Note must be repaid, along with any accrued interest, by or before June 30, 2020. In the event of default, any amount outstanding shall be deemed immediately payable by RA Session II, the Company's President and Chief Executive Officer, as a personal guarantor (see Note 5). For the period from Inception through December 31, 2019, the Company did not incur any expenses associated with the Queen's RGA, and no amounts were due or outstanding under the Note as of December 31, 2019.

Note 5—Related Party Transactions

RA Session II, President and Chief Executive Officer and a member of the Company's board of directors, is a guarantor under the Guaranty and Security Agreement between himself, Queen's and

the Company, and in the event of the Company's failure to fund its obligations under the RGA with Queen's, has personally guaranteed payments due by the Company to Queen's.

Note 6—Stockholders' Deficit

At December 31, 2019, the Company was authorized to issue 10,000,000 shares of common stock with a par value of $0.00001 per share. RA Session II and one other individual were issued 7,950,000 and 50,000 shares of the Company's common stock ("Founders Shares"), respectively, upon the formation of the Company on September 20, 2019. RA Session II and the other individual had not paid the Company for the aggregate par value, which approximated fair value at Inception, for these Founders Shares as of December 31, 2019. All amounts owed for the issuance of these Founders Shares were settled in July 2020.

On November 19, 2019, the Company issued 2,000,000 shares of the Company's common stock to UT System in exchange for a license agreement. The fair value of the shares on the date of issuance was $980,000 in the aggregate.

Except for the issuance of common stock to UT System in connection with the UT Southwestern Agreement, the Company did not grant any equity-based awards during the period from its Inception through December 31, 2019.

The Company amended its certificate of incorporation on March 4, 2020, July 2, 2020 and again on July 28, 2020 such that the total number of shares of common stock authorized to be issued was increased to 30,000,000, and the total number of shares of preferred stock authorized to be issued was increased to 15,647,052, of which 10,000,000 preferred shares are designated Series A preferred stock and 5,647,052 are designated Series B preferred stock.

Note 7—Income Taxes

Provision for income taxes

There is no provision for income taxes because the Company has incurred operating losses and capitalized certain items for income tax purposes since its inception and maintains a full valuation allowance against its net deferred tax assets. The reported amount of income tax expense for the period differs from the amount that would result from applying the federal statutory tax rate to net loss before taxes primarily because of the change in valuation allowance.

Deferred tax assets and valuation allowance

Deferred tax assets reflect the tax effects of NOLs, tax credit carryovers, and temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. At December 31, 2019, the Company's most significant deferred tax asset is intangible costs capitalized for income tax purposes but expensed for financial reporting. At December 31, 2019, the Company had no U.S. federal and state NOL carryforwards.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

A reconciliation of the U.S. statutory federal income tax rate to the Company's effective tax rate is as follows:

	For the period from September 20, 2019 (date of inception) to December 31, 2019
Statutory federal income tax rate	21.0%
Change in valuation allowance	(21.0%)
Income tax provision (benefit)	0.0%

The significant components of the Company's net deferred tax asset are as follows (in thousands):

	December 31, 2019
Deferred tax assets:	
Organizational and start-up costs	$ 28
R&D license	206
Total deferred income tax assets	234
Valuation allowance	(234)
Deferred tax asset, net of allowance	$ —

The Company may be entitled to claim federal and state income tax credits for its 2019 R&D activities, but these amounts have not yet been determined. Any R&D Credits generated by the Company in 2019 would result in an additional deferred tax asset that would be subject to a full valuation allowance. Future changes in ownership may limit the utilization of R&D Credits due to Section 383 of the Internal Revenue Code of 1986, as amended, and similar provisions.

The Company's initial tax year was 2019, which remains open for the assessment of income taxes.

Note 8—Commitments and Contingencies

Litigation

The Company is not a party to any material legal proceedings and is not aware of any pending or threatened claims. From time to time, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities.

Commitments

As of December 31, 2019, the Company was not a party to any leasing agreements.

In the normal course of business, the Company enters into contracts that contain a variety of indemnifications with its employees, licensors, suppliers and service providers. The Company's maximum exposure under these arrangements is unknown at December 31, 2019. The Company does not anticipate recognizing any significant losses relating to these arrangements.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

Note 9—Subsequent Events

The Company has evaluated subsequent events through July 31, 2020, the date that these financial statements were issued. Except for the matters disclosed below and in Note 6, no additional subsequent events had occurred that would require recognition or disclosure in these financial statements.

Related Party Transactions

In January 2020, the Company entered into a secured promissory note with a related party, its President and Chief Executive Officer, RA Session II, for $1.67 million, with 10% interest. The Company secured the note with a first priority security interest in certain assets of the Company. During March 2020, the Company repaid $1.65 million of the note, and the remaining balance was repaid in July 2020. As a result, Mr. Session released his security interest in the collateral.

In March 2020, the Company entered into a services agreement with PBM Capital Group, LLC, an affiliate of PBM TGT Holdings, LLC (the lead investor in the Company's Series A preferred stock financing discussed below, and who has the ability to designate two members of the Company's board of directors), whereby PBM Capital Group, LLC provides accounting and other administrative and management services related to payroll administration, human resources, bookkeeping, preparation of financial statements and tax returns, accounts payable and receivable, and other similar functions for a fee of $2,500 per month.

License Agreements

On February 21, 2020, the Company entered into a license agreement with Queen's (the "Queen's Agreement") to obtain the exclusive perpetual, royalty-bearing license, with the right to sublicense through multiple tiers, under certain patent rights and know-how of Queen's, including certain improvements to such patent rights and know-how, to develop products in any field which use one or more valid claims of the patents licensed under the Queen's Agreement (the "Licensed Patents"), or the technology, information and intellectual property related to the patents licensed under the Queen's Agreement (the "Licensed Technology" and, together with the Licensed Patents, the "Licensed Products"), and to make, have made, use, sell, offer for sale, import and export Licensed Products and otherwise exploit such patents and know-how for use in certain specified indications. In exchange for the rights granted to the Company, the Company made a cash payment of $3.0 million in April 2020 and is obligated to make aggregate cash payments of up to $10.0 million upon the completion of a combination of regulatory milestones and up to $10.0 million upon the completion of a combination of commercial milestones. In further consideration of the rights granted, beginning with the Company's first commercial sale of the Licensed Products, the Company will also pay an annual earned royalty in the low single digits on net sales of Licensed Products, subject to certain customary reductions, and a percentage of non-royalty sublicensing revenue ranging in the low double digits. Royalties are payable, on a Licensed Products-by-Licensed Products and a country-by-country basis, until expiration of the last valid claim of a Licensed Patent covering such Licensed Products in such country and the expiration of any regulatory exclusivity for such Licensed Products in such country.

On April 2, 2020, the Company amended the UT Southwestern Agreement to include the addition of another licensed product and certain indications, and a right of first refusal to the Company over certain patient dosing patents. No additional consideration was transferred in connection with this amendment.

Taysha Gene Therapies, Inc.
Notes to Financial Statements

Convertible Preferred Stock Financing Arrangements

On March 4, 2020, the Company issued and sold 6,000,000 shares of its Series A convertible preferred stock for $18.0 million, or $3.00 per share. The Company also agreed to sell an additional 4,000,000 shares of its Series A convertible preferred stock to these same investors for the same price per share upon the attainment of certain defined clinical milestones. Moreover, such investors had the right, in their sole discretion, to purchase any or all such additional shares whether or not the Company achieved the specified milestones. In June and July 2020, such investors exercised in full their option to purchase these additional shares prior to the achievement of such milestones, and the Company issued and sold to such investors an aggregate of 4,000,000 shares of Series A convertible preferred stock at a purchase price of $3.00 per share for aggregate gross proceeds of $12.0 million. The Company issued and sold 8,166,667 shares to PBM TGT Holdings, LLC and 1,000,000 shares issued to Nolan Capital, LLC which are controlled by members of the Company's board of directors.

In July 2020, the Company entered into a purchase agreement (the "Series B Purchase Agreement") for a private placement of up to 5,647,052 shares of Series B convertible preferred stock. As of July 31, 2020, the Company had sold 5,623,520 of Series B convertible preferred stock at a price of $17.00 per share in multiple closings during the month of July for gross proceeds of approximately $95.6 million. The majority of investors that participated in the Series B Purchase Agreement were new investors.

Equity Incentive Plan

On July 1, 2020, the Company's board of directors approved the 2020 Equity Incentive Plan ("2020 Plan") which permits the granting of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock, restricted stock units and other stock-based awards to employees, directors, officers and consultants. On July 1, 2020, 3,529,412 shares of common stock were authorized for issuance under the 2020 Plan.

On July 1, 2020, options to purchase 2,658,822 shares of common stock under the 2020 Plan were awarded to certain employees and consultants of the Company with an exercise price per share of $0.87, which are generally expected to vest over a 4-year term. RA Session II was also awarded 705,882 restricted stock awards under the 2020 Plan on July 1, 2020, which are expected to vest over a 3-year term.

Other

During the early months of 2020, COVID-19 emerged and has subsequently spread world-wide. The World Health Organization has declared COVID-19 a pandemic resulting in federal, state and local governments and private entities mediating various restrictions, including travel restrictions, restrictions on public gatherings, stay at home orders, and advisories and quarantining people who may have been exposed to the virus. Management is currently evaluating the impact of the COVID-19 pandemic on its future plans and has concluded that while it is reasonably possible that the virus could have a negative effect on the Company's financial position and results of its operations, the specific impact is not readily determinable as of the date of these financial statements.

Taysha Gene Therapies, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share data)
(Unaudited)

	December 31, 2019	June 30, 2020	Pro Forma June 30, 2020
ASSETS			
Current assets:			
Cash and cash equivalents	$ —	$ 11,200	
Prepaid expenses	—	9	
Deferred offering costs	15	90	
Total current assets	15	11,299	
Property and equipment, net	—	19	
Total assets	**$ 15**	**$ 11,318**	
LIABILITIES, CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT			
Current liabilities			
Accounts payable	$ —	$ 1,250	
Accrued expenses	150	1,603	
Due to related party	—	61	
Preferred stock tranche liability	—	17,176	
Total current liabilities	150	20,090	
Total liabilities	150	20,090	
Commitments and contingencies - Note 9			
Convertible preferred stock			
Series A convertible preferred stock, $0.00001 par value per share; no shares authorized, issued and outstanding as of December 31, 2019; 10,000,000 shares authorized, 6,200,000 shares issued and outstanding as of June 30, 2020; no shares authorized, issued or outstanding pro forma as of June 30, 2020	—	18,014	—
Stockholders' deficit			
Common stock, $0.00001 par value per share; 10,000,000 authorized, issued and outstanding as of December 31, 2019; 23,000,000 shares authorized, 10,000,000 issued and outstanding as of June 30, 2020; 23,000,000 shares authorized, 16,200,000 issued and outstanding pro forma as of June 30, 2020	—	—	—
Additional paid-in capital	980	980	18,994
Accumulated deficit	(1,115)	(27,766)	(27,766)
Total stockholders' deficit	(135)	(26,786)	(8,772)
Total liabilities, convertible preferred stock and stockholders' deficit	**$ 15**	**$ 11,318**	

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Condensed Consolidated Statement of Operations
(in thousands, except share and per share data)
(Unaudited)

	For the Six Months Ended June 30, 2020
Operating expenses:	
Research and development	$ 8,576
General and administrative	1,018
Total operating expenses	9,594
Loss from operations	(9,594)
Other expense:	
Change in fair value of preferred stock tranche liability	(17,030)
Interest expense	(27)
Total other expense	(17,057)
Net loss	**$ (26,651)**
Net loss per common share, basic and diluted	$ (2.67)
Weighted-average common shares outstanding, basic and diluted	10,000,000
Pro forma net loss per common share, basic and diluted	$ (1.91)
Pro forma weighted-average shares outstanding, basic and diluted	13,924,176

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Condensed Consolidated Statement of Convertible Preferred Stock and Stockholders' Deficit
(in thousands, except share data)
(Unaudited)

	Series A Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount			
Balance as of December 31, 2019	**—**	**$ —**	**10,000,000**	**$ —**	**$ 980**	**$ (1,115)**	**$ (135)**
Issuance of Series A convertible preferred stock, net of issuance costs of $440 and issuance of preferred stock tranche liability of $1,050	6,200,000	17,110	—	—	—	—	—
Reclassification of preferred stock tranche liability upon issuance of Series A milestone shares	—	904	—	—	—	—	—
Net loss	—	—	—	—	—	(26,651)	(26,651)
Balance as of June 30, 2020	**6,200,000**	**$ 18,014**	**10,000,000**	**$ —**	**$ 980**	**$ (27,766)**	**$ (26,786)**

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Condensed Consolidated Statement of Cash Flows
(in thousands)
(Unaudited)

	For the Six Months Ended June 30, 2020
Cash flows from operating activities	
Net loss	$ (26,651)
Adjustments to reconcile net loss to net cash used in operating activities:	
Change in fair value of preferred stock tranche liability	17,030
Research and development license expense	3,000
Changes in operating assets and liabilities:	
Prepaid expenses	(9)
Accounts payable	1,237
Accrued expenses	1,214
Due to related party	14
Net cash used in operating activities	(4,165)
Cash flows from investing activities	
Purchase of research and development license	(3,000)
Net cash used in investing activities	(3,000)
Cash flows from financing activities	
Proceeds from issuances of Series A convertible preferred stock	18,600
Payment of Series A convertible preferred stock issuance costs	(263)
Proceeds from notes payable to related party	1,673
Repayment of note payable to related party	(1,645)
Net cash provided by financing activities	18,365
Net increase in cash and cash equivalents	11,200
Cash at the beginning of the period	—
Cash at the end of the period	**$ 11,200**
Supplemental disclosure of cash flow information:	
Cash paid for interest	$ 27
Supplemental disclosure of noncash investing and financing activities:	
Deferred offering costs not yet paid	$ 90
Capital expenditures not yet paid	$ 19
Series A convertible preferred stock issuance costs not yet paid	$ 177
Reclassification of preferred stock tranche liability upon share issuance	$ 904
Allocation of preferred stock tranche liability	$ 1,050

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Note 1—Organization and Description of Business Operations

Taysha Gene Therapies, Inc. (the "Company" or "Taysha") was originally formed under the laws of the State of Texas on September 20, 2019 ("Inception"). Taysha converted to a Delaware corporation on February 13, 2020, which had no impact to the Company's par value or issued and authorized capital structure.

Taysha is a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations.

Liquidity and Capital Resources

The Company has incurred operating losses since Inception, and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of June 30, 2020, the Company had an accumulated deficit of $27.8 million.

Between March and July 2020, the Company closed on the sale of an aggregate of 10,000,000 shares of Series A convertible preferred stock for gross proceeds of $30.0 million. Between July and August 2020, the Company closed on the sale of an aggregate of 5,647,048 shares of Series B convertible preferred stock for gross proceeds of $96.0 million (see Note 10).

Due to the sale of its Series A and Series B convertible preferred stock, management believes that its existing financial resources are sufficient to continue operating activities at least one year past the issuance date of these interim condensed consolidated financial statements. Future capital requirements will depend on many factors, including the timing and extent of spending on research and development and the market acceptance of the Company's products. The Company will need to obtain additional financing in order to complete clinical studies and launch and commercialize any product candidates for which it receives regulatory approval. There can be no assurance that such financing will be available or will be at terms acceptable to the Company.

During the early months of 2020, COVID-19 emerged and has subsequently spread worldwide. The World Health Organization has declared COVID-19 a pandemic resulting in federal, state and local governments and private entities implementing various restrictions, including travel restrictions, restrictions on public gatherings, stay at home orders, and advisories and quarantining people who may have been exposed to the virus. The Company has been actively monitoring the novel coronavirus ("COVID-19") situation and its impact globally. Management believes the financial results for the six months ended June 30, 2020 were not significantly impacted by COVID-19. In addition, management believes the remote working arrangements and travel restrictions imposed by various governmental jurisdictions have had limited impact on the Company's ability to maintain internal operations during the six months ended June 30, 2020. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company's business, results of operations and financial condition will depend on future developments that are highly uncertain, including as a result of new information that may emerge concerning COVID-19 and the actions taken to contain it or treat COVID-19.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Note 2—Summary of Significant Accounting Policies

Basis of Presentation

The accompanying interim condensed consolidated financial statements have been prepared in accordance with generally accepted accounting principles in the United States of America ("GAAP") as determined by the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC").

The accompanying interim condensed consolidated financial statements include the accounts of Taysha and its inactive wholly owned U.S. subsidiaries that were incorporated during 2020. All intercompany transactions and balances have been eliminated in consolidation.

The accompanying condensed consolidated balance sheet as of June 30, 2020, the condensed consolidated statements of operations, convertible preferred stock and stockholders' deficit, and cash flows for the six months ended June 30, 2020, are unaudited. The interim condensed consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in management's opinion, include all adjustments consisting of only normal recurring adjustments necessary for the fair statement of the Company's financial position as of June 30, 2020 and its results of operations and cash flows for the six months ended June 30, 2020. The results of operations for the six months ended June 30, 2020 are not necessarily indicative of the results to be expected for the full fiscal year or any other period.

These interim condensed consolidated financial statements should be read in conjunction with the Company's annual financial statements for the period from September 20, 2019 (date of inception) through December 31, 2019 included elsewhere in this prospectus.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. These estimates and assumptions are based on current facts, historical experience as well as other pertinent industry and regulatory authority information, including the potential future effects of COVID-19, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

Significant Accounting Policies

The Company's significant accounting policies are disclosed in the audited financial statements as of December 31, 2019 and for the period from September 20, 2019 (date of inception) through December 31, 2019 included elsewhere in this prospectus. Since the date of those financial statements, there have been no changes to the Company's significant accounting policies except as noted below.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Cash and Cash Equivalents

Cash and cash equivalents consist of funds held in a standard checking account. The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. As of June 30, 2020, the Company does not have any cash equivalents.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to significant concentration of credit risk consist primarily of cash and cash equivalents. Periodically, the Company may maintain deposits in financial institutions in excess of government insured limits. Management believes that the Company is not exposed to significant credit risk as the Company's deposits are held at financial institutions that management believes to be of high credit quality. The Company has not experienced any losses on these deposits.

Fair Value of Financial Instruments

The Company's financial assets and liabilities are accounted for in accordance with ASC 820, *Fair Value Measurements and Disclosures* which defines fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The fair value hierarchy requires an entity to maximize the use of observable inputs when measuring fair value and classifies those inputs into three levels:

Level 1— Observable inputs, such as quoted prices in active markets for identical assets or liabilities.

Level 2—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted prices for similar assets or liabilities; quoted prices in markets that are not active; or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the instrument's anticipated life.

Level 3—Unobservable inputs in which there is little or no market data, which require the reporting entity to develop its own assumptions.

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair values requires more judgement. Accordingly, the degree of judgement exercised by the Company in determining fair value is greatest for instruments categorized as Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The carrying values reported in the Company's condensed consolidated balance sheet for cash and cash equivalents, accounts payable, and accrued expenses are reasonable estimates of their fair values due to the short-term nature of these items.

Refer to Note 5 for further information about Level 3 inputs.

Deferred Offering Costs

Deferred offering costs consist of legal fees incurred through the balance sheet date that are directly related to the Series B convertible preferred stock offering and planned initial public offering

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

("IPO") and will be reflected as issuance costs upon the completion of the planned offerings. Should the planned offerings prove to be unsuccessful, these deferred costs as well as any additional expenses to be incurred will be charged to operations.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation and consist solely of computer equipment. Depreciation expense is recognized using the straight-line method over its estimated useful life of 3 years.

Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance are charged to expense as incurred.

Impairment of Long-Lived Assets

The Company evaluates its long-lived assets, which consist of property and equipment, for impairment whenever events or changes in circumstances indicate that the carrying amount of such assets may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to the future undiscounted net cash flows expected to be generated by the asset. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the carrying amount of the asset exceeds the fair value of the asset. There were no impairment losses recognized during the six months ended June 30, 2020.

Convertible Preferred Stock

The Company records shares of convertible preferred stock at their respective fair values on the dates of issuance, net of issuance costs. The Company has applied the guidance in ASC 480-10-S99-3A, *SEC Staff Announcement: Classification and Measurement of Redeemable Securities* and has therefore classified the Series A convertible preferred stock as mezzanine equity. The convertible preferred stock is recorded outside of stockholders' deficit because, in the event of certain deemed liquidation events considered not solely within the Company's control, such as a merger, acquisition and sale of all or substantially all of the Company's assets (a "Deemed Liquidation Event"), the convertible preferred stock will become redeemable at the option of the holders. In the event of a change of control of the Company, proceeds received from the sale of such shares will be distributed in accordance with the liquidation preferences set forth in the Company's Amended and Restated Certificate of Incorporation. The Company has determined not to adjust the carrying values of the convertible preferred stock to the liquidation preferences of such shares because of the uncertainty of whether or when such an event would occur.

Preferred Stock Tranche Liability

The Company has determined that its obligation to issue, and the Company's investors' right to purchase, additional shares of Series A convertible preferred stock pursuant to the milestone closings (see Note 5) represent a freestanding financial instrument (the "tranche liability"). The tranche liability was initially recorded at fair value. The proceeds from the sale of the convertible preferred stock are first allocated to the fair value of the tranche liability with the remaining proceeds from the sale of the convertible preferred stock allocated to the Series A convertible preferred stock. The tranche liability is

remeasured at each reporting period and upon the exercise or expiration of the obligation, with gains and losses arising from subsequent changes in its fair value recognized in other expense in the condensed consolidated statement of operations. At the time of the exercise or expiration of the tranche liability, any remaining value of the tranche liability is reclassified to convertible preferred stock on the condensed consolidated balance sheet.

Unaudited Pro Forma Financial Information

Immediately prior to the completion of an IPO of the Company's common stock, all outstanding shares of the Company's convertible preferred stock will automatically convert into shares of its common stock. Pro forma basic and diluted net loss per common share has been computed to give effect to the automatic conversion of all outstanding shares of the Company's convertible preferred stock into shares of its common stock. The unaudited pro forma net loss per common share for the six months ended June 30, 2020 has been computed using the weighted-average number of shares of common stock outstanding, including the pro forma effect of the automatic conversion of all outstanding shares of the Company's convertible preferred stock into shares of its common stock as if the IPO had occurred at the beginning of the period or their issuance dates, if later. The basic and diluted net loss per common share does not include (i) the issuance of 3,800,000 shares of the Company's Series A convertible preferred stock and 5,647,048 shares of the Company's Series B convertible preferred stock sold subsequent to June 30, 2020 (see Note 10), and the conversion of these preferred shares into an aggregate of 9,447,048 shares of common stock, and (ii) the shares of common stock expected to be sold in, and related proceeds to be received from, the IPO.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)*, as amended, with guidance regarding the accounting for and disclosure of leases. This update requires lessees to recognize the liabilities related to all leases, including operating leases, with a term greater than 12 months on the balance sheet. This update also requires lessees and lessors to disclose key information about their leasing transactions. This guidance will become effective for the Company for annual reporting periods beginning after December 15, 2021 and interim periods within fiscal years beginning after December 15, 2022. The Company is currently evaluating the impact of this standard on its interim condensed consolidated financial statements.

In December 2019, the FASB issued ASU No. 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes* ("ASU 2019-12"), which is intended to simplify various aspects related to accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in ASC 740 and also clarifies and amends existing guidance to improve consistent application. This guidance is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2020, with early adoption permitted. The Company is currently evaluating the impact of this standard on its interim condensed consolidated financial statements.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Note 3—Supplemental Financial Information

Accrued expenses consisted of the following (in thousands):

	December 31, 2019	June 30, 2020
Accrued research and development	$ 7	$ 666
Accrued legal	122	460
Accrued compensation	—	159
Other	21	318
Total accrued expenses	$ 150	$ 1,603

Note 4—Research, Collaboration and License Agreements

UT Southwestern Agreement

On November 19, 2019, the Company entered into a research, collaboration and license agreement ("UT Southwestern Agreement") with the Board of Regents of the University of Texas System ("UT System") on behalf of The University of Southwestern Medical Center ("UT Southwestern"). Under the UT Southwestern Agreement, UT Southwestern is primarily responsible for preclinical development activities with respect to licensed products for use in certain specified indications (up to IND-enabling studies), and the Company is responsible for all subsequent clinical development and commercialization activities with respect to the licensed products. UT Southwestern will conduct such preclinical activities for a two-year period under mutually agreed upon sponsored research agreements that were entered into beginning in April 2020. During the initial research phase, the Company has the right to expand the scope of specified indications under the UT Southwestern Agreement.

In connection with the UT Southwestern Agreement, the Company obtained an exclusive, worldwide, royalty-free license under certain patent rights of UT Southwestern and a non-exclusive, worldwide, royalty-free license under certain know-how of UT Southwestern, in each case to make, have made, use, sell, offer for sale and import licensed products for use in certain specified indications. Additionally, the Company obtained a non-exclusive, worldwide, royalty-free license under certain patents and know-how of UT Southwestern for use in all human uses, with a right of first refusal to obtain an exclusive license under certain of such patent rights and an option to negotiate an exclusive license under other of such patent rights. The Company is required to use commercially reasonable efforts to develop, obtain regulatory approval for, and commercialize at least one licensed product.

On April 2, 2020, the Company amended the UT Southwestern Agreement to include the addition of another licensed product and certain indications, and a right of first refusal to the Company over certain patient dosing patents. No additional consideration was transferred in connection with this amendment.

The UT Southwestern Agreement expires on a country-by-country and licensed product-by-licensed product basis upon the expiration of the last valid claim of a licensed patent in such country for such licensed product. After the initial research term, the Company may terminate the agreement, on an indication-by-indication and licensed product-by-licensed product basis, at any time upon specified written notice to UT Southwestern. Either party may terminate the agreement upon an uncured material breach of the agreement or insolvency of the other party.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

In November 2019, as partial consideration for the license rights granted under the UT Southwestern Agreement, the Company issued 2,000,000 shares of its common stock, or 20% of its then outstanding fully-diluted common stock, to UT Southwestern. As additional consideration, UT Southwestern was entitled to receive additional shares if their holdings fell below 10% on a fully-diluted basis before or as a result of the completion of a qualified financing. In March 2020, following the initial closing of the Series A convertible preferred stock agreement, which met the definition of such qualified financing, the anti-dilution feature expired and no additional shares were issued. The Company does not have any future milestone or royalty obligations to UT Southwestern under the UT Southwestern Agreement other than costs related to maintenance of patents.

The Company also has the right of first refusal for any shares that UT Southwestern wishes to sell that expires upon an initial public offering or change of control of the Company.

Queen's Agreement

In late December 2019, the Company entered into a research grant agreement ("RGA") with Queen's University at Kingston ("Queen's"), for certain research and development activities related to the generation of AAV9 vector. The Company committed to fund $3.8 million under the RGA with Queen's. The Company agreed to issue Queen's a promise-to-pay note whereby any amounts paid directly by Queen's for the manufacture of the vector for use in the funded research activities, to the extent such amounts have not already been funded by the Company to Queen's, become a loan obligation for the Company (the "Note"), subject to an interest rate of 6%. Any amounts outstanding under the Note must be repaid, along with any accrued interest, by or before June 30, 2020. In the event of default, any amount outstanding shall be deemed immediately payable by RA Session II, the Company's President and Chief Executive Officer, as a personal guarantor (see Note 7). For the six months ended June 30, 2020, the Company paid all expenses associated with the Queen's RGA, thus no amounts were due or outstanding under the Note as of June 30, 2020.

On February 21, 2020, the Company entered into a license agreement with Queen's (the "Queen's Agreement") to obtain the exclusive perpetual, royalty-bearing license, with the right to sublicense through multiple tiers, under certain patent rights and know-how of Queen's, including certain improvements to such patent rights and know-how, to develop products in any field which use one or more valid claims of the patents licensed under the Queen's Agreement (the "Licensed Patents"), or the technology, information and intellectual property related to the patents licensed under the Queen's Agreement (the "Licensed Technology" and, together with the Licensed Patents, the "Licensed Products"), and to make, have made, use, sell, offer for sale, import and export Licensed Products and otherwise exploit such patents and know-how for use in certain specified indications. In exchange for the rights granted to the Company, the Company made a cash payment of $3.0 million in April 2020 which is recorded in research and development expenses in the condensed consolidated statement of operations and included as an investing outflow in the statement of cash flows since the acquired license does not have an alternative future use. The Company is obligated to make aggregate cash payments of up to $10.0 million upon the completion of a combination of regulatory milestones and up to $10.0 million upon the completion of a combination of commercial milestones. In further consideration of the rights granted, beginning with the Company's first commercial sale of the Licensed Products, the Company will also pay an annual earned royalty in the low single digits on net sales of Licensed Products, subject to certain customary reductions, and a percentage of non-royalty sublicensing revenue ranging in the low double digits. Royalties are payable, on a Licensed Products-by-Licensed Products and a country-by-country basis, until expiration of the last valid claim of a Licensed Patent covering such Licensed Products in such country and the expiration of any regulatory exclusivity for such Licensed Products in such country.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Note 5—Convertible Preferred Stock, Tranche Liability and Stockholders' Deficit

Series A convertible preferred stock

At June 30, 2020, convertible preferred stock consisted of the following (in thousands, except per share amounts):

	Shares Authorized	Shares Issued and Outstanding	Issuance Price per Share	Carrying Value	Liquidation Preference
Series A	10,000	6,200	$ 3.00	$18,014	$ 18,600
Total	10,000	6,200		$18,014	$ 18,600

On March 4, 2020, the Company entered into a purchase agreement (the "Series A Purchase Agreement") for a private placement of up to 10,000,000 shares of Series A convertible preferred stock at an original issuance price of $3.00 per share, subject to separate closings, including: (1) 6,000,000 shares at the initial closing on March 4, 2020, and (2) 2,000,000 shares at each of two subsequent closings triggered by the achievement of specific clinical milestones. The Series A Purchase Agreement obligated the Company to issue and sell and the Series A investors to purchase up to a total of 4,000,000 additional shares of Series A convertible preferred stock (the "Milestone Shares") at the same price per share upon the achievement of certain defined clinical milestones (the "tranche liability"). The determination as to whether the milestone events had been met was subject to certification by the Board of Directors. Each Series A investor had the right, but not the obligation, to purchase all or any portion of the Milestone Shares at any time in its sole option and in its sole and absolute discretion, whether or not the Company had achieved the applicable clinical milestone.

On June 30, 2020, several affiliated Series A investors elected to exercise in full their options to purchase their pro-rata portion of the Milestone Shares prior to the Company's achievement of the clinical milestones for gross proceeds of $0.6 million. The remainder of the Series A investors exercised in full their options to purchase their remaining pro-rata portion of the Milestone Shares prior to the Company's achievement of the clinical milestones for gross proceeds of $11.4 million between July 1, 2020 and July 2, 2020.

As of June 30, 2020, 6,200,000 shares of the Series A convertible preferred stock were issued and outstanding. These shares were issued in exchange for gross proceeds of $18.6 million, net of issuance costs of approximately $0.4 million.

Dividends

The holders of Series A convertible preferred stock are not entitled to receive dividends unless declared by the Board of Directors in accordance with the Company's certificate of incorporation, as amended from time to time. No dividends have been declared since inception.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Liquidation preference

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company, holders of Series A convertible preferred stock are entitled to receive, prior and in preference to holders of common stock, an amount equal to the original issuance price of $3.00 per share (subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A convertible preferred stock) plus any declared but unpaid dividends thereon. If upon occurrence of such an event, the assets and funds to be distributed among the holders of Series A convertible preferred stock are insufficient to permit full payment to such holders, the entire assets and funds of the Company legally available for distribution will be distributed ratably among the holders of the Series A convertible preferred stock. All remaining legally available assets of the Company are to be distributed pro rata to the holders of Series A convertible preferred stock and common stock, on an as-converted basis.

Conversion rights

Shares of Series A convertible preferred stock are convertible into such number of fully paid and non-assessable shares of common stock as determined by dividing the Series A convertible preferred stock original issuance price by the conversion price then in effect. The original conversion price per share of the Series A convertible preferred stock is $3.00, subject to adjustment in the event of certain dilutive issuances. The Series A convertible preferred stock original issuance price and conversion price are each subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A convertible preferred stock.

Each share of Series A convertible preferred stock is convertible at any time at the option of the holder at the conversion ratio then in effect. In addition, each share of Series A convertible preferred stock will be automatically converted into common stock at the conversion ratio then in effect upon either (a) the consummation of an underwritten public offering resulting in gross proceeds to the Company of at least $50 million at a price per share of at least $15.00 (subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A convertible preferred stock), or (b) the date and time, or the occurrence of an event, specified by the vote or written consent of the holders of a majority of the then outstanding shares of Series A convertible preferred stock.

Voting rights

Holders of Series A convertible preferred stock are entitled to vote as a single class together with the holders of common stock and have one vote for each share of common stock into which the Series A convertible preferred stock is convertible.

The holders of Series A convertible preferred stock are entitled to elect two directors to the Board of Directors. The holders of Series A convertible preferred stock and the holders of common stock, each voting as a separate class, are entitled to jointly elect one director to the Board of Directors. The holders of common stock, exclusively and as a separate class, are entitled to elect one director to the Board of Directors. The Company has concluded that the holders of its Series A convertible preferred stock control the Board of Directors.

A majority of the outstanding shares of Series A convertible preferred stock is necessary for approving certain protective provisions in the Company's amended and restated certificate of incorporation, including the ability to either increase or decrease the authorized number of directors constituting the Board of Directors.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Series A convertible preferred stock tranche liability

The Company concluded that the tranche liability met the definition of a freestanding financial instrument, as it was legally detachable and separately exercisable from the initial closing of the Series A convertible preferred stock.

The estimated fair value of the tranche liability was determined using a Monte Carlo simulation at the initial issuance date. As of March 4, 2020, the simulations occurred based on the implied aggregate equity value of the Company derived from the Series A convertible preferred stock offering price of $3.00 per share, along with, in part, the following subjective assumptions: risk-free rate of 0.59%, an expected volatility of 80%, the expected term to a liquidity event of 1 year, and a 60% probability of achieving the clinical milestones and timing thereof. Subsequently, the estimated fair value of the tranche liability was determined using a backsolve approach at June 30, 2020, which was calculated based on the aggregate equity value of the Company derived from the Series B convertible preferred stock offering price of $17.00 per share. The subsequent remeasurement also considered, in part, a risk-free rate of 0.17%, an expected volatility of 80%, and the expected term to a liquidity event of 0.5 years.

Based on the analysis, the Company recorded a preferred stock tranche liability of $1.1 million at the issue date to account for the obligation to issue the Milestone Shares at a predetermined fixed price at a future settlement date.

At June 30, 2020, ahead of the anticipated closing of the Series B Purchase Agreement for $17.00 per share that occurred on July 2, 2020 (see Note 10), certain investors elected to exercise in full their options to purchase their pro-rata portion of the Milestone Shares prior to the Company's achievement of the clinical milestones and purchased 200,000 shares of Series A convertible preferred stock. The Company remeasured the fair value of the entire tranche liability at June 30, 2020, and recognized a non-cash expense of $17.0 million in the condensed consolidated statement of operations. As 200,000 of the Milestone Shares were also issued to certain investors on June 30, 2020, the related tranche liability was extinguished, and the Company reclassified $0.9 million to convertible preferred stock on the condensed consolidated balance sheet. The Company concluded that no beneficial conversion feature ("BCF") existed as the effective conversion price of the Series A convertible preferred stock exceeded the fair value of the Company's common stock at each of the commitment dates. Specifically at the commitment date of June 30, 2020, when 200,000 Milestone Shares were issued, the deemed proceeds were equal to the cash proceeds received for the shares of Series A convertible preferred stock and the fair value of the tranche liability that related to the Milestone Shares, or $7.52 per share. As the effective conversion price exceeded the fair value of the Company's common stock, no BCF existed.

The following table provides a reconciliation of the preferred stock tranche liability measured at fair value using Level 3 significant unobservable inputs (dollars in thousands):

Balance at January 1, 2020	$ —
Fair value at issuance of Series A convertible preferred stock (March 2020)	1,050
Change in fair value	17,030
Settlement of preferred stock tranche liability due to partial issuance of Milestone Shares	(904)
Balance at June 30, 2020	$ 17,176
Shares of common stock reserved at June 30, 2020 for issuance of the remaining Milestone Shares	3,800,000

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

Authorized shares and equity awards

The Company amended its certificate of incorporation on March 4, 2020, July 2, 2020 and again on July 28, 2020 such that the total number of shares of common stock authorized to be issued was increased to 30,000,000, and the total number of shares of preferred stock authorized to be issued was increased to 15,647,052, of which 10,000,000 preferred shares are designated Series A convertible preferred stock and 5,647,052 are designated Series B convertible preferred stock.

The Company did not grant any equity-based awards during the six months ended June 30, 2020.

Note 6—Net Loss Per Common Share

Basic net loss per common share is computed by dividing net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding for the period. Diluted net loss per common share excludes the potential impact of the Company's convertible preferred stock because their effect would be anti-dilutive due to the Company's net loss. Since the Company had a net loss in the period presented, basic and diluted net loss per common share are the same.

The table below provides potentially dilutive securities not included in the calculation of the diluted net loss per common share because to do so would be anti-dilutive:

	For the Six Months Ended June 30, 2020
Shares issuable upon conversion of Series A convertible preferred stock	6,200,000
Total	6,200,000

Unaudited Pro Forma Net Loss Per Common Share

The following table represents the calculation of basic and diluted pro forma net loss per common share for the six months ended June 30, 2020 (dollars in thousands, except share and per share data):

	Pro Forma for the Six Months Ended June 30, 2020 (unaudited)
Net loss	$ (26,651)
Weighted-average shares of common stock outstanding used to compute net loss per common share, basic and diluted	10,000,000
Pro forma adjustments to reflect conversion of convertible preferred stock	3,924,176
Weighted-average shares to compute pro forma net loss per common share, basic and diluted	13,924,176
Pro forma net loss common share, basic and diluted	$ (1.91)

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

For the six months ended June 30, 2020, basic and diluted pro forma net loss per common share were the same, as there were no potentially dilutive securities.

Note 7—Related Party Transactions

RA Session II, President and Chief Executive Officer and a member of the Company's board of directors, is a guarantor under the Guaranty and Security Agreement between himself, Queen's and the Company, and in the event of the Company's failure to fund its obligations under the RGA with Queen's, has personally guaranteed payments due by the Company to Queen's.

In January 2020, the Company entered into two secured promissory notes with a related party, its President and Chief Executive Officer, RA Session II, for an aggregate of $1.67 million, with 10% interest. The Company secured the notes with a first priority security interest in certain assets of the Company. During March 2020, the Company repaid $1.65 million of the notes, and the remaining balance of approximately $28,000, which is included in due to related party in the condensed consolidated balance sheet, was repaid in July 2020. As a result, Mr. Session released his security interest in the collateral.

In March 2020, the Company entered into a services agreement with PBM Capital Group, LLC ("PBM"), an affiliate of PBM TGT Holdings, LLC (the lead investor in the Company's convertible Series A preferred stock financing discussed below, and who has the ability to designate two members of the Company's board of directors), whereby PBM provides accounting and other administrative and management services related to payroll administration, human resources, bookkeeping, preparation of financial statements and tax returns, accounts payable and receivable, and other similar functions for a fee of $2,500 per month. As of June 30, 2020, inclusive of certain pass-through direct costs, the Company has approximately $33,000 due to PBM, which is included in due to related party in the condensed consolidated balance sheet.

As of June 30, 2020, 4,900,000 and 600,000 shares of the Series A convertible preferred stock were issued to PBM TGT Holdings, LLC and Nolan Capital, LLC, respectively, which are controlled by members of the Company's board of directors.

Note 8—Income Taxes

Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on its history of operating losses, the Company believes that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for its deferred tax assets as of June 30, 2020 and December 31, 2019.

On March 27, 2020, the U.S. President signed into law the CARES Act, an economic relief package in response to the COVID-19 global pandemic. The CARES Act contains several corporate income tax provisions, including making remaining alternative minimum tax credits immediately refundable; providing a 5-year carryback of net operating loss carryforwards ("NOLs") generated in tax years 2018, 2019, and 2020, and removing the 80% taxable income limitation on utilization of those NOLs if carried back to prior tax years or utilized in tax years beginning before 2021; and temporarily

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liberalizing the interest deductibility rules under Section 163(j) of the Tax Cuts and Jobs Act, by raising the adjusted taxable income limitation from 30% to 50% for tax years 2019 and 2020 and giving taxpayers the election of using 2019 adjusted taxable income for purposes of computing 2020 interest deductibility. The Company is still evaluating the impact but does not currently expect the provisions of the CARES Act to have a material effect on the realizability of deferred income tax assets or tax expense.

As of June 30, 2020, there were no material changes to either the nature or the amounts of the uncertain tax positions previously determined for the period from September 20, 2019 (date of inception) through December 31, 2019.

Note 9—Commitments and Contingencies

Litigation

The Company is not a party to any material legal proceedings and is not aware of any pending or threatened claims. From time to time, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities.

Commitments

As of June 30, 2020, the Company was not a party to any leasing agreements.

In the normal course of business, the Company enters into contracts that contain a variety of indemnifications with its employees, licensors, suppliers and service providers. The Company's maximum exposure under these arrangements is unknown at June 30, 2020. The Company does not anticipate recognizing any significant losses relating to these arrangements.

Note 10—Subsequent Events

The Company has evaluated subsequent events through September 2, 2020, the date that these interim condensed consolidated financial statements were issued. Except for the matters disclosed below, no additional subsequent events had occurred that would require recognition or disclosure in these interim condensed consolidated financial statements.

License Agreement

In August 2020, the Company entered into license and inventory purchase agreements with Abeona Therapeutics Inc. ("Abeona") for worldwide exclusive rights to certain intellectual property rights and know-how relating to the research, development and manufacture of ABO-202, an AAV-based gene therapy for CLN1 disease (also known as infantile Batten disease). Under the terms of the agreements, the Company will make initial cash payments to Abeona of $7.0 million, and up to $26.0 million in regulatory-related milestones and up to $30.0 million in sales-related milestones per licensed product. The Company will also pay an annual earned royalty in the high single digits on net sales of any licensed products for CLN1.

Convertible Preferred Stock Financing Arrangements

In July 2020, the remaining investors in the Series A convertible preferred stock exercised in full their option to purchase their pro-rata portion of the Milestone Shares prior to the achievement of such milestones, and the Company issued and sold to such investors an aggregate of 3,800,000 shares of Series A convertible preferred stock at a purchase price of $3.00 per share for aggregate gross proceeds of $11.4 million. As part of this issuance, the Company issued and sold 3,266,667 shares to PBM TGT Holdings, LLC and 400,000 shares to Nolan Capital, LLC which are controlled by members of the Company's board of directors.

Taysha Gene Therapies, Inc.
Notes to unaudited condensed consolidated financial statements

In July 2020, the Company entered into a purchase agreement (the “Series B Purchase Agreement”) for a private placement of up to 5,647,052 shares of Series B convertible preferred stock. In August 2020, at the close of the Series B convertible preferred stock offering, the Company had sold 5,647,048 of Series B convertible preferred stock at a price of $17.00 per share in multiple closings for gross proceeds of $96.0 million. The majority of investors that participated in the Series B Purchase Agreement were new investors.

The holders of Series B convertible preferred stock have similar voting, dividend and conversion rights as the holders of Series A convertible preferred stock, except that the original issuance price of the Series B convertible preferred stock (subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B convertible preferred stock) is $17.00 per share. The Series B convertible preferred stock is not redeemable except in the event of a Deemed Liquidation Event.

In addition, each share of Series A and Series B convertible preferred stock will be automatically converted into common stock at the conversion ratio then in effect upon either (a) the consummation of an underwritten public offering resulting in gross proceeds to the Company of at least $50 million at a price per share of at least $17.00 (subject to adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A convertible preferred stock), including the approval of at least one Series A Director, or (b) the date and time, or the occurrence of an event, specified by the vote or written consent of (i) the holders of a majority of the then outstanding shares of Series A and Series B convertible preferred stock, and (ii) a majority of the then outstanding shares of Series B convertible preferred stock.

Equity Incentive Plan

On July 1, 2020, the Company’s board of directors approved the 2020 Equity Incentive Plan (“2020 Plan”) which permits the granting of incentive stock options, non-statutory stock options, stock appreciation rights, restricted stock, restricted stock units and other stock-based awards to employees, directors, officers and consultants. On July 1, 2020, 3,529,412 shares of common stock were authorized for issuance under the 2020 Plan.

On July 1, 2020, options to purchase 2,658,822 shares of common stock under the 2020 Plan were awarded to certain employees and consultants of the Company with an exercise price per share of $0.87, all of which were subsequently cancelled. In exchange, the Company awarded 2,312,014 restricted stock units on September 2, 2020, which are expected to vest over a four-year term. The estimated fair value of these restricted stock units at the grant date was $16.23 per share. Such shares are not accounted for as outstanding until they vest. RA Session II was also awarded 705,882 restricted stock awards under the 2020 Plan on July 1, 2020, which are expected to vest over a three-year term. The fair value of these restricted stock awards at the grant date of July 1, 2020 was $5.75 per share.

On September 2, 2020, options to purchase 15,000 shares of common stock under the 2020 Plan were awarded to certain directors of the Company with an exercise price per share of $16.23, which are generally expected to vest over a four-year term and expire in ten years. On this date, the Company also granted an additional 303,921 restricted stock units with a grant date fair value of $16.23 per share.

Shares

Common Stock

Goldman Sachs & Co. LLC **Morgan Stanley** **Jefferies**

Chardan

Through and including , 2020 (the 25th day after the commencement of this offering), all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

PART II

INFORMATION NOT REQUIRED IN PROSPECTUS

Item 13. Other Expenses of Issuance and Distribution.

The following table indicates the expenses to be incurred in connection with the offering described in this registration statement, other than underwriting discounts and commissions, all of which will be paid by us. All amounts are estimated except the Securities and Exchange Commission, or SEC, registration fee, the Financial Industry Regulatory Authority, Inc., or FINRA, filing fee and the Nasdaq Global Market initial listing fee.

	Amount
SEC registration fee	$ 12,980
FINRA filing fee	15,500
Nasdaq Global Market initial listing fee	225,000
Accountants' fees and expenses	*
Legal fees and expenses	*
Blue sky fees and expenses	*
Transfer agent's fees and expenses	*
Printing and engraving expenses	*
Miscellaneous	*
Total expenses	$ *

* To be provided by amendment

Item 14. Indemnification of Directors and Officers.

We are incorporated under the laws of the State of Delaware. Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of directors of a corporation to the corporation or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or such other court shall deem proper.

As permitted by the Delaware General Corporation Law, our amended and restated certificate of incorporation and bylaws to be in effect upon the closing of this offering will provide that: (i) we are required to indemnify our directors to the fullest extent permitted by the Delaware General Corporation

Law; (ii) we may, in our discretion, indemnify our officers, employees and agents as set forth in the Delaware General Corporation Law; (iii) we are required, upon satisfaction of certain conditions, to advance all expenses incurred by our directors in connection with certain legal proceedings; (iv) the rights conferred in the bylaws are not exclusive; and (v) we are authorized to enter into indemnification agreements with our directors, officers, employees and agents.

In connection with this offering, we expect to enter into indemnification agreements with each of our directors and executive officers that require us to indemnify them against expenses, judgments, fines, settlements and other amounts that any such person becomes legally obligated to pay (including with respect to a derivative action) in connection with any proceeding, whether actual or threatened, to which such person may be made a party by reason of the fact that such person is or was a director or officer of us or any of our affiliates, provided such person acted in good faith and in a manner such person reasonably believed to be in, or not opposed to, our best interests. The indemnification agreements will also set forth certain procedures that will apply in the event of a claim for indemnification thereunder. We intend to enter into similar indemnification agreements with our executive officers prior to the completion of this offering. At present, no litigation or proceeding is pending that involves any of our directors or officers regarding which indemnification is sought, nor are we aware of any threatened litigation that may result in claims for indemnification.

We maintain a directors' and officers' liability insurance policy. The policy insures directors and officers against unindemnified losses arising from certain wrongful acts in their capacities as directors and officers and reimburses us for those losses for which we have lawfully indemnified the directors and officers. The policy contains various exclusions.

In addition, the underwriting agreement filed as Exhibit 1.1 to this Registration Statement provides for indemnification by the underwriters of us and our officers and directors for certain liabilities arising under the Securities Act, or otherwise. Our amended and restated investor rights agreement with certain investors also provides for cross-indemnification in connection with the registration of our common stock on behalf of such investors.

Item 15. Recent Sales of Unregistered Securities.

The following list sets forth information regarding all unregistered securities sold by us since our inception through the date of the prospectus that forms a part of this registration statement.

Issuances of Common Stock

In September 2019, we issued an aggregate of 8,000,000 shares of our common stock to two accredited investors at a purchase price of $0.00001 per share, for aggregate consideration of $80.00.

In November 2019, we issued 2,000,000 shares of our common stock to one investor as consideration for entering into a research, collaboration and license agreement.

Issuances of Preferred Stock

In March 2020, we issued an aggregate of 6,000,000 shares of our Series A convertible preferred stock to nine investors at a purchase price of $3.00 per share, for aggregate consideration of $18.0 million.

In June 2020, we issued an aggregate of 200,000 shares of our Series A convertible preferred stock to six investors at a purchase price of $3.00 per share, for aggregate consideration of $0.6 million.

In July 2020, we issued an aggregate of 3,800,000 shares of our Series A convertible preferred stock to three investors at a purchase price of $3.00 per share, for aggregate consideration of $11.4 million.

In July and August 2020, we issued an aggregate of 5,647,048 shares of our Series B convertible preferred stock to 31 investors at a purchase price of $17.00 per share, for aggregate consideration of $96.0 million.

Issuances Pursuant to our Equity Plans

From September 20, 2019 (the date of our inception) through the date of this registration statement, we granted options under our 2020 Equity Incentive Plan to purchase an aggregate of 15,000 shares of common stock, at an exercise price of $16.23 per share, to certain of our employees, directors and consultants. Of these, no shares have been issued upon the exercise of options. We have also granted restricted stock unit awards with respect to 2,615,935 shares of common stock and restricted stock awards with respect to 705,882 shares of common stock under our 2020 Equity Incentive Plan during the same time period.

None of the foregoing transactions involved any underwriters, underwriting discounts or commissions, or any public offering. Unless otherwise specified above, we believe these transactions were exempt from registration under the Securities Act in reliance on Section 4(a)(2) of the Securities Act (and Regulation D promulgated thereunder) or Rule 701 promulgated under Section 3(b) of the Securities Act as transactions by an issuer not involving any public offering or under benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of the securities in each of these transactions represented their intentions to acquire the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were placed on the share certificates issued in these transactions. All recipients had adequate access, through their relationships with us, to information about us. The sales of these securities were made without any general solicitation or advertising.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits.

The exhibits listed below are filed as part of this registration.

Exhibit Number	Description of Exhibit
1.1*	Form of Underwriting Agreement
3.1	Amended and Restated Certificate of Incorporation of the Registrant (as amended and currently in effect)
3.2	Amended and Restated Bylaws of the Registrant (currently in effect)
3.3*	Form of Amended and Restated Certificate of Incorporation of the Registrant (to be effective upon the closing of this offering)
3.4*	Form of Amended and Restated Bylaws of the Registrant (to be effective upon the closing of this offering)
4.1	Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated July 2, 2020
5.1*	Opinion of Cooley LLP
10.1†	Research, Collaboration & License Agreement, by and between the Registrant and The Board of Regents of the University of Texas System on behalf of The University of Texas Southwestern Medical Center, dated as of November 19, 2019
10.2†	Amendment to Research, Collaboration & License Agreement, by and between the Registrant and The Board of Regents of the University of Texas System on behalf of The University of Texas Southwestern Medical Center, dated as of April 2, 2020
10.3†	License Agreement, by and between the Registrant and Queen's University at Kingston, dated as of February 21, 2020

Exhibit Number	Description of Exhibit
10.4†#	License Agreement, by and between the Registrant and Abeona Therapeutics Inc., dated as of August 14, 2020
10.5+	2020 Equity Incentive Plan and Forms of Option Grant Notice and Agreement, Exercise Notice, Early Exercise Notice and Restricted Stock Award Notice
10.6+*	2020 Stock Incentive Plan and Forms of Stock Option Grant Notice, Stock Option Agreement, Restricted Stock Unit Grant Notice and Restricted Stock Unit Award Agreement
10.7+*	2020 Employee Stock Purchase Plan
10.8+*	Form of Indemnification Agreement with Executive Officers and Directors
10.9+	Executive Employment Agreement, by and between the Registrant and RA Session II, dated as of April 1, 2020
23.1	Consent of Deloitte & Touche LLP, independent registered public accounting firm
23.2*	Consent of Cooley LLP (included in Exhibit 5.1)
24.1	Power of Attorney (included on signature page)

+ Indicates management contract or compensatory plan.
† Portions of this agreement (indicated by asterisks) have been omitted because the registrant has determined they are not material and would likely cause competitive harm to the registrant if publicly disclosed.
# Certain schedules to this agreement have been omitted in accordance with Item 601(b)(2) of Regulation S-K. A copy of any omitted schedules will be furnished supplementally to the SEC upon request.
* To be filed by amendment.

(b) Financial Statement Schedules.

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. Undertakings.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized in the City of Dallas, State of Texas, on this 2nd day of September, 2020.

TAYSHA GENE THERAPIES, INC.

By: /s/ RA Session II
RA Session II
President and Chief Executive Officer

KNOW ALL BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints RA Session II and Kamran Alam, and each of them, as his true and lawful agents, proxies and attorneys-in-fact, with full power of substitution and resubstitution, for him or her and in his name, place and stead, in any and all capacities, to (i) act on, sign and file with the Securities and Exchange Commission any and all amendments (including post-effective amendments) to this registration statement together with all schedules and exhibits thereto and any subsequent registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, together with all schedules and exhibits thereto, (ii) act on, sign and file such certificates, instruments, agreements and other documents as may be necessary or appropriate in connection therewith, (iii) act on and file any supplement to any prospectus included in this registration statement or any such amendment or any subsequent registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, and (iv) take any and all actions which may be necessary or appropriate to be done, as fully for all intents and purposes as he might or could do in person, hereby approving, ratifying and confirming all that such agent, proxy and attorney-in-fact or any of his substitutes may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Act of 1933, this registration statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
/s/ RA Session II RA Session II	President, Chief Executive Officer and Director *(Principal Executive Officer)*	September 2, 2020
/s/ Kamran Alam Kamran Alam	Chief Financial Officer *(Principal Financial and Accounting Officer)*	September 2, 2020
/s/ Phillip B. Donenberg Phillip B. Donenberg	Director	September 2, 2020
/s/ Sean P. Nolan Sean P. Nolan	Director	September 2, 2020
/s/ Paul B. Manning Paul B. Manning	Director	September 2, 2020
/s/ Sukumar Nagendran, M.D. Sukumar Nagendran, M.D.	Director	September 2, 2020

Exhibit 3.1

AMENDED AND RESTATED
CERTIFICATE OF INCORPORATION
OF
TAYSHA GENE THERAPIES, INC.

(Pursuant to Sections 242 and 245 of the
General Corporation Law of the State of Delaware)

Taysha Gene Therapies, Inc., a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware (the "**General Corporation Law**"),

DOES HEREBY CERTIFY:

1. That the name of this corporation is Taysha Gene Therapies, Inc., and that this corporation was originally incorporated in Texas pursuant to Texas Business Organization Code.

2. That this corporation subsequently converted to a corporation incorporated under the General Corporation Law on February 13, 2020 under the name Taysha Gene Therapies, Inc.

3. That the Board of Directors duly adopted resolutions proposing to amend and restate the Certificate of Incorporation of this corporation, declaring said amendment and restatement to be advisable and in the best interests of this corporation and its stockholders, and authorizing the appropriate officers of this corporation to solicit the consent of the stockholders therefor, which resolution setting forth the proposed amendment and restatement is as follows:

RESOLVED, that the Certificate of Incorporation of this corporation be amended and restated in its entirety to read as follows:

FIRST: The name of this corporation is Taysha Gene Therapies, Inc. (the "**Corporation**").

SECOND: The address of the registered office of the Corporation in the State of Delaware is 1675 South State Street, Suite B, in the City of Dover, County of Kent, Delaware 19901. The name of its registered agent at such address is Capitol Services, Inc.

THIRD: The nature of the business or purposes to be conducted or promoted is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law.

FOURTH: The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 30,000,000 shares of Common Stock, $0.00001 par value per share ("**Common Stock**") and (ii) 14,411,764 shares of Preferred Stock, $0.00001 par value per share ("**Preferred Stock**").

The following is a statement of the designations and the powers, privileges and rights, and the qualifications, limitations or restrictions thereof in respect of each class of capital stock of the Corporation.

A. COMMON STOCK

1. General. The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth herein.

2. Voting. The holders of the Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders (and written actions in lieu of meetings). There shall be no cumulative voting. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by (in addition to any vote of the holders of one or more series of Preferred Stock that may be required by the terms of this Amended and Restated Certificate of Incorporation (this "**Restated Certificate**")) the affirmative vote of the holders of shares of capital stock of the Corporation representing a majority of the votes represented by all outstanding shares of capital stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law.

B. PREFERRED STOCK

Preferred Stock may be issued from time to time in one or more series, each of such series to consist of such number of shares and to have such terms, rights, powers and preferences, and the qualifications and limitations with respect thereto, as stated or expressed herein. Of the 14,411,764 shares of Preferred Stock that the Corporation has the authority to issue (i) 10,000,000 shares are hereby designated "**Series A Preferred Stock**" and (ii) 4,411,764 shares are hereby designated "**Series B Preferred Stock**" with the following rights, preferences, powers, privileges and restrictions, qualifications and limitations. Unless otherwise indicated, references to "sections" or "subsections" in this Part B of this Article Fourth refer to sections and subsections of Part B of this Article Fourth.

1. Dividends. The Corporation shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Corporation (other than dividends on shares of Common Stock payable in shares of Common Stock) unless (in addition to the obtaining of any consents required elsewhere in this Restated Certificate) the holders of the Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Preferred Stock in an amount at least equal to (i) in the case of a dividend on Common Stock or any class or series that is convertible into Common Stock, that dividend per share of Preferred Stock as would equal the product of (A) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into Common Stock and (B) the number of shares of Common Stock issuable upon conversion of a share of Preferred Stock, in each case calculated on the record date for determination of holders entitled to receive such dividend or (ii) in the case of a dividend on any class or series that is not convertible into Common Stock, at a rate per share of Preferred Stock determined by (A) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to such class or series) and (B) multiplying such fraction by an amount equal to the applicable Original Issue Price (as defined below); provided that, if the Corporation declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Corporation, the dividend payable to the holders of Preferred

Stock pursuant to this Section 1 shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest Preferred Stock dividend. The "**Series A Original Issue Price**" shall mean $3.00 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A Preferred Stock. The "**Series B Original Issue Price**" shall mean $17.00 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B Preferred Stock. The applicable "**Original Issue Price**" shall mean the Series A Original Issue Price in the case of the Series A Preferred Stock and the Series B Original Issue Price in the case of the Series B Preferred Stock.

2. Liquidation, Dissolution or Winding Up; Certain Mergers, Consolidations and Asset Sales.

2.1 Preferential Payments to Holders of Series B Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation, the holders of shares of Series B Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders and in the event of a Deemed Liquidation Event (as defined below), the holders of shares of Series B Preferred Stock then outstanding shall be entitled to be paid out of the consideration payable to stockholders in such Deemed Liquidation Event or out of the Available Proceeds (as defined below), as applicable, before any payment shall be made to the holders of Series A Preferred Stock or Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (i) the Series B Original Issue Price, plus any dividends declared but unpaid on each share of Series B Preferred Stock, and (ii) such amount per share as would have been payable had all shares of Series B Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (the amount payable pursuant to this sentence is hereinafter referred to as the "**Series B Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series B Preferred Stock the full amount to which they shall be entitled under this Section 2.1, the holders of shares of Series B Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.2 Preferential Payments to Holders of Series A Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation, after payment in full of all preferential amounts required to be paid to the holders of Series B Preferred Stock under Section 2.1, the holders of shares of Series A Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders and in the event of a Deemed Liquidation Event, the holders of shares of Series A Preferred Stock then outstanding shall be entitled to be paid out of the consideration payable to stockholders in such Deemed Liquidation Event or out of the Available Proceeds, as applicable, before any payment shall be made to the holders of Common Stock by reason of their ownership thereof, an amount per share equal to the greater of (i) the Series A Original Issue Price, plus any dividends declared but unpaid on each share of Series A Preferred Stock, and (ii) such amount per share as would have been payable had all shares of Series A Preferred Stock been converted into Common Stock pursuant to Section 4 immediately prior to the liquidation, dissolution, winding up

or Deemed Liquidation Event (the amount payable pursuant to this sentence is hereinafter referred to as the "**Series A Liquidation Amount**"). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Series A Preferred Stock the full amount to which they shall be entitled under this Section 2.2, the holders of shares of Series A Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.3 Payments to Holders of Common Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation, after the payment in full of all Series B Liquidation Amounts required to be paid to the holders of shares of Series B Preferred Stock and all Series A Liquidation Amounts required to be paid to the holders of shares of Series A Preferred Stock, the remaining assets of the Corporation available for distribution to its stockholders or, in the case of a Deemed Liquidation Event, the consideration not payable to the holders of shares of Preferred Stock pursuant to Sections 2.1 and 2.2 or the remaining Available Proceeds, as the case may be, shall be distributed among the holders of shares of Common Stock, pro rata based on the number of shares held by each such holder.

2.4 Deemed Liquidation Events.

2.4.1 Definition. Each of the following events shall be considered a "**Deemed Liquidation Event**" unless (i) the holders of at least a majority of the outstanding shares of Preferred Stock, voting together as a single class on an as-converted basis (the "**Requisite Holders**"), and (ii) the holders of at least a majority of the outstanding shares of Series B Preferred Stock, voting as a separate class (the "**Requisite Series B Holders**"), elect otherwise by written notice sent to the Corporation at least fifteen (15) days prior to the effective date of any such event:

(a) a merger or consolidation in which

(i) the Corporation is a constituent party or

(ii) a subsidiary of the Corporation is a constituent party and the Corporation issues shares of its capital stock pursuant to such merger or consolidation,

except any such merger or consolidation involving the Corporation or a subsidiary in which the shares of capital stock of the Corporation outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority, by voting power, of the capital stock of (1) the surviving or resulting corporation; or (2) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or

(b) (1) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Corporation or any subsidiary of the Corporation of all or substantially all the business or assets of the Corporation

and its subsidiaries taken as a whole, or (2) the sale or disposition (whether by merger, consolidation or otherwise, and whether in a single transaction or a series of related transactions) of one or more subsidiaries of the Corporation if substantially all of the assets of the Corporation and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Corporation.

2.4.2 Effecting a Deemed Liquidation Event.

(a) The Corporation shall not have the power to effect a Deemed Liquidation Event referred to in Section 2.4.1(a)(i) unless the agreement or plan of merger or consolidation for such transaction (the "**Merger Agreement**") provides that the consideration payable to the stockholders of the Corporation in such Deemed Liquidation Event shall be paid to the holders of capital stock of the Corporation in accordance with Sections 2.1, 2.2 and 2.3.

(b) In the event of a Deemed Liquidation Event referred to in Section 2.4.1(a)(ii) or 2.4.1(b), if the Corporation does not effect a dissolution of the Corporation under the General Corporation Law within ninety (90) days after such Deemed Liquidation Event, then (i) the Corporation shall send a written notice to each holder of Preferred Stock no later than the ninetieth (90th) day after the Deemed Liquidation Event advising such holders of their right (and the requirements to be met to secure such right) pursuant to the terms of the following clause (ii) to require the redemption of such shares of Preferred Stock, and (ii) if the Requisite Holders so request in a written instrument delivered to the Corporation not later than one hundred twenty (120) days after such Deemed Liquidation Event, the Corporation shall use the consideration received by the Corporation for such Deemed Liquidation Event (net of any retained liabilities associated with the assets sold or technology licensed, as determined in good faith by the Board of Directors of the Corporation (the "**Board of Directors**"))**,** together with any other assets of the Corporation available for distribution to its stockholders, all to the extent permitted by Delaware law governing distributions to stockholders (the "**Available Proceeds**"), on the one hundred fiftieth (150th) day after such Deemed Liquidation Event, to redeem all outstanding shares of Preferred Stock at a price per share equal to the Series B Liquidation Amount or Series A Liquidation amount, as applicable. Notwithstanding the foregoing, in the event of a redemption pursuant to the preceding sentence, if the Available Proceeds are not sufficient to redeem all outstanding shares of Preferred Stock, the Corporation shall redeem each holder's shares of Preferred Stock in accordance with the liquidation preferences set forth in Sections 2.1 and 2.2 to the fullest extent of such Available Proceeds, and shall redeem the remaining shares in accordance with the liquidation preferences set forth in Sections 2.1 and 2.2 as soon as it may lawfully do so under Delaware law governing distributions to stockholders. Prior to the distribution or redemption provided for in this Section 2.4.2(b), the Corporation shall not expend or dissipate the consideration received for such Deemed Liquidation Event, except to discharge expenses incurred in connection with such Deemed Liquidation Event. On or before the applicable redemption date, each holder of shares of Preferred Stock to be redeemed shall, if a holder of shares in certificated form, surrender the certificate or certificates representing such shares (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation, in the manner and at the place designated by the Corporation, and thereupon the Available Proceeds allocable to such shares shall be payable to the

order of the person whose name appears on such certificate or certificates as the owner thereof. If on the applicable redemption date the Available Proceeds payable upon redemption of the shares of Preferred Stock to be redeemed on such date is paid or tendered for payment or deposited with an independent payment agent so as to be available therefor in a timely manner, then notwithstanding that any certificates evidencing any of the shares of Preferred Stock so redeemed shall not have been surrendered, all rights with respect to such shares shall forthwith after the redemption date terminate, except only the right of the holders to receive their allocable share of the Available Proceeds without interest upon surrender of any such certificate or certificates therefor.

2.4.3 Amount Deemed Paid or Distributed. The amount deemed paid or distributed to the holders of capital stock of the Corporation upon any such merger, consolidation, sale, transfer, exclusive license, other disposition or redemption shall be the cash or the value of the property, rights or securities to be paid or distributed to such holders pursuant to such Deemed Liquidation Event. The value of such property, rights or securities shall be determined in good faith by the Board of Directors.

2.4.4 Allocation of Escrow and Contingent Consideration. In the event of a Deemed Liquidation Event pursuant to Section 2.4.1(a)(i), if any portion of the consideration payable to the stockholders of the Corporation is payable only upon satisfaction of contingencies (the "**Additional Consideration**"), the Merger Agreement shall provide that (a) the portion of such consideration that is not Additional Consideration (such portion, the "**Initial Consideration**") shall be allocated among the holders of capital stock of the Corporation in accordance with Sections 2.1, 2.2 and 2.3 as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event; and (b) any Additional Consideration which becomes payable to the stockholders of the Corporation upon satisfaction of such contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with Sections 2.1, 2.2 and 2.3 after taking into account the previous payment of the Initial Consideration as part of the same transaction. For the purposes of this Section 2.4.4, consideration placed into escrow or retained as a holdback to be available for satisfaction of indemnification or similar obligations in connection with such Deemed Liquidation Event shall be deemed to be Additional Consideration.

3. Voting.

3.1 General. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders of the Corporation (or by written consent of stockholders in lieu of meeting), each holder of outstanding shares of Preferred Stock shall be entitled to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Preferred Stock held by such holder are convertible as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of this Restated Certificate, holders of Preferred Stock shall vote together with the holders of Common Stock as a single class and on an as-converted to Common Stock basis.

3.2 Election of Directors. The holders of record of the shares of Series A Preferred Stock, voting exclusively and as a separate class, shall be entitled to elect two (2) directors of the Corporation (the "**Series A Directors**"); the holders of record of the shares of Preferred Stock and the holders of record of the shares of Common Stock, each voting as a separate

class (i.e. the majority of each of the outstanding Preferred Stock, voting together as a single class on an as-converted basis, and the outstanding Common Stock is required for the election of such director), shall be entitled to jointly elect one (1) director of the Corporation (the "**Joint Director**"); the holders of record of the shares of Common Stock, exclusively and as a separate class, shall be entitled to elect one (1) director of the Corporation. Any director elected as provided in the preceding sentence may be removed without cause by, and only by, the affirmative vote of the holders of the shares of the class or series of capital stock entitled to elect such director or directors, given either at a special meeting of such stockholders duly called for that purpose or pursuant to a written consent of stockholders. If the holders of shares of Series A Preferred Stock or Common Stock, as the case may be, fail to elect a sufficient number of directors to fill all directorships for which they are entitled to elect directors, voting exclusively and as a separate class (or if the holders of the shares of Preferred Stock and the holders of the shares of Common Stock, each voting as a separate class, fail to elect the Joint Director), pursuant to the first sentence of this Section 3.2, then any directorship not so filled shall remain vacant until such time as the holders of the Series A Preferred Stock or Common Stock (or, in the case of the Joint Director, each of the holders of the Preferred Stock and the Common Stock), as the case may be, elect a person to fill such directorship by vote or written consent in lieu of a meeting; and no such directorship may be filled by stockholders of the Corporation other than by the stockholders of the Corporation that are entitled to elect a person to fill such directorship, voting exclusively and as a separate class. The holders of record of the shares of Common Stock and of any other class or series of voting stock (including the Preferred Stock), exclusively and voting together as a single class, shall be entitled to elect the balance of the total number of directors of the Corporation. At any meeting held for the purpose of electing a director, the presence in person or by proxy of the holders of a majority of the outstanding shares of the class or series entitled to elect such director shall constitute a quorum for the purpose of electing such director. Except as otherwise provided in this Section 3.2, a vacancy in any directorship filled by the holders of any class or series shall be filled only by vote or written consent in lieu of a meeting of the holders of such class or series or by any remaining director or directors elected by the holders of such class or series pursuant to this Section 3.2.

3.3 Preferred Stock Protective Provisions. At any time when shares of Preferred Stock are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or this Restated Certificate) the written consent or affirmative vote of the Requisite Holders given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.

3.3.1 liquidate, dissolve or wind-up the business and affairs of the Corporation, effect any Deemed Liquidation Event, or consent to any of the foregoing;

3.3.2 amend, alter or repeal any provision of this Restated Certificate or Bylaws of the Corporation (the "**Bylaws**") in a manner adverse to the Preferred Stock;

3.3.3 create, or authorize the creation of, or issue or obligate itself to issue shares of, any additional class or series of capital stock unless the same ranks junior to all series of Preferred Stock with respect to the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends and rights of redemption, or increase the

authorized number of shares of Preferred Stock or increase the authorized number of shares of any additional class or series of capital stock of the Corporation unless the same ranks junior to all series of Preferred Stock with respect to the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends and rights of redemption;

3.3.4 (i) reclassify, alter or amend any existing security of the Corporation that is pari passu with any series Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to such series of Preferred Stock in respect of any such right, preference, or privilege or (ii) reclassify, alter or amend any existing security of the Corporation that is junior to any series Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or pari passu with such series of Preferred Stock in respect of any such right, preference or privilege;

3.3.5 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) redemptions of or dividends or distributions on the Preferred Stock as expressly authorized herein, (ii) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock and (iii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof;

3.3.6 create, or hold capital stock in, any subsidiary that is not wholly owned (either directly or through one or more other subsidiaries) by the Corporation, or permit any subsidiary to create, or authorize the creation of, or issue or obligate itself to issue, any shares of any class or series of capital stock, or sell, transfer or otherwise dispose of any capital stock of any direct or indirect subsidiary of the Corporation, or permit any direct or indirect subsidiary to sell, lease, transfer, exclusively license or otherwise dispose (in a single transaction or series of related transactions) of all or substantially all of the assets of such subsidiary; or

3.3.7 increase or decrease the authorized number of directors constituting the Board of Directors.

3.4 Series B Preferred Stock Protective Provisions. At any time when shares of Series B Preferred Stock are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or this Restated Certificate) the written consent or affirmative vote of the Requisite Series B Holders given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.

3.4.1 amend, alter, repeal or waive any provision of this Restated Certificate or Bylaws in a manner adverse to the Series B Preferred Stock;

3.4.2 increase the authorized number of shares of Series B Preferred Stock;

3.4.3 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) redemptions of or dividends or distributions on the Preferred Stock as expressly authorized herein, (ii) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock and (iii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof;

3.4.4 (i) reclassify, alter or amend any existing security of the Corporation that is pari passu with the Series B Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to the Series B Preferred Stock in respect of any such right, preference, or privilege or (ii) reclassify, alter or amend any existing security of the Corporation that is junior to the Series B Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or pari passu with the Series B Preferred Stock in respect of any such right, preference or privilege; or

3.4.5 consummate a Deemed Liquidation Event in which the per share proceeds to the holders of Series B Preferred Stock are less than the Series B Original Issue Price in respect of such shares of Series B Preferred Stock.

4. Optional Conversion. The holders of the Preferred Stock shall have conversion rights as follows (the "**Conversion Rights**"):

4.1 Right to Convert.

4.1.1 Conversion Ratio. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and non-assessable shares of Common Stock as is determined by dividing the applicable Original Issue Price by the applicable Conversion Price (as defined below) in effect at the time of conversion. The "**Series A Conversion Price**" shall initially be equal to $3.00. Such initial Series A Conversion Price, and the rate at which shares of Series A Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below. The "**Series B Conversion Price**" shall initially be equal to $17.00. Such initial Series B Conversion Price, and the rate at which shares of Series B Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below. The applicable "**Conversion Price**" shall mean the Series A Conversion Price in the case of the Series A Preferred Stock and the Series B Conversion Price in the case of the Series B Preferred Stock.

4.1.2 Termination of Conversion Rights. In the event of a liquidation, dissolution or winding up of the Corporation or a Deemed Liquidation Event, the Conversion Rights shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable on such event to the holders of Preferred Stock.

4.2 Fractional Shares. No fractional shares of Common Stock shall be issued upon conversion of the Preferred Stock. In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the fair market value of a share of Common Stock as determined in good faith by the Board of Directors. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the aggregate number of shares of Common Stock issuable upon such conversion.

4.3 Mechanics of Conversion.

4.3.1 Notice of Conversion. In order for a holder of Preferred Stock to voluntarily convert shares of Preferred Stock into shares of Common Stock, such holder shall (a) provide written notice to the Corporation's transfer agent at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent) that such holder elects to convert all or any number of such holder's shares of Preferred Stock and, if applicable, any event on which such conversion is contingent and (b) if such holder's shares are certificated, surrender the certificate or certificates for such shares of Preferred Stock (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate), at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent). Such notice shall state such holder's name or the names of the nominees in which such holder wishes the shares of Common Stock to be issued. If required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in writing. The close of business on the date of receipt by the transfer agent (or by the Corporation if the Corporation serves as its own transfer agent) of such notice and, if applicable, certificates (or lost certificate affidavit and agreement) shall be the time of conversion (the "**Conversion Time**"), and the shares of Common Stock issuable upon conversion of the specified shares shall be deemed to be outstanding of record as of such date. The Corporation shall, as soon as practicable after the Conversion Time (i) issue and deliver to such holder of Preferred Stock, or to his, her or its nominees, a certificate or certificates (or notice of issuance if such shares are uncertificated) for the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof and a certificate for the number (if any) of the shares of Preferred Stock represented by the surrendered certificate that were not converted into Common Stock, (ii) pay in cash such amount as provided in Section 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted.

4.3.2 Reservation of Shares. The Corporation shall at all times when shares of Preferred Stock shall be outstanding, reserve and keep available out of its authorized but unissued capital stock, for the purpose of effecting the conversion of the Preferred Stock, such number of its duly authorized shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion

of all then outstanding shares of the Preferred Stock, the Corporation shall take such corporate action as may be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes, including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to this Restated Certificate. Before taking any action which would cause an adjustment reducing the applicable Conversion Price below the then par value of the shares of Common Stock issuable upon conversion of the Preferred Stock, the Corporation will take any corporate action which may, in the opinion of its counsel, be necessary in order that the Corporation may validly and legally issue fully paid and non-assessable shares of Common Stock at such adjusted applicable Conversion Price.

4.3.3 Effect of Conversion. All shares of Preferred Stock which shall have been surrendered for conversion as herein provided shall no longer be deemed to be outstanding and all rights with respect to such shares shall immediately cease and terminate at the Conversion Time, except only the right of the holders thereof to receive shares of Common Stock in exchange therefor, to receive payment in lieu of any fraction of a share otherwise issuable upon such conversion as provided in Section 4.2 and to receive payment of any dividends declared but unpaid thereon. Any shares of Preferred Stock so converted shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.

4.3.4 No Further Adjustment. Upon any such conversion, no adjustment to the applicable Conversion Price shall be made for any declared but unpaid dividends on the Preferred Stock surrendered for conversion or on the Common Stock delivered upon conversion.

4.3.5 Taxes. The Corporation shall pay any and all issue and other similar taxes that may be payable in respect of any issuance or delivery of shares of Common Stock upon conversion of shares of Preferred Stock pursuant to this Section 4. The Corporation shall not, however, be required to pay any tax which may be payable in respect of any transfer involved in the issuance and delivery of shares of Common Stock in a name other than that in which the shares of Preferred Stock so converted were registered, and no such issuance or delivery shall be made unless and until the person or entity requesting such issuance has paid to the Corporation the amount of any such tax or has established, to the satisfaction of the Corporation, that such tax has been paid.

4.4 Adjustments to Conversion Price for Diluting Issues.

4.4.1 Special Definitions. For purposes of this Article Fourth, the following definitions shall apply:

(a) "**Option**" shall mean rights, options or warrants to subscribe for, purchase or otherwise acquire Common Stock or Convertible Securities.

(b) "**Series B Original Issue Date**" shall mean the date on which the first share of Series B Preferred Stock was issued.

(c) "**Convertible Securities**" shall mean any evidences of indebtedness, shares or other securities directly or indirectly convertible into or exchangeable for Common Stock, but excluding Options.

(d) "**Additional Shares of Common Stock**" shall mean all shares of Common Stock issued (or, pursuant to Section 4.4.3 below, deemed to be issued) by the Corporation after the Series B Original Issue Date, other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, "**Exempted Securities**"):

(i) shares of Common Stock, Options or Convertible Securities issued as a dividend or distribution on Preferred Stock;

(ii) shares of Common Stock, Options or Convertible Securities issued by reason of a dividend, stock split, split-up or other distribution on shares of Common Stock that is covered by Section 4.5, 4.6, 4.7 or 4.8**;**

(iii) Common Stock, including Options therefor (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization affecting such shares), issued or issuable to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries, pursuant to the terms of any plan, agreement or arrangement approved by the Board of Directors; or

(iv) shares of Common Stock or Convertible Securities actually issued upon the exercise of Options or shares of Common Stock actually issued upon the conversion or exchange of Convertible Securities, in each case provided such issuance is pursuant to the terms of such Option or Convertible Security.

4.4.2 No Adjustment of Conversion Price. No adjustment in the applicable Conversion Price for a series of Preferred Stock shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of a majority of the outstanding shares of such series of Preferred Stock agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock.

4.4.3 Deemed Issue of Additional Shares of Common Stock.

(a) If the Corporation at any time or from time to time after the Series B Original Issue Date shall issue any Options or Convertible Securities (excluding Options or Convertible Securities which are themselves Exempted Securities) or shall fix a record date for the determination of holders of any class of securities entitled to receive any such Options or Convertible Securities, then the maximum number of shares of Common Stock (as set forth in the instrument relating thereto, assuming the satisfaction of any conditions to exercisability, convertibility or exchangeability but without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or, in the case of Convertible Securities and Options therefor, the conversion or exchange of such Convertible Securities, shall be deemed to be Additional Shares of Common Stock issued as of the time of such issue or, in case such a record date shall have been fixed, as of the close of business on such record date.

(b) If the terms of any Option or Convertible Security, the issuance of which resulted in an adjustment to the applicable Conversion Price pursuant to the terms of Section 4.4.4, are revised as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase or decrease in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (2) any increase or decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then, effective upon such increase or decrease becoming effective, the applicable Conversion Price computed upon the original issue of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto) shall be readjusted to such applicable Conversion Price as would have obtained had such revised terms been in effect upon the original date of issuance of such Option or Convertible Security. Notwithstanding the foregoing, no readjustment pursuant to this clause (b) shall have the effect of increasing the applicable Conversion Price to an amount which exceeds the lower of (i) the applicable Conversion Price in effect immediately prior to the original adjustment made as a result of the issuance of such Option or Convertible Security, or (ii) the applicable Conversion Price that would have resulted from any issuances of Additional Shares of Common Stock (other than deemed issuances of Additional Shares of Common Stock as a result of the issuance of such Option or Convertible Security) between the original adjustment date and such readjustment date.

(c) If the terms of any Option or Convertible Security (excluding Options or Convertible Securities which are themselves Exempted Securities), the issuance of which did not result in an adjustment to the applicable Conversion Price pursuant to the terms of Section 4.4.4 (either because the consideration per share (determined pursuant to Section 4.4.5) of the Additional Shares of Common Stock subject thereto was equal to or greater than the applicable Conversion Price then in effect, or because such Option or Convertible Security was issued before the Series B Original Issue Date), are revised after the Series B Original Issue Date as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase in the number of shares of Common Stock issuable upon the exercise, conversion or exchange of any such Option or Convertible Security or (2) any decrease in the

consideration payable to the Corporation upon such exercise, conversion or exchange, then such Option or Convertible Security, as so amended or adjusted, and the Additional Shares of Common Stock subject thereto (determined in the manner provided in Section 4.4.3(a) shall be deemed to have been issued effective upon such increase or decrease becoming effective.

(d) Upon the expiration or termination of any unexercised Option or unconverted or unexchanged Convertible Security (or portion thereof) which resulted (either upon its original issuance or upon a revision of its terms) in an adjustment to the applicable Conversion Price pursuant to the terms of Section 4.4.4, the applicable Conversion Price shall be readjusted to such applicable Conversion Price as would have obtained had such Option or Convertible Security (or portion thereof) never been issued.

(e) If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, is calculable at the time such Option or Convertible Security is issued or amended but is subject to adjustment based upon subsequent events, any adjustment to the applicable Conversion Price provided for in this Section 4.4.3 shall be effected at the time of such issuance or amendment based on such number of shares or amount of consideration without regard to any provisions for subsequent adjustments (and any subsequent adjustments shall be treated as provided in clauses (b) and (c) of this Section 4.4.3). If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, cannot be calculated at all at the time such Option or Convertible Security is issued or amended, any adjustment to the applicable Conversion Price that would result under the terms of this Section 4.4.3 at the time of such issuance or amendment shall instead be effected at the time such number of shares and/or amount of consideration is first calculable (even if subject to subsequent adjustments), assuming for purposes of calculating such adjustment to the applicable Conversion Price that such issuance or amendment took place at the time such calculation can first be made.

4.4.4 Adjustment of Conversion Price Upon Issuance of Additional Shares of Common Stock. In the event the Corporation shall at any time or from time to time after the Series B Original Issue Date issue Additional Shares of Common Stock (including Additional Shares of Common Stock deemed to be issued pursuant to Section 4.4.3), without consideration or for a consideration per share less than the applicable Conversion Price in effect immediately prior to such issuance or deemed issuance, then the applicable Conversion Price shall be reduced, concurrently with such issue, to a price (calculated to the nearest one-hundredth of a cent) determined in accordance with the following formula:

$$CP_2 = CP_1 * (A + B) \div (A + C).$$

For purposes of the foregoing formula, the following definitions shall apply:

(a) "CP_2" shall mean the applicable Conversion Price in effect immediately after such issuance or deemed issuance of Additional Shares of Common Stock

(b) "CP_1" shall mean the applicable Conversion Price in effect immediately prior to such issuance or deemed issuance of Additional Shares of Common Stock;

(c) "A" shall mean the number of shares of Common Stock outstanding immediately prior to such issuance or deemed issuance of Additional Shares of Common Stock (treating for this purpose as outstanding all shares of Common Stock issuable upon exercise of Options outstanding immediately prior to such issuance or deemed issuance or upon conversion or exchange of Convertible Securities (including the Preferred Stock) outstanding (assuming exercise of any outstanding Options therefor) immediately prior to such issue);

(d) "B" shall mean the number of shares of Common Stock that would have been issued if such Additional Shares of Common Stock had been issued or deemed issued at a price per share equal to CP_1 (determined by dividing the aggregate consideration received by the Corporation in respect of such issue by CP_1); and

(e) "C" shall mean the number of such Additional Shares of Common Stock issued in such transaction.

4.4.5 Determination of Consideration. For purposes of this Section 4.4, the consideration received by the Corporation for the issuance or deemed issuance of any Additional Shares of Common Stock shall be computed as follows:

(a) Cash and Property: Such consideration shall:

(i) insofar as it consists of cash, be computed at the aggregate amount of cash received by the Corporation, excluding amounts paid or payable for accrued interest;

(ii) insofar as it consists of property other than cash, be computed at the fair market value thereof at the time of such issue, as determined in good faith by the Board of Directors; and

(iii) in the event Additional Shares of Common Stock are issued together with other shares or securities or other assets of the Corporation for consideration which covers both, be the proportion of such consideration so received, computed as provided in clauses (i) and (ii) above, as determined in good faith by the Board of Directors.

(b) Options and Convertible Securities. The consideration per share received by the Corporation for Additional Shares of Common Stock deemed to have been issued pursuant to Section 4.4.3, relating to Options and Convertible Securities, shall be determined by dividing:

(i) The total amount, if any, received or receivable by the Corporation as consideration for the issue of such Options or

Convertible Securities, plus the minimum aggregate amount of additional consideration (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such consideration) payable to the Corporation upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities, by

(ii) the maximum number of shares of Common Stock (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities.

4.4.6 Multiple Closing Dates. In the event the Corporation shall issue on more than one date Additional Shares of Common Stock that are a part of one transaction or a series of related transactions and that would result in an adjustment to the applicable Conversion Price pursuant to the terms of Section 4.4.4, and such issuance dates occur within a period of no more than ninety (90) days from the first such issuance to the final such issuance, then, upon the final such issuance, the applicable Conversion Price shall be readjusted to give effect to all such issuances as if they occurred on the date of the first such issuance (and without giving effect to any additional adjustments as a result of any such subsequent issuances within such period).

4.5 Adjustment for Stock Splits and Combinations. If the Corporation shall at any time or from time to time after the Series B Original Issue Date effect a subdivision of the outstanding Common Stock, the applicable Conversion Price in effect immediately before that subdivision shall be proportionately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding. If the Corporation shall at any time or from time to time after the Series B Original Issue Date combine the outstanding shares of Common Stock, the applicable Conversion Price in effect immediately before the combination shall be proportionately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in the aggregate number of shares of Common Stock outstanding. Any adjustment under this subsection shall become effective at the close of business on the date the subdivision or combination becomes effective.

4.6 Adjustment for Certain Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series B Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable on the Common Stock in additional shares of Common Stock, then and in each such event the applicable Conversion Price in effect immediately before such event shall be decreased as of the time of such issuance or, in the event such a record date shall have been fixed, as of the close of business on such record date, by multiplying the applicable Conversion Price then in effect by a fraction:

(1) the numerator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date, and

(2) the denominator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date plus the number of shares of Common Stock issuable in payment of such dividend or distribution.

Notwithstanding the foregoing (a) if such record date shall have been fixed and such dividend is not fully paid or if such distribution is not fully made on the date fixed therefor, the applicable Conversion Price shall be recomputed accordingly as of the close of business on such record date and thereafter the applicable Conversion Price shall be adjusted pursuant to this subsection as of the time of actual payment of such dividends or distributions; and (b) that no such adjustment shall be made if the holders of Preferred Stock simultaneously receive a dividend or other distribution of shares of Common Stock in a number equal to the number of shares of Common Stock as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.7 Adjustments for Other Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series B Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable in securities of the Corporation (other than a distribution of shares of Common Stock in respect of outstanding shares of Common Stock) or in other property and the provisions of Section 1 do not apply to such dividend or distribution, then and in each such event the holders of Preferred Stock shall receive, simultaneously with the distribution to the holders of Common Stock, a dividend or other distribution of such securities or other property in an amount equal to the amount of such securities or other property as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.8 Adjustment for Merger or Reorganization, etc. Subject to the provisions of Section 2.4, if there shall occur any reorganization, recapitalization, reclassification, consolidation or merger involving the Corporation in which the Common Stock (but not the Preferred Stock) is converted into or exchanged for securities, cash or other property (other than a transaction covered by Sections 4.4, 4.6 or 4.7), then, following any such reorganization, recapitalization, reclassification, consolidation or merger, each share of Preferred Stock shall

thereafter be convertible in lieu of the Common Stock into which it was convertible prior to such event into the kind and amount of securities, cash or other property which a holder of the number of shares of Common Stock of the Corporation issuable upon conversion of one share of Preferred Stock immediately prior to such reorganization, recapitalization, reclassification, consolidation or merger would have been entitled to receive pursuant to such transaction; and, in such case, appropriate adjustment (as determined in good faith by the Board of Directors) shall be made in the application of the provisions in this Section 4 with respect to the rights and interests thereafter of the holders of the Preferred Stock, to the end that the provisions set forth in this Section 4 (including provisions with respect to changes in and other adjustments of the applicable Conversion Price) shall thereafter be applicable, as nearly as reasonably may be, in relation to any securities or other property thereafter deliverable upon the conversion of the Preferred Stock.

4.9 Certificate as to Adjustments. Upon the occurrence of each adjustment or readjustment of the applicable Conversion Price pursuant to this Section 4, the Corporation at its expense shall, as promptly as reasonably practicable but in any event not later than ten (10) days thereafter, compute such adjustment or readjustment in accordance with the terms hereof and furnish to each holder of Preferred Stock a certificate setting forth such adjustment or readjustment (including the kind and amount of securities, cash or other property into which the Preferred Stock is convertible) and showing in detail the facts upon which such adjustment or readjustment is based. The Corporation shall, as promptly as reasonably practicable after the written request at any time of any holder of Preferred Stock (but in any event not later than ten (10) days thereafter), furnish or cause to be furnished to such holder a certificate setting forth (i) the applicable Conversion Price then in effect, and (ii) the number of shares of Common Stock and the amount, if any, of other securities, cash or property which then would be received upon the conversion of Preferred Stock.

4.10 Notice of Record Date. In the event:

(a) the Corporation shall take a record of the holders of its Common Stock (or other capital stock or securities at the time issuable upon conversion of the Preferred Stock) for the purpose of entitling or enabling them to receive any dividend or other distribution, or to receive any right to subscribe for or purchase any shares of capital stock of any class or any other securities, or to receive any other security; or

(b) of any capital reorganization of the Corporation, any reclassification of the Common Stock of the Corporation, or any Deemed Liquidation Event; or

(c) of the voluntary or involuntary dissolution, liquidation or winding-up of the Corporation,

then, and in each such case, the Corporation will send or cause to be sent to the holders of the Preferred Stock a notice specifying, as the case may be, (i) the record date for such dividend, distribution or right, and the amount and character of such dividend, distribution or right, or (ii) the effective date on which such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up is proposed to take place, and the time, if any is to be fixed, as of which the holders of record of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of the Preferred Stock) shall be entitled to exchange their shares of Common Stock (or such other capital stock or securities) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, transfer, dissolution,

liquidation or winding-up, and the amount per share and character of such exchange applicable to the Preferred Stock and the Common Stock. Such notice shall be sent at least ten (10) days prior to the record date or effective date for the event specified in such notice.

5. Mandatory Conversion.

5.1 Trigger Events. Upon either (a) the closing of the sale of shares of Common Stock to the public at a price per share of at least $17.00 (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Common Stock), in a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least $50,000,000 of gross proceeds to the Corporation and in connection with such offering the Common Stock is listed for trading on the Nasdaq Stock Market's National Market, the New York Stock Exchange or another exchange or marketplace approved by the Board of Directors, including the approval of at least one of the Series A Directors or (b) the date and time, or the occurrence of an event, specified by vote or written consent of (i) the Requisite Holders and (ii) the Requisite Series B Holders (the time of such closing or the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the "**Mandatory Conversion Time**"), then (i) all outstanding shares of Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Section 4.1.1. and (ii) such shares may not be reissued by the Corporation.

5.2 Procedural Requirements. All holders of record of shares of Preferred Stock shall be sent written notice of the Mandatory Conversion Time and the place designated for mandatory conversion of all such shares of Preferred Stock pursuant to this Section 5. Such notice need not be sent in advance of the occurrence of the Mandatory Conversion Time. Upon receipt of such notice, each holder of shares of Preferred Stock in certificated form shall surrender his, her or its certificate or certificates for all such shares (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation at the place designated in such notice. If so required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or by his, her or its attorney duly authorized in writing. All rights with respect to the Preferred Stock converted pursuant to Section 5.1, including the rights, if any, to receive notices and vote (other than as a holder of Common Stock), will terminate at the Mandatory Conversion Time (notwithstanding the failure of the holder or holders thereof to surrender any certificates at or prior to such time), except only the rights of the holders thereof, upon surrender of any certificate or certificates of such holders (or lost certificate affidavit and agreement) therefor, to receive the items provided for in the next sentence of this Section 5.2. As soon as practicable after the Mandatory Conversion Time and, if applicable, the surrender of any certificate or certificates (or lost certificate affidavit and agreement) for Preferred Stock, the Corporation shall (a) issue and deliver to such holder, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable on such conversion in accordance with the provisions hereof and (b) pay cash as provided in Section 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and the payment of any declared but unpaid dividends on the shares of Preferred Stock converted. Such converted Preferred Stock shall

be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock accordingly.

6. Redeemed or Otherwise Acquired Shares. Any shares of Preferred Stock that are redeemed or otherwise acquired by the Corporation or any of its subsidiaries shall be automatically and immediately cancelled and retired and shall not be reissued, sold or transferred. Neither the Corporation nor any of its subsidiaries may exercise any voting or other rights granted to the holders of Preferred Stock following redemption.

7. Waiver. Except as otherwise provided herein, any of the rights, powers, preferences and other terms of the Preferred Stock set forth herein may be waived on behalf of all holders of Preferred Stock by the affirmative written consent or vote of the holders of at least a majority of the shares of Preferred Stock then outstanding, voting together as a single class on an as-converted basis.

8. Notices. Any notice required or permitted by the provisions of this Article Fourth to be given to a holder of shares of Preferred Stock shall be mailed, postage prepaid, to the post office address last shown on the records of the Corporation, or given by electronic communication in compliance with the provisions of the General Corporation Law, and shall be deemed sent upon such mailing or electronic transmission.

FIFTH: Subject to any additional vote required by this Restated Certificate or the Bylaws, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, repeal, alter, amend and rescind any or all of the Bylaws.

SIXTH: Subject to any additional vote required by this Restated Certificate, the number of directors of the Corporation shall be determined in the manner set forth in the Bylaws. Each director shall be entitled to one vote on each matter presented to the Board of Directors; provided, however, that, so long as the holders of Series A Preferred Stock are entitled to elect any Series A Directors, the affirmative vote of at least one Series A Director shall be required for the authorization by the Board of Directors of any of the matters set forth in Section 5.4 of the Amended and Restated Investors' Rights Agreement, dated as of July 2, 2020, by and among the Corporation and the other parties thereto, as such agreement may be amended from time to time.

SEVENTH: Elections of directors need not be by written ballot unless the Bylaws of the Corporation shall so provide.

EIGHTH: Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws may provide. The books of the Corporation may be kept outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the Bylaws.

NINTH: To the fullest extent permitted by law, a director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director. If the General Corporation Law or any other law of the State of Delaware is amended after approval by the stockholders of this Article Ninth to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law as so amended.

Any repeal or modification of the foregoing provisions of this Article Ninth by the stockholders of the Corporation shall not adversely affect any right or protection of a director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

TENTH: The following indemnification provisions shall apply to the persons enumerated below.

1. Right to Indemnification of Directors and Officers. The Corporation shall indemnify and hold harmless, to the fullest extent permitted by applicable law as it presently exists or may hereafter be amended, any person (an "**Indemnified Person**") who was or is made or is threatened to be made a party or is otherwise involved in any action, suit or proceeding, whether civil, criminal, administrative or investigative (a "**Proceeding**"), by reason of the fact that such person, or a person for whom such person is the legal representative, is or was a director or officer of the Corporation or, while a director or officer of the Corporation, is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation or of a partnership, joint venture, limited liability company, trust, enterprise or nonprofit entity, including service with respect to employee benefit plans, against all liability and loss suffered and expenses (including attorneys' fees) reasonably incurred by such Indemnified Person in such Proceeding. Notwithstanding the preceding sentence, except as otherwise provided in Section 3 of this Article Tenth, the Corporation shall be required to indemnify an Indemnified Person in connection with a Proceeding (or part thereof) commenced by such Indemnified Person only if the commencement of such Proceeding (or part thereof) by the Indemnified Person was authorized in advance by the Board of Directors.

2. Prepayment of Expenses of Directors and Officers. The Corporation shall pay the expenses (including attorneys' fees) incurred by an Indemnified Person in defending any Proceeding in advance of its final disposition, provided, however, that, to the extent required by law, such payment of expenses in advance of the final disposition of the Proceeding shall be made only upon receipt of an undertaking by the Indemnified Person to repay all amounts advanced if it should be ultimately determined that the Indemnified Person is not entitled to be indemnified under this Article Tenth or otherwise.

3. Claims by Directors and Officers. If a claim for indemnification or advancement of expenses under this Article Tenth is not paid in full within thirty (30) days after a written claim therefor by the Indemnified Person has been received by the Corporation, the Indemnified Person may file suit to recover the unpaid amount of such claim and, if successful in whole or in part, shall be entitled to be paid the expense of prosecuting such claim. In any such action the Corporation shall have the burden of proving that the Indemnified Person is not entitled to the requested indemnification or advancement of expenses under applicable law.

4. Indemnification of Employees and Agents. The Corporation may indemnify and advance expenses to any person who was or is made or is threatened to be made or is otherwise involved in any Proceeding by reason of the fact that such person, or a person for whom such person is the legal representative, is or was an employee or agent of the Corporation or, while an

employee or agent of the Corporation, is or was serving at the request of the Corporation as a director, officer, employee or agent of another corporation or of a partnership, joint venture, limited liability company, trust, enterprise or nonprofit entity, including service with respect to employee benefit plans, against all liability and loss suffered and expenses (including attorneys' fees) reasonably incurred by such person in connection with such Proceeding. The ultimate determination of entitlement to indemnification of persons who are non-director or officer employees or agents shall be made in such manner as is determined by the Board of Directors in its sole discretion. Notwithstanding the foregoing sentence, the Corporation shall not be required to indemnify a person in connection with a Proceeding initiated by such person if the Proceeding was not authorized in advance by the Board of Directors.

5. Advancement of Expenses of Employees and Agents. The Corporation may pay the expenses (including attorneys' fees) incurred by an employee or agent in defending any Proceeding in advance of its final disposition on such terms and conditions as may be determined by the Board of Directors.

6. Non-Exclusivity of Rights. The rights conferred on any person by this Article Tenth shall not be exclusive of any other rights which such person may have or hereafter acquire under any statute, provision of this Restated Certificate, the Bylaws, or any agreement, or pursuant to any vote of stockholders or disinterested directors or otherwise.

7. Other Indemnification. The Corporation's obligation, if any, to indemnify any person who was or is serving at its request as a director, officer or employee of another corporation, partnership, limited liability company, joint venture, trust, organization or other enterprise shall be reduced by any amount such person may collect as indemnification from such other corporation, partnership, limited liability company, joint venture, trust, organization or other enterprise.

8. Insurance. The Board of Directors may, to the full extent permitted by applicable law as it presently exists, or may hereafter be amended from time to time, authorize an appropriate officer or officers to purchase and maintain at the Corporation's expense insurance: (a) to indemnify the Corporation for any obligation which it incurs as a result of the indemnification of directors, officers and employees under the provisions of this Article Tenth; and (b) to indemnify or insure directors, officers and employees against liability in instances in which they may not otherwise be indemnified by the Corporation under the provisions of this Article Tenth.

9. Amendment or Repeal. Any repeal or modification of the foregoing provisions of this Article Tenth shall not adversely affect any right or protection hereunder of any person in respect of any act or omission occurring prior to the time of such repeal or modification. The rights provided hereunder shall inure to the benefit of any Indemnified Person and such person's heirs, executors and administrators.

ELEVENTH: The Corporation renounces, to the fullest extent permitted by law, any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An "**Excluded Opportunity**" is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of (i) any director of the Corporation who is not an employee of the Corporation or any of its subsidiaries, or (ii) any holder of Preferred Stock or any partner, member, director, stockholder, employee, affiliate or agent of any such holder, other than someone who is an employee of the Corporation or any of its subsidiaries (collectively, the persons referred to in

clauses (i) and (ii) are "**Covered Persons**"), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person's capacity as a director of the Corporation while such Covered Person is performing services in such capacity. Any repeal or modification of this Article Eleventh will only be prospective and will not affect the rights under this Article Eleventh in effect at the time of the occurrence of any actions or omissions to act giving rise to liability. Notwithstanding anything to the contrary contained elsewhere in this Restated Certificate, the affirmative vote of the holders of at least a majority of the shares of Preferred Stock then outstanding, voting together as a single class on an as-converted basis, will be required to amend or repeal, or to adopt any provisions inconsistent with this Article Eleventh.

TWELFTH: Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery in the State of Delaware shall be the sole and exclusive forum for any stockholder (including a beneficial owner) to bring (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of fiduciary duty owed by any director, officer or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim against the Corporation, its directors, officers or employees arising pursuant to any provision of the Delaware General Corporation Law or the Corporation's certificate of incorporation or bylaws or (iv) any action asserting a claim against the Corporation, its directors, officers or employees governed by the internal affairs doctrine, except for, as to each of (i) through (iv) above, any claim as to which the Court of Chancery determines that there is an indispensable party not subject to the jurisdiction of the Court of Chancery (and the indispensable party does not consent to the personal jurisdiction of the Court of Chancery within ten days following such determination), which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery, or for which the Court of Chancery does not have subject matter jurisdiction. If any provision or provisions of this Article Twelfth shall be held to be invalid, illegal or unenforceable as applied to any person or entity or circumstance for any reason whatsoever, then, to the fullest extent permitted by law, the validity, legality and enforceability of such provisions in any other circumstance and of the remaining provisions of this Article Twelfth (including, without limitation, each portion of any sentence of this Article Twelfth containing any such provision held to be invalid, illegal or unenforceable that is not itself held to be invalid, illegal or unenforceable) and the application of such provision to other persons or entities and circumstances shall not in any way be affected or impaired thereby.

* * *

4. That the foregoing amendment and restatement was approved by the holders of the requisite number of shares of this corporation in accordance with Section 228 of the General Corporation Law.

5. That this Restated Certificate, which restates and integrates and further amends the provisions of this Corporation's Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the General Corporation Law.

IN WITNESS WHEREOF, this Amended and Restated Certificate of Incorporation has been executed by a duly authorized officer of this corporation on this 2nd day of July, 2020.

By: /s/ R.A. Session II
Name: R.A. Session II
Title: President

CERTIFICATE OF AMENDMENT OF
CERTIFICATE OF INCORPORATION OF
TAYSHA GENE THERAPIES, INC.

Taysha Gene Therapies, Inc., a corporation organized and existing under and by virtue of the General Corporation Law of the State of Delaware (the "**General Corporation Law**"), hereby certifies that:

1. The name of the corporation is Taysha Gene Therapies, Inc. (the "**Corporation**"), and that this Corporation was originally incorporated in Texas pursuant to the Texas Business Organization Code.

2. This Corporation was subsequently converted into a corporation incorporated under the General Corporation Law on February 13, 2020 under the name Taysha Gene Therapies, Inc.

3. The Board of Directors of the Corporation, acting in accordance with the provisions of Sections 141 and 242 of the General Corporation Law, adopted resolutions amending the certificate of incorporation as follows:

The first paragraph of Article is amended and restated to read in its entirety as follows:

"**FOURTH**: The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 30,000,000 shares of Common Stock, $0.00001 par value per share ("**Common Stock**") and (ii) 15,647,052 shares of Preferred Stock, $0.00001 par value per share ("**Preferred Stock**")."

The first paragraph of Section B of Article FOURTH is amended and restated to read in its entirety as follows:

"B. PREFERRED STOCK

Preferred Stock may be issued from time to time in one or more series, each of such series to consist of such number of shares and to have such terms, rights, powers and preferences, and the qualifications and limitations with respect thereto, as stated or expressed herein. Of the 15,647,052 shares of Preferred Stock that the Corporation has the authority to issue (i) 10,000,000 shares are hereby designated "**Series A Preferred Stock**" and (ii) 5,647,052 shares are hereby designated "**Series B Preferred Stock**" with the following rights, preferences, powers, privileges and restrictions, qualifications and limitations. Unless otherwise indicated, references to "sections" or "subsections" in this Part B of this Article Fourth refer to sections and subsections of Part B of this Article Fourth."

4. This Certificate of Amendment was duly adopted by the stockholders of the Corporation in accordance with the provisions of Sections 228 and 242 of the General Corporation Law.

[Signature Page Follows]

IN WITNESS WHEREOF, the Corporation has caused this Certificate of Amendment to be signed by a duly authorized officer as of July 28, 2020.

TAYSHA GENE THERAPIES, INC.

By: /s/ R.A. Session II
Name: R.A. Session II
Title: Chief Executive Officer

AMENDED AND RESTATED BYLAWS OF TAYSHA GENE THERAPIES, INC. (A DELAWARE CORPORATION)

MARCH 4, 2020

ARTICLE I
STOCKHOLDERS

1.1 Place of Meetings. All meetings of stockholders shall be held at such place (if any) within or without the State of Delaware as may be determined from time to time by the Board of Directors or, if not determined by the Board of Directors, by the Chairman of the Board, the President, or the Chief Executive Officer; provided that the Board of Directors may, in its sole discretion, determine that any meeting of stockholders shall not be held at any place but shall be held solely by means of remote communication in accordance with Section 1.12.

1.2 Annual Meeting. The annual meeting of stockholders for the election of directors and for the transaction of such other business as may properly be brought before the meeting shall be held on a date to be fixed by the Board of Directors at a time and place to be fixed by the Board of Directors and stated in the notice of the meeting.

1.3 Special Meetings. Special meetings of stockholders may be called at any time by the Board of Directors, the Chairman of the Board, the Chief Executive Officer, or the President or the holders of record of not less than 10% of all shares entitled to cast votes at the meeting, for any purpose or purposes prescribed in the notice of the meeting and shall be held at such place, on such date and at such time as the Board of Directors may fix. Business transacted at any special meeting of stockholders shall be confined to the purpose or purposes stated in the notice of meeting.

1.4 Notice of Meetings.

(a) Written notice of each meeting of stockholders, whether annual or special, shall be given not less than ten (10) nor more than sixty (60) days before the date on which the meeting is to be held, to each stockholder entitled to vote at such meeting as of the record date fixed by the Board of Directors for determining the stockholders entitled to notice of the meeting, except as otherwise provided herein or as required by law (meaning here and hereafter, as required from time to time by the Delaware General Corporation Law or the Certificate of Incorporation of the corporation). The notice of any meeting shall state the place, if any, date and hour of the meeting, and the means of remote communication, if any, by which stockholders and proxy holders may be deemed to be present in person and vote at such meeting. The notice of a special meeting shall state, in addition, the purpose or purposes for which the meeting is called. If mailed, notice is given when deposited in the United States mail, postage prepaid, directed to the stockholder at his address as it appears on the records of the corporation. If mailed, notice is given when deposited in the United States mail, postage prepaid, directed to the stockholder at his address as it appears on the records of the corporation.

(b) Notice to stockholders may be given by personal delivery, mail, or, with the consent of the stockholder entitled to receive notice, by facsimile or other means of electronic transmission. If mailed, such notice shall be delivered by postage prepaid envelope directed to each

stockholder at such stockholder's address as it appears in the records of the corporation and shall be deemed given when deposited in the United States mail. Notice given by electronic transmission pursuant to this subsection shall be deemed given: (1) if by facsimile telecommunication, when directed to a facsimile telecommunication number at which the stockholder has consented to receive notice; (2) if by electronic mail, when directed to an electronic mail address at which the stockholder has consented to receive notice; (3) if by posting on an electronic network together with separate notice to the stockholder of such specific posting, upon the later of (A) such posting and (B) the giving of such separate notice; and (4) if by any other form of electronic transmission, when directed to the stockholder. An affidavit of the secretary or an assistant secretary or of the transfer agent or other agent of the corporation that the notice has been given by personal delivery, by mail, or by a form of electronic transmission shall, in the absence of fraud, be prima facie evidence of the facts stated therein.

(c) Notice of any meeting of stockholders need not be given to any stockholder if waived by such stockholder either in a writing signed by such stockholder or by electronic transmission, whether such waiver is given before or after such meeting is held. If such a waiver is given by electronic transmission, the electronic transmission must either set forth or be submitted with information from which it can be determined that the electronic transmission was authorized by the stockholder.

1.5 Voting List. The officer who has charge of the stock ledger of the corporation shall prepare, at least ten (10) days before each meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, arranged in alphabetical order for each class of stock, and showing the mailing address of each stockholder and the number of shares registered in the name of each stockholder. The corporation shall not be required to include electronic mail addresses or other electronic contact information on such list. Such list shall be open to the examination of any stockholder, for any purpose germane to the meeting, during ordinary business hours, for a period of at least ten (10) days prior to the meeting, in the manner provided by law. If the meeting is held at a place, the list shall be produced and kept at the time and place of the meeting during the whole time of the meeting, and may be examined by any stockholder who is present. This list shall determine the identity of the stockholders entitled to vote at any meeting and the number of shares held by each of them.

1.6 Quorum. Except as otherwise provided by law or these Bylaws, the holders of a majority of the shares of the capital stock of the corporation entitled to vote at the meeting, present in person or represented by proxy, shall constitute a quorum for the transaction of business. Where a separate class vote by a class or classes or series is required, a majority of the shares of such class or classes or series present in person or represented by proxy shall constitute a quorum entitled to take action with respect to that vote on that matter.

1.7 Adjournments. Any meeting of stockholders may be adjourned to any other time and to any other place at which a meeting of stockholders may be held under these Bylaws by the Chairman of the meeting or, in the absence of such person, by any officer entitled to preside at or to act as Secretary of such meeting, or by the holders of a majority of the shares of stock present or represented at the meeting and entitled to vote, although less than a quorum. When a meeting is adjourned to another place, date or time, written notice need not be given of the adjourned meeting if the date, time and place, if any, thereof, and the means of remote communication, if any, by which stockholders and proxy holders may be deemed to be present in person and vote at such adjourned meeting, are announced at the meeting at which the adjournment is taken; provided, however, that if the date of any adjourned meeting is more than thirty (30) days after the date for which the meeting was originally noticed, or if a new record date is fixed for determining the stockholders entitled to vote at the adjourned meeting in accordance with Section 4.5, written notice of the place, if any, date and time of the adjourned meeting and the means of remote communication, if any, by which stockholders and proxy holders may be deemed to be present in person and vote at such adjourned meeting, shall be given in conformity herewith. At the adjourned meeting, the corporation may transact any business which might have been transacted at the original meeting.

1.8 Voting and Proxies. Each stockholder shall have one vote for each share of stock entitled to vote held of record by such stockholder and a proportionate vote for each fractional share so held, unless otherwise provided by law or in the Certificate of Incorporation. Each stockholder of record entitled to vote at a meeting of stockholders may vote in person or may authorize any other person or persons to vote or act for such stockholder by a written proxy executed by the stockholder or the stockholder's authorized agent or by an electronic transmission permitted by law and delivered to the Secretary of the corporation. No stockholder may authorize more than one proxy for his shares. Any copy, facsimile transmission or other reliable reproduction of the writing or electronic transmission created pursuant to this section may be substituted or used in lieu of the original writing or electronic transmission for any and all purposes for which the original writing or transmission could be used, provided that such copy, facsimile transmission or other reproduction shall be a complete reproduction of the entire original writing or electronic transmission.

1.9 Action at Meeting.

(a) At any meeting of stockholders for the election of one or more directors at which a quorum is present, the election shall be determined by a plurality of the votes cast by the stockholders entitled to vote at the election.

(b) All other matters shall be determined by a majority in voting power of the shares present in person or represented by proxy and entitled to vote on the matter (or if there are two or more classes of stock entitled to vote as separate classes, then in the case of each such class, a majority of the shares of each such class present in person or represented by proxy and entitled to vote on the matter shall decide such matter), provided that a quorum is present, except when a different vote is required by express provision of law, the Certificate of Incorporation or these Bylaws.

(c) All voting, including on the election of directors, but excepting where otherwise required by law, may be by a voice vote; provided, however, that upon demand therefor by a stockholder entitled to vote or the stockholder's proxy, a vote by ballot shall be taken. Each ballot shall state the name of the stockholder or proxy voting and such other information as may be required under the procedures established for the meeting. The corporation may, and to the extent required by law, shall, in advance of any meeting of stockholders, appoint one or more inspectors to act at the meeting and make a written report thereof. The corporation may designate one or more persons as an alternate inspector to replace any inspector who fails to act. If no inspector or alternate is able to act at a meeting of stockholders, the person presiding at the meeting may, and to the extent required by law, shall, appoint one or more inspectors to act at the meeting. Each inspector, before entering upon the discharge of his duties, shall take and sign an oath to faithfully execute the duties of inspector with strict impartiality and according to the best of his ability.

1.10 Conduct of Business. At every meeting of the stockholders, the Chairman of the Board, or, in his absence, the Chief Executive Officer, or the President, or, in his absence, such other person as may be appointed by the Board of Directors, shall act as chairman. The Secretary of the corporation or a person designated by the chairman of the meeting shall act as secretary of the meeting. Unless otherwise approved by the chairman of the meeting, attendance at the stockholders' meeting is restricted to stockholders of record, persons authorized in accordance with Section 1.8 of these Bylaws to act by proxy, and officers of the corporation.

The chairman of the meeting shall call the meeting to order, establish the agenda, and conduct the business of the meeting in accordance therewith or, at the chairman's discretion, the business of the meeting may be conducted otherwise in accordance with the wishes of the stockholders in attendance. The date and time of the opening and closing of the polls for each matter upon which the stockholders will vote at the meeting shall be announced at the meeting.

1.11 Stockholder Action Without Meeting. Any action which may be taken at any annual or special meeting of stockholders may be taken without a meeting and without prior notice, if a consent in writing, setting forth the actions so taken, is signed by the holders of outstanding shares having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted. All such consents shall be filed with the Secretary of the corporation and shall be maintained in the corporate records. Prompt notice of the taking of a corporate action without a meeting by less than unanimous written consent shall be given to those stockholders who have not consented in writing.

An electronic transmission consenting to an action to be taken and transmitted by a stockholder, or by a proxy holder or other person authorized to act for a stockholder, shall be deemed to be written, signed and dated for the purpose of this Section 1.11, provided that such electronic transmission sets forth or is delivered with information from which the corporation can determine (a) that the electronic transmission was transmitted by the stockholder or by a person authorized to act for the stockholder and (b) the date on which such stockholder or authorized person transmitted such electronic transmission. The date on which such electronic transmission is transmitted shall be deemed to be the date on which such consent was signed. No consent given by electronic transmission shall be deemed to have been delivered until such consent is reproduced in paper form and until such paper form shall be delivered to the corporation by delivery to its registered office in the State of Delaware, its principal place of business or an officer or agent of the corporation having custody of the books in which proceedings of meetings of stockholders are recorded.

1.12 Meetings by Remote Communication. If authorized by the Board of Directors, and subject to such guidelines and procedures as the Board of Directors may adopt, stockholders and proxy holders not physically present at a meeting of stockholders may, by means of remote communication, participate in the meeting and be deemed present in person and vote at the meeting, whether such meeting is to be held at a designated place or solely by means of remote communication, provided that (a) the corporation shall implement reasonable measures to verify that each person deemed present and permitted to vote at the meeting by means of remote communication is a stockholder or proxy holder, (b) the corporation shall implement reasonable measures to provide such stockholders and proxy holders a reasonable opportunity to participate in the meeting and to vote on matters submitted to the stockholders, including an opportunity to read or hear the proceedings of the meeting substantially concurrently with such proceedings, and (c) if any stockholder or proxy holder votes or takes other action at the meeting by means of remote communication, a record of such vote or other action shall be maintained by the corporation.

ARTICLE II
BOARD OF DIRECTORS

2.1 General Powers. The business and affairs of the corporation shall be managed by or under the direction of a Board of Directors, who may exercise all of the powers of the corporation except as otherwise provided by law or the Certificate of Incorporation. In the event of a vacancy on the Board of Directors, the remaining directors, except as otherwise provided by law, may exercise the powers of the full Board of Directors until the vacancy is filled.

2.2 Number and Term of Office. Subject to the rights of the holders of any series of preferred stock to elect directors under specified circumstances, the number of directors shall initially be five (5) and, thereafter, shall be fixed from time to time exclusively by the Board of Directors pursuant to a resolution adopted by a majority of the total number of authorized directors (whether or not there exist any vacancies in previously authorized directorships at the time any such resolution is presented to the Board for adoption). All directors shall hold office until the next annual meeting of stockholders and until their respective successors are elected, except in the case of the death, resignation or removal of any director.

2.3 Vacancies and Newly Created Directorships. Subject to the rights of the holders of any series of preferred stock then outstanding, newly created directorships resulting from any increase in the authorized number of directors or any vacancies in the Board of Directors resulting from death, resignation, retirement, disqualification or other cause (other than removal from office by a vote of the stockholders) may be filled only by a majority vote of the directors then in office, though less than a quorum, by the sole remaining director, or, to the extent required by the Certificate of Incorporation or if there are no directors, by the stockholders, and directors so chosen shall hold office for a term expiring at the next annual meeting of stockholders. No decrease in the number of authorized directors shall shorten the term of any incumbent director.

2.4 Resignation. Any director may resign by delivering notice in writing or by electronic transmission to the Chief Executive Officer**,** President, Chairman of the Board or Secretary. Such resignation shall be effective upon receipt unless it is specified to be effective at some other time or upon the happening of some other event.

2.5 Removal. Subject to the rights of the holders of any series of preferred stock then outstanding, any directors, or the entire Board of Directors, may be removed from office at any time, with or without cause, by the affirmative vote of the holders of a majority of the voting power of all of the outstanding shares of capital stock entitled to vote generally in the election of directors, voting together as a single class. Vacancies in the Board of Directors resulting from such removal may be filled by a majority of the directors then in office, though less than a quorum, by the sole remaining director, or by the stockholders at the next annual meeting or at a special meeting called in accordance with Section 1.3 above. Directors so chosen shall hold office until the next annual meeting of stockholders.

2.6 Regular Meetings. Regular meetings of the Board of Directors may be held without notice at such time and place, either within or without the State of Delaware, as shall be determined from time to time by the Board of Directors; provided that any director who is absent when such a determination is made shall be given notice of the determination. A regular meeting of the Board of Directors may be held without notice immediately after and at the same place as the annual meeting of stockholders.

2.7 Special Meetings. Special meetings of the Board of Directors may be called by the Chairman of the Board, the Chief Executive Officer**,** the President or two or more directors and may be held at any time and place, within or without the State of Delaware.

2.8 Notice of Special Meetings. Notice of any special meeting of directors shall be given to each director by whom it is not waived by the Secretary or by the officer or one of the directors calling the meeting. Notice shall be duly given to each director by whom it is not waived by (a) giving notice to such director in person or by telephone, electronic transmission or voice message system at least 24 hours in advance of the meeting, (b) sending a facsimile to his last known facsimile number, or delivering written notice by hand to his last known business or home address, at least 24 hours in advance of the meeting, or (c) mailing written notice to his last known business or home address at least three (3) days in advance of the meeting. A notice or waiver of notice of a meeting of the Board of Directors need not specify the purposes of the meeting. Unless otherwise indicated in the notice thereof, any and all business may be transacted at a special meeting.

2.9 Participation in Meetings by Telephone Conference Calls or Other Methods of Communication. Directors or any members of any committee designated by the directors may participate in a meeting of the Board of Directors or such committee by means of conference telephone or other communications equipment by means of which all persons participating in the meeting can hear each other, and participation by such means shall constitute presence in person at such meeting.

2.10 Quorum. A majority of the total number of authorized directors shall constitute a quorum at any meeting of the Board of Directors. In the absence of a quorum at any such meeting, a majority of the directors present may adjourn the meeting from time to time without further notice other than announcement at the meeting, until a quorum shall be present. Interested directors may be counted in determining the presence of a quorum at a meeting of the Board of Directors or at a meeting of a committee which authorizes a particular contract or transaction.

2.11 Action at Meeting. At any meeting of the Board of Directors at which a quorum is present, the vote of a majority of those present shall be sufficient to take any action, unless a different vote is specified by law, the Certificate of Incorporation or these Bylaws.

2.12 Action by Written Consent. Any action required or permitted to be taken at any meeting of the Board of Directors or of any committee of the Board of Directors may be taken without a meeting if all members of the Board of Directors or committee, as the case may be, consent to the action in writing or by electronic transmission, and the writings or electronic transmissions are filed with the minutes of proceedings of the Board of Directors or committee. Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form.

2.13 Committees. The Board of Directors may designate one or more committees, each committee to consist of one or more of the directors of the corporation, with such lawfully delegated powers and duties as it therefor confers, to serve at the pleasure of the Board of Directors. The Board of Directors may designate one or more directors as alternate members of any committee, who may replace any absent or disqualified member at any meeting of the committee. In the absence or disqualification of a member of a committee, the member or members of the committee present at any meeting and not disqualified from voting, whether or not he or they constitute a quorum, may unanimously appoint another member of the Board of Directors to act at the meeting in the place of any such absent or disqualified member. Any such committee, to the extent provided in the resolution of the Board of Directors and subject to the provisions of the Delaware General Corporation Law, shall have and may exercise all the powers and authority of the Board of Directors in the management of the business and affairs of the corporation and may authorize the seal of the corporation to be affixed to all papers which

may require it. Each such committee shall keep minutes and make such reports as the Board of Directors may from time to time request. Except as the Board of Directors may otherwise determine, any committee may make rules for the conduct of its business, but unless otherwise provided by such rules, its business shall be conducted as nearly as possible in the same manner as is provided in these Bylaws for the Board of Directors.

2.14 Compensation of Directors. Directors may be paid such compensation for their services and such reimbursement for expenses of attendance at meetings as the Board of Directors may from time to time determine. No such payment shall preclude any director from serving the corporation or any of its parent or subsidiary corporations in any other capacity and receiving compensation for such service.

2.15 Nomination of Director Candidates. Subject to the rights of holders of any class or series of preferred stock then outstanding, nominations for the election of Directors may be made by (a) the Board of Directors or a duly authorized committee thereof or (b) any stockholder entitled to vote in the election of directors.

ARTICLE III
OFFICERS

3.1 Enumeration. The officers of the corporation shall consist of a Chief Executive Officer, a President, a Secretary, a Treasurer, a Chief Financial Officer and such other officers with such other titles as the Board of Directors shall determine, including, at the discretion of the Board of Directors, a Chairman of the Board and one or more Vice Presidents and Assistant Secretaries. The Board of Directors may appoint such other officers as it may deem appropriate.

3.2 Election. Officers shall be elected annually by the Board of Directors at its first meeting following the annual meeting of stockholders. Officers may be appointed by the Board of Directors at any other meeting.

3.3 Qualification. No officer need be a stockholder. Any two or more offices may be held by the same person.

3.4 Tenure. Except as otherwise provided by law, by the Certificate of Incorporation or by these Bylaws, each officer shall hold office until his successor is elected and qualified, unless a different term is specified in the vote appointing the officer, or until his earlier death, resignation or removal.

3.5 Resignation and Removal. Any officer may resign by delivering his written resignation to the corporation at its principal office or to the President or Secretary. Such resignation shall be effective upon receipt unless it is specified to be effective at some other time or upon the happening of some other event. Any officer elected by the Board of Directors may be removed at any time, with or without cause, by the Board of Directors.

3.6 Chairman of the Board. The Board of Directors may appoint a Chairman of the Board. If the Board of Directors appoints a Chairman of the Board, he shall perform such duties and possess such powers as are assigned to the Chairman by the Board of Directors and these Bylaws. Unless otherwise provided by the Board of Directors, he shall preside at all meetings of the Board of Directors.

3.7 Chief Executive Officer. The Chief Executive Officer of the corporation shall, subject to the direction of the Board of Directors, have general supervision, direction and control of the business and

the officers of the corporation. He shall preside at all meetings of the stockholders and, in the absence or nonexistence of a Chairman of the Board, at all meetings of the Board of Directors. He shall have the general powers and duties of management usually vested in the chief executive officer of a corporation, including general supervision, direction and control of the business and supervision of other officers of the corporation, and shall have such other powers and duties as may be prescribed by the Board of Directors or these Bylaws.

3.8 President. Subject to the direction of the Board of Directors and such supervisory powers as may be given by these Bylaws or the Board of Directors to the Chairman of the Board or the Chief Executive Officer, if such titles be held by other officers, the President shall have general supervision, direction and control of the business and supervision of other officers of the corporation. Unless otherwise designated by the Board of Directors, the President shall be the Chief Executive Officer of the corporation. The President shall have such other powers and duties as may be prescribed by the Board of Directors or these Bylaws. He shall have power to sign stock certificates, contracts and other instruments of the corporation which are authorized and shall have general supervision and direction of all of the other officers, employees and agents of the corporation, other than the Chairman of the Board and the Chief Executive Officer.

3.9 Vice Presidents. Any Vice President shall perform such duties and possess such powers as the Board of Directors**,** the Chief Executive Officer or the President may from time to time prescribe. In the event of the absence, inability or refusal to act of the President, the Vice President (or if there shall be more than one, the Vice Presidents in the order determined by the Board of Directors) shall perform the duties of the President and when so performing shall have all the powers of and be subject to all the restrictions upon the President. The Board of Directors may assign to any Vice President the title of Executive Vice President, Senior Vice President or any other title selected by the Board of Directors.

3.10 Secretary and Assistant Secretaries. The Secretary shall perform such duties and shall have such powers as the Board of Directors or the President may from time to time prescribe. In addition, the Secretary shall perform such duties and have such powers as are set forth in these Bylaws and as are incident to the office of the Secretary, including, without limitation, the duty and power to give notices of all meetings of stockholders and special meetings of the Board of Directors, to keep a record of the proceedings of all meetings of stockholders and the Board of Directors, to maintain a stock ledger and prepare lists of stockholders and their addresses as required, to be custodian of corporate records and the corporate seal and to affix and attest to the same on documents.

Any Assistant Secretary shall perform such duties and possess such powers as the Board of Directors, the Chief Executive Officer, the President or the Secretary may from time to time prescribe. In the event of the absence, inability or refusal to act of the Secretary, the Assistant Secretary (or if there shall be more than one, the Assistant Secretaries in the order determined by the Board of Directors) shall perform the duties and exercise the powers of the Secretary.

In the absence of the Secretary or any Assistant Secretary at any meeting of stockholders or directors, the person presiding at the meeting shall designate a temporary secretary to keep a record of the meeting.

3.11 Treasurer. The Treasurer shall perform such duties and have such powers as are incident to the office of treasurer, including without limitation, the duty and power to keep and be responsible for all funds and securities of the corporation, to maintain the financial records of the corporation, to deposit funds of the corporation in depositories as authorized, to disburse such funds as authorized, to make proper accounts of such funds, and to render as required by the Board of Directors accounts of all such transactions and of the financial condition of the corporation.

3.12 Chief Financial Officer. The Chief Financial Officer shall perform such duties and shall have such powers as may from time to time be assigned to the Chief Financial Officer by the Board of Directors**,** the Chief Executive Officer or the President. Unless otherwise designated by the Board of Directors, the Chief Financial Officer shall be the Treasurer of the corporation.

3.13 Salaries. Officers of the corporation shall be entitled to such salaries, compensation or reimbursement as shall be fixed or allowed from time to time by the Board of Directors.

3.14 Delegation of Authority. The Board of Directors may from time to time delegate the powers or duties of any officer to any other officers or agents, notwithstanding any provision hereof.

ARTICLE IV
CAPITAL STOCK

4.1 Issuance of Stock. Subject to the provisions of the Certificate of Incorporation, the whole or any part of any unissued balance of the authorized capital stock of the corporation or the whole or any part of any unissued balance of the authorized capital stock of the corporation held in its treasury may be issued, sold, transferred or otherwise disposed of by vote of the Board of Directors in such manner, for such consideration and on such terms as the Board of Directors may determine.

4.2 Certificates of Stock. Every holder of stock of the corporation shall be entitled to have a certificate, in such form as may be prescribed by law and by the Board of Directors, certifying the number and class of shares of stock owned by such stockholder in the corporation. Each such certificate shall be signed by, or in the name of, the corporation by any two (2) authorized officers of the corporation. Any or all of the signatures on the certificate may be a facsimile.

Each certificate for shares of stock which are subject to any restriction on transfer pursuant to the Certificate of Incorporation, the Bylaws, applicable securities laws or any agreement among any number of stockholders or among such holders and the corporation shall have conspicuously noted on the face or back of the certificate either the full text of the restriction or a statement of the existence of such restriction.

4.3 Transfers. Except as otherwise established by rules and regulations adopted by the Board of Directors, and subject to applicable law, shares of stock may be transferred on the books of the corporation by the surrender to the corporation or its transfer agent of the certificate representing such shares properly endorsed or accompanied by a written assignment or power of attorney properly executed, and with such proof of authority or authenticity of signature as the corporation or its transfer agent may reasonably require. Except as may be otherwise required by law, the Certificate of Incorporation or the Bylaws, the corporation shall be entitled to treat the record holder of stock as shown on its books as the owner of such stock for all purposes, including the payment of dividends and the right to vote with respect to such stock, regardless of any transfer, pledge or other disposition of such stock until the shares have been transferred on the books of the corporation in accordance with the requirements of these Bylaws.

4.4 Lost, Stolen or Destroyed Certificates. The corporation may issue a new certificate in place of any previously issued certificate alleged to have been lost, stolen, or destroyed, upon such terms and conditions as the Board of Directors may prescribe, including the presentation of reasonable evidence of such loss, theft or destruction and the giving of such indemnity as the Board of Directors may require for the protection of the corporation or any transfer agent or registrar.

4.5 Record Date. The Board of Directors may fix in advance a record date for the determination of the stockholders entitled to notice of and to vote at any meeting of stockholders or to express consent to corporate action in writing without a meeting, or entitled to receive payment of any dividend or other distribution or allotment of any rights in respect of any change, concession or exchange of stock, or for the purpose of any other lawful action. Such record date shall not precede the date on which the resolution fixing the record date is adopted and shall not be more than 60 nor less than 10 days before the date of such meeting, nor more than 60 days prior to any other action to which such record date relates.

If no record date is fixed by the Board of Directors, the record date for determining the stockholders entitled to notice of and to vote at a meeting of stockholders shall be the close of business on the day before the date on which notice is given, or, if notice is waived, the close of business on the day before the date on which the meeting is held. If no record date is fixed by the Board of Directors, the record date for determining stockholders entitled to express consent to corporate action in writing without a meeting when no prior action by the Board of Directors is necessary, shall be the date on which the first written consent is expressed. The record date for determining stockholders for any other purpose shall be the close of business on the day on which the Board of Directors adopts the resolution relating to such purpose.

A determination of stockholders of record entitled to notice of and to vote at a meeting of stockholders shall apply to any adjournment of the meeting; provided, however, that the Board of Directors may fix a new record date for the adjourned meeting.

ARTICLE V
GENERAL PROVISIONS

5.1 Fiscal Year. The fiscal year of the corporation shall be as fixed by the Board of Directors.

5.2 Corporate Seal. The corporate seal shall be in such form as shall be approved by the Board of Directors.

5.3 Waiver of Notice. Whenever any notice whatsoever is required to be given by law, by the Certificate of Incorporation or by these Bylaws, a waiver of such notice either in writing signed by the person entitled to such notice or such person's duly authorized attorney, or by electronic transmission or any other method permitted under the Delaware General Corporation Law, whether before, at or after the time stated in such waiver, or the appearance of such person or persons at such meeting in person or by proxy, shall be deemed equivalent to such notice. Neither the business nor the purpose of any meeting need be specified in such a waiver. Attendance at any meeting shall constitute waiver of notice except attendance for the sole purpose of objecting to the timeliness or manner of notice.

5.4 Actions with Respect to Securities of Other Corporations. Except as the Board of Directors may otherwise designate, the Chief Executive Officer or President or any officer of the corporation authorized by the Chief Executive Officer or President shall have the power to vote and otherwise act on behalf of the corporation, in person or by proxy, and may waive notice of, and act as, or appoint any person or persons to act as, proxy or attorney-in-fact to this corporation (with or without power of substitution) at any meeting of stockholders or shareholders (or with respect to any action of stockholders) of any other corporation or organization, the securities of which may be held by this corporation and otherwise to exercise any and all rights and powers that this corporation may possess by reason of this corporation's ownership of securities in such other corporation or other organization.

5.5 Evidence of Authority. A certificate by the Secretary, or an Assistant Secretary, or a temporary Secretary, as to any action taken by the stockholders, directors, a committee or any officer or representative of the corporation shall as to all persons who rely on the certificate in good faith be conclusive evidence of such action.

5.6 Certificate of Incorporation. All references in these Bylaws to the Certificate of Incorporation shall be deemed to refer to the Certificate of Incorporation of the corporation, as amended and in effect from time to time.

5.7 Severability. Any determination that any provision of these Bylaws is for any reason inapplicable, illegal or ineffective shall not affect or invalidate any other provision of these Bylaws.

5.8 Pronouns. All pronouns used in these Bylaws shall be deemed to refer to the masculine, feminine or neuter, singular or plural, as the identity of the person or persons may require.

5.9 Notices. Except as otherwise specifically provided herein or required by law, all notices required to be given to any stockholder, director, officer, employee or agent of the corporation shall be in writing and may in every instance be effectively given by hand delivery to the recipient thereof, by depositing such notice in the mails, postage paid, or by sending such notice by commercial courier service, or by facsimile or other electronic transmission, provided that notice to stockholders by electronic transmission shall be given in the manner provided in Section 232 of the Delaware General Corporation Law. Any such notice shall be addressed to such stockholder, director, officer, employee or agent at his last known address as the same appears on the books of the corporation. The time when such notice shall be deemed to be given shall be the time such notice is received by such stockholder, director, officer, employee or agent, or by any person accepting such notice on behalf of such person, if delivered by hand, facsimile, other electronic transmission or commercial courier service, or the time such notice is dispatched, if delivered through the mails. Without limiting the manner by which notice otherwise may be given effectively, notice to any stockholder shall be deemed given: (a) if by facsimile, when directed to a number at which the stockholder has consented to receive notice; (b) if by electronic mail, when directed to an electronic mail address at which the stockholder has consented to receive notice; (c) if by a posting on an electronic network together with separate notice to the stockholder of such specific posting, upon the later of (i) such posting and (ii) the giving of such separate notice; (d) if by any other form of electronic transmission, when directed to the stockholder; and (e) if by mail, when deposited in the mail, postage prepaid, directed to the stockholder at such stockholder’s address as it appears on the records of the corporation.

5.10 Reliance Upon Books, Reports and Records. Each director, each member of any committee designated by the Board of Directors, and each officer of the corporation shall, in the performance of his duties, be fully protected in relying in good faith upon the books of account or other records of the corporation, as provided by law, including reports made to the corporation by any of its officers, by an independent certified public accountant, or by an appraiser selected with reasonable care.

5.11 Time Periods. In applying any provision of these Bylaws which require that an act be done or not done a specified number of days prior to an event or that an act be done during a period of a specified number of days prior to an event, calendar days shall be used, the day of the doing of the act shall be excluded, and the day of the event shall be included.

5.12 Facsimile Signatures. In addition to the provisions for use of facsimile signatures elsewhere specifically authorized in these Bylaws, facsimile signatures of any officer or officers of the corporation may be used whenever and as authorized by the Board of Directors or a committee thereof.

5.13 Forum for Certain Actions. Unless the corporation consents in writing to the selection of an alternative forum, the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of the corporation, (ii) any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee of the corporation to the corporation or the corporation's stockholders, (iii) any action asserting a claim against the corporation arising pursuant to any provision of the Delaware General Corporation Law or the corporation's Certificate of Incorporation or Bylaws, or (iv) any action asserting a claim against the corporation governed by the internal affairs doctrine shall be a state or federal court located within the state of Delaware, in all cases subject to the court's having personal jurisdiction over the indispensable parties named as defendants. Any person or entity purchasing or otherwise acquiring any interest in shares of capital stock of the corporation shall be deemed to have notice of and consented to the provisions of this Section 5.13.

ARTICLE VI
AMENDMENTS

6.1 By the Board of Directors. Except as otherwise set forth in these Bylaws, these Bylaws may be altered, amended or repealed or new Bylaws may be adopted by the affirmative vote of a majority of the directors present at any regular or special meeting of the Board of Directors at which a quorum is present.

6.2 By the Stockholders. Except as otherwise set forth in these Bylaws, these Bylaws may be altered, amended or repealed or new Bylaws may be adopted by the affirmative vote of the holders of at least a majority of the voting power of all of the shares of capital stock of the corporation issued and outstanding and entitled to vote generally in any election of directors, voting together as a single class. Such vote may be held at any annual meeting of stockholders, or at any special meeting of stockholders provided that notice of such alteration, amendment, repeal or adoption of new Bylaws shall have been stated in the notice of such special meeting.

ARTICLE VII
INDEMNIFICATION OF DIRECTORS AND OFFICERS

7.1 Right to Indemnification. Each person who was or is made a party or is threatened to be made a party to or is involved in any action, suit or proceeding, whether civil, criminal, administrative or investigative ("proceeding"), by reason of the fact that he or a person of whom he is the legal representative, is or was a director or officer of the corporation or is or was serving at the request of the corporation as a director or officer of another corporation, or as a controlling person of a partnership, joint venture, trust or other enterprise, including service with respect to employee benefit plans, whether the basis of such proceeding is alleged action in an official capacity as a director or officer, or in any other capacity while serving as a director or officer, shall be indemnified and held harmless by the corporation to the fullest extent authorized by the Delaware General Corporation Law, as the same exists or may hereafter be amended (but, in the case of any such amendment, only to the extent that such amendment permits the corporation to provide broader indemnification rights than such law permitted the corporation to provide prior to such amendment) against all expenses, liability and loss reasonably incurred or

suffered by such person in connection therewith and such indemnification shall continue as to a person who has ceased to be a director or officer and shall inure to the benefit of his heirs, executors and administrators; provided, however, that except as provided in Section 7.2 of this Article VII, the corporation shall indemnify any such person seeking indemnity in connection with a proceeding (or part thereof) initiated by such person only if (a) such indemnification is expressly required to be made by law, (b) the proceeding (or part thereof) was authorized by the Board of Directors of the corporation, (c) such indemnification is provided by the corporation, in its sole discretion, pursuant to the powers vested in the corporation under the Delaware General Corporation Law, or (d) the proceeding (or part thereof) is brought to establish or enforce a right to indemnification or advancement under an indemnity agreement or any other statute or law or otherwise as required under Section 145 of the Delaware General Corporation Law. The rights hereunder shall be contract rights and shall include the right to be paid expenses incurred in defending any such proceeding in advance of its final disposition; provided, however, that the payment of such expenses incurred by a director or officer of the corporation in his capacity as a director or officer (and not in any other capacity in which service was or is tendered by such person while a director or officer, including, without limitation, service to an employee benefit plan) in advance of the final disposition of such proceeding, shall be made only upon delivery to the corporation of an undertaking, by or on behalf of such director or officer, to repay all amounts so advanced if it should be determined ultimately by final judicial decision from which there is no further right to appeal that such director or officer is not entitled to be indemnified under this section or otherwise.

7.2 Right of Claimant to Bring Suit. If a claim under Section 7.1 is not paid in full by the corporation within sixty (60) days after a written claim has been received by the corporation, or 20 days in the case of a claim for advancement of expenses, the claimant may at any time thereafter bring suit against the corporation to recover the unpaid amount of the claim and, if such suit is not frivolous or brought in bad faith, the claimant shall be entitled to be paid also the expense of prosecuting such claim. It shall be a defense to any such action (other than an action brought to enforce a claim for expenses incurred in defending any proceeding in advance of its final disposition where the required undertaking, if any, has been tendered to this corporation) that the claimant has not met the standards of conduct which make it permissible under the Delaware General Corporation Law for the corporation to indemnify the claimant for the amount claimed. Neither the failure of the corporation (including its Board of Directors, independent legal counsel, or its stockholders) to have made a determination prior to the commencement of such action that indemnification of the claimant is proper in the circumstances because he has met the applicable standard of conduct set forth in the Delaware General Corporation Law, nor an actual determination by the corporation (including its Board of Directors, independent legal counsel or its stockholders) that the claimant has not met such applicable standard of conduct, shall be a defense to the action or create a presumption that claimant has not met the applicable standard of conduct. In any suit brought by the corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the corporation shall be entitled to recover such expenses upon a final judicial decision from which there is no further right to appeal that the indemnitee has not met any applicable standard for indemnification set forth in the Delaware General Corporation Law. In any suit brought by the indemnitee to enforce a right to indemnification or to an advancement of expenses hereunder, or brought by the corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the burden of proving that the indemnitee is not entitled to be indemnified, or to such advancement of expenses, shall be on the corporation.

7.3 Indemnification of Employees and Agents. The corporation may, to the extent authorized from time to time by the Board of Directors, grant rights to indemnification, and to the advancement of related expenses, to any employee or agent of the corporation to the fullest extent of the provisions of this Article VII with respect to the indemnification of and advancement of expenses to directors and officers of the corporation.

7.4 Non-Exclusivity of Rights. The rights conferred on any person in this Article VII shall not be exclusive of any other right which such persons may have or hereafter acquire under any statute, provision of the Certificate of Incorporation, Bylaw, agreement, vote of stockholders or disinterested directors or otherwise.

7.5 Indemnification Contracts. The Board of Directors is authorized to enter into a contract with any director, officer, employee or agent of the corporation, or any person serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, including employee benefit plans, providing for indemnification rights equivalent to or, if the Board of Directors so determines, greater than, those provided for in this Article VII.

7.6 Insurance. The corporation may maintain insurance to the extent reasonably available, at its expense, to protect itself and any such director, officer, employee or agent of the corporation or another corporation, partnership, joint venture, trust or other enterprise against any such expense, liability or loss, whether or not the corporation would have the power to indemnify such person against such expense, liability or loss under the Delaware General Corporation Law.

7.7 Effect of Amendment. Any amendment, repeal or modification of any provision of this Article VII shall not adversely affect any right or protection of an indemnitee or his successor in respect of any act or omission occurring prior to such amendment, repeal or modification.

[Remainder of page intentionally left blank]

CERTIFICATE BY SECRETARY OF ADOPTION

OF AMENDED AND RESTATED BYLAWS

OF

TAYSHA GENE THERAPIES, INC.

The undersigned hereby certifies that he is the duly elected, qualified and acting Secretary of Taysha Gene Therapies, Inc., a Delaware corporation (the "Corporation"), and that the foregoing Amended and Restated Bylaws were adopted as the Bylaws of the Corporation as of the date first written above by the Board of Directors of the Corporation (the "Board").

That the foregoing Bylaws constitute the Amended and Restated Bylaws of the Corporation duly adopted and approved by written consent of the Board in lieu of a meeting.

IN WITNESS WHEREOF, I have hereunder subscribed my name as of the date first written above.

/s/ R.A. Session II
R.A. Session II, Secretary

Exhibit 4.1

Certain information has been excluded from this agreement (indicated by "[***]") because Taysha Gene Therapies, Inc. has determined such information (i) is not material and (ii) would be competitively harmful if publicly disclosed.

AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

THIS AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT (this "**Agreement**"), is made as of the 2nd day of July, 2020, by and among Taysha Gene Therapies, Inc., a Delaware corporation (the "**Company**"), each of the investors listed on Schedule A hereto, each of which is referred to in this Agreement as an "**Investor**", each of the stockholders listed on Schedule B hereto, each of whom is referred to herein as a "**Key Holder**" and any Additional Purchaser (as defined in the Purchase Agreement) that becomes a party to this Agreement in accordance with Section 6.9 hereof.

RECITALS

WHEREAS, the Key Holders and certain of the Investors (the "**Existing Investors**") hold shares of the Company's Series A Preferred Stock and/or shares of Common Stock and possess registration rights, information rights, rights of first offer, and other rights pursuant to that certain Investors' Rights Agreement dated as of March 4, 2020, by and among the Company and such Existing Investors (the "**Prior Agreement**");

WHEREAS, the Existing Investors and Key Holders are holders of at least a majority of the Registrable Securities of the Company (as defined in the Prior Agreement), and desire to amend and restate the Prior Agreement in its entirety and to accept the rights created pursuant to this Agreement in lieu of the rights granted to them under the Prior Agreement; and

WHEREAS, certain of the Investors are parties to that certain Series B Preferred Stock Purchase Agreement of even date herewith by and among the Company and such Investors (the "**Purchase Agreement**"), under which certain of the Company's and such Investors' obligations are conditioned upon the execution and delivery of this Agreement by such Investors and the Existing Investors and Key Holders holding at least a majority of the Registrable Securities, and the Company.

NOW, THEREFORE, the Key Holders and the Existing Investors hereby agree that the Prior Agreement shall be amended and restated, and the parties hereby agree as follows:

1. Definitions. For purposes of this Agreement:

1.1 "**Affiliate**" means (i) with respect to any specified Person, any other Person who, directly or indirectly, controls, is controlled by, or is under common control with such Person, including without limitation any general partner, managing member, manager, officer or director of such Person or any venture capital fund or other investment fund now or hereafter existing that is controlled by one or more general partners or managing members of, or shares the same management company or investment adviser with, such Person, and (ii) with respect to any specified Person that is a partnership, limited partnership, corporation or limited liability company, any Person that is a partner, general partner, limited partner, shareholder or member thereof. For purposes of this definition, one or more Persons will be deemed to be under common control if they have granted to one of such Persons (whether by agreement, granting of a power-of-attorney, or otherwise) the ability to exercise all rights, receive all notices, and take any action under this Agreement.

1.2 "**Board of Directors**" means the board of directors of the Company.

1.3 "**Certificate of Incorporation**" means the Company's Amended and Restated Certificate of Incorporation, as amended and/or restated from time to time.

1.4 "**Common Stock**" means shares of the Company's common stock, par value $0.00001 per share.

1.5 "**Competitor**" means a Person engaged, directly or indirectly (including through any partnership, limited liability company, corporation, joint venture or similar arrangement (whether now existing or formed hereafter)), in gene therapies for rare diseases, but shall not include (i) PBM, (ii) any Fidelity Investor or (iii) any financial investment firm or collective investment vehicle that, together with its Affiliates, holds less than twenty percent (20%) of the outstanding equity of any Competitor and does not, nor do any of its Affiliates, have a right to designate any members of the board of directors of any Competitor. Competitor specifically excludes the Board of Regents of the University of Texas System (the "**Regents**").

1.6 "**Damages**" means any loss, damage, claim or liability (joint or several) to which a party hereto may become subject under the Securities Act, the Exchange Act, or other federal or state law, insofar as such loss, damage, claim or liability (or any action in respect thereof) arises out of or is based upon: (i) any untrue statement or alleged untrue statement of a material fact contained in any registration statement of the Company, including any preliminary prospectus or final prospectus contained therein or any amendments or supplements thereto; (ii) an omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading; or (iii) any violation or alleged violation by the indemnifying party (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities law, or any rule or regulation promulgated under the Securities Act, the Exchange Act, or any state securities law.

1.7 "**Derivative Securities**" means any securities or rights convertible into, or exercisable or exchangeable for (in each case, directly or indirectly)**,** Common Stock, including options and warrants.

1.8 "**Exchange Act**" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

1.9 "**Excluded Registration**" means (i) a registration relating to the sale or grant of securities to employees of the Company or a subsidiary pursuant to a stock option, stock purchase, equity incentive or similar plan; (ii) a registration relating to an SEC Rule 145 transaction; (iii) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities; or (iv) a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered.

1.10 "**Fidelity**" means Fidelity Management & Research Company and any successor or affiliated registered investment advisor to the Fidelity Investors.

1.11 "**Fidelity Investors**" means the Investors that are advisory or sub-advisory clients of Fidelity.

1.12 "**FOIA Party**" means a Person that, in the determination of the Board of Directors, may be subject to, and thereby required to disclose non-public information furnished by or relating to the Company under, the Freedom of Information Act, 5 U.S.C. 552 ("**FOIA**"), any state public records access law, any state or other jurisdiction's laws similar in intent or effect to FOIA, or any other similar statutory or regulatory requirement.

1.13 "**Form S-1**" means such form under the Securities Act as in effect on the date hereof or any successor registration form under the Securities Act subsequently adopted by the SEC.

1.14 "**Form S-3**" means such form under the Securities Act as in effect on the date hereof or any registration form under the Securities Act subsequently adopted by the SEC that permits forward incorporation of substantial information by reference to other documents filed by the Company with the SEC.

1.15 "**GAAP**" means generally accepted accounting principles in the United States as in effect from time to time.

1.16 "**Holder**" means any holder of Registrable Securities who is a party to this Agreement.

1.17 "**Immediate Family Member**" means a child, stepchild, grandchild, parent, stepparent, grandparent, spouse, sibling, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including, adoptive relationships, of a natural person referred to herein.

1.18 "**Initiating Holders**" means, collectively, Holders who properly initiate a registration request under this Agreement.

1.19 "**IPO**" means the Company's first underwritten public offering of its Common Stock under the Securities Act.

1.20 "**Key Employee**" means R.A. Session II.

1.21 "**Key Holder Registrable Securities**" means (i) the 10,000,000 shares of Common Stock held by the Key Holders as of the date hereof, and (ii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right, or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of such shares.

1.22 "**Major Investor**" means any Investor that, individually or together with such Investor's Affiliates, holds at least 666,666 shares of Registrable Securities (as adjusted for any stock split, stock dividend, combination, or other recapitalization or reclassification effected after the date hereof).

1.23 "**New Securities**" means, collectively, equity securities of the Company, whether or not currently authorized, as well as rights, options, or warrants to purchase such equity securities, or securities of any type whatsoever that are, or may become, convertible or exchangeable into or exercisable for such equity securities.

1.24 "**PBM**" means PBM TGT Holdings, LLC and its Affiliates.

1.25 "**Person**" means any individual, corporation, partnership, trust, limited liability company, association or other entity.

1.26 "**Preferred Stock**" means, collectively, shares of the Company's Series A Preferred Stock and Series B Preferred Stock.

1.27 "**Registrable Securities**" means (i) the Common Stock issuable or issued upon conversion of the Preferred Stock; (ii) any Common Stock, or any Common Stock issued or issuable (directly or indirectly) upon conversion and/or exercise of any other securities of the Company**,** acquired by the Investors after the date hereof; (iii) the Key Holder Registrable Securities, provided, however, that such Key Holder Registrable Securities shall not be deemed Registrable Securities and the Key Holders shall not be deemed Holders for the purposes of Sections 2.1 (and any other applicable Section with respect to registrations under Section 2.1), 2.10, 3.1, 3.2, 4.1 (other than with respect to the Regents so long as the Regents are a Major Investor) and 6.6 (other than with respect to the Regents' rights under Section 6.6(c)); and (iv) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right, or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clauses (i) and (ii) above; excluding in all cases, however, any Registrable Securities sold by a Person in a transaction in which the applicable rights under this Agreement are not assigned pursuant to Section 6.1, and excluding for purposes of Section 2 any shares for which registration rights have terminated pursuant to Section 2.13 of this Agreement.

1.28 "**Registrable Securities then outstanding**" means the number of shares determined by adding the number of shares of outstanding Common Stock that are Registrable Securities and the number of shares of Common Stock issuable (directly or indirectly) pursuant to then exercisable and/or convertible securities that are Registrable Securities.

1.29 "**Restricted Securities**" means the securities of the Company required to be notated with the legend set forth in Section 2.12(b) hereof.

1.30 "**SEC**" means the Securities and Exchange Commission.

1.31 "**SEC Rule 144**" means Rule 144 promulgated by the SEC under the Securities Act.

1.32 "**SEC Rule 145**" means Rule 145 promulgated by the SEC under the Securities Act.

1.33 "**Securities Act**" means the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

1.34 "**Selling Expenses**" means all underwriting discounts, selling commissions, and stock transfer taxes applicable to the sale of Registrable Securities, and fees and disbursements of counsel for any Holder, except for the fees and disbursements of the Selling Holder Counsel borne and paid by the Company as provided in Section 2.6.

1.35 "**Series A Director**" means any director of the Company that the holders of record of the Series A Preferred Stock are entitled to elect, exclusively and as a separate class, pursuant to the Certificate of Incorporation.

1.36 "**Series A Preferred Stock**" means shares of the Company's Series A Preferred Stock, par value $0.00001 per share.

1.37 "**Series B Preferred Stock**" means shares of the Company's Series B Preferred Stock, par value $0.00001 per share.

2. Registration Rights. The Company covenants and agrees as follows:

2.1 Demand Registration.

(a) Form S-1 Demand. If at any time after the earlier of (i) five (5) years after the date of this Agreement or (ii) one hundred eighty (180) days after the effective date of the registration statement for the IPO, the Company receives a request from Holders of a majority of the Registrable Securities then outstanding that the Company file a Form S-1 registration statement with respect to Registrable Securities, the anticipated aggregate offering price, net of Selling Expenses, of which exceeds $10 million, then the Company shall (x) within ten (10) days after the date such request is given, give notice thereof (the "**Demand Notice**") to all Holders other than the Initiating Holders; and (y) as soon as practicable, and in any event within sixty (60) days after the date such request is given by the Initiating Holders, file a Form S-1 registration statement under the Securities Act covering all Registrable Securities that the Initiating Holders requested to be registered and any additional Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Sections 2.1(c) and 2.3.

(b) Form S-3 Demand. If at any time when it is eligible to use a Form S-3 registration statement, the Company receives a request from Holders of at least a majority of the Registrable Securities then outstanding that the Company file a Form S-3 registration statement with respect to outstanding Registrable Securities of such Holders having an anticipated aggregate offering price, net of Selling Expenses, of at least $5 million, then the Company shall (i) within ten (10) days after the date such request is given, give a Demand Notice to all Holders other than the Initiating Holders; and (ii) as soon as practicable, and in any event within forty-five (45) days after the date such request is given by the Initiating Holders, file a Form S-3 registration statement under the Securities Act covering all Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Sections 2.1(c) and 2.3.

(c) Notwithstanding the foregoing obligations, if the Company furnishes to Holders requesting a registration pursuant to this Section 2.1 a certificate signed by the Company's chief executive officer stating that in the good faith judgment of the Board of Directors it would be materially detrimental to the Company and its stockholders for such registration statement to either become effective or remain effective for as long as such registration statement otherwise would be required to remain effective, because such action would (i) materially interfere with a significant acquisition, corporate reorganization, or other similar transaction involving the Company; (ii) require premature disclosure of material information that the Company has a bona fide business purpose for preserving as confidential; or (iii) render the Company unable to comply with requirements under the Securities Act or Exchange Act, then the Company shall have the right to defer taking action with respect to such filing, and any time periods with respect to filing or effectiveness thereof shall be tolled correspondingly, for a period of not more than one hundred twenty (120) days after the request of the Initiating Holders is given; provided, however, that the Company may not invoke this right more than once in any twelve (12) month period; and provided further that the Company shall not register any securities for its own account or that of any other stockholder during such one hundred twenty (120) day period other than an Excluded Registration.

(d) The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Section 2.1(a) (i) during the period that is sixty (60) days before the Company's good faith estimate of the date of filing of, and ending on a date that is one hundred eighty (180) days after the effective date of, a Company-initiated registration, provided that the Company is actively employing in good faith commercially reasonable efforts to cause such registration statement to become effective; (ii) after the Company has effected one (1) registration pursuant to Section 2.1(a); or (iii) if the Initiating Holders propose to dispose of shares of Registrable Securities that may be immediately registered on Form S-3 pursuant to a request made pursuant to Section 2.1(b). The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Section 2.1(b) (i) during the period that is thirty (30) days before the Company's good faith estimate of the date of filing of, and ending on a date that is ninety (90) days after the effective date of, a Company-initiated registration, provided that the Company is actively employing in good faith commercially reasonable efforts to cause such registration statement to become effective; or (ii) if the Company has effected two registrations pursuant to Section 2.1(b) within the twelve (12) month period immediately preceding the date of such request. A registration shall not be counted as "effected" for purposes of this Section 2.1(d) until such time as the applicable registration statement has been declared effective by the SEC, unless the Initiating Holders withdraw their request for such registration, elect not to pay the registration expenses therefor, and forfeit their right to one demand registration statement pursuant to Section 2.6, in which case such withdrawn registration statement shall be counted as "effected" for purposes of this Section 2.1(d); provided, that if such withdrawal is during a period the Company has deferred taking action pursuant to Section 2.1(c), then the Initiating Holders may withdraw their request for registration and such registration will not be counted as "effected" for purposes of this Section 2.1(d).

2.2 Company Registration. If the Company proposes to register (including, for this purpose, a registration effected by the Company for stockholders other than the Holders) any of its Common Stock under the Securities Act in connection with the public offering of such securities solely for cash (other than in an Excluded Registration), the Company shall, at such time,

promptly give each Holder notice of such registration. Upon the request of each Holder given within twenty (20) days after such notice is given by the Company, the Company shall, subject to the provisions of Section 2.3, cause to be registered all of the Registrable Securities that each such Holder has requested to be included in such registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Section 2.2 before the effective date of such registration, whether or not any Holder has elected to include Registrable Securities in such registration. The expenses (other than Selling Expenses) of such withdrawn registration shall be borne by the Company in accordance with Section 2.6.

2.3 Underwriting Requirements.

(a) If, pursuant to Section 2.1, the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to Section 2.1, and the Company shall include such information in the Demand Notice. The underwriter(s) will be selected by the Board of Directors and shall be reasonably acceptable to a majority in interest of the Initiating Holders. In such event, the right of any Holder to include such Holder's Registrable Securities in such registration shall be conditioned upon such Holder's participation in such underwriting and the inclusion of such Holder's Registrable Securities in the underwriting to the extent provided herein. All Holders proposing to distribute their securities through such underwriting shall (together with the Company as provided in Section 2.4(e)) enter into an underwriting agreement in customary form with the underwriter(s) selected for such underwriting. Notwithstanding any other provision of this Section 2.3, if the managing underwriter(s) advise the Initiating Holders in writing that marketing factors require a limitation on the number of shares to be underwritten, then the Initiating Holders shall so advise all Holders of Registrable Securities that otherwise would be underwritten pursuant hereto, and the number of Registrable Securities that may be included in the underwriting shall be allocated among such Holders of Registrable Securities, including the Initiating Holders, in proportion (as nearly as practicable) to the number of Registrable Securities owned by each Holder or in such other proportion as shall mutually be agreed to by all such selling Holders; provided, however, that the number of Registrable Securities held by the Holders to be included in such underwriting shall not be reduced unless Key Holder Registrable Securities and all other securities are first entirely excluded from the underwriting. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest one hundred (100) shares.

(b) In connection with any offering involving an underwriting of shares of the Company's capital stock pursuant to Section 2.2, the Company shall not be required to include any of the Holders' Registrable Securities in such underwriting unless the Holders accept the terms of the underwriting as agreed upon between the Company and its underwriters, and then only in such quantity as the underwriters in their sole discretion determine will not jeopardize the success of the offering by the Company. If the total number of securities, including Registrable Securities, requested by stockholders to be included in such offering exceeds the number of securities to be sold (other than by the Company) that the underwriters in their reasonable discretion determine is compatible with the success of the offering, then the Company shall be required to include in the offering only that number of such securities, including Registrable Securities, which the underwriters and the Company in their sole discretion determine will not jeopardize the success of the offering. If the underwriters determine that less than all of the

Registrable Securities requested to be registered can be included in such offering, then the Registrable Securities that are included in such offering shall be allocated among the selling Holders in proportion (as nearly as practicable to) the number of Registrable Securities owned by each selling Holder or in such other proportions as shall mutually be agreed to by all such selling Holders. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest one hundred (100) shares. Notwithstanding the foregoing, in no event shall (i) the number of Registrable Securities included in the offering be reduced unless all other securities (other than securities to be sold by the Company) are first entirely excluded from the offering, (ii) the number of Registrable Securities included in the offering be reduced below thirty percent (30%) of the total number of securities included in such offering, unless such offering is the IPO, in which case the selling Holders may be excluded further if the underwriters make the determination described above and no other stockholder's securities are included in such offering, or (iii) notwithstanding (ii) above, any Registrable Securities which are not Key Holder Registrable Securities be excluded from such underwriting unless all Key Holder Registrable Securities are first excluded from such offering. For purposes of the provision in this Section 2.3(b) concerning apportionment, for any selling Holder that is a partnership, limited liability company, or corporation, the partners, members, retired partners, retired members, stockholders, and Affiliates of such Holder, or the estates and Immediate Family Members of any such partners, retired partners, members, and retired members and any trusts for the benefit of any of the foregoing Persons, shall be deemed to be a single "selling Holder," and any pro rata reduction with respect to such "selling Holder" shall be based upon the aggregate number of Registrable Securities owned by all Persons included in such "selling Holder," as defined in this sentence.

(c) For purposes of Section 2.1, a registration shall not be counted as "effected" if, as a result of an exercise of the underwriter's cutback provisions in Section 2.3(a), fewer than fifty percent (50%) of the total number of Registrable Securities that Holders have requested to be included in such registration statement are actually included.

2.4 Obligations of the Company. Whenever required under this Section 2 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a registration statement with respect to such Registrable Securities and use its commercially reasonable efforts to cause such registration statement to become effective and, upon the request of the Holders of a majority of the Registrable Securities registered thereunder, keep such registration statement effective for a period of up to one hundred twenty (120) days or, if earlier, until the distribution contemplated in the registration statement has been completed; provided, however, that (i) such one hundred twenty (120) day period shall be extended for a period of time equal to the period the Holder refrains, at the request of an underwriter of Common Stock (or other securities) of the Company, from selling any securities included in such registration;

(b) prepare and file with the SEC such amendments and supplements to such registration statement, and the prospectus used in connection with such registration statement, as may be necessary to comply with the Securities Act in order to enable the disposition of all securities covered by such registration statement;

(c) furnish to the selling Holders such numbers of copies of a prospectus, including a preliminary prospectus, as required by the Securities Act, and such other documents as the Holders may reasonably request in order to facilitate their disposition of their Registrable Securities;

(d) use its commercially reasonable efforts to register and qualify the securities covered by such registration statement under such other securities or blue-sky laws of such jurisdictions as shall be reasonably requested by the selling Holders; provided that the Company shall not be required to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, unless the Company is already subject to service in such jurisdiction and except as may be required by the Securities Act;

(e) in the event of any underwritten public offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the underwriter(s) of such offering;

(f) use its commercially reasonable efforts to cause all such Registrable Securities covered by such registration statement to be listed on a national securities exchange or trading system and each securities exchange and trading system (if any) on which similar securities issued by the Company are then listed;

(g) provide a transfer agent and registrar for all Registrable Securities registered pursuant to this Agreement and provide a CUSIP number for all such Registrable Securities, in each case not later than the effective date of such registration;

(h) promptly make available for inspection by the selling Holders, any underwriter(s) participating in any disposition pursuant to such registration statement, and any attorney or accountant or other agent retained by any such underwriter or selected by the selling Holders, all financial and other records, pertinent corporate documents, and properties of the Company, and cause the Company's officers, directors, employees, and independent accountants to supply all information reasonably requested by any such seller, underwriter, attorney, accountant, or agent, in each case, as necessary or advisable to verify the accuracy of the information in such registration statement and to conduct appropriate due diligence in connection therewith;

(i) notify each selling Holder, promptly after the Company receives notice thereof, of the time when such registration statement has been declared effective or a supplement to any prospectus forming a part of such registration statement has been filed; and

(j) after such registration statement becomes effective, notify each selling Holder of any request by the SEC that the Company amend or supplement such registration statement or prospectus.

In addition, the Company shall ensure that, at all times after any registration statement covering a public offering of securities of the Company under the Securities Act shall have become effective, its insider trading policy shall provide that the Company's directors may implement a trading program under Rule 10b5-1 of the Exchange Act.

2.5 Furnish Information. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 2 with respect to the Registrable Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself, the Registrable Securities held by it, and the intended method of disposition of such securities as is reasonably required to effect the registration of such Holder's Registrable Securities.

2.6 Expenses of Registration. All expenses (other than Selling Expenses) incurred in connection with registrations, filings, or qualifications pursuant to Section 2, including all registration, filing, and qualification fees; printers' and accounting fees; fees and disbursements of counsel for the Company; and the reasonable fees and disbursements, not to exceed $50,000, of one counsel for the selling Holders ("**Selling Holder Counsel**"), shall be borne and paid by the Company; provided, however, that the Company shall not be required to pay for any expenses of any registration proceeding begun pursuant to Section 2.1 if the registration request is subsequently withdrawn at the request of the Holders of a majority of the Registrable Securities to be registered (in which case all selling Holders shall bear such expenses pro rata based upon the number of Registrable Securities that were to be included in the withdrawn registration), unless the Holders of a majority of the Registrable Securities agree to forfeit their right to one registration pursuant to Sections 2.1(a) or 2.1(b), as the case may be; provided further that if, at the time of such withdrawal, the Holders shall have learned of a material adverse change in the condition, business, or prospects of the Company from that known to the Holders at the time of their request and have withdrawn the request with reasonable promptness after learning of such information then the Holders shall not be required to pay any of such expenses and shall not forfeit their right to one registration pursuant to Sections 2.1(a) or 2.1(b). All Selling Expenses relating to Registrable Securities registered pursuant to this Section 2 shall be borne and paid by the Holders pro rata on the basis of the number of Registrable Securities registered on their behalf.

2.7 Delay of Registration. No Holder shall have any right to obtain or seek an injunction restraining or otherwise delaying any registration pursuant to this Agreement as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 2.

2.8 Indemnification. If any Registrable Securities are included in a registration statement under this Section 2:

(a) To the extent permitted by law, the Company will indemnify and hold harmless each selling Holder, and the partners, members, officers, directors, and stockholders of each such Holder; legal counsel, accountants and investment advisers for each such Holder; any underwriter (as defined in the Securities Act) for each such Holder; and each Person, if any, who controls such Holder or underwriter within the meaning of the Securities Act or the Exchange Act, against any Damages, and the Company will pay to each such Holder, underwriter, controlling Person, or other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Section 2.8(a) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Company, which consent shall not be unreasonably withheld, nor shall the Company be liable for any Damages to the extent that they

arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of any such Holder, underwriter, controlling Person, or other aforementioned Person expressly for use in connection with such registration.

(b) To the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, and each of its directors, each of its officers who has signed the registration statement, each Person (if any), who controls the Company within the meaning of the Securities Act, legal counsel and accountants for the Company, any underwriter (as defined in the Securities Act), any other Holder selling securities in such registration statement, and any controlling Person of any such underwriter or other Holder, against any Damages, in each case only to the extent that such Damages arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of such selling Holder expressly for use in connection with such registration; and each such selling Holder will pay to the Company and each other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Section 2.8(b) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Holder, which consent shall not be unreasonably withheld; and provided further that in no event shall the aggregate amounts payable by any Holder by way of indemnity or contribution under Sections 2.8(b) and 2.8(d) exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of fraud or willful misconduct by such Holder.

(c) Promptly after receipt by an indemnified party under this Section 2.8 of notice of the commencement of any action (including any governmental action) for which a party may be entitled to indemnification hereunder, such indemnified party will, if a claim in respect thereof is to be made against any indemnifying party under this Section 2.8, give the indemnifying party notice of the commencement thereof. The indemnifying party shall have the right to participate in such action and, to the extent the indemnifying party so desires, participate jointly with any other indemnifying party to which notice has been given, and to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain one separate counsel, with the fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such action. The failure to give notice to the indemnifying party within a reasonable time of the commencement of any such action shall relieve such indemnifying party of any liability to the indemnified party under this Section 2.8, only to the extent that such failure materially prejudices the indemnifying party's ability to defend such action. The failure to give notice to the indemnifying party will not relieve it of any liability that it may have to any indemnified party otherwise than under this Section 2.8.

(d) To provide for just and equitable contribution to joint liability under the Securities Act in any case in which either: (i) any party otherwise entitled to indemnification hereunder makes a claim for indemnification pursuant to this Section 2.8 but it is judicially

determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case, notwithstanding the fact that this Section 2.8 provides for indemnification in such case, or (ii) contribution under the Securities Act may be required on the part of any party hereto for which indemnification is provided under this Section 2.8, then, and in each such case, such parties will contribute to the aggregate losses, claims, damages, liabilities, or expenses to which they may be subject (after contribution from others) in such proportion as is appropriate to reflect the relative fault of each of the indemnifying party and the indemnified party in connection with the statements, omissions, or other actions that resulted in such loss, claim, damage, liability, or expense, as well as to reflect any other relevant equitable considerations. The relative fault of the indemnifying party and of the indemnified party shall be determined by reference to, among other things, whether the untrue or allegedly untrue statement of a material fact, or the omission or alleged omission of a material fact, relates to information supplied by the indemnifying party or by the indemnified party and the parties' relative intent, knowledge, access to information, and opportunity to correct or prevent such statement or omission; provided, however, that, in any such case (x) no Holder will be required to contribute any amount in excess of the public offering price of all such Registrable Securities offered and sold by such Holder pursuant to such registration statement, and (y) no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; and provided further that in no event shall a Holder's liability pursuant to this Section 2.8(d), when combined with the amounts paid or payable by such Holder pursuant to Section 2.8(b), exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of willful misconduct or fraud by such Holder.

(e) Notwithstanding the foregoing, to the extent that any provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with the underwritten public offering are in conflict with the foregoing provisions, such provisions in the underwriting agreement shall control.

(f) Unless otherwise superseded by an underwriting agreement entered into in connection with the underwritten public offering, the obligations of the Company and Holders under this Section 2.8 shall survive the completion of any offering of Registrable Securities in a registration under this Section 2, and otherwise shall survive the termination of this Agreement.

2.9 Reports Under Exchange Act. With a view to making available to the Holders the benefits of SEC Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell securities of the Company to the public without registration or pursuant to a registration on Form S-3, the Company shall:

(a) make and keep available adequate current public information, as those terms are understood and defined in SEC Rule 144, at all times after the effective date of the registration statement filed by the Company for the IPO;

(b) use commercially reasonable efforts to file with the SEC in a timely manner all reports and other documents required of the Company under the Securities Act and the Exchange Act (at any time after the Company has become subject to such reporting requirements); and

(c) furnish to any Holder, so long as the Holder owns any Registrable Securities, forthwith upon request (i) to the extent accurate, a written statement by the Company that it has complied with the reporting requirements of SEC Rule 144 (at any time after ninety (90) days after the effective date of the registration statement filed by the Company for the IPO), the Securities Act, and the Exchange Act (at any time after the Company has become subject to such reporting requirements), or that it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after the Company so qualifies); and (ii) such other information as may be reasonably requested in availing any Holder of any rule or regulation of the SEC that permits the selling of any such securities without registration (at any time after the Company has become subject to the reporting requirements under the Exchange Act) or pursuant to Form S-3 (at any time after the Company so qualifies to use such form).

2.10 Limitations on Subsequent Registration Rights. From and after the date of this Agreement, the Company shall not, without the prior written consent of the Holders of a majority of the Registrable Securities then outstanding, enter into any agreement with any holder or prospective holder of any securities of the Company that would (i) provide to such holder or prospective holder the right to include securities in any registration on other than a subordinate basis after all Holders have had the opportunity to include in the registration and offering all shares of Registrable Securities that they wish to so include; or (ii) allow such holder or prospective holder to initiate a demand for registration of any securities held by such holder or prospective holder; provided that this limitation shall not apply to Registrable Securities acquired by any additional Investor that becomes a party to this Agreement in accordance with Section 6.9.

2.11 "Market Stand-off" Agreement. Each Holder hereby agrees that it will not, without the prior written consent of the managing underwriter, during the period commencing on the date of the final prospectus relating to the IPO, and ending on the date specified by the Company and the managing underwriter (such period not to exceed one hundred eighty (180) days), (i) lend; offer; pledge; sell; contract to sell; sell any option or contract to purchase; purchase any option or contract to sell; grant any option, right, or warrant to purchase; or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable (directly or indirectly) for Common Stock held immediately before the effective date of the registration statement for the IPO or (ii) enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of such securities, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or other securities, in cash, or otherwise. The foregoing provisions of this Section 2.11 (A) shall apply only to the IPO, (B) shall not apply to the sale of shares acquired in the IPO or in the open market on or after the IPO, (C) shall not apply to the sale of any shares to an underwriter pursuant to an underwriting agreement, or the transfer of any shares to any trust for the direct or indirect benefit of the Holder or the immediate family of the Holder, provided that the trustee of the trust agrees to be bound in writing by the restrictions set forth herein, and provided further that any such transfer shall not involve a disposition for value, and (D) shall be applicable to the Holders only if all officers and directors and all stockholders individually owning more than one percent (1%) of the Company's outstanding Common Stock (after giving effect to conversion into Common Stock of all outstanding Preferred Stock) are

subject to the same restrictions. The underwriters in connection with such registration are intended third-party beneficiaries of this Section 2.11 and shall have the right, power and authority to enforce the provisions hereof as though they were a party hereto. Each Holder further agrees to execute such agreements as may be reasonably requested by the underwriters in connection with such registration that are consistent with this Section 2.11 or that are necessary to give further effect thereto. In the event that the Company or the managing underwriter waives or terminates any of the restrictions contained in this Section 2.11 or in a lock-up agreement with respect to the securities of any Holder, officer, director or greater than one-percent stockholder of the Company (in any such case, the "**Released Securities**"), the restrictions contained in this Section 2.11 and in any lock-up agreements executed by the Investors shall be waived or terminated, as applicable, to the same extent and with respect to the same percentage of securities of each Investor as the percentage of Released Securities represent with respect to the securities held by the applicable Holder, officer, director or greater than one-percent stockholder.

2.12 Restrictions on Transfer.

(a) The Preferred Stock and the Registrable Securities shall not be sold, pledged, or otherwise transferred, and the Company shall not recognize and shall issue stop-transfer instructions to its transfer agent with respect to any such sale, pledge, or transfer, except upon the conditions specified in this Agreement, which conditions are intended to ensure compliance with the provisions of the Securities Act. A transferring Holder will cause any proposed purchaser, pledgee, or transferee of the Preferred Stock and the Registrable Securities held by such Holder to agree to take and hold such securities subject to the provisions and upon the conditions specified in this Agreement. Notwithstanding the foregoing, the Company shall not require any transferee of shares pursuant to an effective registration statement or, following the IPO, SEC Rule 144 to be bound by the terms of this Agreement.

(b) Each certificate, instrument, or book entry representing (i) the Preferred Stock, (ii) the Registrable Securities, and (iii) any other securities issued in respect of the securities referenced in clauses (i) and (ii), upon any stock split, stock dividend, recapitalization, merger, consolidation, or similar event, shall (unless otherwise permitted by the provisions of Section 2.12(c)) be notated with a legend substantially in the following form:

THE SECURITIES REPRESENTED HEREBY HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. SUCH SHARES MAY NOT BE SOLD, PLEDGED, OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR A VALID EXEMPTION FROM THE REGISTRATION AND PROSPECTUS DELIVERY REQUIREMENTS OF SAID ACT.

THE SECURITIES REPRESENTED HEREBY MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY.

The Holders consent to the Company making a notation in its records and giving instructions to any transfer agent of the Restricted Securities in order to implement the restrictions on transfer set forth in this Section 2.12.

(c) The holder of such Restricted Securities, by acceptance of ownership thereof, agrees to comply in all respects with the provisions of this Section 2. Before any proposed sale, pledge, or transfer of any Restricted Securities, unless there is in effect a registration statement under the Securities Act covering the proposed transaction or, following the IPO, the transfer is made pursuant to SEC Rule 144, the Holder thereof shall give notice to the Company of such Holder's intention to effect such sale, pledge, or transfer. Each such notice shall describe the manner and circumstances of the proposed sale, pledge, or transfer in sufficient detail and, if reasonably requested by the Company, shall be accompanied at such Holder's expense by either (i) a written opinion of legal counsel who shall, and whose legal opinion shall, be reasonably satisfactory to the Company, addressed to the Company, to the effect that the proposed transaction may be effected without registration under the Securities Act; (ii) a "no action" letter from the SEC to the effect that the proposed sale, pledge, or transfer of such Restricted Securities without registration will not result in a recommendation by the staff of the SEC that action be taken with respect thereto; or (iii) any other evidence reasonably satisfactory to counsel to the Company to the effect that the proposed sale, pledge, or transfer of the Restricted Securities may be effected without registration under the Securities Act, whereupon the Holder of such Restricted Securities shall be entitled to sell, pledge, or transfer such Restricted Securities in accordance with the terms of the notice given by the Holder to the Company. The Company will not require such a legal opinion or "no action" letter (x) in any transaction in compliance with SEC Rule 144; or (y) in any transaction in which such Holder distributes Restricted Securities to an Affiliate of such Holder for no consideration; provided that, other than in connection with a transaction in compliance with SEC Rule 144 following the IPO, each transferee agrees in writing to be subject to the terms of this Section 2.12. Each certificate, instrument, or book entry representing the Restricted Securities transferred as above provided shall be notated with, except if such transfer is made pursuant to SEC Rule 144 or pursuant to an effective registration statement, the appropriate restrictive legend set forth in Section 2.12(b), except that such certificate instrument, or book entry shall not be notated with such restrictive legend if, in the opinion of counsel for such Holder and the Company, such legend is not required in order to establish compliance with any provisions of the Securities Act.

2.13 Termination of Registration Rights. The right of any Holder to request registration or inclusion of Registrable Securities in any registration pursuant to Sections 2.1 or 2.2 shall terminate upon the earliest to occur of:

(a) the closing of a Deemed Liquidation Event, as such term is defined in the Certificate of Incorporation;

(b) such time after consummation of the IPO as Rule 144 or another similar exemption under the Securities Act is available for the sale of all of such Holder's shares without limitation during a three-month period without registration; and

(c) the third (3rd) anniversary of the IPO.

3. Information Rights.

3.1 Delivery of Financial Statements. The Company shall deliver to each Major Investor, provided that the Board of Directors has not reasonably determined that such Major Investor is a Competitor of the Company:

(a) as soon as practicable, but in any event within one-hundred twenty (120) days after the end of each fiscal year of the Company (i) a balance sheet as of the end of such year, (ii) statements of income and of cash flows for such year, and (iii) a statement of stockholders' equity as of the end of such year, all such financial statements audited and certified by independent public accountants of regionally recognized standing selected by the Company;

(b) as soon as practicable, but in any event within forty-five (45) days after the end of each of the first three (3) quarters of each fiscal year of the Company, unaudited statements of income and cash flows for such fiscal quarter, and an unaudited balance sheet as of the end of such fiscal quarter, all prepared in accordance with GAAP (except that such financial statements may (i) be subject to normal year-end audit adjustments; and (ii) not contain all notes thereto that may be required in accordance with GAAP);

(c) as soon as practicable, but in any event within forty-five (45) days after the end of each quarter of each fiscal year of the Company, a statement showing the number of shares of each class and series of capital stock and securities convertible into or exercisable for shares of capital stock outstanding at the end of the period, the Common Stock issuable upon conversion or exercise of any outstanding securities convertible or exercisable for Common Stock and the exchange ratio or exercise price applicable thereto, and the number of shares of issued stock options and stock options not yet issued but reserved for issuance, if any, all in sufficient detail as to permit the Holders to calculate their respective percentage equity ownership in the Company, and certified by the controller of the Company as being true, complete, and correct;

(d) as soon as practicable, but in any event thirty (30) days before the end of each fiscal year, a budget and business plan for the next fiscal year (collectively, the "**Budget**"), approved by the Board of Directors and prepared on a monthly basis, including balance sheets, income statements, and statements of cash flow for such months and, promptly after prepared, any other budgets or revised budgets prepared by the Company; and

(e) such other information relating to the financial condition, business, prospects, or corporate affairs of the Company as any Major Investor may from time to time reasonably request; provided, however, that the Company shall not be obligated under this Section 3.1(d) to provide information (i) that the Company reasonably determines in good faith to be a trade secret or confidential information (unless covered by an enforceable confidentiality agreement, in a form acceptable to the Company); or (ii) the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.

If, for any period, the Company has any subsidiary whose accounts are consolidated with those of the Company, then in respect of such period the financial statements delivered pursuant to the foregoing sections shall be the consolidated and consolidating financial statements of the Company and all such consolidated subsidiaries.

Notwithstanding anything else in this Section 3.1 to the contrary, the Company may cease providing the information set forth in this Section 3.1 during the period starting with the date sixty (60) days before the Company's good-faith estimate of the date of filing of a registration statement if it reasonably concludes it must do so to comply with the SEC rules applicable to such registration statement and related offering; provided that the Company's covenants under this Section 3.1 shall be reinstated at such time as the Company is no longer actively employing its commercially reasonable efforts to cause such registration statement to become effective.

3.2 Inspection. The Company shall permit each Major Investor (provided that the Board of Directors has not reasonably determined that such Major Investor is a Competitor of the Company), at such Major Investor's expense, to visit and inspect the Company's properties; examine its books of account and records; and discuss the Company's affairs, finances, and accounts with its officers, during normal business hours of the Company as may be reasonably requested by the Major Investor; provided, however, that the Company shall not be obligated pursuant to this Section 3.2 to provide access to any information that it reasonably and in good faith considers to be a trade secret or confidential information (unless covered by an enforceable confidentiality agreement, in form acceptable to the Company) or the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.

3.3 Termination of Information Rights. The covenants set forth in Section 3.1 and Section 3.2 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, or (iii) upon the closing of a Deemed Liquidation Event, as such term is defined in the Certificate of Incorporation, whichever event occurs first; provided, however, that in the event the covenants set forth in Section 3.1 terminate upon a Deemed Liquidation Event, if the consideration received by the Investors in such Deemed Liquidation Event is not solely in the form of cash and/or publicly traded securities, the Company will use commercially reasonable efforts to ensure that the Major Investors receive financial information from the acquiring company or other successor to the Company comparable to those set forth in Section 3.1.

3.4 Confidentiality. Each Investor agrees that such Investor will keep confidential and will not disclose, divulge, or use for any purpose (other than to monitor its investment in the Company) any confidential information obtained from the Company pursuant to the terms of this Agreement (including notice of the Company's intention to file a registration statement), unless such confidential information (a) is known or becomes known to the public in general (other than as a result of a breach of this Section 3.4 by such Investor), (b) is or has been independently developed or conceived by such Investor without use of the Company's confidential information, or (c) is or has been made known or disclosed to such Investor by a third party without a breach of any obligation of confidentiality such third party may have to the Company; provided, however, that an Investor may disclose confidential information (i) to its attorneys, accountants, consultants, and other professionals to the extent necessary to obtain their services in connection with monitoring its investment in the Company; (ii) to any prospective purchaser of any Registrable Securities from such Investor, if such prospective purchaser agrees to be bound by the provisions of this Section 3.4; (iii) to any Affiliate, partner, member, stockholder, or wholly owned subsidiary of such Investor in the ordinary course of business, provided that such Investor informs such Person that such information is confidential and directs such Person to maintain the

confidentiality of such information; or (iv) as may otherwise be required by law, regulation, rule, court order or subpoena, provided that such Investor promptly notifies the Company of such disclosure and takes reasonable steps to minimize the extent of any such required disclosure.

4. Rights to Future Stock Issuances.

4.1 Right of First Offer. Subject to the terms and conditions of this Section 4.1 and applicable securities laws, if the Company proposes to offer or sell any New Securities, the Company shall first offer such New Securities to each Major Investor. A Major Investor shall be entitled to apportion the right of first offer hereby granted to it in such proportions as it deems appropriate, among (i) itself, (ii) its Affiliates, and (iii) its beneficial interest holders, such as limited partners, members or any other Person having "beneficial ownership," as such term is defined in Rule 13d-3 promulgated under the Exchange Act, of such Major Investor; provided that each such Affiliate (x) is not a Competitor or FOIA Party, unless such party's purchase of New Securities is otherwise consented to by the Board of Directors, and (y) agrees to enter into this Agreement and each of the Amended and Restated Voting Agreement and Amended and Restated Right of First Refusal and Co-Sale Agreement of even date herewith among the Company, the Investors and the other parties named therein, as an "**Investor**" under each such agreement (provided that any FOIA Party shall not be entitled to any rights as a Major Investor under Sections 3.1, 3.2 and 4.1 hereof).

(a) The Company shall give notice (the "**Offer Notice**") to each Major Investor, stating (i) its bona fide intention to offer such New Securities, (ii) the number of such New Securities to be offered, and (iii) the price and terms, if any, upon which it proposes to offer such New Securities.

(b) By notification to the Company within twenty (20) days after the Offer Notice is given, each Major Investor may elect to purchase or otherwise acquire, at the price and on the terms specified in the Offer Notice, up to that portion of such New Securities which equals the proportion that the Common Stock then held by such Major Investor (including all shares of Common Stock then issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of the Preferred Stock and any other Derivative Securities then held by such Major Investor) bears to the total Common Stock of the Company then outstanding (assuming full conversion and/or exercise, as applicable, of all Preferred Stock and any other Derivative Securities then outstanding). At the expiration of such twenty (20) day period, the Company shall promptly notify each Major Investor that elects to purchase or acquire all the shares available to it (each, a "**Fully Exercising Investor**") of any other Major Investor's failure to do likewise. During the ten (10) day period commencing after the Company has given such notice, each Fully Exercising Investor may, by giving notice to the Company, elect to purchase or acquire, in addition to the number of shares specified above, up to that portion of the New Securities for which Major Investors were entitled to subscribe but that were not subscribed for by the Major Investors which is equal to the proportion that the Common Stock issued and held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of Preferred Stock and any other Derivative Securities then held, by such Fully Exercising Investor bears to the Common Stock issued and held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of the Preferred Stock and any other Derivative Securities then held, by all Fully Exercising Investors who wish to purchase such unsubscribed shares. The closing of any sale pursuant to this Section 4.1(b) shall occur within the later of ninety (90) days of the date that the Offer Notice is given and the date of initial sale of New Securities pursuant to Section 4.1(c).

(c) If all New Securities referred to in the Offer Notice are not elected to be purchased or acquired as provided in Section 4.1(b), the Company may, during the ninety (90) day period following the expiration of the periods provided in Section 4.1(b), offer and sell the remaining unsubscribed portion of such New Securities to any Person or Persons at a price not less than, and upon terms no more favorable to the offeree than, those specified in the Offer Notice. If the Company does not enter into an agreement for the sale of the New Securities within such period, or if such agreement is not consummated within thirty (30) days of the execution thereof, the right provided hereunder shall be deemed to be revived and such New Securities shall not be offered unless first reoffered to the Major Investors in accordance with this Section 4.1.

(d) The right of first offer in this Section 4.1 shall not be applicable to (i) Exempted Securities (as defined in the Certificate of Incorporation); (ii) shares of Common Stock issued in the IPO; and (iii) the issuance of shares of Series B Preferred Stock to Purchasers pursuant to Section 1.3 of the Purchase Agreement.

(e) Notwithstanding any provision hereof to the contrary, in lieu of complying with the provisions of this Section 4.1, the Company may elect to give notice to the Major Investors within thirty (30) days after the issuance of New Securities. Such notice shall describe the type, price, and terms of the New Securities. Each Major Investor shall have twenty (20) days from the date notice is given to elect to purchase up to the number of New Securities that would, if purchased by such Major Investor, maintain such Major Investor's percentage-ownership position, calculated as set forth in Section 4.1(b) before giving effect to the issuance of such New Securities. The closing of such sale shall occur within sixty (60) days of the date notice is given to the Major Investors.

4.2 Termination. The covenants set forth in Section 4.1 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, or (ii) upon the closing of a Deemed Liquidation Event, as such term is defined in the Certificate of Incorporation, whichever event occurs first.

5. Additional Covenants.

5.1 Insurance. The Company shall use its commercially reasonable efforts to maintain, from financially sound and reputable insurers Directors and Officers liability insurance and term "key-person" insurance on R.A. Session II, in an amount and on terms and conditions satisfactory to the Board of Directors, until such time as the Board of Directors determines that such insurance should be discontinued. The key-person policy shall name the Company as loss payee, and neither policy shall be cancelable by the Company without prior approval by the Board of Directors. Each Key Holder (other than the Regents) hereby covenants and agrees that, to the extent such Key Holder is named under such key-person policy, such Key Holder will execute and deliver to the Company, as reasonably requested, a written notice and consent form with respect to such policy.

5.2 Employee Agreements. The Company will cause (i) each Person now or hereafter employed by it or by any subsidiary (or engaged by the Company or any subsidiary as a consultant/independent contractor) with access to confidential information and/or trade secrets to enter into a nondisclosure and proprietary rights assignment agreement; and (ii) each Key Employee to enter into a noncompetition and nonsolicitation agreement, substantially in the form approved by the Board of Directors with a term not to exceed one year. In addition, the Company shall not amend, modify, terminate, waive, or otherwise alter, in whole or in part, any of the above-referenced agreements or any restricted stock agreement between the Company and any employee, without the consent of at least one of the Series A Directors.

5.3 Employee Stock. Unless otherwise approved by the Board of Directors, including a Series A Director, all future employees and consultants of the Company who purchase, receive options to purchase, or receive awards of shares of the Company's capital stock after the date hereof shall be required to execute restricted stock or option agreements, as applicable, providing for (i) vesting of shares over a four (4) year period, with the first twenty-five percent (25%) of such shares vesting following twelve (12) months of continued employment or service, and the remaining shares vesting in equal monthly installments over the following thirty-six (36) months, and (ii) a market stand-off provision substantially similar to that in Section 2.11. Without the prior approval by the Board of Directors, including a Series A Director, the Company shall not amend, modify, terminate, waive or otherwise alter, in whole or in part, any stock purchase, stock restriction or option agreement with any existing employee or service provider if such amendment would cause it to be inconsistent with this Section 5.3. In addition, unless otherwise approved by the Board of Directors, including a Series A Director, the Company shall retain (and not waive) a "right of first refusal" on employee transfers until the Company's IPO and shall have the right to repurchase unvested shares at cost upon termination of employment of a holder of restricted stock.

5.4 Matters Requiring Investor Director Approval. So long as the holders of Series A Preferred Stock are entitled to elect any Series A Directors, the Company hereby covenants and agrees with each of the Investors that it shall not, without approval of the Board of Directors, which approval must include the affirmative vote of at least one (1) of the Series A Directors:

(a) make, or permit any subsidiary to make, any loan or advance to, or own any stock or other securities of, any subsidiary or other corporation, partnership, or other entity unless it is wholly owned by the Company;

(b) make, or permit any subsidiary to make, any loan or advance to any Person, including, without limitation, any employee or director of the Company or any subsidiary, except advances and similar expenditures in the ordinary course of business or under the terms of an employee stock or option plan approved by the Board of Directors;

(c) guarantee, directly or indirectly, or permit any subsidiary to guarantee, directly or indirectly, any indebtedness except for trade accounts of the Company or any subsidiary arising in the ordinary course of business;

(d) make any investment inconsistent with any investment policy approved by the Board of Directors;

(e) incur any aggregate indebtedness in excess of $100,000 that is not already included in a budget approved by the Board of Directors, other than trade credit incurred in the ordinary course of business;

(f) otherwise enter into or be a party to any transaction with any director, officer, or employee of the Company or any "associate" (as defined in Rule 12b-2 promulgated under the Exchange Act) of any such Person, including without limitation any "management bonus" or similar plan providing payments to employees in connection with a Deemed Liquidation Event, as such term is defined in the Certificate of Incorporation, except for (i) transactions contemplated by this Agreement and the Purchase Agreement or (ii) transactions made in the ordinary course of business and pursuant to reasonable requirements of the Company's business and upon fair and reasonable terms that are approved by a majority of the Board of Directors;

(g) hire, terminate, or change the compensation of the executive officers, including approving any option grants or stock awards to executive officers;

(h) change the principal business of the Company, enter new lines of business, or exit the current line of business;

(i) sell, assign, license, pledge, or encumber material technology or intellectual property, other than licenses granted in the ordinary course of business; or

(j) enter into any corporate strategic relationship involving the payment, contribution, or assignment by the Company or to the Company of money or assets greater than $100,000.

5.5 Board Matters. Unless otherwise determined by the vote of a majority of the directors then in office, the Board of Directors shall meet at least quarterly in accordance with an agreed-upon schedule. The Company shall reimburse the directors for all reasonable out-of-pocket travel expenses incurred (consistent with the Company's travel policy) in connection with attending meetings of the Board of Directors. Each Series A Director shall be entitled in such person's discretion to be a member of any committee of the Board of Directors.

5.6 Successor Indemnification. If the Company or any of its successors or assignees consolidates with or merges into any other Person and is not the continuing or surviving corporation or entity of such consolidation or merger, then to the extent necessary, proper provision shall be made so that the successors and assignees of the Company assume the obligations of the Company with respect to indemnification of members of the Board of Directors as in effect immediately before such transaction, whether such obligations are contained in the Company's Bylaws, the Certificate of Incorporation, or elsewhere, as the case may be.

5.7 Indemnification Matters. The Company hereby acknowledges that one (1) or more of the directors nominated to serve on the Board of Directors by the Investors (each an "**Investor Director**") may have certain rights to indemnification, advancement of expenses and/or insurance provided by one or more of the Investors and certain of their Affiliates (collectively, the "**Investor Indemnitors**"). The Company hereby agrees (a) that it is the indemnitor of first resort (*i.e.*, its obligations to any such Investor Director are primary and any obligation of the Investor

Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by such Investor Director are secondary), (b) that it shall be required to advance the full amount of expenses incurred by such Investor Director and shall be liable for the full amount of all expenses, judgments, penalties, fines and amounts paid in settlement by or on behalf of any such Investor Director to the extent legally permitted and as required by the Company's Certificate of Incorporation or Bylaws of the Company (or any agreement between the Company and such Investor Director), without regard to any rights such Investor Director may have against the Investor Indemnitors, and, (c) that it irrevocably waives, relinquishes and releases the Investor Indemnitors from any and all claims against the Investor Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Investor Indemnitors on behalf of any such Investor Director with respect to any claim for which such Investor Director has sought indemnification from the Company shall affect the foregoing and the Investor Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of such Investor Director against the Company. The Investor Directors and the Investor Indemnitors are intended third-party beneficiaries of this Section 5.7 and shall have the right, power and authority to enforce the provisions of this Section 5.7 as though they were a party to this Agreement.

5.8 Right to Conduct Activities. The Company hereby agrees and acknowledges that PBM TGT Holdings, LLC (together with its Affiliates) and each Fidelity Investor (together with its Affiliates) (collectively, the "**Fund Investors**") are professional investment organizations, and as such review the business plans and related proprietary information of many enterprises, some of which may compete directly or indirectly with the Company's business (as currently conducted or as currently propose to be conducted). The Company hereby agrees that, to the extent permitted under applicable law, the Fund Investors shall not be liable to the Company for any claim arising out of, or based upon, (i) the investment by any Fund Investor in any entity competitive with the Company, or (ii) actions taken by any partner, officer, employee or other representative of a Fund Investor to assist any such competitive company, whether or not such action was taken as a member of the board of directors of such competitive company or otherwise, and whether or not such action has a detrimental effect on the Company; provided, however, that the foregoing shall not relieve (x) any of the Investors from liability associated with the unauthorized disclosure of the Company's confidential information obtained pursuant to this Agreement, or (y) any director or officer of the Company from any liability associated with his or her fiduciary duties to the Company. The Company acknowledges that the execution of this Agreement and the access to the Company's confidential information shall in no way be construed to prohibit or restrict the Fund Investors, their investment advisers or their investment advisers' other investment advisory clients from maintaining, making or considering investments in such other enterprises, or from otherwise operating in the ordinary course of business, provided that such Fund Investor does not disclose the Company's confidential information in pursuit of such activities.

5.10 Termination of Covenants. The covenants set forth in this Section 5, except for Sections 5.6, 5.7 and 5.8, shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO or (ii) upon a Deemed Liquidation Event, as such term is defined in the Certificate of Incorporation, whichever event occurs first.

6. Miscellaneous.

6.1 Successors and Assigns. The rights under this Agreement may be assigned (but only with all related obligations) by a Holder to a transferee of Registrable Securities that (i) is an Affiliate of a Holder; (ii) is a Holder's Immediate Family Member or trust for the benefit of an individual Holder or one or more of such Holder's Immediate Family Members or (iii) would hold, after giving effect to any transfer of shares of Registrable Securities thereto by such Holder, all of the Registrable Securities held by such Holder; provided, however, that (x) the Company is, within a reasonable time after such transfer, furnished with written notice of the name and address of such transferee and the Registrable Securities with respect to which such rights are being transferred; and (y) such transferee agrees in a written instrument delivered to the Company to be bound by and subject to the terms and conditions of this Agreement, including the provisions of Section 2.11. For the purposes of determining the number of shares of Registrable Securities held by a transferee, the holdings of a transferee (1) that is an Affiliate or stockholder of a Holder; (2) who is a Holder's Immediate Family Member; or (3) that is a trust for the benefit of an individual Holder or such Holder's Immediate Family Member shall be aggregated together and with those of the transferring Holder; provided further that all transferees who would not qualify individually for assignment of rights shall, as a condition to the applicable transfer, establish a single attorney-in-fact for the purpose of exercising any rights, receiving notices, or taking any action under this Agreement. The terms and conditions of this Agreement inure to the benefit of and are binding upon the respective successors and permitted assignees of the parties. Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and permitted assignees any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided herein.

6.2 Governing Law. This Agreement shall be governed by the internal law of the State of Delaware, without regard to conflict of law principles that would result in the application of any law other than the law of the State of Delaware, provided, however, that Texas substantive law (without regard to any choice of law doctrines) shall govern in all events the Regents' rights of sovereign immunity, any limitations on the extent of any indemnity provisions that purport to apply to the Regents, their governmental authority, and the treatment of information in the Regents' possession under the Texas Public Information Act.

6.3 Counterparts. This Agreement may be executed in two (2) or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, *e.g.*, www.docusign.com) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes**.**

6.4 Titles and Subtitles. The titles and subtitles used in this Agreement are for convenience only and are not to be considered in construing or interpreting this Agreement.

6.5 Notices.

(a) All notices and other communications given or made pursuant to this Agreement shall be in writing and shall be deemed effectively given upon the earlier of actual

receipt or (i) personal delivery to the party to be notified; (ii) when sent, if sent by electronic mail or facsimile during the recipient's normal business hours, and if not sent during normal business hours, then on the recipient's next business day; (iii) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid; or (iv) one (1) business day after the business day of deposit with a nationally recognized overnight courier, freight prepaid, specifying next-day delivery, with written verification of receipt. All communications shall be sent to the respective parties at their addresses as set forth on Schedule A or Schedule B (as applicable) hereto, or to the principal office of the Company and to the attention of the Chief Executive Officer, in the case of the Company, or to such email address, facsimile number, or address as subsequently modified by written notice given in accordance with this Section 6.5. If notice is given to the Company, a copy shall also be sent to Cooley LLP, 1299 Pennsylvania Ave, NW, Suite 700, Washington, DC 20004, Attention: Brooke Nussbaum.

(b) Consent to Electronic Notice. Each Investor and Key Holder consents to the delivery of any stockholder notice pursuant to the Delaware General Corporation Law (the "DGCL"), as amended or superseded from time to time, by electronic transmission pursuant to Section 232 of the DGCL (or any successor thereto) at the electronic mail address or the facsimile number set forth below such Investor's or Key Holder's name on the Schedules hereto, as updated from time to time by notice to the Company, or as on the books of the Company. To the extent that any notice given by means of electronic transmission is returned or undeliverable for any reason, the foregoing consent shall be deemed to have been revoked until a new or corrected electronic mail address has been provided, and such attempted Electronic Notice shall be ineffective and deemed to not have been given. Each Investor and Key Holder agrees to promptly notify the Company of any change in such stockholder's electronic mail address, and that failure to do so shall not affect the foregoing.

6.6 Amendments and Waivers. Any term of this Agreement may be amended, modified or terminated and the observance of any term of this Agreement may be waived (either generally or in a particular instance, and either retroactively or prospectively) only with the written consent of the Company and the holders of at least a majority of the Registrable Securities then outstanding; provided that the Company may in its sole discretion waive compliance with Section 2.12(c) (and the Company's failure to object promptly in writing after notification of a proposed assignment allegedly in violation of Section 2.12(c) shall be deemed to be a waiver); and provided further that any provision hereof may be waived by any waiving party on such party's own behalf, without the consent of any other party. Notwithstanding the foregoing, (a) this Agreement may not be amended, modified or terminated and the observance of any term hereof may not be waived with respect to any Investor without the written consent of such Investor, unless such amendment, modification, termination, or waiver applies to all Investors in the same fashion (it being agreed that a waiver of the provisions of Section 4 with respect to a particular transaction shall be deemed to apply to all Investors in the same fashion if such waiver does so by its terms, notwithstanding the fact that certain Investors may nonetheless, by agreement with the Company, purchase securities in such transaction; provided, however, that where following such a waiver any Major Investor, by agreement with the Company, purchases securities in such transaction, such waiver shall not be effective as to any other Major Investor who did not waive such provision unless such Major Investor is also permitted to participate in such offering on a pro rata basis (based on the level of participation of the Major Investor purchasing the largest portion of such Major Investor's pro rata share), in accordance with the other provisions set forth in Section 4, but in no event in

excess of the number of New Securities that such Major Investor would have been entitled to purchase in the absence of such waiver), (b) Sections 3.1 and 3.2, Section 4 and any other section of this Agreement applicable to the Major Investors (including this clause (b) of this Section 6.6) may not be amended, modified, terminated or waived without the written consent of the holders of at least a majority of the Registrable Securities then outstanding and held by the Major Investors, (c) the provisions in this Agreement that relate to the acknowledgements, rights and privileges unique to the Regents shall not be amended without the prior written consent of the Regents, (d) Sections 2.11, 3.1 and 3.3, and any provision in this Agreement that relates to the acknowledgements, rights and privileges unique to the Fidelity Investors, shall not be amended in a manner adverse to the Fidelity Investors without the prior written consent of the Fidelity Investors holding a majority of the Registerable Securities held by all Fidelity Investors and (e) Sections 2.11, 3.1 and 3.3, and any provision in this Agreement that relates to the acknowledgements, rights and privileges unique to PBM, shall not be amended in a manner adverse to PBM without the prior written consent of PBM. Further, this Agreement may not be amended, modified or terminated, and no provision hereof may be waived, in each case, in any way which would adversely affect the rights of the Key Holders hereunder in a manner disproportionate to any adverse effect such amendment, modification, termination or waiver would have on the rights of the Investors hereunder, without also the written consent of the holders of at least a majority of the Registrable Securities held by the Key Holders. Notwithstanding the foregoing, Schedule A hereto may be amended by the Company from time to time to add transferees of any Registrable Securities in compliance with the terms of this Agreement without the consent of the other parties; and Schedule A hereto may also be amended by the Company after the date of this Agreement without the consent of the other parties to add information regarding any additional Investor who becomes a party to this Agreement in accordance with Section 6.9. The Company shall give prompt notice of any amendment, modification or termination hereof or waiver hereunder to any party hereto that did not consent in writing to such amendment, modification, termination, or waiver. Any amendment, modification, termination, or waiver effected in accordance with this Section 6.6 shall be binding on all parties hereto, regardless of whether any such party has consented thereto. No waivers of or exceptions to any term, condition, or provision of this Agreement, in any one or more instances, shall be deemed to be or construed as a further or continuing waiver of any such term, condition, or provision.

6.7 Severability. In case any one or more of the provisions contained in this Agreement is for any reason held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality, or unenforceability shall not affect any other provision of this Agreement, and such invalid, illegal, or unenforceable provision shall be reformed and construed so that it will be valid, legal, and enforceable to the maximum extent permitted by law.

6.8 Aggregation of Stock. All shares of Registrable Securities held or acquired by Affiliates shall be aggregated together for the purpose of determining the availability of any rights under this Agreement and such Affiliated persons may apportion such rights as among themselves in any manner they deem appropriate.

6.9 Additional Investors. Notwithstanding anything to the contrary contained herein, if the Company issues additional shares of the Company's Series B Preferred Stock after the date hereof, whether pursuant to the Purchase Agreement or otherwise, any purchaser of such shares of Series B Preferred Stock may become a party to this Agreement by executing and

delivering an additional counterpart signature page to this Agreement, and thereafter shall be deemed an "Investor" for all purposes hereunder. No action or consent by the Investors shall be required for such joinder to this Agreement by such additional Investor, so long as such additional Investor has agreed in writing to be bound by all of the obligations as an "Investor" hereunder.

6.10 Entire Agreement. This Agreement (including any Schedules and Exhibits hereto) constitutes the full and entire understanding and agreement among the parties with respect to the subject matter hereof, and any other written or oral agreement relating to the subject matter hereof existing between the parties is expressly canceled. Upon the effectiveness of this Agreement, the Prior Agreement shall be deemed amended and restated in its entirety by this Agreement and shall be of no further force or effect.

6.11 Dispute Resolution. The parties (a) hereby irrevocably and unconditionally submit to the jurisdiction of the state courts of the State of Delaware and to the jurisdiction of the United States District Court for the District of Delaware for the purpose of any suit, action or other proceeding arising out of or based upon this Agreement, (b) agree not to commence any suit, action or other proceeding arising out of or based upon this Agreement except in the state courts of the State of Delaware or the United States District Court for the District of Delaware, and (c) hereby waive, and agree not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, to the extent permitted by law, any claim that it is not subject personally to the jurisdiction of the above-named courts, that its property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court.

WAIVER OF JURY TRIAL: EACH PARTY (OTHER THAN THE REGENTS) HEREBY WAIVES ITS RIGHTS TO A JURY TRIAL OF ANY CLAIM OR CAUSE OF ACTION BASED UPON OR ARISING OUT OF THIS AGREEMENT, THE OTHER TRANSACTION DOCUMENTS, THE SECURITIES OR THE SUBJECT MATTER HEREOF OR THEREOF. THE SCOPE OF THIS WAIVER IS INTENDED TO BE ALL-ENCOMPASSING OF ANY AND ALL DISPUTES THAT MAY BE FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS TRANSACTION, INCLUDING, WITHOUT LIMITATION, CONTRACT CLAIMS, TORT CLAIMS (INCLUDING NEGLIGENCE), BREACH OF DUTY CLAIMS, AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THIS SECTION HAS BEEN FULLY DISCUSSED BY EACH OF THE PARTIES HERETO AND THESE PROVISIONS WILL NOT BE SUBJECT TO ANY EXCEPTIONS. EACH PARTY HERETO HEREBY FURTHER WARRANTS AND REPRESENTS THAT SUCH PARTY HAS REVIEWED THIS WAIVER WITH ITS LEGAL COUNSEL, AND THAT SUCH PARTY KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS FOLLOWING CONSULTATION WITH LEGAL COUNSEL.

The prevailing party shall be entitled to reasonable attorney's fees, costs, and necessary disbursements in addition to any other relief to which such party may be entitled; provided, however, that to the extent the non-prevailing party is the Regents, the prevailing party may make no recovery pursuant to this Section 6.11 from the Regents; and provided further, that the Regents may make no recovery pursuant to this Section 6.11. Each of the parties to this Agreement (other than the Regents) consents to personal jurisdiction for any equitable action sought in the U.S. District Court for the District of Delaware or any court of the State of Delaware having subject matter jurisdiction.

6.12 Delays or Omissions. No delay or omission to exercise any right, power, or remedy accruing to any party under this Agreement, upon any breach or default of any other party under this Agreement, shall impair any such right, power, or remedy of such nonbreaching or nondefaulting party, nor shall it be construed to be a waiver of or acquiescence to any such breach or default, or to any similar breach or default thereafter occurring, nor shall any waiver of any single breach or default be deemed a waiver of any other breach or default theretofore or thereafter occurring. All remedies, whether under this Agreement or by law or otherwise afforded to any party, shall be cumulative and not alternative.

6.13 Acknowledgment. The Company, the Key Holders and the Investors each acknowledge that the Regents are an agency of the State of Texas and under the constitution and the laws of the State of Texas possess certain rights and privileges, are subject to certain limitations and restrictions, and only have such authority as is granted to them under the constitution and laws of the State of Texas. Notwithstanding any provision hereof, nothing in this Agreement is intended to be, nor will it be construed to be, a waiver of the sovereign immunity of the State of Texas or a prospective waiver or restriction of any of the rights, remedies, claims, and privileges of the State of Texas. Moreover, notwithstanding the generality or specificity of any provision hereof, the provisions of this Agreement as they pertain to the Regents are enforceable only to the extent authorized by the constitution and laws of the State of Texas; accordingly, to the extent any provision hereof conflicts with the constitution or laws of the State of Texas or exceeds the right, power or authority of the Regents to agree to such provision, then that provision will not be enforceable against the Regents or the State of Texas.

[Signature Page(s) to Follow]

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

TAYSHA GENE THERAPIES, INC.

By: /s/ R.A. Session II
Name: R.A. Session II
Title: President

KEY HOLDERS:

Signature: /s/ R.A. Session II
Name: R.A. Session II

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

KEY HOLDERS:

Signature: /s/ Dan Janiak
Name: Dan Janiak

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

PBM TGT Holdings, LLC

By: PBM Capital Group, LLC, its manager

By: /s/ Paul B. Manning
Name: Paul B. Manning
Title: CEO

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

Nolan Capital, LLC

By: /s/ Sean Nolan
Name: Sean Nolan
Title: President

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund

By: /s/ Chris Maher
Name: Chris Maher
Title: Authorized Signatory

Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund

By: /s/ Chris Maher
Name: Chris Maher
Title: Authorized Signatory

Fidelity Growth Company Commingled Pool

By: Fidelity Management Trust Company, as Trustee

By: /s/ Chris Maher
Name: Chris Maher
Title: Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

Fidelity Mt. Vernon Street Trust: Fidelity Growth Company K6 Fund

By: /s/ Chris Maher
Name: Chris Maher
Title: Authorized Signatory

Fidelity Select Portfolios: Biotechnology Portfolio

By: /s/ Chris Maher
Name: Chris Maher
Title: Authorized Signatory

Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund

By: /s/ Chris Maher
Name: Chris Maher
Title: Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

Sands Capital Life Sciences Pulse Fund, LLC

By: /s/ Jonathan Goodman
Name: Jonathan Goodman
Title: General Counsel

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

Octagon Investments Master Fund LP

By: Octagon Capital Advisors LP, its Investment Manager

By: /s/ Ting Jia
Name: Ting Jia
Title: Managing Member

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

VENROCK HEALTHCARE CAPITAL PARTNERS III, L.P.

By: VHCP Management III, LLC, its general partner
By: VR Advisor, LLC, its manager

By: /s/ Nimish Shah
Name: Nimish Shah
Title: Authorized Signatory

VHCP CO-INVESTMENT HOLDINGS III, LLC

By: VHCP Management III, LLC, its manager
By: VR Advisor, LLC, its manager

By: /s/ Nimish Shah
Name: Nimish Shah
Title: Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

GC&H Investments, LLC

By: /s/ Jim Kindler
Name: Jim Kindler
Title: Manager

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

Perceptive Life Sciences Master Fund LTD

By: /s/ James H. Mannix
Name: James H. Mannix
Title: Chief Operating Officer

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

GV 2019, L.P.
By: GV 2019 GP, L.P., its General Partner
By: GV 2019 GP, L.L.C., its General Partner

By: /s/ Daphne M. Chang
Name: Daphne M. Chang
Title: Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

By: /s/ Doug Jennings
Name: Doug Jennings

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

By: /s/ Ryan Walker
Name: Ryan Walker

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

By: /s/ Suku Nagendran
Name: Sukumar Nagendran

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

By: /s/ Suyash Prasad
Name: Suyash Prasad

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first written above.

INVESTORS:

By: /s/ Lauren Murdza
Name: Lauren Murdza

SCHEDULE A

INVESTORS

Name and Address

PBM TGT Holdings, LLC

Nolan Capital, LLC

Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund

Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund

Fidelity Growth Company Commingled Pool

Fidelity Mt. Vernon Street Trust : Fidelity Growth Company K6 Fund

Fidelity Select Portfolios: Biotechnology Portfolio

Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund

Sands Capital Life Sciences Pulse Fund, LLC

Octagon Investments Master Fund LP

Venrock Healthcare Capital Partners III, L.P.

VHCP Co-Investment Holdings III, LLC

GC&H Investments, LLC

Perceptive Life Sciences Master Fund LTD

GV 2019, L.P.

Doug Jennings

Ryan Walker

Sukumar Nagendran

Suyash Prasad

Lauren Murdza

Chardan Healthcare Investments, LLC

Bios Fund II NT, LP

Bios Fund II QP, LP

Bios Fund II, LP

Bios Fund III NT, LP

Bios Fund III QP, LP

Bios Fund III, LP

Invus Public Equities, LP

Casdin Partners Master Fund, L.P.

PBM Capital Group, LLC

Franklin Strategic Series – Franklin Biotechnology Discovery Fund

Franklin Templeton Investment Funds – Franklin Biotechnology Discover Fund

ArrowMark Life Science Fund, LP

Meridian Small Cap Growth Fund

BlackRock Health Sciences Opportunities Portfolio, a Series of BlackRock Funds

BLACKROCK HEALTH SCIENCES TRUST

BlackRock Global Funds – World Healthscience Fund

BLACKROCK HEALTH SCIENCES MASTER UNIT TRUST

BLACKROCK HEALTH SCIENCES TRUST II

BLACKROCK HEALTH SCIENCES MASTER UNIT TRUST

BLACKROCK HEALTH SCIENCES TRUST II

Anthony Lamb

Emily Ware

Ralph Shifflett

Joshua Moerman

Lee Williams

Russell Polsky

Mark Blaze

Candace Taylor

Christa Cosner

Gregory MacDonald

David Zawitz

David Glover

David Halloran

Debra Walker

Diana Smalls

Elizabeth Kodi

Gary Massengill

Jessica DeGraff

Joseph Pedersen

Melissa Woodruff

Patrick Nolan

Shannon Chen

Stephanie Culley

Sung You

Casey Steffan

Christopher Henry

Jodi Mills

Joseph Wrege

Joshua Batman

Juan Rodriguez

Kevin Miller

Matthew Powell

Cynthia Moravec

Jeffrey Kopocis

Joan Oehmke

Kristen Heckel

Luisa Assink

Phillip Edwards

Roger Varner

Ronald Buchanan

Teresa Hastings

Travis Coyner

Wendie Charles

Peter Reines

Thaddeus J. Spinks, VMD

Jennifer Reynolds

Al Novak

Carr Family LLC

Brendan Clancy

Peter Taylor

Jonathan Leach

Tom Selinger

Chuck Koeniger

Richard Gilliam

Ray Caterino Jr.

Benj Garrett

Linda Ricca

Joe Hall

Sean Stalfort

Jay Stalfort

John P. Williamson, Jr.

Steven Goldman

John A Stalfort III 2018 Irrevocable Trust

Jayson Rieger

Dry Pond Partners, LLC

The Don and Jenna Mosman Revocable Living Trust, dated September 21, 2018

Joseph Pedersen

Melissa Woodruff

Gary Massengill

Christa Cosner

Sung You

David Glover

BKB Growth Investments, LLC

SCHEDULE B

KEY HOLDERS

R.A. Session II

The Board of Regents of the University of Texas System

Dan Janiak

JOINDER AGREEMENT TO THE
AMENDED AND RESTATED
INVESTORS' RIGHTS AGREEMENT

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/s/ Anthony Lamb
Name: Anthony Lamb

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Emily Ware
Name: Emily Ware

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Ralph Shifflett
Name: Ralph Shifflett

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/s/ Joshua Moerman
Name: Joshua Moerman

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/s/ Lee Williams
Name: Lee Williams

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/s/ Russell Polsky
Name: Russell Polsky

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Mark Blaze
Name: Mark Blaze

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Candace Taylor
Name: Candace Taylor

JOINDER AGREEMENT TO THE
AMENDED AND RESTATED
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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Christa Cosner
Name: Christa Cosner

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Gregory MacDonald
Name: Gregory MacDonald

JOINDER AGREEMENT TO THE
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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ David Zawitz
Name: David Zawitz

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ David Glover
Name: David Glover

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ David Halloran
Name: David Halloran

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/s/ Debra Walker
Name: Debra Walker

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Diana Smalls

Name: Diana Smalls

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/s/ Elizabeth Kodi
Name: Elizabeth Kodi

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/s/ Gary Massengill
Name: Gary Massengill

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/s/ Jessica DeGraff
Name: Jessica DeGraff

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Joseph Pedersen
Name: Joseph Pedersen

JOINDER AGREEMENT TO THE
AMENDED AND RESTATED
INVESTORS' RIGHTS AGREEMENT

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Melissa Woodruff
Name: Melissa Woodruff

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Patrick Nolan
Name: Patrick Nolan

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Shannon Chen
Name: Shannon Chen

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Stephanie Culley
Name: Stephanie Culley

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Sung You
Name: Sung You

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Casey Steffan
Name: Casey Steffan

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/s/ Christopher Henry
Name: Christopher Henry

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Jodi Mills
Name: Jodi Mills

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Joseph Wrege
Name: Joseph Wrege

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Joshua Batman
Name: Joshua Batman

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Juan Rodriguez
Name: Juan Rodriguez

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/s/ Kevin Miller
Name: Kevin Miller

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/s/ Matthew Powell
Name: Matthew Powell

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/s/ Cynthia Moravec
Name: Cynthia Moravec

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/s/ Jeffrey Kopocis
Name: Jeffrey Kopocis

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/s/ Joan Oehmke
Name: Joan Oehmke

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/s/ Kristen Heckel
Name: Kristen Heckel

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/s/ Luisa Assink

Name: Luisa Assink

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/s/ Phillip Edwards
Name: Phillip Edwards

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/s/ Roger Varner
Name: Roger Varner

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/s/ Ronald Buchanan
Name: Ronald Buchanan

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/s/ Teresa Hastings
Name: Teresa Hastings

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/s/ Travis Coyner
Name: Travis Coyner

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/s/ Wendie Charles

Name: Wendie Charles

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/s/ Peter Reines

Name: Peter Reines

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/s/ Thaddeus J. Spinks, VMD
Name: Thaddeus J. Spinks, VMD

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Jennifer Reynolds
Name: Jennifer Reynolds

JOINDER AGREEMENT TO THE
AMENDED AND RESTATED
INVESTORS' RIGHTS AGREEMENT

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Al Novak
Name: Al Novak

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Carr Family LLC

/s/ John Carr
Name: John Carr
Title: Manager

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/s/ Brendan Clancy
Name: Brendan Clancy

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/s/ Peter Taylor
Name: Peter Taylor

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/s/ Jonathan H Leach VMD

Name: Jonathan H Leach VMD

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Tom Selinger
Name: Tom Selinger

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/s/ Chuck Koeniger
Name: Chuck Koeniger

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Richard Gilliam
Name: Richard Gilliam

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Ray Caterino Jr.
Name: Ray Caterino Jr.

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Benj Garrett
Name: Benj Garrett

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Linda Ricca
Name: Linda Ricca

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Joe Hall
Name: Joe Hall

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Sean Stalfort
Name: Sean Stalfort

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Jay Stalfort
Name: Jay Stalfort

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/s/ John P. Williamson, Jr.
Name: John P. Williamson, Jr.

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/s/ Steven Goldman
Name: Steven Goldman

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John A Stalfort III 2018 Irrevocable Trust

/s/ Gineane H Stalfort
Name: Gineane H Stalfort
Title: Trustee

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/s/ Jayson Rieger
Name: Jayson Rieger

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Dry Pond Partners, LLC

/s/ Scott C. Sullivan

Name: Scott C. Sullivan
Title Manager

/s/ Bruce B. Cameron, IV

Name: Bruce B. Cameron, IV
Title: Manager

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/s/ Don Mosman
Name: Don Mosman

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/s/ Joseph Pedersen
Name: Joseph Pedersen

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/s/ Melissa Woodruff
Name: Melissa Woodruff

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/s/ Gary Massengill
Name: Gary Massengill

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/s/ Christa Cosner
Name: Christa Cosner

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Accordingly, the undersigned has executed and delivered this Joinder Agreement to be effective as of August 24, 2020.

/s/ Sung You
Name: Sung You

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/s/ David Glover
Name: David Glover

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BKB Growth Investments, LLC

/s/ Paul B. Manning
Name: Paul B. Manning
Title: Manager

/s/ Bradford Manning
Name: Bradford Manning
Title: Manager

Certain information has been excluded from this agreement (indicated by "[***]") because Taysha Gene Therapies, Inc. has determined such information (i) is not material and (ii) would be competitively harmful if publicly disclosed.

RESEARCH, COLLABORATION & LICENSE AGREEMENT
AGT. NO. 2020-0029/L3720-Taysha

This Research, Collaboration & License Agreement (this "**Agreement**") is dated as of November 19, 2019 (the "**Effective Date**") by and between The Board of Regents of the University of Texas System ("**System**"), an agency of the State of Texas, on behalf of The University of Texas Southwestern Medical Center ("**UT Southwestern**"), a component institution of System ("**Licensor**"), and Taysha Gene Therapies, Inc. a corporation organized under the laws of the state of Texas ("**Licensee**"). Licensor and Licensee may be referred to herein as a "**Party**" or, collectively, as "**Parties**".

RECITALS:

WHEREAS, Licensee is a biotechnology company with expertise in the development, manufacture and commercialization of human therapeutic products for treatment of genetic disorders.

WHEREAS, UT Southwestern, through Dr. Steven Gray, Dr. Berge Minassian and the Gray Laboratory, have technology and expertise in the research and development of gene therapy products.

WHEREAS, the Research Program contemplated by this Agreement is of mutual interest to Licensee and Licensor and may benefit Licensee and Licensor through the creation or discovery of new inventions and the development and commercialization of Licensed Products (as defined below).

NOW, THEREFORE, in consideration of the various promises and undertakings set forth herein, the Parties agree as follows:

ARTICLE 1
DEFINITIONS

Unless otherwise specifically provided herein, the following terms shall have the following meanings:

1.1 "**AAV**" means adeno-associated virus.

1.2 "**Affiliate**" means a Person that controls, is controlled by or is under common control with a Party, but only for so long as such control exists. For the purposes of this Section 1.2, the word "control" (including, with correlative meaning, the terms "controlled by" or "under common control with") means the actual power, either directly or indirectly through one or more intermediaries, to direct the management and policies of such Person, whether by the ownership of more than fifty percent (50%) of the voting stock of such Person, or by contract or otherwise.

1.3 "**Calendar Year**" means each successive period of twelve (12) months commencing on January 1 and ending on December 31.

1.4 **"cGMP"** means those current practices, as amended from time to time, related to the manufacture of pharmaceutical products and any precursors thereto promulgated in guidelines and regulations of standard compilations including the GMP Rules of the World Health Organization, the United States Code of Federal Regulations, the Guide to Inspection of Bulk Pharmaceutical Chemicals (established by the United States Department of Health and Human Services), the Pharmaceutical Inspection Convention, and the European Community Guide to Good Manufacturing Practice in the production of pharmaceutical products, and equivalent guidelines, regulations and standards in the Territory, as such guidelines, regulations and standards may be amended from time to time.

1.5 "**Clinical Study**" means a Phase 1 Study, Phase 1/2 Study, Phase 2 Study, or Phase 3 Study, or such other study in humans that is conducted in accordance with good clinical practices and is designed to generate data in support or maintenance of an application for Regulatory Approval.

1.6 "**CNS Monogenic Rare Disorder**" means a rare disease or condition (as determined in accordance with Sec. 526 of the FD&C Act or corresponding Laws outside the United States) with significant morbidity in the central nervous system (CNS) or peripheral nervous system (PNS) caused by a gain-of-function or loss-of-function mutation in a specific gene.

1.7 "**Commercially Reasonable Efforts**" means the efforts and resources that [***] would use for [***] of similar commercial potential and similar stage of development, taking into account [***].

1.8 "**Contract Quarter**" means the three-month periods ending on March 31, June 30, September 30 and December 31, or any stub-period thereof at the commencement of this Agreement or the expiration or termination of this Agreement.

1.9 "**Controlled**" means, with respect to intellectual property rights, that a Party or one of its Affiliates owns or has a license or sublicense to such intellectual property rights and has the ability to provide, grant a license or sublicense to, or assign its right, title and interest in and to, such intellectual property rights as provided for in the Agreement without violating the terms of any other agreement or other arrangement with any Third Party.

1.10 "**EMA**" means the European Medicines Agency and any successor entity thereto.

1.11 "**Exclusive Option Indications**" means each of the following indications for a Gene Therapy Product: [***].

1.12 "**FDA**" means the United States Food and Drug Administration and any successor entity thereto.

1.13 "**FD&C Act**" means the United States Federal Food, Drug and Cosmetic Act, as amended.

1.14 "**Field of Use**" means [***] uses in humans. For clarity, any and all uses in non-humans, including any and all veterinary uses in companion animals and livestock species, is excluded from the Field of Use.

1.15 "**FIH**" means on a Licensed Product-by-Licensed Product basis, a first in human Clinical Study for a Licensed Product.

1.16 "**First Commercial Sale**" means, on a country-by-country basis, the first commercial transfer or disposition for value of Licensed Product in such country to a Third Party by Licensee, or any of its Affiliates or Sublicensees, in each case, after Regulatory Approvals have been obtained for such country.

1.17 "**Gene Therapy Product**" means a pharmaceutical product (or proposed or prospective pharmaceutical product) that [***]. For clarity, Gene Therapy Products do not include [***].

1.18 "**Governmental Body**" means any: (a) nation, principality, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (b) federal, provincial, state, local, municipal, foreign or other government; (c) governmental or quasi-governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, board, instrumentality, officer, official, representative,

organization, unit, body or entity and any court or other tribunal); (d) multi-national or supranational organization or body; or (e) individual, entity, or body exercising, or entitled to exercise, any executive, legislative, judicial, administrative, regulatory, police, military or taxing authority or power of any nature.

1.19 "**Gray Laboratory**" means Dr. Steven Gray and Dr. Berge Minassian and all individuals who are under the direct or indirect supervision or control of Dr. Steven Gray or Dr. Berge Minassian at Licensor (as a group or individual basis as applicable herein).

1.20 "**IND**" means an Investigational New Drug Application as defined in the FD&C Act and the regulations promulgated thereunder, or (b) the equivalent application to the equivalent regulatory authority in any other regulatory jurisdiction, including a Clinical Trial Authorization ("**CTA**") to the European Medicines Agency, the filing of which is necessary to initiate or conduct clinical testing of a pharmaceutical product in humans in such jurisdiction.

1.21 "**Indication**" means each of the following indications for a Gene Therapy Product: (a) Named Indications and (b) Exclusive Option Indications added to the Agreement through exercise of an Exclusive Indication Option. "Indication" shall not include indications for which a Research Program SRA has not been timely executed pursuant to Section 2.2.1, an indication for which a Research Program has expired pursuant to Section 2.2.5, nor an indication that has failed as provided in Section 2.8.

1.22 "**Know-How**" means intellectual property, data, results, pre-clinical and clinical protocols and study data, chemical structures, chemical sequences, information, inventions, formulas, techniques, methods, processes, procedures and developments. "Know-How" does not include Licensor Patent Rights claiming any of the foregoing. "Know-How" also does not include Licensed Materials.

1.23 "**Law**" or "**Laws**" means all applicable laws, statutes, rules, regulations, ordinances and other pronouncements having the binding effect of law of any Governmental Body.

1.24 "**Licensed Know-How**" means all Know-How and Licensed Material that is Controlled by Licensor and (a) developed by the Gray Laboratory as of the Effective Date or (b) developed in the Gray Laboratory under the Research Program or pursuant to any Research Program SRA (including but not limited to all Research Results), and in each case (a) and (b) is necessary or reasonably useful to develop, make, use, sell, offer for sale or import a Licensed Product for the Indication in the Field of Use.

1.25 "**Licensed Material**" means biological or chemical materials that are Controlled by Licensor and necessary or useful to exploit the licenses granted to Licensee under the Agreement as of the Effective Date or at any time during the Term. Such biological and chemical materials include cell lines (including but not limited to the [***]), viral seed stocks, product-specific reference materials, platform or product specific assay controls and reagents that are not available as standard commercial items.

1.26 "**Licensed Product**" means any (a) process, service or method covered by a Valid Claim or Licensed Know-How, or whose use or practice would, absent the License, constitute an infringement, inducement of infringement or contributory infringement of any Valid Claim, or would infringe a Valid Claim once issued ("**Method**"); (b) article, composition, apparatus, substance, chemical or any other material covered by a Valid Claim or Licensed Know-How, or whose manufacture, import, use offer for sale or sale would, absent the License, constitute an infringement, inducement of infringement or contributory infringement of any Valid Claim or

would infringe a Valid Claim once issued; or (c) service, article, composition, apparatus, chemical, substance or any other material made, used or sold by or utilizing or practicing a Method, or (d) any product that incorporates or makes use or is made through use of material Licensed Know-How.

1.27 "**Licensor Patent Rights**" means (a) the Patent Rights listed in Exhibit A Controlled by Licensor as of the Effective Date, (b) the Patent Rights Controlled by Licensor that are necessary or useful for the development of Licensed Products, including but not limited to Patent Rights that claim a method of manufacturing or producing a component contained in a Licensed Product; (c) any Patent Rights Controlled by Licensor and conceived and reduced to practice by Dr. Gray, Dr. Minassian or the Gray Laboratory in the conduct of a Research Program; (d) any Patent Rights conceived and reduced to practice in the conduct of a Research Program SRA; (e) any corresponding foreign Patent Rights to the foregoing.

1.28 "**Named Indications**" means: [***].

1.29 "**Patent Rights**" means (a) patents and patent applications, together with any unlisted patents and patent applications claiming priority thereto, and any continuations, continuations-in-part, reissues, reexamination certificates, substitutions, divisionals, supplementary protection certificates, renewals, registrations, extensions including all confirmations, revalidations, patents of addition, PCTs, and pediatric exclusivity periods and all foreign counterparts thereof, and any patents issued or issuing with respect to any of the foregoing and (b) all official correspondence relating to the foregoing.

1.30 "**Person**" means any natural person, corporation, firm, business trust, joint venture, association, organization, company, partnership or other business entity, or any government or agency or political subdivision thereof.

1.31 "**Phase 1 Study**" means a clinical study of a drug candidate in human patients with the primary objective of characterizing its safety, tolerability, and pharmacokinetics and identifying a recommended dose and regimen for future studies as described in 21 C.F.R. §312.21(a), or a comparable clinical study prescribed by the relevant regulatory authority in a country other than the United States. The drug candidate can be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.32 "**Phase 1/2 Study**" means a clinical study of a drug candidate in diseased human patients that satisfies the requirements of a Phase 1 Study and a Phase 2 Study.

1.33 "**Phase 2 Study**" means a clinical study of a drug candidate in human patients with the primary objective of characterizing its activity in a specific disease state as well as generating more detailed safety, tolerability, and pharmacokinetics information as described in 21 C.F.R. §312.21(b), or a comparable clinical study prescribed by the relevant Regulatory Authority in a country other than the United States including a human clinical study that is also designed to satisfy the requirements of 21 C.F.R. §312.21(a) or corresponding foreign regulations and is subsequently optimized or expanded to satisfy the requirements of 21 C.F.R. §312.21(b) (or corresponding foreign regulations) or otherwise to enable a Phase 3 Clinical Study (e.g., a phase 1/2 trial). The relevant drug candidate may be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.34 "**Phase 3 Study**" means a clinical study of a drug candidate in human patients that incorporates accepted endpoints for confirmation of statistical significance of efficacy and safety with the aim

to obtain Regulatory Approval in any country as described in 21 C.F.R. 312.21(c), or a comparable clinical study prescribed by the relevant Regulatory Authority in a country other than the United States. The relevant drug candidate may be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.35 "**Quarterly Payment Deadline**" means the day that is sixty (60) days after the last day of any particular Contract Quarter.

1.36 "**Regulatory Approval**" means, with respect to a product in any regulatory jurisdiction, approval from the applicable Regulatory Authority sufficient for the manufacture, distribution, use, marketing and sale of such pharmaceutical product in such jurisdiction in accordance with Laws. "Regulatory Approval" does not include authorization by a Regulatory Authority to conduct named patient, compassionate use or other similar activities.

1.37 "**Regulatory Authority**" means any Governmental Body, including the FDA or EMA, or any successor agency thereto, that has responsibility for granting any licenses or approvals or granting pricing or reimbursement approvals necessary for the marketing and sale of a pharmaceutical product in any country.

1.38 "**Research Program SRAs**" means the sponsored research agreements setting forth the Parties' roles and responsibilities for an Indication in furtherance of the Research Program, and as may be amended from time to time with written approval of the JSC or (to the extent provided herein) both Parties.

1.39 "**Research Program**" means the pre-clinical discovery, research, and development program of Licensed Products in the Field of Use for the Indications funded by Licensee and to be conducted by the Parties hereunder.

1.40 "**Research Results**" means all any and all ideas, information, inventions, developments, animate and inanimate materials, including live animals, discoveries, software, know-how, methods, techniques, formulae, data, software, processes, methodologies, techniques, biological materials, software and works of authorship, whether patentable or copyrightable, that are first conceived, discovered, developed, reduced to practice, or generated in the performance of the Research Program by the Gray Laboratory, including any unpatentable inventions discovered, developed or conceived in the conduct of the Research Program. Research Results expressly excludes Licensor Patent Rights.

1.41 "**Sublicensee**" means a Person (including any Affiliate) to which a Sublicense is granted pursuant to the terms of Section 3.4.

1.42 "**Sublicense Documents**" means any and all agreements, amendments or written understandings entered into with a Sublicensee (including any of its Affiliates) that are directly or indirectly related to a Sublicense, Licensor Patent Rights or Licensed Product.

1.43 "**Tax**" means all taxes, duties, fees, premiums, assessments, imposts, levies, rates, withholdings, dues, government contributions and other charges of any kind whatsoever, whether direct or indirect, together with all interest, penalties, fines, additions to tax or other additional amounts, imposed by any Governmental Body.

1.44 "**Third Party**" means any Person other than Licensor, Licensee or any of their respective Affiliates.

1.45 "**Transition Point**" or "**TP**" means on a Licensed Product-by-Licensed Product basis the date on which the IND-enabling studies for such Licensed Product under the Research Program have been completed and immediately prior to filing of the IND, unless otherwise agreed by the Parties.

1.46 "**United States**" or "**US**" means the United States of America, its territories and possessions.

1.47 "**Valid Claim**" means a claim of (a) an issued and unexpired patent in Licensor Patent Rights which claim has not been revoked or held unenforceable or invalid by a decision of a court of governmental agency of competent jurisdiction from which no further appeal can be taken or has been taken within the time allowed for appeal, and has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer; or (b) a pending patent application (that has been pending for no more than [***] from the filing date of such application) that is included in Licensor Patent Rights which was filed and is being prosecuted in good faith, and has not been abandoned or finally disallowed without the possibility of appeal or re-filing of the application.

1.48 **Other Terms**. The definition of each of the following terms is set forth in the section of the Agreement indicated below:

Defined Term	Section
Agreement	Introductory Clause
Bankruptcy Action	10.3.2
Confidential Information	7.1
CTA	1.21
Disclosing Party	7.1
Effective Date	Introductory Clause
Exclusive Indication Option	2.5
Exclusive Option Period	2.5
Failed Indication	2.8
Initial Financing	5.7
Infringement Notice	6.3.1
Joint Steering Committee ("JSC")	2.9.1
License	3.1
Licensee	Introductory Clause
Limited Exclusivity Covenant	2.7
Licensor	Introductory Clause
Licensor Indemnitees	9.1.1
Method	1.27
Ongoing Patent Costs	6.2.2
Party or Parties	Introductory Clause
Patent Costs	6.2.1
Patent Counsel	6.1.1
Progress Report	5.8
Prosecution Request	6.1.2
Receiving Party	7.1
Research Term	2.2.1
R&D Extension Term	2.2.1
Sublicense	3.4.1
System	Introductory Clause
Term	10.1
UT Southwestern	Introductory Clause

ARTICLE 2
COLLABORATION PROGRAMS; GOVERNANCE

2.1 **Overall Project**. The Parties desire to collaborate with respect to the pre-clinical development of Licensed Products, as set forth in more detail in this Article 2, in each Indication within the Field of Use, with the goal of identifying one or more Licensed Products for clinical development and commercialization in each Indication. As more specifically outlined herein, Licensor will be responsible, in consultation with Licensee through the JSC, for preclinical development activities, including IND-enabling studies, GMP clinical manufacturing, natural history studies, FIH Clinical Studies and compassionate use studies (if authorized by Licensee) and all activities set forth in Research Program SRAs that are not identified therein as Licensee's responsibilities. Licensee will be responsible for regulatory strategy and operations, clinical development, GMP commercial scale manufacture, process development, commercial development, business development, corporate infrastructure and commercialization of all Licensed Product.

2.2 **Research**.

2.2.1 During the period of [***] following the Effective Date ("**Research Term**"), Licensee shall provide research and development funding to Licensor pursuant to Research Program SRAs to fund the Research Program for each Indication. The Parties shall enter into one or more Research Program SRAs within [***] after the Effective Date to fund Licensor's pre-clinical research and development activities for the [***] Named Indications. The Parties shall enter into additional Research Program SRAs as additional Indications are added to the Research Program as provided in this Agreement. Licensee shall also provide early discovery funding to Licensor pursuant to Research Program SRAs to fund early discovery research for each of the Exclusive Option Indications within [***] after the Effective Date. Upon mutual agreement of the Parties, the Research Term may be extended for additional terms with respect to one or more Indications or Exclusive Option Indications as determined by the Parties ("**R&D Extension Term,**" which shall extend the Research Term) on terms to be mutually agreed to by the Parties. For clarity, neither Party is under any obligation to extend the Research Term.

2.2.2 Licensor shall conduct the Research Program in accordance with the Research Program SRAs and the other terms and conditions of this Agreement. Except as otherwise provided in this Agreement or any of the Research Program SRAs by specific reference to the applicable provision of this Agreement, if any provision contained in this Agreement conflicts with any provision in any Research Program SRA, the provision contained in this Agreement shall govern and control.

2.2.3 The JSC shall review the Research Program SRAs at least [***] per Calendar Year. Subject to compliance with Section 2.3.2, the JSC may amend the Research Program plans executed under a Research Program SRA at any time, including amendments to include further activities, including corresponding revisions to the budget provided for therein.

2.2.4 Licensor shall maintain records of the results of the Research Program in sufficient detail and in good scientific manner appropriate for patent purposes to properly reflect all

work done and results achieved; provided that maintenance of records in accordance with Licensor's policies shall be deemed to satisfy this requirement. Licensor will provide task-based, scientific reports of the progress and results of the Research Program on a schedule to be agreed to in writing by the Parties. Upon Licensee's reasonable request, Licensor will disclose and deliver Research Results to Licensee, and will provide Licensee with such additional information and technical assistance as may be reasonably needed for Licensee to interpret and use such Research Results. Licensor shall maintain records of the use of the funds provided by Licensee pursuant to a Research Program SRA as specified in the applicable Research Program SRA and shall make such records available to Licensee upon reasonable notice during Licensor's normal business hours.

2.2.5 The Parties hereby acknowledge that there are inherent uncertainties involved in the research and development of products and such uncertainties form part of the business risk involved in undertaking the Research Program. Accordingly, in the event that upon completion of the portion of a Research Program SRA associated with a specific Indication, the Parties do not develop or identify a suitable candidate to propose as a development candidate for that Indication, then the corresponding portion of the Research Program shall expire, and neither Party shall have any further obligations to the other under that portion of the Research Program or the applicable Research Program SRA.

2.2.6 Each Party will have the right to engage Third Party subcontractors to perform certain of its obligations under this Agreement; provided that Licensor's right to engage Third Party subcontractors is subject to Licensee's prior written consent. Any subcontractor to be engaged by a Party to perform a Party's obligations set forth in the Agreement must meet the qualifications typically required by such Party for the performance of work similar in scope and complexity to the subcontracted activity and will enter into such Party's standard nondisclosure agreement consistent with such Party's standard practices which agreement shall be as least as protective as the nondisclosure obligations set forth herein. Any Party engaging a subcontractor hereunder will remain responsible and obligated for such activities and will not grant rights to such subcontractor that interfere with the rights of the other Party under this Agreement. Furthermore, if Licensor engages any subcontractor to perform any activities that would otherwise be performed by Dr. Gray, Dr. Minassian or the Gray Laboratory, any research, development, or other innovation performed or made by such subcontractor in connection with that engagement will, for purposes of this Agreement, be deemed to have been performed or made by Dr. Gray, the Gray Laboratory or Dr. Minassian, as the case may be.

2.3 **Funding of the Research Program**.

2.3.1 Each Research Program SRA entered into between the Parties shall include a budget for the applicable portion of the Research Program.

2.3.2 If at any time Licensor determines that it will require additional funds for any Research Program SRA then in effect, it will notify Licensee and provide a good faith estimate and itemized budget of the additional amount. If the Parties cannot agree on an updated budget for the applicable Research Program SRA, the Parties shall submit the dispute to the JSC for review and determination. Notwithstanding the foregoing: [***].

2.4 **Unavailability of Dr. Steven Gray or Dr. Minassian**. If either Steven Gray, PhD or Berge Minassian, MD becomes unavailable for any reason, Licensor may propose another member of its faculty who is acceptable to Licensee, to oversee the performance of Licensor's portion of

the Research Program. If Licensee agrees to the proposed substitution, the Parties will amend the definition of the Gray Laboratory (effective on a going-forward basis) to describe the group within Licensor that will be continuing the Research Program. If a substitute faculty member (or faculty members) acceptable to Licensee has not been agreed upon within [***] after Steven Gray, PhD or Berge Minassian MD is no longer available to oversee and support the performance of the Research Program, or if the Parties have not agreed upon an amendment to the definition of the Gray Laboratory within that same time period, Licensee may transfer all outstanding Research Program SRAs to Licensee.

2.5 **Exclusive Indication Option**. For a period of [***] following the Effective Date ("**Exclusive Option Period**"), Licensee will have the exclusive option to include in the Research Program any of the Exclusive Option Indications ("**Exclusive Indication Option**"). Licensee may exercise its Exclusive Indication Option to add any number of the Exclusive Option Indications during the Exclusive Option Period by providing written notice to Licensor, and may add one or more of the Exclusive Option Indications at different times during such period. Within [***] following notice of the exercise of such Exclusive Indication Option, the Licensor will develop and propose a work plan and budget for the preclinical and development costs through IND-enabling studies to be conducted at Licensor for a Licensed Product for such Exclusive Option Indication(s) to supplement any previously conducted research under a Research Program SRA. Within [***] of Licensee's receipt of such budget and work plan, Licensee shall decide whether to proceed with the exercise of such Exclusive Indication Option. If Licensee proceeds, Licensee shall provide written notice to Licensor and the Parties shall enter into an amendment to the current Research Program SRA for such Exclusive Option Indication for such additional work and such Exclusive Option Indication shall become an Indication under the terms of this Agreement.

2.6 **Natural History Studies**. During the Research Term, Licensor, through the Gray Laboratory, may conduct natural history studies relating to the Research Program, subject to the Parties' having reached mutual agreement upon the terms of the funding, rights to the data arising from such studies and other terms in a Research Program SRA.

2.7 **Exclusivity**. On an Indication-by-Indication basis during the Research Term for such Indication until the earlier of (a) transfer of the Research Program to Licensee and (b) completion of all tasks for such Indication by Licensee under the Research Program SRA, for such Indication plus an additional [***] after achievement of TP for such Indication, Licensor will ensure that Dr. Gray, Dr. Minassian, and the Gray Laboratory shall not collaborate with any commercial Third Party to develop another Gene Therapy Product for the same Indication (as such Indication is updated throughout the agreement when additional indications are included pursuant to this Agreement) ("**Limited Exclusivity Covenant**").

2.8 **Program Failure**. In the event that the portion of the Research Program associated with any Licensed Product for any Indication fails at a key decision point during the Research Program, as such failure is objectively defined in the work plan for such project, and a decision is subsequently made by the Licensee to discontinue further development of the program for such Indication ("**Failed Indication**"), any remaining funding allocated for the Failed Indication program pursuant to a Research Program SRA (minus wind-down and non-cancellable expenses) will be promptly returned to Licensee or allocated as set forth in the Research Program SRA.

2.9 **Governance**.

2.9.1 **Joint Steering Committee**.

(a) Formation; Composition. Within [***] of the Effective Date, the Parties will establish a joint steering committee (the "**Joint Steering Committee**" or "**JSC**") comprised of [***] representatives from each Party with sufficient seniority within the applicable Party to make decisions arising within the scope of the JSC's responsibilities. The JSC may change its size from time to time by mutual consent of its members, provided that the JSC will consist at all times of an equal number of representatives of each of Licensor and Licensee. Each Party may replace its JSC representatives at any time upon written notice to the other Party.

(b) Specific Responsibilities. The JSC will:

(i) oversee the Research Program, including but not limited to any Research Program SRAs entered into between the Parties;

(ii) approve (once acceptable to the JSC) any amendments to Research Program SRAs [***], subject to clause (b) of Section 2.3.2;

(iii) determine whether Licensor or Licensee will contract directly with subcontractors;

(iv) use good-faith efforts to resolve any disagreement between the Parties relating to the Research Program and any Research Program SRA;

(v) establish such additional subcommittees as it deems necessary to achieve the objectives and intent of the Research Program; and

(vi) use good-faith efforts to resolve other issues presented to it by either Party pertaining to the administration of the Research Program or other matters covered by this Agreement.

For clarity, once a development candidate for an Indication is named, the JSC shall meet and determine if Licensee or Licensor is better suited to continue with the drug development process and if any obligations of a Party pursuant to an SRA should be transferred to the other Party in order to develop such development candidate more efficiently.

(c) Reporting. Each Party shall keep the JSC informed on the progress of the activities under the Research Program then currently ongoing under a Research Program SRA, including delivering quarterly written updates of its progress under any Research Program SRAs to the JSC at least [***] in advance of each JSC meeting.

(d) Meetings. During the performance of Research Program SRAs by Licensor, the JSC will meet at least quarterly. Following the completion of Licensor's performance of the Research Program SRAs, the Parties may agree to meet to discuss items previously addressed by the JSC, but the JSC shall no longer meet for such Indication. The JSC may meet in person, by videoconference or by teleconference. Notwithstanding the foregoing, at least [***] meetings per Calendar Year will be in person unless the parties mutually agree in writing to waive such requirement. In-person JSC meetings will be held at locations at Licensor's facilities in Dallas. Meetings of the JSC will be effective only if all representatives of each Party are present or participating in such meeting. The JSC shall keep accurate minutes of its deliberations which shall record all proposed

decisions and all actions recommended or taken. The secretary of the JSC (as appointed by the members of the JSC) shall be responsible for the preparation of draft minutes. Draft minutes shall be sent to all members of the JSC within [***] after each meeting and shall be approved, if appropriate, at the next meeting. All records of the JSC shall at all times be available to both Licensor and Licensee.

(e) Decision-Making. The representatives from each Party on the JSC will have, collectively, one (1) vote on behalf of that Party. If the JSC is unable to reach agreement on any issue or matter for which it is responsible, such disputed matter will be escalated to Licensee's Chief Executive Officer and Licensor or their designee, for discussion in good faith. In the event that after escalation the Parties are unable to reach agreement with respect to the disputed matter, then [***]. If Licensee assumes performance of Licensor's obligations under a Research Program SRA with respect to a particular Indication, Licensee shall pay Licensor all costs through the date of transition to Licensee per the budget of the Research Program SRA for such Indication.

ARTICLE 3
LICENSES AND OTHER RIGHTS

3.1 **Grant of License**. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee and its Affiliates the following (collectively, the "**License**"):

3.1.1 an exclusive, worldwide, royalty-free, right and license (with the right to sublicense through multiple tiers, subject to the provisions of Section 3.4), under Licensor Patent Rights to make, have made, use, sell, offer for sale and import Licensed Products for the Indications in the Field of Use during the Term; and

3.1.2 a non-exclusive, world-wide, royalty-free, right and license (with the right to sublicense through multiple tiers subject to the provisions of Section 3.4), under Licensed Know-How and Licensed Materials (and Licensor's intellectual property rights therein) to use and practice the same in order to make, have made, use, sell, offer for sale and import Licensed Products for the Indications in the Field of Use during the Term.

3.2 **Retained Rights**. Notwithstanding the License, Licensor retains the right under Licensor Patent Rights to: (a) conduct educational, non-commercial research, clinical activities and patient care activities itself, including, but not limited to sponsored research, and (b) authorize non-commercial Third Parties to conduct educational, non-commercial research and clinical activities and patient care activities; provided that in all cases clinical research may not be conducted with a specific vector and transgene combination once such is identified in a Research Program SRA or by the JSC, except as permitted in a clinical study agreement between the Parties or otherwise permitted by the Licensee.

3.3 **U.S. Government Rights**. The License is expressly subject to all applicable provisions of any license to the United States Government executed by Licensor and is subject to any overriding obligations to the United States Federal Government under 35 U.S.C. §§200-212, applicable governmental implementing regulations, and the U.S. Government sponsored research agreement or other guidelines, including that products that result from intellectual property funded by the United States Federal Government that are sold in the United States be substantially manufactured in the United States.

3.4 **Grant of Sublicense by Licensee**.

3.4.1 Licensor grants to Licensee the right to grant sublicenses, in whole or in part, under the License (each, a "**Sublicense**") subject to the terms and conditions of this Agreement and specifically this Section 3.4. The term Sublicense shall include any grant of rights under the License by a Sublicensee to any downstream Third Party, such downstream Third Party shall also be considered a Sublicensee for purposes of this Agreement.

3.4.2 All Sublicenses will (a) be issued in writing, and (b) to the extent applicable, include all of the rights of Licensor and require the performance of obligations due to Licensor (and, if applicable, the U.S. Government under 35 U.S.C. §§200-212). A Sublicense Document shall not grant rights in the Licensor Patent Rights, Licensed Know-How, or Licensed Materials that exceed the scope and rights granted to Licensee hereunder. Sublicensee must agree in writing to be bound by the applicable terms and conditions of this Agreement and shall indicate that Licensor is a third party beneficiary of the Sublicense Document.

3.4.3 Licensee shall deliver to Licensor a true, complete, and correct copy of each Sublicense Document within [***] days following the applicable execution, modification, or termination of such Sublicense Document which agreement may be redacted to remove confidential information of sublicensee. If the Sublicense Document is not in English, Licensee shall provide Licensor an accurate English translation in addition to a copy of the original agreement.

3.5 **Delivery of Know-How.** Licensor shall upon Licensee's reasonable request and at Licensee's cost and expense, disclose and deliver Licensed Know-How and/or Licensed Materials to Licensee, and will provide such technical assistance as may be reasonably needed for Licensee to interpret and use such Licensed Know-How and/or Licensed Materials for the exploitation of the License.

3.6 **No Implied License.** Each Party acknowledges that the rights and licenses granted in this Agreement are limited to the scope expressly granted. Accordingly, except for the rights expressly granted under this Agreement, no right, title, or interest of any nature whatsoever is granted whether by implication, estoppel, reliance, or otherwise, by either Party to the other Party. All rights with respect to any know-how, patent or other intellectual property right rights that are not specifically granted herein are reserved to the owner thereof.

ARTICLE 4
FINANCIAL PROVISIONS

4.1 **Equity Issuance**. On the Effective Date, and in partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee and Licensor shall enter into an Equity Issuance Agreement in the form attached as Exhibit B. Licensee shall issue Licensor shares of common stock of Licensee equal to [***] of the fully-diluted common stock of Licensee with assignable participation rights included in such Equity Issuance Agreement. The calculation of fully-diluted ownership in this Article 4 shall include all shares of common stock outstanding as of the date in question, assuming conversion of all convertible debt and equity and the exercise of all authorized capital stock and equity rights, including options, warrants, calls, rights, commitments, and agreements of any character (including any Simple Agreement for Future Equity) obligating Company to issue, deliver, or sell any shares of capital stock or that Company is obligated or committed to issue, deliver, or sell, including any such capital stock or equity rights that an investor has agreed may be authorized to be issued pursuant to any incentive compensation plan after the closing of a financing.

4.2 [***].

ARTICLE 5
CLINICAL DEVELOPMENT, REGULATORY AFFAIRS; COMMERCIALIZATION

5.1 **Development**. Licensee will be responsible for all post-TP Licensed Product development activities including all clinical testing, regulatory approvals, marketing and commercialization (except for manufacturing of Licensed Products in support of these activities, which may be performed by Licensee and Licensor, pursuant to a mutually agreed upon manufacturing agreement with Licensee).

5.2 **Clinical**. Licensee shall be responsible for all clinical activities and lead regulatory interactions for the Licensed Products for each Indication under this Agreement. Without limiting the foregoing, Licensee may, but does not have the obligation, to contract with Licensor to conduct a FIH Clinical Study for each Licensed Product developed under the Research Program. Licensee will consider in good faith using Licensor as a study site for one or more studies where Licensor can reasonably demonstrate that Licensor's capabilities and costs are reasonably comparable to other potential study sites. If Licensor is willing and able to conduct a Clinical Study for a Licensed Product developed under the Research Program, the Parties will negotiate a separate clinical study agreement and a separate clinical study budget prior to initiation of such Clinical Study.

5.3 **Commercialization**. Licensee will have sole responsibility for and sole decision-making over all commercialization activities of the Licensed Products for the Indications in the Field of Use, and will be solely responsible for the associated costs of such commercialization activities.

5.4 **Manufacturing**. Except as otherwise provided in this Agreement or in a Research Program SRA, Licensee will have responsibility for and decision-making authority over all manufacturing activities and associated costs for the clinical development (including GMP manufacturing for Clinical Studies, even if performed by Licensor) and commercialization of the Licensed Products in the Field of Use for the Indications post-TP for such Licensed Product. The Parties may enter into a manufacturing agreement for GMP manufacturing of Licensed Products (including components thereof).

5.5 **Regulatory**.

5.5.1 Licensee will have responsibility for and decision-making over regulatory activities for the Licensed Products for the Indications in the Field of Use. Licensee will have the right to conduct all communications with Regulatory Authorities, including all meetings, conferences and discussions (including advisory committee meetings), with regard to Licensed Products for the Indications in the Field of Use. Licensee will lead and have control over preparing and submitting all regulatory filings related to the Licensed Products for the Indications in the Field of Use, including all applications for Regulatory Approval. Licensee will own any and all applications for Regulatory Approvals (including INDs), Regulatory Approvals, and other regulatory filings related to the Licensed Product for the Indication in the Field of Use, which will be held in the name of Licensee or its designees.

5.5.2 Licensor will cooperate with any reasonable request from Licensee with respect to obtaining any Regulatory Approval for a Licensed Product for the Indication in the Field of Use including, at Licensee's cost: (a) making its faculty, employees, consultants and other staff available to assist Licensee upon reasonable notice, (b) responding to questions raised by Licensee, and (c) making available to Licensee, in the form requested by Licensee, information related to the Licensed Products that is necessary to prepare, file, obtain and maintain any Regulatory Approval for such Licensed Product.

5.6 **General Diligence**. Licensee by itself or through its Affiliates and Sublicensees will use Commercially Reasonable Efforts to actively develop, obtain Regulatory Approval and commercialize at least one Licensed Product within the Field of Use. This obligation shall not prohibit Licensee from terminating this Agreement at any time with respect to any Indication, after which Licensee's obligation under this Section 5.6 shall only apply to the remaining Indications.

5.7 [***].

5.8 **Progress Reports**. After the performance of Research Program SRAs by Licensor but prior to the First Commercial Sale of a Licensed Product in the respective Indication, Licensee on an [***] basis, but in no event later than [***], shall submit to Licensor a progress report (each, a "**Progress Report**") covering Licensee's (and any Affiliates' and Sublicensees') activities related to the development of all Licensed Products in each Indication and the obtaining of Regulatory Approvals necessary for commercialization of Licensed Products.

5.9 **Government and Economic Development Reporting**. If Licensor requests, Licensee will provide information for Licensor's Government and economic development reporting purposes, including the following:

5.9.1 Number and geographic location of new full-time employees created during the past Contract Year; total number and geographic location of full time employees of Licensee at the end of such Contract Year;

5.9.2 Dollar amount of new equity financing received by Licensee during the past Contract Year, and current capitalization, including number and class of outstanding securities;

5.9.3 Location and square footage of facilities; and

5.9.4 Other information required under Federal and state law.

ARTICLE 6
INTELLECTUAL PROPERTY

6.1 **Patent Filing Prosecution and Maintenance**.

6.1.1 Licensor Patent Rights will be held in the name of Licensor and obtained with counsel authorized to represent Licensor and mutually agreed upon by the Parties ("**Patent Counsel**"). Licensor will provide Licensee with advance copies of filings of the Licensor Patent Rights and will implement any reasonable comments or suggestions by Licensee with respect to same. Licensor will instruct Patent Counsel to copy Licensee on all correspondence related to Licensor Patent Rights (including copies of each patent application, office action, response to office action, request for terminal disclaimer, and request for reissue or reexamination of any patent or patent application) and to interact with Licensee with respect to the preparation, filing, prosecution and maintenance of Licensor Patent Rights. In the event of a disagreement between Licensee and Licensor regarding a filing for the Licensor Patent Rights or any strategy relating to the filing, prosecution or maintenance of the Licensor Patent Rights, Licensee shall have final decision making with respect to the strategy and any such filing, prosecution or maintenance. Provided that

Licensee is not in breach with respect to reimbursement of Patent Costs, Licensor shall not allow any Licensor Patent Rights for which Licensee is licensed to lapse or become abandoned without Licensee's written authorization under this Agreement, even if such lapse or abandonment is associated with the filing of continuations, divisionals, or the like that substitute for the lapsed application. For the purposes of this Agreement, "maintenance" of the Licensor Patent Rights includes *inter partes* patent review proceedings before the USPTO or a similar patent administration outside the US. For further clarity, validity challenges raised in infringement litigation will be handled per Section 6.3, Infringement.

6.1.2 Licensee has the right to request a country filing via a written request to Licensor [***] prior to the deadline set by the patent office in the territory in which filing is to take place ("**Prosecution Request**").

6.2 **Patent Costs**.

6.2.1 Licensee will bear all Patent Costs incurred during the Term after the Effective Date ("**Ongoing Patent Costs**"), on a pro rata basis (as described below) where applicable. Notwithstanding anything to the contrary in this Agreement, Licensee shall have no obligation to reimburse or bear any Patent Costs for any *inter partes* patent review proceedings or other post-grant proceedings before the USPTO or any similar patent administration outside the US, to the extent such proceedings are undertaken or such Patent Costs are otherwise incurred without Licensee's consent or without Licensee's involvement in such proceedings. For Licensor Patent Rights that are also licensed to one or more other commercial Third Parties, Licensee shall be responsible for payment to Licensor of a pro rata share of such Ongoing Patent Costs based on the number of such licensees for such Licensor Patent Rights. "**Patent Costs**" means all out-of-pocket costs for the filing, prosecution and maintenance of Licensor Patent Rights, including all accrued attorney fees, expenses, official and filing fees incurred after the Effective Date.

6.2.2 Licensee shall pay such Patent Costs within [***] of receipt of an invoice for such patent actions. For clarity, this Section 6.2.2 shall not apply during any period during the Term where a separate agreement between Licensor and Licensee is in effect regarding direct billing of Licensee by Patent Counsel.

6.2.3 Licensee may, upon [***] advance written notice to Licensor, elect to abandon any patent or patent application included in the Licensor Patent Rights. Upon the effective date of such notice, such patent or patent application shall no longer be part of the License granted to Licensee in Section 3.1 or the Licensor Patent Rights for purposes of this Agreement (including the provisions of this Section 6.2).

6.3 **Infringement**.

6.3.1 If either Party believes that an infringement by a Third Party with respect to any Licensor Patent Right is occurring or may potentially occur, the knowledgeable Party will provide the other Party with (a) written notice of such infringement or potential infringement and (b) evidence of such infringement or potential infringement (the "**Infringement Notice**"). Licensee shall not notify such a Third Party (including the infringer) of infringement or put such Third Party on notice of the existence of Licensor Patent Rights without first obtaining the written consent of Licensor, which consent will not be unreasonably withheld, conditioned or delayed. Both Licensor and Licensee will use reasonable efforts to cooperate with each other to terminate such infringement without litigation.

6.3.2 If infringing activity of potential commercial significance has not been abated within [***] following the date the Infringement Notice for such activity was provided, then during the period in which, and in the jurisdiction where, Licensee is the sole licensee of such infringed Licensor Patent Right, Licensee may institute suit for patent infringement of a Licensor Patent Right against the infringer. Licensor may voluntarily join such suit at Licensee's reasonable expense, but may not thereafter commence suit against the infringer for the acts of infringement that are the subject of Licensee's suit or any judgment rendered in such suit. If in a suit initiated by Licensee, Licensor is involuntarily joined other than by Licensee, then Licensee will pay any costs reasonably incurred by Licensor arising out of such suit, including any reasonable legal fees of counsel that Licensor selects and retains to represent it in the suit. Licensee shall be free to enter into a settlement, consent judgment or other voluntary disposition, provided that any settlement, consent judgment or other voluntary disposition that (i) limits the scope, validity or enforcement of Licensor Patent Rights or (ii) admits fault or wrongdoing on the part of Licensor must be approved in advance by Licensor in writing. Licensor shall provide Licensee notice of its approval or denial within [***] of any request for such approval by Licensee, provided that (x) in the event Licensor wishes to deny such approval, such notice shall include a detailed written description of Licensor's reasonable objections to the proposed settlement, consent judgment, or other voluntary disposition and (y) Licensor shall be deemed to have approved of such proposed settlement, consent judgment, or other voluntary disposition in the event it fails to provide such notice within such [***] period in accordance herewith.

6.3.3 If, within [***] following the date the Infringement Notice was provided, infringing activity of potential commercial significance has not been abated and if Licensee has not brought suit against the infringer, then Licensor may institute suit for patent infringement against the infringer. If Licensor institutes such suit, then Licensee may not join such suit without the prior written consent of Licensor and may not thereafter commence suit against the infringer for the acts of infringement that are the subject of Licensor's suit or any judgment rendered in such suit.

6.3.4 Notwithstanding Sections 6.3.2 and 6.3.3, (a) in the event that any Licensor Patent Rights are infringed by a Third Party prior to the First Commercial Sale of a Licensed Product in the United States or (b) if any of the infringed Licensor Patent Rights are also licensed by Licensor to a Third Party prior to any enforcement action being taken by either Party regarding such infringement, the Parties shall discuss, and will mutually agree, in writing, as to how to handle such infringement by such Third Party.

6.3.5 Any recovery or settlement received in connection with any suit will first be shared by Licensor and Licensee equally to cover any litigation costs each incurred [***]. Any remaining recoveries shall be allocated to Licensee; except for any suit that is initiated by Licensor and in which Licensee was not a party in the litigation, then such recovery in excess of litigation costs will belong to Licensor.

6.3.6 Each Party will reasonably cooperate and assist with the other in litigation proceedings instituted hereunder but at the expense of the Party who initiated the suit (unless such suit is being jointly prosecuted by the Parties). For clarity, such requirement does not require a Party to join a suit unless otherwise specifically required under this Agreement.

6.4 **Patent Marking**. Licensee shall place in a conspicuous location on any Licensed Product (or its packaging where appropriate and practicable) made or sold under this Agreement a patent notice in accordance with the Laws concerning the marking of patented articles where such Licensed Product is made or sold, as applicable.

6.5 **Research Program SRAs**. For clarity, all intellectual property arising from or generated during the performance of a Research Program SRA shall be governed by the terms and conditions of this Agreement.

ARTICLE 7
CONFIDENTIALITY & PUBLICATION

7.1 **Confidential Information**. Licensee shall not disclose Confidential Information to Licensor unless it is anticipated to be necessary or useful to the performance of the Research Program or is reasonably responsive to Licensee's reporting obligations under this Agreement or any other request of Licensor. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that, during the Term and for [***] thereafter, the receiving Party (the "**Receiving Party**") and its Affiliates will keep confidential and will not publish or otherwise disclose or use for any purpose, other than as provided for in this Agreement, any confidential or proprietary information or materials, patentable or otherwise, in any form (written, oral, photographic, electronic, magnetic, or otherwise), including trade secrets, Know-How, inventions or discoveries, proprietary information, formulae, processes, techniques and information relating to the past, present and future marketing, financial, and research and development activities of any product or potential product or useful technology of the Disclosing Party or its Affiliates and the pricing thereof (collectively, "**Confidential Information**"), which is disclosed to it by the other Party (the "**Disclosing Party**") or its Affiliates or otherwise received or accessed by a Receiving Party in the course of performing its obligations or exercising its rights under this Agreement.

7.2 **Exceptions to Confidentiality**. "Confidential Information" does not include information that (a) was in the lawful knowledge and possession of the Receiving Party or its Affiliates prior to the time it was disclosed to, or learned by, the Receiving Party or its Affiliates, or was otherwise developed independently by the Receiving Party or its Affiliates, as evidenced by written records kept in the ordinary course of business, or other documentary proof of actual use by the Receiving Party or its Affiliates; (b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party or its Affiliates, as evidenced by written records of the Receiving Party or its Affiliates; (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party or its Affiliates in breach of this Agreement; or (d) was disclosed to the Receiving Party or its Affiliates, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party or its Affiliates not to disclose such information to others. In the event a Receiving Party is required to make a disclosure under Law or regulation, the order of a court of competent jurisdiction, or the rules of the U.S. Securities and Exchange Commission (including by reason of any securities offering by Licensee), any stock exchange or listing entity, such disclosure will not constitute a breach of this Article 7 provided such Receiving Party shall provide prompt written notice to the Disclosing Party and take all reasonable steps to limit the extent of the disclosure and obtain confidential treatment for any remaining required disclosure.

7.3 **Publications**. Licensor shall have the first right to publish, publicly present or otherwise publicly disclose Research Results for any purpose, subject to the following provisions; provided, however, that if Licensee provides written request to Licensor to publish, publicly present or otherwise

publicly disclose the Research Results, and Licensor does not publish, publicly present or otherwise publicly disclose such Research Results within [***] of such written request, Licensee shall be entitled to publish, publicly present or otherwise publicly disclose such Research Results in accordance with the provisions of this Section 7.3. Any disagreement regarding publications of Research Results during the Research Term shall be determined by the JSC. Licensor shall furnish the Licensee with a copy of any proposed publication, presentation, or other public disclosure at least [***] in advance of the date of such presentation or public disclosure or the submission of said proposed publication in order for Licensee to review and comment on said proposed publication, presentation, or other public disclosure to (a) determine whether such contains any Licensee Confidential Information and (b) enable Licensee to identify any Licensor intellectual property that it wishes Licensor to file patent applications on or to seek other intellectual property protection for. If within the [***] review period (i) Licensee notifies Licensor in writing that the Licensee requires deletion of Licensee Confidential Information from the publication, presentation, or other disclosure, the Parties will cooperate to modify the same to ensure Licensee Confidential Information is not disclosed or (ii) if Licensee requests in writing that publication, presentation, or other disclosure be delayed to allow for patent filings or other intellectual property protection on certain items in the proposed publication, presentation, or other disclosure, Licensor shall delay the same for up to [***] to allow for the filing of patent applications or other intellectual property protection.

ARTICLE 8
REPRESENTATIONS, WARRANTIES AND COVENANTS

8.1 **Mutual Representations and Warranties**. Each Party represents and warrants to the other Party that, as of the Effective Date:

8.1.1 such Party is duly organized and validly existing under the Laws of the jurisdiction of its incorporation or organization;

8.1.2 such Party has taken all action necessary to authorize the execution and delivery of this Agreement and the performance of its obligations under this Agreement;

8.1.3 this Agreement is a legal and valid obligation of such Party, binding upon such Party and enforceable against such Party in accordance with the terms of this Agreement, except as enforcement may be limited by applicable bankruptcy, fraudulent conveyance, insolvency, reorganization, moratorium and other laws relating to or affecting creditors' rights generally and by general equitable principles; and

8.1.4 such Party has all right, power and authority to enter into this Agreement, to perform its obligations under this Agreement.

8.2 **Representation and Warranties of Licensor.** Except for the rights, if any, of the Governmental Body, Licensor represents and warrants to Licensee that: (i) Licensor is the owner or agent of the entire right, title, and interest in and to Licensor Patent Rights (other than the right, title and interest of any joint owner identified in Exhibit A), (ii) Licensor has the right to grant licenses hereunder, (iii) to the knowledge of Licensor's designated office for technology commercialization, Licensor has not knowingly granted and will not grant licenses or other rights under the Licensor Patent Rights that are in conflict with the terms and conditions in the Agreement and (iv) to the knowledge of Licensor's designated office for technology commercialization, Licensor has not received: (a) any written notice that any of the Licensor Patent Rights are invalid or unenforceable, or (b) any written notice of any actual or threatened claims that the use of or practice of the Licensor Patent Rights, Licensor Know-How, or Licensor Material infringes or misappropriates (or has infringed or misappropriated) the intellectual property of any Third Party.

8.3 **Disclaimer of Representations and Warranties**. Other than the representations and warranties provided in Sections 8.1 and 8.2 above, **NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, WHETHER EXPRESS OR IMPLIED, AND EXPLICITLY DISCLAIMS ANY REPRESENTATION AND WARRANTY**.

ARTICLE 9
INDEMNIFICATION; INSURANCE AND LIMITATION OF LIABILITY

9.1 **Indemnification by Licensee**.

9.1.1 Licensee shall defend, indemnify and hold Licensor and its respective trustees, officers, faculty, students, employees, contractors and agents (the "**Licensor Indemnitees**") harmless from and against any and all liability, damage, loss, cost or expense, to the extent directly arising from Third Party claims or suits to the extent directly arising from:

(a) the gross negligence, recklessness or wrongful intentional acts or omissions of Licensee, its Affiliates or Sublicensees and its or their respective directors, officers, employees and agents, in connection with Licensee's performance of its obligations or exercise of its rights under this Agreement;

(b) any material breach of this Agreement by Licensee; or

(c) the development, manufacturing (unless manufactured by Licensor) or commercialization of a Licensed Product by or on behalf of Licensee or its Affiliates or Sublicensees;

provided that Licensee's obligations pursuant to this Section 9.1 shall not apply to the extent such claims or suits result from the gross negligence or willful misconduct of any of Licensor Indemnitees as determined by a court of law.

9.1.2 As a condition to a Licensor Indemnitee's right to receive indemnification under this Section 9.1, Licensor shall: (a) promptly notify Licensee as soon as it becomes aware of a claim or suit for which indemnification may be sought pursuant hereto; (b) reasonably cooperate, and cause the individual Licensor Indemnitees to reasonably cooperate, with Licensee in the defense, settlement or compromise of such claim or suit; and (c) permit the Licensee to control the defense, settlement or compromise of such claim or suit, including the right to select defense counsel. In no event, however, may Licensee compromise or settle any claim or suit in a manner which (a) admits fault or negligence on the part of Licensor or any other Licensor Indemnitee; (b) commits Licensor or any other Licensor Indemnitee to take, or forbear to take, any action, without the prior written consent of Licensor, or (c) grant any rights under the Licensor Patent Rights except for Sublicenses permitted under Article 2. Licensor shall reasonably cooperate with Licensee and its counsel in the course of the defense of any such suit, claim or demand, such cooperation to include without limitation using reasonable efforts to provide or make available documents, information and witnesses.

9.2 **Insurance**. Prior to commencement of a Clinical Study of a Licensed Product, Licensee shall maintain insurance during the Term, at its sole cost and expense, of the types and in amounts which

are reasonable and customary in the U.S. Pharmaceutical industry for companies of comparable size and activities obtained from a reputable insurer to protect against potential liabilities and risk arising out of the activities to be performed under this Agreement and upon such terms (including coverages and deductible limits) as are customary in the U.S. pharmaceutical industry generally for that activities to be conducted by Licensee under this Agreement. Upon written request from Licensor, Licensee shall provide written evidence (e.g., certificates) of such insurance to Licensor.

9.3 **LIMITATION OF LIABILITY**. EXCEPT FOR DAMAGES ARISING FROM A BREACH OF ARTICLE 7, IN NO EVENT SHALL EITHER PARTY BE LIABLE TO THE OTHER PARTY OR ANY OF ITS AFFILIATES FOR SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES, INCLUDING LOSS OF PROFITS, WHETHER IN CONTRACT, WARRANTY, TORT, NEGLIGENCE, STRICT LIABILITY OR OTHERWISE ARISING OUT OF OR RELATING TO THIS AGREEMENT, THE TRANSACTIONS CONTEMPLATED HEREIN OR ANY BREACH HEREOF.

ARTICLE 10
TERM AND TERMINATION

10.1 **Term**. The term of this Agreement (the "**Term**") shall commence on the Effective Date and, unless terminated sooner as provided below, shall continue in full force and effect on a country-by-country and Licensed Product-by-Licensed Product basis until the expiration of the last Valid Claim of the Licensor Patent Rights in such country for such Licensed Product. Upon expiration of such Agreement with respect to such Licensed Product, Licensee shall have a fully-paid up right and license.

10.2 **Termination of the Agreement for Convenience**. At any time after the Research Term, Licensee may, at its convenience, terminate this Agreement in its entirety or terminate this Agreement with respect to any Licensed Product for an Indication in the Field of Use on a Licensed Product-by-Licensed Product basis, upon providing at least [***] prior written notice to Licensor of such termination.

10.3 **Termination For Cause**.

10.3.1 If either Party materially breaches any of its material obligations under this Agreement, the non-breaching Party may give to the breach Party a written notice specifying the nature of the default, requiring it to cure such breach, and stating its intention to terminate this Agreement. If such breach is not cured within [***] of such notice, such termination shall become effective upon a notice of termination by the terminating Party thereafter. Provided however, if there is a dispute on whether a material breach occurred or if a breach was material such termination is tolled pending resolution of such dispute and the License shall remain in effect during such period.

10.3.2 Either Party may terminate this Agreement, upon written notice, with immediate effect if, at any time, the other Party is unable to pay its debts when they come due, or files in any court or agency pursuant to any statute or regulation of any state, country or jurisdiction, a petition for the appointment of a receiver or trustee of such Party or of its assets, or if such Party proposes a written agreement of composition or extension of its debts, or if such Party is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition is not dismissed within [***] after the filing thereof, or if such Party is a party to any dissolution or liquidation, or if such Party makes an assignment for the benefit of its creditors of all or substantially all its assets (in each case, "**Bankruptcy Action**").

10.4 **Effects of Termination**.

10.4.1 Notwithstanding the termination of this Agreement, the following provisions shall survive: Sections 6.2, and 10.4 and Articles 1, 7, 8, 9, and 11. Furthermore, where this Agreement is terminated in relation to fewer than all of the Indications or Licensed Products (as provided above), it will remain in effect as to the non-terminated Indication(s) or non-terminated Licensed Product(s).

10.4.2 Termination of this Agreement shall not relieve the Parties of any obligation or liability that, at the time of termination, has already accrued hereunder, or which is attributable to a period prior to the effective date of such termination. Termination of this Agreement shall not preclude either Party from pursuing all rights and remedies it may have hereunder or at Law or in equity with respect to any breach of this Agreement nor prejudice either Party's right to obtain performance of any obligation.

10.4.3 If this Agreement is terminated for any reason, all outstanding Sublicenses (including all Sublicense Documents for each Sublicense) not in default will be assigned by Licensee to Licensor, and such assignment will be accepted by Licensor. Each assigned Sublicense will remain in full force and effect with Licensor as the licensor or sublicensor instead of Licensee, but the duties and obligations of Licensor under the assigned Sublicenses will not be greater than the duties of Licensor under this Agreement, and the rights of Licensor under the assigned Sublicenses will not be less than the rights of Licensor under this Agreement, including all financial consideration and other rights of Licensor. Licensor may, at its reasonable discretion, amend such outstanding Sublicenses to contain the terms and conditions found in this Agreement. [***]. Where this Agreement is terminated in relation to fewer than all of the Indications or Licensed Products (as provided above), the foregoing paragraph will not apply to the extent any Sublicense pertains to any non-terminated Indication(s) or Licensed Product(s).

10.4.4 Within [***] days of termination of this Agreement or any Indication (other than termination by Licensee pursuant to Section 10.3), Licensee shall pay Licensor all costs through the effective termination date per any Research Program SRA as well as all commitments related to the performance of the Research Program SRAs (i.e., all costs or non-cancellable commitments incurred prior to the receipt, or issuance, by Licensor of the notice of termination, and the cost of each employee, student and faculty member to the extent supported under the Research Program SRA until the effective date of termination; and subject to Licensor's written notification to Licensee and Licensee's acknowledgement of all costs and non-cancellable commitments as they arise) incurred by Licensor under this Agreement, or for the terminated Indication, as applicable.

ARTICLE 11
ADDITIONAL PROVISIONS

11.1 **Relationship of the Parties**. Nothing in this Agreement is intended or shall be deemed, for financial, tax, legal or other purposes, to constitute a partnership, agency, joint venture or employer-employee relationship between the Parties. The Parties are independent contractors and at no time will either Party make commitments or incur any charges or expenses for or on behalf of the other Party.

11.2 **Expenses**. Except as otherwise provided in this Agreement, [***].

11.3 **Third Party Beneficiary**. The Parties agree that each Sublicensee is a third party beneficiary of this Agreement with respect to Section 10.4.3.

11.4 **Use of Names**. Licensee, its Affiliates and Sublicensees may not use the name, logo, seal, trademark, or service mark (including any adaptation of them) of Licensor or any Licensor school, organization, employee, student or representative, without the prior written consent of Licensor. Notwithstanding the foregoing, Licensee may use the name of Licensor in a non-misleading and factual manner solely in (a) executive summaries, business plans, offering memoranda and other similar documents used by Licensee for the purpose of raising financing for the operations of Licensee as related to Licensed Product, or entering into commercial contracts with Third Parties, but in such case only to the extent necessary to inform a reader that the Licensor Patent Rights and/or Licensed Know-How (subject to the provisions of Section 7) has been licensed by Licensee from Licensor, and/or that Licensee is collaborating with Licensor on the Research Program, and to inform a reader of the identity and published credentials of inventors of intellectual property, and (b) any securities reports required to be filed with the Securities and Exchange Commission.

11.5 **No Discrimination**. Neither Licensor nor Licensee will discriminate against any employee or applicant for employment because of race, color, sex, sexual or affectional preference, age, religion, national or ethnic origin, handicap, or veteran status.

11.6 **Successors and Assignment**. The terms and provisions hereof shall inure to the benefit of, and be binding upon, the Parties and their respective successors and permitted assigns. Licensee may assign or transfer this Agreement or any of its rights or obligations created hereunder, in whole or in part to (a) an Affiliate, or (b) to any Third Party, without the prior written consent of Licensor.

11.7 **Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments and to do all such other acts as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

11.8 **Entire Agreement of the Parties; Amendments**. This Agreement, the Exhibits and Appendices or Schedules hereto, and the Equity Issuance Agreement constitute and contain the entire understanding and agreement of the Parties respecting the subject matter hereof and cancel and supersede any and all prior negotiations, correspondence, understandings and agreements between the Parties, whether oral or written, regarding such subject matter. No waiver, modification or amendment of any provision of this Agreement shall be valid or effective unless made in a writing referencing this Agreement and signed by a duly authorized officer of each Party.

11.9 **Governing Law**. This Agreement shall be governed by and interpreted in accordance with the laws of the state of Texas, excluding application of any conflict of laws principles that would require application of the law of a jurisdiction outside of the state of Texas. Nothing in this Agreement shall constitute a waiver of sovereign immunity by System or UT Southwestern.

11.10 **Dispute Resolution**. If a dispute arises between the Parties concerning this Agreement, then the Parties will confer, as soon as practicable, in an attempt to resolve the dispute (in accordance with the provisions of Section 2.9 as applicable); provided, however, that the foregoing will not prohibit either Party from (a) immediately seeking and/or obtaining preliminary injunctive or other equitable relief for any actual or threatened breach of confidentiality by the other Party or (b) submitting a dispute (subject to the provisions of Section 2.9 as applicable) to the jurisdiction of, and venue in, the state and Federal courts located in the Northern District of Texas.

11.11 **Notices and Deliveries**. Any notice, request, approval or consent required or permitted to be given under this Agreement shall be in writing and directed to a Party at its address or facsimile number shown below or such other address or facsimile number as such Party shall have last given by notice to the other Party. A notice will be deemed received: if delivered personally, on the date of delivery; if mailed, [***] after deposit in the United States mail; if sent via courier, [***] after deposit with the courier service; or if sent via facsimile, upon receipt of confirmation of transmission provided that a confirming copy of such notice is sent by certified mail, postage prepaid, return receipt requested.

For Licensor

UT Southwestern Medical Center
[***]

For Licensee:

Taysha Gene Therapies, Inc.
[***]

with a copy to

[***]

11.12 **Waiver**. A waiver by either Party of any of the terms and conditions of this Agreement in any instance shall not be deemed or construed to be a waiver of such term or condition for the future, or of any other term or condition hereof. Except as otherwise provided herein, all rights, remedies, undertakings, obligations and agreements contained in this Agreement shall be cumulative and none of them shall be in limitation of any other remedy, right, undertaking, obligation or agreement of either Party.

11.13 **Severability**. When possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under law, but if any provision of this Agreement is held to be prohibited by or invalid under law, such provision will be ineffective only to the extent of such prohibition or invalidity, without invalidating the remainder of this Agreement. The Parties shall make a good faith effort to replace the invalid or unenforceable provision with a valid one which in its economic effect is most consistent with the invalid or unenforceable provision.

11.14 **Export Compliance**. Licensee understands that the Arms Export Control Act (AECA), including its implementing International Traffic In Arms Regulations (ITAR), and the Export Administration Act (EAA), including its Export Administration Regulations (EAR), are some (but not all) of the laws and regulations that comprise the U.S. export laws and regulations. Licensee further understands that the U.S. export laws and regulations include (but are not limited to): (a) ITAR and EAR product/service/data-specific requirements; (b) ITAR and EAR ultimate destination-specific requirements; (c) ITAR and EAR end user-specific requirements; (d) Foreign Corrupt Practices Act; and (e) anti-boycott laws and regulations. Licensee will comply with all then-current applicable export laws and regulations of the U.S. Government (and other applicable U.S. laws and regulations) pertaining to the Licensed Products (including any associated products, items, articles, computer software, media, services, technical data, and other information). Licensee certifies that it will not, directly or indirectly, export (including any deemed export), nor re-export (including any deemed re-export) the Licensed Products (including any associated products, items, articles, computer software, media, services, technical data, and other information) in violation of applicable U.S. laws and regulations. Licensee will include a provision in its agreements, substantially similar to this Section 11.14, with its Sublicensees, third party wholesalers and distributors, and physicians, hospitals or other healthcare providers who purchase a Licensed Product, requiring that these parties comply with all then-current applicable U.S. export laws and regulations and other applicable U.S. laws and regulations.

11.15 **Interpretation**. The words “include,” “includes” and “including” shall be deemed to be followed by the phrase “without limitation.” All references herein to Articles, Sections, Schedules and Exhibits shall be deemed references to Articles and Sections of, Schedules and Exhibits to, this Agreement unless the context shall otherwise require. Unless the context otherwise requires, countries shall include territories. References to any specific Law or article, section or other division thereof, shall be deemed to include the then-current amendments or any replacement Law thereto.

11.16 **Counterparts**. This Agreement may be executed in counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. A facsimile or a portable document format (PDF) copy of this Agreement, including the signature pages, will be deemed an original.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have executed this Agreement as of the Effective Date.

BOARD OF REGENTS OF THE
UNIVERSITY OF TEXAS SYSTEM

By /s/ Arnim Dontes
Arnim Dontes
Executive Vice President for Business Affairs
UT Southwestern Medical Center

Date 11/21/2019

TAYSHA GENE THERAPIES, INC.

By /s/ RA Session II
RA Session II
Founder

Date 11/19/19

Approved as to Content:

By /s/ Claire Aldridge
Claire Aldridge, Ph.D.
Associate Vice President,
Commercialization and Business Development
UT Southwestern Medical Center

Date 11/20/2019

[Signature Page to Research, Collaboration & License Agreement]

Exhibit A

Licensor Patent Rights

[***]

Exhibit B

Equity Issuance Agreement

[***]

Certain information has been excluded from this agreement (indicated by "[***]") because Taysha Gene Therapies, Inc. has determined such information (i) is not material and (ii) would be competitively harmful if publicly disclosed.

AMENDMENT TO RESEARCH, COLLABORATION & LICENSE AGREEMENT
AGT NO. 2020-0029/L3729-Taysha

This Amendment to the November 19, 2019 Research, Collaboration & License Agreement ("**Amendment**") is entered into as of April 2, 2020 (the "**Amendment Effective Date**") by and between The Board of Regents ("**Board**") of The University of Texas System ("**System**"), an agency of the State of Texas whose address is 210 West 7th Street, Austin, Texas 78701, on behalf of The University of Texas Southwestern Medical Center ("**UT Southwestern**"), a component institution of System ("**Licensor**"), and Taysha Gene Therapies, Inc., a Texas corporation ("**Licensee**"). Licensor and Licensee are referred to collectively as the "**Parties**" and individually as a "**Party**". All capitalized terms used but not defined herein shall have the meaning ascribed to such term in the Agreement.

WHEREAS, the Parties entered into a certain Research, Collaboration & License Agreement (AGT No. 2020-0029/L3729-Taysha) ("**Agreement**"); and

WHEREAS, the Parties wish to amend the Agreement to include additional license grants to newly developed intellectual property rights of UT Southwestern.

NOW, THEREFORE, in consideration of the various promises and undertakings set forth herein, the Parties agree as follows:

1. **Amendments**. The Parties hereby agree to amend the Agreement as follows:

a. The following definitions are hereby amended as follows:

(i) The definition of "**Gray Laboratory**" shall include the following language at the end of the definition ", including but not limited to, Sara Sinnett, Ryan Butler, Marc Diamond and Rachel Bailey."

(ii) The definition of "**Licensed Product**" shall include "Licensed Capsid Know-How or Licensed Redosing Know-How" after each instance "Licensed Know-How" is used in such definition.

(iii) The definition of "**Named Indications**" shall include the following addition indications "[***]."

(iv) The definition of "**Valid Claim**" shall include "Licensor Capsid Patent Rights or Licensor Redosing Patent Rights" after each instance "Licensor Patent Rights" is used in such definition.

b. The following definitions are added to Section 1 of the Agreement:

1.49 "**AAV Capsid Platform**" means the proprietary AAV capsid platform Controlled by Licensor and conceived and reduced to practice by the Gray Laboratory and further described in the study entitled "[***]."

1.50 "**Licensed Capsid Know-How**" means all Know-How and Licensed Material that is Controlled by Licensor and (a) relating to the AAV Capsid Platform, and (b) developed by the Gray Laboratory, and in each case (a) and (b) is necessary or reasonably useful to develop, make, use, sell, offer for sale, or import a Licensed Product in the Field of Use.

1.51 "**Licensed Redosing Know-How**" means all Know-How and Licensed Material that is Controlled by Licensor and (a) relating to the Redosing Technology, and (b) developed by the Gray Laboratory, and in each case (a) and (b) is necessary or reasonably useful to develop, make, use, sell, offer for sale, or import a Licensed Product in the Field of Use.

1.52 "**Licensor Capsid Patent Rights**" means the (a) the Patent Rights Controlled by Licensor that are necessary or useful for the development of Licensed Product, including but not limited to Patent Rights that claim a method of manufacturing or producing a component contained in a Licensed Product, (b) any Patent Rights Controlled by Licensor and conceived and reduced to practice by Dr. Gray, Dr. Minassian, or the Gray Laboratory and relating to the AAV Capsid Platform, and (c) any corresponding foreign Patent Rights to the foregoing.

1.53 "**Licensor Redosing Patent Rights**" means the (a) the Patent Rights Controlled by Licensor that are necessary or useful for the development of a Licensed Product, including but not limited to Patent Rights that claim a method of manufacturing or producing a component contained in a Licensed Product, (b) any Patent Rights Controlled by Licensor and conceived and reduced to practice by Dr. Gray, Dr. Minassian, or the Gray Laboratory and relating to the Redosing Technology, and (c) any corresponding foreign Patent Rights to the foregoing.

1.54 "**Redosing Indications**" means the following indications: [***].

1.55 "**Redosing Technology**" means the proprietary [***].

c. Section 3.1 addressing the License is revised to add the following subsections 3.1.3 through 3.1.6 to the Agreement:

3.1.3 an exclusive, world-wide, royalty-free right and license (with the right to sublicense through multiple tiers subject to the provisions of Section 3.4), under the Licensor Redosing Patent Rights to make, have made, use, sell, offer for sale, and import Licensed Products for the Indications and Redosing Indications in the Field of Use during the Term;

3.1.4 a non-exclusive, world-wide, royalty-free right and license (with the right to sublicense through multiple tiers subject to the provisions of Section 3.4), (a) under the Licensed Redosing Know-How, to make, have made, use sell, offer for sale and import Licensed Products for all indications, including the Indications and Redosing Indications, in the Field of Use during the Term and (b) subject to Section 3.7, under the Licensor Redosing Patent Rights to make, have made, use, sell, offer for sale, and import Licensed Products for any indication in the Field of Use during the Term;

3.1.5 an exclusive, world-wide, royalty-free right and license (with the right to sublicense through multiple tiers subject to the provisions of Section 3.4), under the Licensor Capsid Patent Rights to make, have made, use, sell, offer for sale, and import Licensed Products for the Indications in the Field of Use during the Term; and

3.1.6 a non-exclusive, world-wide, royalty-free right and license (with the right to sublicense through multiple tiers subject to the provisions of Section 3.4), (a) under the Licensed Capsid Know-How to make, have made, use sell, offer for sale and import Licensed Products for all indications in the Field of Use during the Term, and (b) subject to Section 3.8, under the Licensor Capsid Patent Rights to make, have made, use, sell, offer for sale, and import Licensed Products for any indication in the Field of Use during the Term.

d. The remaining provisions of Article 3 shall apply to all license grants set forth in Section 3.1 for Licensor Capsid Patent Rights, Licensor Redosing Patent Rights, Licensed Capsid Know-How and Licensed Redosing Know-How as such provisions are set forth in such sections for Licensor Patent Rights and Licensed Know-How, including the provisions of Retained Rights (Section 3.2), Government Rights (Section 3.3), Grant of Sublicense by Licensee (Section 3.4), Delivery of Know-How (Section 3.5) and No Implied License (Section 3.6).

e. The following Section 3.7 is added to the Agreement:

3.7 **Right of First Refusal**. Licensor grants to Licensee a right of first refusal ("**ROFR**") to obtain an exclusive license under the Licensor Redosing Patent Rights for a Licensed Product in the Field of Use for any indication other than the Indications or Redosing Indications. Licensor shall provide written notice to Licensee prior to any negotiation with a Third Party for a non-exclusive license to any indication other than the Indications or Redosing Indications under the Licensor Redosing Patent Rights. Licensee may exercise the ROFR at any time during the Term by providing written notice ("**ROFR Notice**") to Licensor, or within [***] of Licensee's receipt of a written notice from Licensor that a Third Party is interested in obtaining a non-exclusive license under the Licensor Redosing Patent Rights for an indication in the Field of Use ("**Third Party Notice**"). Upon Licensee's exercise of the ROFR, the Parties shall negotiate in good faith terms of an exclusive license (or amendment to this Agreement) in such other indication. In the event Licensor and Licensee are unable to enter into an agreement after [***] from the ROFR Notice ("**ROFR Negotiation Period**") or Licensee does not provide a ROFR Notice within [***] of receipt of a Third Party Notice, then Licensor will have the right to enter into an agreement with a Third Party to grant non-exclusive rights with respect to the Licensor Redosing Patent Rights to a Third Party in such indication that was the subject of the ROFR Notice. For clarity, if Licensee and Licensor do not enter into an agreement for an exclusive license grant for the indication that is subject of the ROFR Notice, the non-exclusive license grant to Licensee set forth in Section 3.1.4(b) shall remain intact and Licensor may only grant a non-exclusive license to such Third Party for such indication.

f. The following Section 3.8 is added to the Agreement:

3.8 **Licensor Capsid Patent Rights**. At any time during the Term, Licensor may provide written notice to Licensee that a Third Party is interested in obtaining an exclusive license grant under the Licensor Capsid Patent Rights for an indication that is subject to the non-exclusive license grant set forth in Section 3.1.6(b). Upon receipt of written notice, Licensee may, in its sole discretion, provide to Licensor written notice to exclude such indication from the non-exclusive license grant set forth in Section 3.1.6(b). Upon receipt of such written notice, the Parties shall negotiate in good faith an amendment to the Agreement. For clarity, Licensee is not obligated to exclude such indication or enter into an amendment to exclude such indication.

g. The provisions of Article 6, Sections 8.2, 8.3, 10.1, and 11.4 shall apply to Licensor Capsid Patent Rights, Licensed Capsid Know-How, Licensor Redosing Patent Rights and Licensed Redosing Know-How as such provisions are set forth in such Article and Sections for Licensor Patent Rights and Licensor Know-How.

2. This Amendment along with the Agreement contains the entire understanding between the Parties and supersedes any and all prior agreements, understandings, and arrangements whether written or oral between the Parties with respect to the matters contained in the Agreement and this Amendment. No amendments, changes, modifications or alterations of the terms and conditions of this Amendment shall be binding upon any Party, unless provided in writing and signed by an authorized representative of each Party.

3. All terms and conditions of the Agreement not changed by this Amendment shall remain in full force and effect.

4. Signatures on this Amendment may be communicated by e-mail transmission and shall be binding upon the Parties upon receipt by transmitting the same by e-mail, which signatures shall be deemed originals. If executed in counterparts, the Amendment shall be effective as if simultaneously executed.

[*Signature Page Follows*]

IN WITNESS WHEREOF, Licensor and Licensee have entered into this Amendment effective as of the Amendment Effective Date.

BOARD OF REGENTS OF THE
UNIVERSITY OF TEXAS SYSTEM

By /s/ Arnim Dontes
Arnim Dontes
Executive Vice President for Business Affairs
UT Southwestern Medical Center

Date 4/3/2020

TAYSHA GENE THERAPIES, INC.

By /s/ RA Session II
Name: RA Session II
Title: Founder

Date 4/2/2020

Approved as to Content:

By /s/ Claire Aldridge
Claire Aldridge, Ph.D.
Associate Vice President,
Commercialization and Business Development
UT Southwestern Medical Center

Date 4/3/2020

Exhibit 10.3

Certain information has bee excluded from this agreement (indicated by "[***]") because Taysha Gene Therapies, Inc. has determined such information (i) is not material and (ii) would be competitively harmful if publicly disclosed.

LICENSE AGREEMENT

This License Agreement ("Agreement") is effective on this 21st day of February 2020 (the "Effective Date").

Between:

QUEEN'S UNIVERSITY AT KINGSTON

(hereinafter referred to as "Licensor")

and

Taysha Gene Therapies Inc.
(hereinafter referred to as "Licensee")

BACKGROUND:

Whereas the Licensed Technology (as defined below) was made in the course of research at the Licensor (or an affiliated research institution or hospital) by the Principal Investigator(s) (as defined below).

Whereas the Principal Investigators have assigned all right, title and interest in the Licensed Technology to the Licensor.

Whereas Licensor has rights under the Licensed Patents and Licensed Technology (as defined below) to make, have made, use, offer for sale, sell and import Licensed Product and otherwise exploit the Licensed Patents and Licensed Technology and to license to others under the Licensed Patents and Licensed Technology to make, have made, use, offer for sale, sell and import Licensed Product and otherwise exploit the Licensed Patents and Licensed Technology.

Subject to the terms and conditions set out in this Agreement, Licensee desires to obtain from Licensor and Licensor wishes to grant to Licensee an exclusive license under the Licensed Patents and Licensed Technology to make, have made, use, offer for sale, sell and import the Licensed Product and otherwise exploit the Licensed Patents and Licensed Technology pursuant to the terms and conditions of this Agreement.

NOW THEREFORE, in consideration of the foregoing premises, the mutual covenants and obligations hereinafter contained, and other good and valuable consideration, Licensor and Licensee agree as follows.

Article 1 – Definitions

For the purposes of this Agreement, the following capitalized terms, words, and phrases, when used in either the singular or plural, shall have the following meanings:

1.1 "**Affiliate**" means, with respect to a party to this Agreement, any Entity which controls, is controlled by, or is under common control with such party, in each case, for so long as such control exists, where the term "control" means direct or indirect possession of (a) at least fifty percent (50%) of the voting securities or comparable equity interest by or in such Entity or (b) the power to direct affirmatively the management and policies of such Entity.

1.2 "**Calendar Quarter**" means a period of three (3) months in the Gregorian Calendar ending on the last day of March, June, September, or December.

1.3 "**Calendar Year**" means the period of four Calendar Quarters ending on the last day of December.

1.4 **"Clinical Trial"** means a Phase I Study, a Phase I/II Study, a Phase II Study, or a Phase III Study, or such other clinical study in human subjects that is permitted by the FDA or other applicable regulatory authority and is designed to generate data in support of or maintenance of an application for regulatory approval.

1.5 "**Commercial Sale**" means the sale or other transfer of a Licensed Product following Marketing Approval by the appropriate and applicable governmental agency for the country in which the sale is to be made. The transfer of such Licensed Product to a third party for use in research and development (including in Clinical Trials for regulatory approval), as marketing samples to develop or promote such Licensed Product, or for compassionate use or other donations shall not constitute a Commercial Sale. A transfer between any selling party, where the intent is to further sell, transfer or provide services to others, shall not be considered a Commercial Sale of a Licensed Product.

1.6 "**Confidential Information**" means any confidential or proprietary information of a party disclosed under this Agreement by such party (the "Disclosing Party") to the other party (the "Receiving Party"), whether or not designated as being confidential or proprietary, for which reasonable precautions have been taken to maintain the secrecy of such information, including but not limited to information relating to any scientific or engineering information or research project, work in process, future developments, names of suppliers and customers, marketing and business plans relating to either party or other confidential or proprietary information that a reasonable person would understand to be confidential or proprietary information of such Disclosing Party, whether in oral, written, graphic or electronic form. Confidential Information of the Disclosing Party shall not include information that is, as demonstrated by written documentation: (i) known to the other party prior to disclosure by the Disclosing Party without a prior duty of confidentiality to the Receiving Party; (ii) disclosed in published literature; (iii) generally known or available to industry; (iv) obtained by the Receiving

Party from a third party who is not in breach of any confidentiality obligations to the Disclosing Party; or (v) independently developed by the Receiving Party without any reference to or use of the Confidential Information of the Disclosing Party.

1.7 "**Covered by the Licensed Patents**" means that, in respect of a Licensed Product, the manufacture, use, or sale of such Licensed Product would infringe, but for the License granted hereunder, a Valid Claim in the Licensed Patents in the country in which such product is manufactured, used or sold.

1.8 "**Entity**" means a corporation, an association, a joint venture, a partnership, a trust, a business, an individual, a government or political subdivision thereof, including an agency, or any other organization which can exercise independent legal standing.

1.9 "**Fair Market Value**" means the [***].

1.10 "**Field of Use**" means all fields of use.

1.11 "**Foundation**" means New Hope Research Foundation, Inc.

1.12 **"Gross Revenues"** means all amounts [***] by Licensee, any of its Affiliates or Sublicensees arising from a Commercial Sale.

1.13 **"Improvements"** means any improvement, idea, design, concept, technique, discovery or invention encumbered by the specification of the Licensed Patents or the Licensed Technology, including for the treatment of Tay-Sachs disease or Sandhoff's Disease, whether or not patentable, copyrightable, or otherwise protectable as intellectual property, developed by the Principal Investigator during the Principal Investigator's employment by Queen's University at any time during the term of this Agreement.

1.14 "**Know-How**" means trade secrets, Confidential Information of Licensor, and other useful, technical information, including without limitation knowledge, know-how, procedures, devices, methods, formulas, software, designs, techniques, processes, and inventions not known to the public, related to the Licensed Patents as described in Schedule B. "Know-How" does not include the Licensed Patents claiming any of the foregoing.

1.15 "**License**" means the license rights granted in Article 2 of this Agreement.

1.16 "**Licensed Patents**" means the patents or patent applications identified in Schedule A, all patents and patent applications that claim priority (directly or indirectly, in whole or in part) thereto, any and all Canadian and foreign applications or patents corresponding thereto, continuations, continuations-in-part, divisions, patents of addition, reissues, re-examinations, supplementary protection certificates, substitutions, renewals, term restorations, revalidations, or extensions of the foregoing.

1.17 "**Licensed Product**" means any product for use in the Field of Use whose development, making, having made, use, sale, offer for sale, import, export, and distribution would, in the absence of the License from Licensor, infringe one or more Valid Claims of the Licensed Patents and/or uses the Licensed Technology.

1.18 "**Licensed Technology**" means all of the technology, information and intellectual property related to the Licensed Patents that is owned or controlled by Licensor, including without limitation all Know-How and Improvements, as described in Schedule B.

1.19 **"Marketing Approval"** means all approvals, licenses, registrations or authorizations of the applicable governmental or regulatory authority(ies) in a jurisdiction necessary for the manufacture, use, storage, import, marketing and sale of a Licensed Product in such jurisdiction. For jurisdictions where governmental or other similar approval of pricing and/or reimbursement is reasonably necessary for marketing in such jurisdiction, Marketing Approval shall not be deemed to occur until such pricing or reimbursement approval is obtained.

1.20 "**Net Sales**" means [***].

1.21 "**Phase I Study**" means a clinical study of a drug candidate in human patients with the primary objective of characterizing its safety, tolerability, and pharmacokinetics and identifying a recommended dose and regimen for future studies as described in 21 C.F.R. §312.21(a), or a comparable clinical study prescribed by the relevant regulatory authority in a country other than the United States. The drug candidate can be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.22 "**Phase I/II Study**" means a clinical study of a drug candidate in diseased human patients that satisfies the requirements of a Phase 1 Study and a Phase 2 Study.

1.23 "**Phase II Study**" means a clinical study of a drug candidate in human patients with the primary objective of characterizing its activity in a specific disease state as well as generating more detailed safety, tolerability, and pharmacokinetics information as described in 21 C.F.R. §312.21(b), or a comparable clinical study prescribed by the relevant regulatory authority in a country other than the United States including a human clinical study that is also designed to satisfy the requirements of 21 C.F.R. §312.21(a) or corresponding foreign regulations and is subsequently optimized or expanded to satisfy the requirements of 21 C.F.R. §312.21(b) (or corresponding foreign regulations) or otherwise to enable a Phase III Clinical Study (e.g., a Phase I/II Study). The relevant drug candidate may be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.24 "**Phase III Study**" means a clinical study of a drug candidate in human patients that incorporates accepted endpoints for confirmation of statistical significance of efficacy and safety with the aim to obtain Marketing Approval in any country as described in 21 C.F.R. 312.21(c), or a comparable clinical study prescribed by the relevant regulatory authority in a country other than the United States. The relevant drug candidate may be administered to patients as a single agent or in combination with other investigational or marketed agents.

1.25 "**Principal Investigator**" means the inventor of the Licensed Technology whom are or were faculty members, cross-appointees, employees and/or students enrolled at Licensor when the Licensed Technology was created and the named inventor of the Licensed Patents.

1.26 "**Queen's**" means Queen's University at Kingston, Ontario.

1.27 **"Registration Study"** means clinical study of a drug candidate in human patients that satisfies both of the following ((i) and (ii)): (i) such clinical study establishes that such Licensed Product has an acceptable safety and efficacy profile for its use, and to determine warnings, precautions, or adverse reactions that are associated with such Licensed Product and is intended to support a Marketing Approval of such Licensed Product; and (ii) such clinical study is sufficient to support the filing of Marketing Approval for such Licensed Product in the United States pursuant to the requirements of the United States Food and Drug Administration or Marketing Approval granted by a regulatory authority in any country.

1.28 "**Regulatory Exclusivity**" means, with respect to any country, legal exclusive marketing rights granted by a regulatory authority in such country with respect to a Licensed Product, including but not limited to data exclusivity, pediatric exclusivity, chemical entity exclusivity and/or orphan drug designation/exclusivity.

1.29 "**ROFN Improvements**" means other than Improvements, any improvement, idea, design, concept, technique, discovery or invention, whether or not patentable, copyrightable, or otherwise protectable as intellectual property that has been assigned, or contractually committed to be assigned pursuant to an intellectual property agreement ("IPA") or similar agreement, to Licensor. For clarity, ROFN Improvements include next generation technology or improvements to the Licensed Patents or Licensed Technology that are not Improvements but conceived or reduced to practice during the term of the Agreement by Principal Investigator of the Licensed Patents or Licensed Technology for the treatment of Tay-Sachs disease or Sandhoff's Disease.

1.30 "**Royalty Term**" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the period beginning on the first Commercial Sale of a Licensed Product in a country and ending on the later of (a) the expiration of the last Valid Claim covering such Licensed Product in such country and (b) the expiration of Regulatory Exclusivity for such Licensed Product in such country.

1.31 "**Sublicensee**" means any Entity that is granted a sublicense under the Licensed Technology and/or the Licensed Patents by Licensee.

1.32 "**Sublicensing Revenue**" means [***].

1.33 "**Territory**" means worldwide.

1.34 "**Valid Claim**" means: (a) a claim in an issued and unexpired patent that has not been held invalid or unenforceable by the final, unappealable decision of a court, or similar legal entity, of competent jurisdiction, or (b) a claim in a pending patent application within the Licensed Patents that is actively being prosecuted and has not been abandoned, expired or rejected without the possibility of appeal or refiling. A patent application pending for more than [***] shall not be considered to have a Valid Claim for purposes of this Agreement unless and until a patent with respect to such patent application issues with such claim.

1.35 **Other Terms**. The definition of each of the following terms is set forth in the section of the Agreement indicated below:

Defined Term	Section
Agreement	Preamble
Applicable Laws	6.2
Effective Date	Preamble
Force Majeure Event	12.6
Insurance Coverage	8.5
Licensee	Preamble
Licensor	Preamble
Licensor Patent	7.1
Maximum Anti-Stacking Reduction	4.2
Overdue Payment	5.3
Priority Review	4.5
Priority Review Voucher	4.5
ROFN	2.6.1
Sublicense Agreement	2.5.1
Sublicensing Revenue	4.4

Article 2– Grant of Rights

2.1 *License* - Subject to the terms and conditions of this Agreement, and where Licensor may lawfully grant such license rights, Licensor hereby grants to Licensee the exclusive, perpetual, royalty-bearing right and license (with the right to sublicense through multiple tiers), in the Territory, under Licensed Technology and the Licensed Patents in the Field of Use, (i) to develop Licensed Products, (ii) to use and otherwise exploit the Licensed Technology and the Licensed Patents, and (iii) to make, have made, use, sell, offer for sale, import, export and distribute Licensed Products.

2.2 *Third Party Compulsory License*. In the event that Licensor is required pursuant to an order, ruling of finding of any court, regulatory entity or other governmental entity within a jurisdiction (other than Licensor) to grant a third party a license under the Licensed Technology to make, have made, use, sell, offer for sale, import, export and distribute a Licensed Product, then Licensor shall immediately notify Licensee in writing. The Parties shall adjust the amounts set forth in Article 4 commensurate with the impact of value decrease resulting from conversion of such license grant from an exclusive license to co-exclusive license or non-exclusive license pursuant to the terms of the license granted to such third party.

2.3 *Rights Retained by Licensor* - Notwithstanding the foregoing, Licensor retains the right to use and practice the Licensed Technology and the Licensed Patents within the Field of Use for non-commercial research including non-commercial preclinical research, and/or academic purposes only (including publishing scientific findings from research related to the Licensed Technology), which right it may transfer only to Queen's and Kingston Health Sciences Centre or any institutions Queen's or KHSC is performing collaborative research with for the same purposes, so long as Licensor, in mutual agreement with Licensee, reasonably determines that such transfer to collaborative researchers would not compromise the objectives of any Clinical Trials for the Licensed Products. Notwithstanding the foregoing, in all cases clinical research may not be conducted by Licensor, Queen's and Kingston Health Sciences Centre or any institutions Queen's or KHSC with a specific vector and transgene combination once such is identified for a Licensed Product, except as permitted in a clinical study agreement between the Parties or otherwise permitted by the Licensee in writing.

2.4 *No right in Licensed Patents and Licensed Technology* - Licensee agrees and acknowledges that it acquires no rights in the Licensed Patents or the Licensed Technology except the License expressly granted under this Agreement.

2.5 *Rights to Sublicense* -The License granted under this Agreement specifically includes the right of Licensee to grant sublicenses through multiple tiers. Licensee agrees that any sublicense it grants to any third party shall be granted under the following conditions:

2.5.1 Any sublicense grant of rights under the Licensed Technology and/or the Licensed Patents shall be restricted to the Field of Use and shall be under terms and conditions as set out in this Agreement (the "Sublicense Agreement"). Each Sublicense Agreement

shall specifically reference this Agreement and all rights retained by Licensor and contain the following:

(i) Sublicensee's acknowledgement of the Licensor's rights under Sections 2.3, 8.1, 8.3 and 8.4; and

(ii) A provision that upon expiry or termination of this Agreement, the rights granted to any Sublicensee shall terminate; provided, however, that any validly issued sublicense shall survive any expiration or termination of this Agreement provided that the Sublicensee agrees to be bound by the applicable terms of the Agreement with respect to activities of the Sublicensee under such Sublicense Agreement.

2.5.2 Within [***] after the execution of the Sublicense Agreement, Licensee shall forward to Licensor a fully executed copy of the Sublicense Agreement, which may be redacted to the extent the terms thereof are not necessary to determine compliance with this Agreement. Should the Sublicense Agreement be written in a language other than English, Licensee shall provide Licensor with an English translation of the Sublicense Agreement. Should the Licensee grant sublicenses that includes terms and conditions that are not consistent with this Agreement, the sublicense shall be null and void.

2.6 *Right of First Negotiation*.

2.6.1 Licensor hereby grants Licensee an exclusive right of first negotiation ("**Right of First Negotiation"** or "**ROFN**") during the term of this Agreement to license the right to under ROFN Improvements in the Field of Use, (i) to develop licensed products, (ii) to use and otherwise exploit the ROFN Improvements, and (iii) to make, have made, use, sell, offer for sale, import, export and distribute licensed products ("**ROFN Products**").

2.6.2 Licensor shall not license, sell, assign, transfer, covenant or otherwise grant any rights to a the ROFN Product in the Field of Use in any country without first offering Licensee in writing the right to include such ROFN Improvements in a ROFN Product or a Licensed Product under this Agreement, including the license grants of Section 2.1, as provided in this Section 2.6 ("**ROFN Offer Notice**"). Licensee may exercise its ROFN for a ROFN Product by providing written notice to Licensor of its exercise of the ROFN within [***] of receipt of the ROFN Offer Notice from Licensor. Prior to Licensor negotiating with or entertaining offers from a Third Party to license, sell, assign, transfer, covenant or otherwise grant any rights to, or otherwise seeking directly or indirectly to exploit, any ROFN Product, Licensor shall first notify Licensee with a ROFN Offer Notice and shall negotiate solely and in good faith with Licensee to grant Licensee a license pursuant of similar scope to the license grants of Section 2.1 for such ROFN Product for a period commencing with the date Licensee provides notice to Licensor of its exercise of the ROFN and expiring [***] thereafter.

2.6.3 If the Parties are unable to agree on substantive terms within the [***] period of negotiation (the "**ROFN Period**") despite good faith efforts, Licensor shall be free to enter into an agreement with a Third Party for the license of Licensor's rights in the ROFN Products by such Third Party, as applicable, provided that the financial terms of such agreement shall be more favorable to Licensor in the aggregate than those financial terms last offered by or to Licensee (or substantially similar terms).

Article 3– Term

3.1 *Term* - The term of this Agreement shall commence on the Effective Date and, unless otherwise terminated under this Agreement, shall extend until the expiration of the last Royalty Term (the "**Term**"). Following expiration of the Term (but not earlier termination), the License shall survive and become non-exclusive, perpetual, irrevocable, fully paid-up and royalty-free.

Article 4 – Licensing Consideration

4.1 *License Issue Fee* – Licensee shall pay Licensor a one-time, non-refundable license fee of Three Million ($3,000,000 USD) within [***]. The license issue fee shall be non-refundable and may not be credited towards the payment of other consideration that Licensee is obligated to pay Licensor under this Agreement.

4.2 *Annual Earned Royalties* – In partial consideration for the License granted in this Agreement, Licensee shall pay to Licensor, on a Licensed Product-by-Licensed Product and country-by-country basis, during the Royalty Term, an annual earned royalty of [***] of Net Sales of Licensed Products.

For clarity, the foregoing royalties shall only be payable during the applicable Royalty Term. If the Licensee, its Affiliate or a Sublicensee becomes obligated to pay additional amounts to third parties with respect to a Licensed Product, the Licensee may deduct [***] of the amount owing to such third parties from the royalties owing to the Licensor with respect to such Licensed Product; provided that under no circumstance shall the royalties due to the Licensor be less than [***] of the royalties otherwise owed to the Licensor without such deduction.

4.3 *Milestone Payments* - In partial consideration for the license granted herein, Licensee shall pay to Licensor the following amounts within [***] of achievement of the following development milestones. For clarity, all milestones shall be payable only once regardless of whether milestones are achieved for multiple Licensed Products.

Date or Event	Amount
[***]	[***]

4.4 *Sublicensing Revenue* - In addition to all other royalties, fees and payments payable hereunder, Licensee will pay to Licensor a percentage of the Sublicensing Revenue as follows: [***]. Licensee shall make such payment to Licensor within [***] after Licensee's receipt of such payment from such Sublicensee.

4.5 *Revenues From Sale of FDA Priority Review Voucher* – In addition to all other royalties, fees and payments payable hereunder, Licensee will pay to Licensor [***], capped at [***] (within [***] following any such sale), of all amounts received by Licensee or Sublicensee, where such consideration is exchanged for the sale of a granted FDA issued Priority Review Voucher or successor program. "Priority Review Voucher" means the Priority Review (as defined below) voucher issued by the United States Secretary of Health and Human Services to the Licensee in connection with Marketing Approval of a Licensed Product as evidenced by publication in the Federal Register (or successor publication) that entitles the holder of such voucher to Priority Review of a single human drug application submitted to the FDA. "Priority Review" means a priority review of and action upon a human drug application by the FDA not later than six (6) months after the filing of such application to the FDA, as defined in and pursuant to the Federal Food, Drug and Cosmetic Act and regulations promulgated thereunder.

4.6 *Right of First Negotiation to Purchase Royalty Stream* – Licensor shall grant Licensee a [***] Right of First Negotiation to match any third-party offer to purchase the royalty stream from any Licensed Product.

4.7 *Payment of Plasmid*- Licensee shall reimburse Licensor $221,300 USD to cover the cost of plasmid production by Aldevron within [***] following [***].

4.8 *Taxes* - All amounts set out above in this Article 4 are exclusive of taxes such as Goods and Services Tax, Provincial Sales Tax, Harmonized Sales Tax or any other tax eligible thereon, which shall be payable by Licensee to Licensor. Licensee will withhold only the minimum amount of taxes specified by Licensee's country of taxation and any tax treaty between Canada and Licensee's country of taxation. If applicable, Licensee will cooperate with Licensor in completing all prescribed forms to claim a reduced amount of taxes withheld under the tax treaty, if any, between Canada and Licensee's country of taxation. Licensee will immediately notify Licensor of any change in the residence status or upon registration for the purposes of the Canadian excise Tax Act.

4.9 *Offset.* In the event that Foundation, its directors, trustees, officers, employees, successors or assigns threatens or brings a claim, action or dispute against Licensee relating to the Licensed Patents, Licensed Technology or Licensed Product, Licensee may offset all amounts expended by Licensee and any sublicensee in responding to, defending and settling such claim, action or dispute (including, but not limited to, attorneys' fees and settlement amounts) against amounts due and payable to Licensor from Licensee and its sublicensees under this Article 4.

Article 5 – Payments and Reports

5.1 *Payments* - Unless otherwise specified in this Agreement, all undisputed amounts due to Licensor shall be paid within [***] following the end of the Calendar Quarter in which such payment accrues or Licensee otherwise incurs the obligation to pay such undisputed amounts. All such payments shall be remitted to Licensor's address given in the notification provision of this Agreement or to such other address as Licensor shall direct. All payments to Licensor shall be remitted using the following payment information:

[***]

5.2 *Currency* - All amounts due to Licensor under this Agreement are in USD and are to be paid in USD. With respect to Net Sales received by Licensee in currency other than USD, calculations required to ascertain amounts due Licensor and any currency conversions necessary to make payment of amounts due Licensor shall be made using an average of the official closing exchange rate quoted by the Wall Street Journal on the last day of each month in the applicable Calendar Quarter. If such currency conversion is not reasonably possible, Licensee shall immediately inform Licensor and Licensee, shall deposit any amounts owed to Licensor, in the currency of the Net Sales received, in the bank or trust association of the country of such currency designated by Licensor.

5.3 *Late Fees* - If any undisputed payment is made more than [***] late after the date such payment is due (an "Overdue Payment"), Licensee shall pay to Licensor interest on an Overdue Payment at a rate of [***]. Such interest will accrue on an Overdue Payment from the [***] after the payment was due. Accrued interest will be due and payable on the first day of each month after interest begins to accrue, until full payment of all Overdue Payments and accrued interest.

5.4 *Reports* - During the term of this Agreement and for [***] thereafter, Licensee shall keep, at its own expense, accurate books of account using accepted accounting procedures, detailing the data necessary to calculate any payments due Licensor from Licensee under this Agreement. Each payment made to Licensor shall be accompanied by a written report summarizing the data used to calculate the amounts

paid. Each report pertaining to royalty payments for the applicable accounting period shall specifically include the following, as applicable:

(i) Gross Revenue amounts;

(ii) Net Sales; and

(iii) Amount of royalties due, broken down by category and country; and

All reports under this Agreement will be treated as Licensee's Confidential Information. If during any reporting period, no Net Sales are invoiced, billed, or received and no payment is due Licensor, Licensee shall nevertheless timely submit a written report to Licensor stating that no Net Sales were invoiced, billed, or received and no funds are due Licensor.

5.5 *Examination of Records* - Upon at least [***] written notice, Licensor, at its own cost and expense, through an independent auditor reasonably acceptable to Licensee, may inspect and examine such records and books of account of Licensee as are necessary, and where Licensee grants sublicenses or any other grant of rights in the Licensed Technology, it shall obtain a right for Licensor's independent auditor reasonably acceptable to such Sublicensee, to examine such records and books of account of Sublicensees on Licensor's behalf, as are necessary to verify the accuracy of Licensee's royalty payments. Such right may be exercised [***] during any twelve-month period. Such examination may be performed at any time within [***] after the end of the reporting period to which the books of account pertain, and shall be performed during normal business hours at the major place of business or at such other site as may be agreed upon by Licensor and Licensee or the party who is subject to the inspection. Licensor may make abstracts or copies of such books of account solely for its use in performing the examination and such copies shall be considered the Confidential Information of Licensee.

5.6 *Result of Examination* - If any examination of Licensee's records shows that Licensee has paid more than required under this Agreement, any excess amounts shall, at Licensee's option, be promptly refunded or credited against future royalties with interest from the date of overpayment [***]. If any examination of Licensee's records shows that Licensee has paid less than required under this Agreement, Licensee shall promptly pay the additional amount due together with interest as required under this Agreement for late payments. If the amount of underpayment exceeds [***] of the amount which should have been paid, Licensee shall also pay all reasonable, out-of-pockets costs incurred by Licensor with respect to such examination.

5.7 *Reimbursement of plasmid materials* – The proposed Sponsored Research Agreement shall contain a provision for the reimbursement of $221,300 USD with respect to the cost of manufacturing the plasmid material. In the event a Sponsored Research Agreement is not executed within [***] of the Effective Date, Licensee agrees to reimburse Licensor directly in the amount of $221,300 USD.

Article 6 – Performance

6.1 *Licensee Efforts* - During the term of this Agreement, Licensee shall use commercially reasonable efforts to exploit the Licensed Technology in the Field of Use in the Territory in countries where it is commercially reasonable to develop Licensed Products hereunder. As a minimum, Licensee agrees to achieve the following:

[***].

6.2 Notwithstanding the foregoing, any delays arising from unexpected scientific difficulties or regulatory delays shall be reasonably excused, provided that the Licensee provides timely notice of such situation and promptly uses commercially reasonable efforts to mitigate such situation.

If Licensee is unable to perform any of the above provisions in Article 6.1, then Licensor, after providing Licensee with written notice of Licensee's failure to perform such obligations and Licensee failing within [***] of receiving such notice to cure such failure or otherwise reach agreement with Licensor on a procedure to remedy the failure, has the right and option to either:

(i) terminate this Agreement; or

(ii) reduce Licensee's Field of Use; or

(iii) convert this exclusive license to a non-exclusive license,

and this right, if exercised by Licensor, supersedes the rights granted in Article 2.

[***], Licensee shall provide an annual report to Licensor summarizing progress achieved on commercializing the Licensed Technology.

Licensee shall comply with all applicable international, federal, national, state, provincial, and local laws and regulations, including, for certainty, any applicable export control, embargo, anti-corruption, and international treaties regulating the sale or use of Licensed Products or containing the Licensed Technology ("Applicable Laws"). Without limiting the generality of the above, Licensee acknowledges that the Licensed Products and any related Licensed Technology may be subject to export controls imposed by Canada, the United States and other Applicable Laws, and may require disclosure to and permits or licenses to be issued by the government of Canada, the government of the United States and/or other applicable government agencies and departments ("*Government*"). Licensee shall obtain, maintain and comply with all required Government licenses and permits in connection with its use of the Licensed Technology and any manufacturing, marketing, sale, transportation and delivery of Licensed Products. Licensee acknowledges that its covenant to comply with Applicable Laws and obtain, maintain and comply with all requisite Government permits is fundamental condition of the entering into of this License by the Licensor.

Licensee shall not take any actions to invalidate the Licensed Patents or have them declared unenforceable.

Article 7 Intellectual Property

7.1 *Licensed Patents* – Unless otherwise provided herein, the Licensed Patents will be held in the name of Licensor and obtained with counsel or patent agent(s) authorized to represent Licensor and mutually agreed upon by the parties. Licensor shall provide Licensee with (a) copies of all relevant documentation related to the prosecution of the Licensed Patents so that Licensee may be informed and appraised of and meaningfully consulted as to their filing, continuing prosecution and maintenance and (b) reasonable opportunity to advise Licensor on the filing, continuing prosecution and maintenance, and to comment on all relevant matters relating thereto, including review of filings and draft responses prior to filing with the applicable patent office. In the event of a disagreement between Licensor and Licensee regarding the filing, prosecution or maintenance of the Licensed Patent Rights, Licensee shall have final decision making authority with respect to such disputed issue. In the event that Licensee does not agree that any given patent application or patent should be made, prosecuted or maintained (hereinafter referred to as a "Licensor Patent"), Licensor shall have the right unilaterally to make, prosecute and maintain such Licensor Patent, and Licensee shall not have any rights to such Licensor Patent. In the event that Licensor elects to abandon any patent or patent application within the Licensed Patents, it shall notify Licensee at least [***] in advance of the associated statutory deadline with the applicable patent office. In the event Licensee disagrees with such abandonment, Licensor shall continue prosecution or maintenance, as the case may be, of such patent or patent application.

7.1.1 Licensor shall use its reasonable efforts to amend any patent application to include claims reasonably requested by Licensee and required to protect the Licensed Products.

7.1.2 Licensee agrees to pay all costs and legal fees incurred by Licensor on behalf of Licensee for the filing, prosecution, maintenance and taxes for the Licensed Patents (other than Licensor Patents). Such costs will include the reasonable and documented -of Licensor's internal patent agents, out-of-pocket costs for the filing, prosecution and maintenance of the Licensed Patents, including in the event of an Inter Partes Review by the US patent office (or similar process in a country outside the US) reasonable and documented attorneys' fees, expenses, official and filing fees. All reasonable and documented expenses incurred after the Effective Date for such prosecution, maintenance and taxes shall be reimbursed to Licensor by Licensee within [***] of invoicing.

7.2 *Patent Markings* - Licensee shall comply with all applicable United States and foreign statutes relating to the marking of Licensed Product(s) and/or related packaging with patent pending, patent number(s), copyrights, or other intellectual property notices and legends required to maintain the intellectual property rights licensed under this Agreement.

Article 8 – Warranties

8.1 *Licensor Warranties* - Licensor represents and warrants as of the Effective Date:

8.1.1 to the best of its knowledge after due inquiry, applying a standard of care consistent with the practices of other Canadian public research and educational institutions, and not the standard of care exercised by or in a pharmaceutical industry research facility:

(i) Licensor has identified all inventors of the Licensed Patents and Licensed Technology and the entire right and title to the Licensed Technology and the Licensed Patents have been properly assigned by the inventors and developers of such technology and patent applications to Licensor;

(ii) Licensor is the owner or agent of the entire right, title and interest in and to the Licensed Patents; and

(iii) Licensor has the right to grant the License hereunder, subject to the provisions of Article 2.

8.1.2 Licensor has not granted any rights under the Licensed Patents and Licensed Technology prior to the Effective Date that would be inconsistent with the rights granted to Licensee herein, subject to the provisions of Article 2;

8.1.3 Licensor has not received any written notice that any of the Licensed Patents are invalid or unenforceable; and

8.1.4 Licensor has not received any written notice of any actual or threatened claims from any Entity other than the Foundation that the use of or practice of the Licensed Patents or Licensed Technology infringes or misappropriates (or has infringed or misappropriated) the intellectual property of any third party.

8.2 *Licensee Warranties* - Licensee represents and warrants:

(i) Licensee has full right and authority to enter into and fully perform its rights and obligations under this Agreement; and

(ii) entering into this Agreement will not conflict with any other obligation of Licensee.

8.3 *Limitation on Licensor's Warranties* – Other than the representations and warranties provided in this Article 8, LICENSOR MAKES NO REPRESENTATIONS OR

WARRANTIES, EXPRESS, IMPLIED OR STATUTORY, WITH RESPECT TO THE LICENSED TECHNOLOGY. ALL LICENSOR DELIVERABLES ARE MADE AVAILABLE TO LICENSEE STRICTLY ON AN "AS IS" BASIS. LICENSOR DOES NOT WARRANT THAT THE LICENSED TECHNOLOGY IS ERROR FREE OR THAT IT WILL MEET LICENSEE'S REQUIREMENTS. ALL IMPLIED WARRANTIES AND CONDITIONS OF MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE ARE EXPRESSLY DISCLAIMED AND EXCLUDED. THE ENTIRE RISK AS TO THE RESULTS AND PERFORMANCE OF THE LICENSED TECHNOLOGY, DELIVERABLES, AND ANY PRODUCTS, SERVICES OR METHODS BASED ON THE LICENSED TECHNOLOGY IS ASSUMED BY LICENSEE. OTHER THAN AS EXPRESSLY SET OUT IN THIS ARTICLE 8, LICENSOR DOES NOT MAKE ANY WARRANTIES OR REPRESENTATIONS, EXPRESS OR IMPLIED, CONCERNING THE PATENTABILITY OF ANY LICENSED TECHNOLOGY, THE VALIDITY OF ANY LICENSED PATENT(S), THE SCOPE OF ANY LICENSED PATENT(S) CLAIMS, OR WHETHER OR NOT THE EXERCISE OF THE RIGHTS LICENSED UNDER THIS AGREEMENT WILL OR WILL NOT RESULT IN INFRINGEMENT OF ANY INTELLECTUAL PROPERTY RIGHTS OF THIRD PARTIES.

8.4 *Licensee's Indemnification*

8.4.1 Licensee hereby indemnifies and undertakes to defend Licensor, its shareholders, directors, trustees, officers, employees, students and agents and hold them harmless against all third party claims, suits, proceedings, demands, actions of any nature or kind whatsoever including any actions for infringement of a third party's intellectual property rights, as referred to in Article 9.1 by a third party (other than the Foundation, its directors, trustees, officers, employees, successors or assigns), damages, judgments, costs, expenses and fees (including but without limitation, reasonable legal expenses) or liability of any kind brought by a third party or otherwise related to third party claims arising out of or in any way associated with the development, sub-licensing, use, manufacture, marketing and sale of the Licensed Products or with the use of Licensed Technology, except, in each case, to the extent such third party claim or liability arises out of or is in any way associated with the gross negligence or willful misconduct of Licensor or breach of this Agreement by Licensor. The obligations of Licensee under this Section 8.4 shall survive the termination of this Agreement.

8.4.2 As a condition to a Licensor right to receive indemnification under Section 8.4.1, Licensor shall: (i) promptly notify Licensee as soon as it becomes aware of a claim or suit for which indemnification may be sought; (ii) reasonably cooperate, and cause the individual Licensor indemnitees to reasonably cooperate, with Licensee in the defense, settlement or compromise of such claim or suit; and (iii) permit Licensee to control the defense, settlement or compromise of such claim or suit, including the right to select defense counsel. In no event, however, may

Licensee compromise or settle any claim or suit in a manner which (A) admits fault or negligence on the part of Licensor or any other Licensor indemnitee; (B) commits Licensor or any other Licensor indemnitee to take, or forbear to take, any action, without the prior written consent of Licensor, or (C) grant any rights under the Licensed Patents except for Sublicenses as permitted herein. Licensor shall reasonably cooperate with Licensee and its counsel in the course of the defense of any such suit, claim or demand, such cooperation to include without limitation using reasonable efforts to provide or make available documents, information and witnesses.

8.5 *Insurance* - Licensee shall obtain and maintain, at its sole cost and expense, general liability insurance, product and product liability insurance in an amount reasonably sufficient to protect against activities related to this Agreement, including coverage for, without limitation, its indemnification obligations under Article 8.4 (the "Insurance Coverage"). Prior to any distribution or Commercial Sale of a Licensed Product Licensee shall obtain and maintain Insurance Cover at a minimum face amount of $[***] per occasion and an aggregate of $[***]. Upon request, Licensee shall furnish to Licensor a certificate evidencing compliance with these insurance obligations and coverages.

8.6 *Limitation of Liability* -

8.6.1 Subject to Sections 8.6.2 and 8.6.3, the liability of either party in relation to this Agreement shall in all circumstances be limited to direct damages and neither party shall be liable for any special, consequential, indirect, incidental, exemplary or punitive damages, whether in contract, tort or otherwise resulting from any cause of action whatsoever, including negligence, gross negligence, negligent misrepresentation, loss of profit, strict liability, operation of law, and/or fundamental breach or other theory of law.

8.6.2 Section 8.6.1 shall not apply in respect of damages arising from a breach of Article 10 or willful misconduct.

8.6.3 Except for a breach of Article 10 or willful misconduct, in no event shall Licensor's aggregate liability for claims arising out of or in connection with this Agreement exceed an amount equal to the amounts paid under Article 4 of this Agreement.

Article 9 Infringement

9.1 *Licensed Technology Infringes Third-Party's Rights* - Should any third party threaten or make a claim that the manufacture, use, or sale of a Licensed Product or the use of the Licensed Technology by Licensee infringes or constitutes wrongful use of such third party's intellectual property rights, Licensee shall give Licensor prompt written notice detailing as many facts as possible concerning such claim. Licensor agrees, at its own

expense, to provide all reasonable and necessary documents, in the possession of Licensor, and reasonable access at Licensor's offices during business hours to personnel of Licensor with a knowledge of the dispute, to Licensee in any attempt by Licensee to resolve such claims.

9.2 *Third-Party Infringement* - Licensor and Licensee agree that, should either party become aware of any actual or potential infringement or wrongful or unauthorized use of the Licensed Technology or the Licensed Patents, that party becoming aware will give the other party prompt written notice detailing as many facts as possible concerning such infringement or potential infringement. During the Term, Licensee, at its own expense, shall have the right to prosecute infringement or wrongful or unauthorized use of the Licensed Technology and/or the Licensed Patents in the Field of Use in the Territory. Licensee shall have the right to join Licensor as a party plaintiff in any action brought by Licensee to enforce the Licensed Technology and/or Licensed Patents. Should Licensee join Licensor, Licensee shall pay all out-of-pocket expenses incurred by Licensor in its participation in the action. Licensee shall recover and retain any and all damages recovered from such an action. Licensor shall provide to Licensee all information reasonably required to assist with the prosecution of such action. The foregoing rights shall be subject to the continuing right of Licensor to intervene in an action commenced by Licensee at Licensor's own expense.

9.3 *Licensor Enforcement* - Should Licensee not commence an action against a third party that is infringing or wrongfully using the Licensed Technology and/or Licensed Patents within 180 days of receiving or giving notice of such infringement, then Licensor may commence an action in its own name against such third party. Licensor shall have the right to join Licensee as a party plaintiff in any action brought by Licensor to enforce the Licensed Technology and/or Licensed Patents intellectual property rights. Should Licensor join Licensee, Licensor shall pay all out-of-pocket expenses incurred by Licensee in its participation in the action. Licensee shall provide to Licensor all information reasonably required to assist with the prosecution of such action. Licensor shall recover and retain any and all damages recovered from such an action.

Article 10 - Confidential Information

10.1 *Confidential Information* - The parties may exchange Confidential Information to each other in connection with this Agreement. The Receiving Party shall safeguard the Confidential Information received from the Disclosing Party and shall not disclose it to anyone within the Receiving Party's organization without a "need to know" and without being bound to a written confidentiality agreement signed on terms no less stringent than set out herein, or, if to third parties, without appropriate written confidentiality agreements being signed on terms no less stringent than set out herein. Each party agrees to take reasonable precautions to protect the other party's Confidential Information and preserve and shall use at least the same degree of care

and precaution as is customarily used to protect its own Confidential Information and which in any event shall be a prudent and reasonable degree of care. Each party agrees not to use the other party's Confidential Information other than as expressly set forth herein.

10.2 Notwithstanding Section 10.1, a Party may disclose Confidential Information (a) to regulatory authorities as reasonably needed to develop and/or obtain or maintain regulatory approvals of Licensed Products, (b) to its Sublicensees as reasonably needed to research, develop and/or commercialize Licensed Products, under terms of confidentiality that are no less restrictive than those set forth in this Agreement, (c) to prospective sublicensees, strategic partners, merger partners or acquirers, existing and potential investors and in each case, their respective professional advisors, in connection with evaluation and/or negotiation of possible sublicense, corporate partnering, merger, asset purchase or other similar transactions, provided that any such disclosure shall be subject to a written confidentiality agreement with terms of non-disclosure no less restrictive than those set forth in this Agreement, or (d) as reasonably needed to conduct or defend any litigation relating to this Agreement, the Licensed Products or such party's rights hereunder. Furthermore, if the Receiving Party of Confidential Information becomes legally compelled to disclose any Confidential Information in order to comply with applicable law or with an order issued by a court or regulatory body with competent jurisdiction, the recipient shall (i) provide prompt written notice to the Disclosing Party so that the Disclosing Party may seek a protective order or other appropriate remedy or waive its rights under this Section; and (ii) disclose only that portion of Confidential Information that is legally required to furnish; provided that, in connection with such disclosure, the recipient shall use commercially reasonable efforts to obtain assurance that confidential treatment will be given with respect to such Confidential Information. If any party is required to file this Agreement with any governmental body, such party shall redact the terms of this Agreement to the extent possible in order to keep particularly sensitive provisions confidential.

Article 11 – Termination

11.1 *Licensor's Right to Terminate* - Licensor may, at its option, terminate this Agreement immediately and without notice if [***], or (b) Licensee files in any court or agency, pursuant to any statute or regulation of any state or country, a petition in bankruptcy or other Insolvency Event or for reorganization or for an arrangement or for the appointment of a receiver or trustee of Licensee, or if Licensee is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition shall not be dismissed within [***] after the filing thereof. For purposes of this Section 11.1, an "Insolvency Event" shall mean Licensee (a) commenced a voluntary proceeding under any insolvency law, or (b) had an involuntary proceeding commenced against it under any insolvency law which has continued undismissed or

unstayed for [***], or (c) had a receiver, trustee or similar official appointed for it or for any substantial part of its property, or (d) had an order for relief entered with respect to it by a court of competent jurisdiction under any insolvency law.

11.2 *Mutual Termination*. Either party may, at its option, terminate this Agreement if the other party: (a) becomes subject to, any proceeding under any Bankruptcy and Insolvency Act or any other statute of any state or country relating to insolvency or the protection of creditor's rights; or (b) is in material breach or default of any obligation under this Agreement and fails to cure such breach or default, or satisfy that such breach or default has been cured, or otherwise reach agreement with the non-breaching party on a procedure to remedy the breach or default within [***] after receiving notice from the non-breaching party to cure.

11.3 *Licensee's Right to Terminate* - Licensee may, at its option, terminate this Agreement on a Licensed Product-by-Licensed Product, country-by-country basis or in its entirety upon providing Licensor [***] written notice.

11.4 *Obligations on Termination* - Termination of this Agreement shall not release Licensor or Licensee from any obligation or liability to the other which shall have matured or accrued prior to termination. The following rights and obligations, in addition to others as expressly provided herein, shall survive termination:

(i) Licensee shall make all reports as required herein prior to termination and shall submit a termination report as reasonably requested by Licensor within [***] of such request;

(ii) Licensee shall pay all royalties or other payments due Licensor accrued for payment prior to termination, within [***] of the effective date of termination and shall promptly remit royalties or other payments due Licensor;

(iii) Licensee shall maintain all records required to be kept herein for the period before termination, and shall allow Licensor examination privileges as set forth in Article 5;

(iv) any licenses, releases, or agreements of non-assertion running in favour of end users of Licensed Products;

(v) all claims and causes of action one party may have against the other; and

(vi) all obligations to preserve and maintain the confidentiality of Confidential Information, except as required by court orders or by law or to satisfy government regulations.

Upon termination of this Agreement: (i) Licensee shall cease making, using, selling, marketing any Licensed Product (subject to the sell-off rights); and (ii) both parties shall destroy any Confidential Information disclosed by the other party and provide a certificate to such other party verifying such destruction. In addition, Licensee shall have the right, upon termination of this Agreement, to sell-off any inventory of Licensed Product for a period of [***], or as mutually agreed upon by the parties in writing.

Article 12 - General Provisions

12.1 *Assignment* - This Agreement shall be binding upon and enure to the benefit of the parties hereto and their respective successor and permitted assigns. Except as may be expressly permitted in this Agreement, neither party may assign this Agreement or any of its rights, duties or obligations to any person or entity without first obtaining the written consent of the other party, which consent shall not be unreasonably withheld; provided, however, that either party may assign this agreement without such consent (a) to an affiliate or (b) in connection with the sale of all or substantially all of its business or assets to which this agreement relates, whether by merger, consolidation, reorganization, stock sale, change of control, operation of law or otherwise.

12.2 *Intentionally Omitted.*

12.3 *Counterparts* - This agreement may be executed in any number of counterparts, each of which together shall be deemed an original, but all of which together shall constitute one and the same instrument. For purposes of executing this agreement, a facsimile (including a PDF delivered via email) copy of this agreement, including the signature pages, will be deemed an original.

12.4 *Dispute Resolution* - Licensor and Licensee shall provide the other party with prompt written notice of any controversy or dispute arising out of or in connection with this Agreement, its interpretation, performance or termination. Both parties do hereby agree to make best efforts to amicably resolve any such dispute within a reasonable time after receiving written notice of the existence of such dispute.

12.5 *Entire Agreement* - This Agreement (including without limitation the Background section and the schedules and exhibits attached hereto) and any documents incorporated by reference herein, constitute the entire agreement and understanding between Licensor and Licensee pertaining to the subject matter hereof and supersede all prior or collateral agreements, understandings, negotiations and discussions pertaining to such subject matter, whether oral or written or in electronic form. Except as specifically set out herein, there are no conditions, representations, warranties, undertakings, promises, inducements, or agreements whether direct, indirect, collateral, express or implied made by Licensor to Licensee. No modification, supplement or waiver of this Agreement shall be binding unless executed in writing by authorized officers of each party.

12.6 *Force Majeure* - Neither Licensor nor Licensee shall be in default of the terms of this Agreement because the party delays performance or fails to perform such terms; provided such delay or failure is not the result of the party's intentional or negligent acts or omissions, but the result of causes beyond the reasonable control of such party (any such cause is a "Force Majeure Event"). Causes reasonably beyond the control of Licensor and Licensee shall include, but not be limited to, revolutions; civil disobedience; fires; acts of God, war, or public enemies; blockades; embargoes; strikes; labour disputes; governmental, administrative or judicial orders, delays in transit or deliveries. In the event of a Force Majeure Event, each party shall be allowed a reasonable period of time to fulfil its obligations hereunder, however, where such Force Majeure Event continues more than 180 days, the party not seeking to rely on the Force Majeure Event to excuse its non-performance, may terminate this Agreement.

12.7 *Further Assurances* - The parties agree that each of them shall, upon reasonable request of the other, do or cause to be done all further lawful acts, deeds and assurances whatsoever required for the better performance of the terms and conditions of this Agreement.

12.8 *Headings* - The article, section and subsection titles and headings contained in this Agreement are for convenience and reference only. Such titles and headings do not form a part of this Agreement, shall not define or limit the scope of the articles, sections or subsections, and shall not affect the construction or interpretation of any of the articles, sections or subsections.

12.9 *Notices* - All notices, reports, payments, requests, consents, demands and other communications between Licensor and Licensee, pertaining to subjects related to this Agreement, shall be in writing and shall be deemed duly given and effective: when actually received by mail or personal delivery; or when mailed by prepaid registered or certified mail to the receiving party at the address set forth below, or to such other address as may be later designated by written notice from either party to the other party:

Licensor's notification address:

Queen's University – Office of Partnerships & Innovation
[***]

Licensee's notification address:

Taysha Gene Therapies, Inc.
[***]

With copy to:
[***]

12.10 *Publicity* - Neither party will use the name of the other party, or of Licensor, in any publicity without the prior written approval of the party being so named. Such approval from either party shall not be unreasonably withheld or delayed. Notwithstanding the foregoing, Licensee may use the name of Licensor in a non-misleading and factual manner solely in (a) executive summaries, business plans, offering memoranda and other similar documents used by Licensee for the purpose of raising financing for the operations of Licensee as related to Licensed Products, or entering into commercial contracts with third parties, but in such case only to the extent necessary to inform a reader that the Licensed Patents and Licensed Technology have been licensed by Licensee from Licensor, and to inform a reader of the identity and published credentials of inventors of intellectual property, and (b) any securities reports required to be filed with the Securities and Exchange Commission. Licensor may use the name of Licensee in a non-misleading and factual manner to provide aggregate technology transfer revenues in University public reports.

12.11 *Severability* - In the event that any provision or term of this Agreement shall be held by a court to be illegal or unenforceable, all of the other terms and provisions hereof shall remain in full force and effect, except that if the provision or term held to be illegal or unenforceable is also held to be a material part of this Agreement such that the party in whose favour the material term or provision was stipulated herein would not have entered into this Agreement without such term or provision, then the party in whose favour the material term or provision was stipulated shall have the option upon such a holding, to terminate this Agreement.

12.12 Waiver of Rights - In order to be effective, any waiver, by either party, of any right under this Agreement, must be in writing signed by an authorized representative of the party making the waiver. No such waiver or failure of Licensor or Licensee to enforce a right or strict performance under this Agreement shall be deemed to be a waiver or forbearance which would in any way prevent Licensor or Licensee from subsequently asserting or exercising any such rights, making a claim not specifically waived, or requiring strict performance of this Agreement. No such waiver or failure to enforce shall affect the validity of this Agreement or be a continuing waiver excusing compliance with any provision of this Agreement in the future.

- Signatures Follow -

IN WITNESS WHEREOF, Licensor and Licensee have caused this Agreement to be executed by their duly authorized representative**:**

Queen's:	/s/ James Banting	Feb. 21, 2020
	Name: James Banting Title: Assistant Vice-Principal (Partnerships and Innovation)	Date
Licensor:	/s/ RA Session II	2/25/2020
	Name: RA Session II Title: Founder	Date

Schedule A

List of Patents and Applications

[***]

Schedule B

List of Know-How

"**Know-How**" means trade secrets, Confidential Information, and other useful, technical information, including without limitation knowledge, know-how, procedures, devices, methods, formulas, software, designs, techniques, processes, and inventions not known to the public, related to the Licensed Patents. Know-How will include all public and non-public data and be available to Licensee sufficient detail to support regulatory filings. Know-How shall include, but is not limited to the following:

[***]

Exhibit 10.4

Certain information has been excluded from this agreement (indicated by "[***]") because Taysha Gene Therapies, Inc. has determined such information (i) is not material and (ii) would be competitively harmful if publicly disclosed.

LICENSE AGREEMENT

DATED AS OF AUGUST 14, 2020

BY AND BETWEEN

ABEONA THERAPEUTICS, INC.

AND

TAYSHA GENE THERAPIES, INC.

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LICENSE AGREEMENT

This License Agreement (this "**Agreement**") is dated as of August 14, 2020 (the "**Effective Date**") by and between Abeona Therapeutics Inc., a Delaware corporation having its principal place of business at 1330 Avenue of the Americas, Suite 33A, New York, NY 10019 ("**Licensor**"), and Taysha Gene Therapies, Inc., a Delaware corporation having a place of business at 2280 Inwood Road, Dallas, TX 75325 ("**Company**"). Licensor and Company may be referred to herein as a "**Party**" or, collectively, as "**Parties**".

RECITALS:

WHEREAS, Licensor is a fully integrated gene and cell therapy company engaged, *inter alia*, in the development of gene therapy technologies relating to CLN1 Disease;

WHEREAS, Company is engaged in the research, development, manufacturing and commercialization of gene therapy products and is interested in Developing, manufacturing and Commercializing Licensed Products; and

WHEREAS, Company desires to license from Licensor, and Licensor wishes to exclusively license to Company rights to Develop, manufacture and Commercialize Licensed Products in the Field in the Territory.

NOW, THEREFORE, in consideration of the various promises and undertakings set forth herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties, intending to be legally bound, hereby agree as follows:

ARTICLE 1
DEFINITIONS

Unless otherwise specifically provided herein, the following terms shall have the following meanings:

1.1 "**Affiliate**" means a Person who controls, is controlled by or is under common control with a Party, but only for so long as such control exists. For the purposes of this Section 1.1, the word "control" (including, with correlative meaning, the terms "controlled by" or "under the common control with") means the actual power, either directly or indirectly through one or more intermediaries, to direct the management and policies of such Person or entity, whether by the ownership of more than fifty percent (50%) of the voting stock of such entity, or by contract or otherwise.

1.2 "**BLA**" means a Biologics License Application submitted pursuant to the requirements of the FDA, as more fully defined in 21 C.F.R. § 601, and any equivalent application submitted in any country in the Territory, including all additions, deletions or supplements thereto, and as any and all such requirements may be amended, or supplanted, at any time.

1.3 "**Calendar Quarter**" means each three (3) month period commencing January 1, April 1, July 1 or October 1 of any year; provided, however, that (a) the first Calendar Quarter of the Term shall extend from the Effective Date to the end of the first full Calendar Quarter thereafter, and (b) the last Calendar Quarter of the Term shall end upon the expiration or termination of this Agreement.

1.4 "**Calendar Year**" means the period beginning on the 1st of January and ending on the 31st of December of the same year; provided, however, that (a) the first Calendar Year of the Term shall commence on the Effective Date and end on the 31st of December of the same year and (b) the last Calendar Year of the Term shall commence on the 1st of January of the Calendar Year in which this Agreement terminates or expires and end on the date of termination or expiration of this Agreement.

1.5 "**CLN1 Disease**" means infantile neuronal ceroid lipofuscinosis, also known as infantile Batten disease, caused by mutations in the palmitoyl protein thioesterase 1 (PPT1) gene.

1.6 "**cGMP Inventory**" means Licensor's inventory of CLN1 Plasmid that has been manufactured in accordance with (a) standards promulgated by any Regulatory Authority having jurisdiction over the manufacture of Licensed Product, including those Current Good Manufacturing Practices promulgated by the FDA and related regulations, as amended, or any successor laws thereto ("**cGMP**"), required to be followed in connection with the manufacture of biopharmaceutical products; (b) standards promulgated by any Regulatory Authority having jurisdiction over the manufacture of Licensed Product, in the form of draft or final guidance documents (including advisory opinions, compliance policy guides and guidelines); and (c) such other industry standards as may be agreed upon by the Parties as applicable to Licensed Product.

1.7 "**Clinical Trial**" means a clinical trial in human subjects that has been approved by a Regulatory Authority and Institutional Review Board or Ethics Committee, and is designed to measure the safety and/or efficacy of Licensed Product. Clinical Trials shall include, but are not limited to, Pivotal Trials.

1.8 "**Combination Product**" means any product comprised of a combination of a Licensed Product and at least one other product where the other product is an active ingredient, device, delivery system or other separately sold product co-packaged such that the Licensed Product and other product is integrated in a singular product.

1.9 "**Commercialization**" or "**Commercialize**" means any and all activities undertaken before and after Regulatory Approval of a BLA for the Licensed Product that relate to the marketing, promoting, distributing, importing or exporting for sale, offering for sale, and selling of the Licensed Product, and interacting with Regulatory Authorities regarding the foregoing.

1.10 "**Commercially Reasonable Efforts**" means: (a) with respect to the efforts to be expended by a Party with respect to any objective, such reasonable, diligent, and good faith efforts as such Party would normally use to accomplish a similar objective under similar circumstances; and (b) with respect to any objective relating to Development or Commercialization of the Licensed Product by a Party, the application by such Party, consistent with the exercise of its prudent scientific and business judgment, of diligent efforts and resources to fulfill the obligation in issue, consistent with the level of efforts a similarly situated pharmaceutical company would devote to a product at a similar stage in its product life as the Licensed Product and having profit potential and strategic value comparable to that of the Licensed Product, taking into account, without limitation, commercial, legal and regulatory factors, target product profiles, product labeling, past performance, the regulatory environment and competitive market conditions in the therapeutic area, safety and efficacy of the Licensed Product, the strength of its proprietary position and such other factors as such Party may reasonably consider, all based on conditions then prevailing. For clarity, Commercially Reasonable Efforts will not mean that a Party guarantees that it will actually accomplish the applicable task or objective.

1.11 "**Confidential Information**" of a Party, means information relating to the business, operations or products of a Party or any of its Affiliates, including any Know-How, that such Party discloses to the other Party under this Agreement, or otherwise becomes known to the other Party by virtue of this Agreement.

1.12 "**Controlled**" means, with respect to (a) Patents, (b) Know-How or (c) biological, chemical or physical material, that a Party or one of its Affiliates owns or has a license or sublicense to such Patents, Know-How or material (or in the case of material, has the right to physical possession of such material) and has the ability to grant a license or sublicense to, or assign its right, title and interest in and to, such Patents, Know-How or material as provided for in this Agreement without violating the terms of any agreement or other arrangement with any Third Party.

1.13 "**Cover**", "**Covering**" or "**Covered**" means, with respect to Licensed Product, that the making, using, selling, or offering for sale of Licensed Product would, but for a license granted in this Agreement under the Licensed Patents, infringe a Valid Claim of the Licensed Patents.

1.14 "**Development**" or "**Develop**" means, with respect to the Licensed Product, the performance of all pre-clinical and clinical development (including toxicology, pharmacology, test method development and stability testing, process development, formulation development, quality control development, statistical analysis), Clinical Trials (excluding Clinical Trials conducted after Regulatory Approval of a BLA), manufacturing and regulatory activities that are required to obtain Regulatory Approval of Licensed Product in the Territory.

1.15 "**Executive Officers**" means, together, [***] of Company and the [***] of Licensor.

1.16 "**Existing Third-Party Agreement(s)**" means the agreement(s) set forth on Schedule 1.16.

1.17 "**FDA**" means the United States Food and Drug Administration or a successor federal agency thereto.

1.18 "**Field**" means, and is limited to, the practice of the Licensed Patents, Licensed Material, and Licensed Know-How for gene therapy for the prevention, treatment or diagnosis of CLN1 Disease in humans.

1.19 "**First Commercial Sale**" of Licensed Product(s) means any transfer for value in an arms-length transaction to an independent Third Party distributor, agent or end user in a country after obtaining all Regulatory Approvals from applicable Regulatory Authorities required for the manufacture, importation, marketing, promotion, pricing, reimbursement and sale of the Licensed Product(s) in such country.

1.20 "**Governmental Body**" means any: (a) nation, principality, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (b) federal, state, local, municipal, foreign or other government; (c) governmental or quasi-governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, board, instrumentality, officer, official, representative, organization, unit, body or entity and any court or other tribunal); (d) multi-national or supranational organization or body; or (e) individual, entity, or body exercising, or entitled to exercise, any executive, legislative, judicial, administrative, regulatory, police, military or taxing authority or power of any nature.

1.21 "**IND**" means an investigational new drug application submitted to applicable Regulatory Authorities for approval to commence Clinical Trials in a given jurisdiction.

1.22 "**Know-How**" means any: (a) scientific or technical information, results or data of any type whatsoever, in any tangible or intangible form whatsoever, that is not in the public domain or otherwise publicly known, including, without limitation, discoveries, inventions, trade secrets, devices, databases, practices, protocols, regulatory filings, methods, processes (including manufacturing processes, specification and techniques), techniques, concepts, ideas, specifications, formulations, formulae, data (including pharmacological, biological, chemical, toxicological, clinical and analytical information, quality control, trial and stability data), case reports forms, medical records, data analyses, reports, studies and procedures, designs for experiments and tests and results of experimentation and testing (including results of research or development), summaries and information contained in submissions to and information from ethical committees, or Regulatory Authorities, and manufacturing process and development information, results and data, whether or not patentable, all to the extent not claimed or disclosed in a patent or patent application; and (b) compositions of matter including, without limitation, plasmids, viruses, cells, cell lines, assays, animal models and physical, biological or chemical material, including drug substance samples, intermediates of drug substance samples, drug product samples and intermediates of drug product samples. The fact that an item is known to the public shall not be taken to exclude the possibility that a compilation including the item, and/or a development relating to the item, is (and remains) not known to the public. "Know-How" includes any rights including copyright, database or design rights protecting such Know-How. "Know-How" excludes Patents.

1.23 "**Law**" or "**Laws**" means all applicable laws, statutes, rules, regulations, ordinances and other pronouncements having the binding effect of law of any Governmental Body.

1.24 "**Licensed Know-How**" means all Know-How that is Controlled by Licensor or any of its Affiliates, as of the Effective Date that is necessary or useful in the research, Development, manufacture, use, or Commercialization of Licensed Products. The Licensed Know-How shall include all Know-How set forth on Schedule 1.24.

1.25 "**Licensed Materials**" means the chemical, biological or physical materials that are set forth on Schedule 1.25.

1.26 "**Licensed Patents**" means the Patents listed on Schedule 1.26, (b) the Patent Rights (as defined by the UNC Agreement) to the extent not already set forth on Schedule 1.26, and (c) any Patents Controlled by Licensor or its Affiliates as of the Effective Date (to the extent not already set forth on Schedule 1.26 as of the Effective Date) or during the Term that are necessary or useful to research, Develop, manufacture, use or Commercialize a Licensed Product with an AAV vector containing a nucleic acid transgene encoding a PPT1 polypeptide. Licensor shall update Schedule 1.26 from time-to-time to include any new Patents Controlled by Licensor during the Term pursuant to clauses (b) and (c)..

1.27 "**Licensed Product**" means any product, or component part thereof for which the manufacture, use, importation or sale of which, absent the license granted under this Agreement, would infringe a Valid Claim of a Licensed Patent and/or that incorporate the Licensed Material.

1.28 "**Licensed Technology**" means the Licensed Patents, the Licensed Know-How and the Licensed Materials. For the avoidance of doubt, the Licensed Technology includes any Licensed Patents, Licensed Know-How and/or Licensed Materials Controlled by Licensor pursuant to any Existing Third-Party Agreement.

1.29 "**Market Exclusivity**" means an exclusive benefit by grant or an exclusion under or from the FDA, European Medicines Agency or other similar Regulatory Authority, relating to a Licensed Product, including orphan drug protection or data exclusivity.

1.30 "**Net Sales**" means [***].

1.31 "**Out-of-Pocket Expenses**" means expenses actually paid by a Party or its Affiliate to any Third Party; provided, that "**Out-of-Pocket Expenses**" shall not include expenses paid to any consultants (or service providers of like kind), except for travel expenses associated with a consultant (or service provider of like kind).

1.32 "**Patents**" means: (a) an issued or granted patent, including any extension, supplemental protection certificate, registration, confirmation, reissue, reexamination or renewal thereof; (b) a pending patent application, including any continuation, divisional, continuation-in-part, substitute or provisional application thereof; and (c) all counterparts or foreign equivalents of any of the foregoing issued by or filed in any country or other jurisdiction.

1.33 "**Person**" means any natural person, corporation, firm, business trust, joint venture, association, organization, company, partnership or other business entity, or any government or agency or political subdivision thereof.

1.34 "**Pivotal Trial**" means either (a) a Clinical Trial, the principal purpose of which is to demonstrate clinically and statistically the efficacy and safety of a Licensed Product as a basis for obtaining Regulatory Approval, and has been recognized and approved as such by the Regulatory Authority, or (b) a Clinical Trial of a Licensed Product on a sufficient number of subjects that satisfies both of the following (i) and (ii): (i) such trial is designed to establish that such Licensed Product has an acceptable safety and efficacy profile for its intended use, and to determine warnings, precautions, and adverse reactions that are associated with such Licensed Product, which trial is intended to support Regulatory Approval of such Licensed Product; and (ii) such trial is a registration trial intended to be sufficient to support the filing of an application for Regulatory Approval for such Licensed Product in the US, or applicable country, as evidenced by (A) an agreement with or statement from the FDA or its foreign equivalent, or (B) other guidance or minutes issued by the FDA or its foreign equivalent, for such registration trial.

1.35 "**Price Approvals**" means, in those countries in the Territory where Regulatory Authorities may approve or determine pricing and/or pricing reimbursement for pharmaceutical products, such pricing and/or pricing reimbursement approval or determination.

1.36 "**Regulatory Authority**" means the FDA or any foreign equivalent.

1.37 "**Regulatory Approval**" means any and all approvals, licenses, registrations, or authorizations of the relevant Regulatory Authority, including Price Approvals, necessary for the Development, manufacture, use, storage, import, transport or Commercialization of Licensed Product in a particular country or jurisdiction. For the avoidance of doubt, Regulatory Approval to Commercialize Licensed Product shall include Price Approval.

1.38 "**Royalty Term**" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the period from the First Commercial Sale of such Licensed Product in such country until the later of (a) the expiration or revocation or complete rejection of the last to expire or to be revoked or to be completely rejected of any Licensed Patent Covering such Licensed Product in the country in which the Licensed Product is sold, (b) the loss of Market Exclusivity in the country in which such Licensed Product is sold, or (c) if no Licensed Patent exists in the country where the Licensed Product is sold Covering the manufacture, use or sale of the Licensed Product and no Market Exclusivity exists in the country in which the Licensed Product is sold, ten (10) years from the First Commercial Sale of such Licensed Product in such country.

1.39 "**Sublicensee**" means any Person other than an Affiliate of Company to which Company (or its Affiliates) grants a sublicense under Licensed Patents or Licensed Know-How.

1.40 "**Tax**" or "**Taxes**" means any federal, state, local or foreign income, gross receipts, license, payroll, employment, excise, severance, stamp, occupation, premium, windfall profits, environmental, customs duties, capital stock, franchise, profits, withholding, social security, unemployment, disability, real property, personal property, sales, use, transfer, registration, value added, alternative or add-on minimum, estimated, or other tax of any kind whatsoever, including any interest, penalty, or addition thereto, whether disputed or not.

1.41 "**Territory**" means all the countries of the world.

1.42 "**Third Party**" means any Person other than Licensor, Company or any of their respective Affiliates.

1.43 "**Third Party License Agreement**" means any agreement entered into by a Party or its Affiliate with a Third Party, or any amendment or supplement thereto, in each case following the Effective Date, whereby royalties, fees or other payments are to be made by a Party or its Affiliate to such Third Party in connection with the grant of rights under intellectual property rights Controlled by such Third Party, which rights are necessary or useful to research, Develop, manufacture, have made, import, export, use or Commercialize Licensed Product.

1.44 "**United States**" or "**US**" means the United States of America, its territories and possessions.

1.45 "**USD**" or "**$**" means the lawful currency of the United States.

1.46 "**UNC Agreement**" means the license agreement entered into by Licensor and the University of North Carolina at Chapel Hill ("**University**") dated September 16, 2016 and referencing "CLN1 Genome Designs for AAV Vectors, University File 16-0076," as attached hereto as Schedule 1.46.

1.47 "**Valid Claim**" means, with respect to the Licensed Patents, (a) any unexpired claim of an issued Patent that has not been found to be unpatentable, unenforceable or invalid by a court or other governmental agency of competent jurisdiction that is unappealable or unappealed in the time allowed for appeal; or (b) a claim of a pending application, which application claims a first priority no more than [***] prior to the date upon which pendency is determined. For purposes of clarification, if a claim in an application has been pending for more than [***] from its priority date, and a Patent subsequently issues containing such claim, then upon issuance of the Patent, the claim shall thereafter be considered a Valid Claim.

1.48 **Other Terms**. The definition of each of the following terms is set forth in the section of the Agreement indicated below:

Defined Term	Section
"**Action**"	6.3(b)(i)
"**Agreement**"	Preamble
"**cGMP**"	1.6
"**CM&C Know-How**"	2.4
"**Company**"	Preamble
"**Company Indemnitees**"	9.2
"**Company IP**"	10.4(b)(ii)(6)
"**Company Patent**"	6.2(d)

"**Development Support**"	3.2
"**Effective Date**"	Preamble
"**Existing IND**"	2.5
"**Insolvency Event**"	10.3.2
"**Licensor**"	Preamble
"**Licensor Indemnitees**"	9.1
"**Manufacturing Technology Transfer**"	2.4
"**Manufacturing Technology Transfer Plan**"	2.4
"**Party**" and "**Parties**"	Preamble
"**Representatives**"	3.2
"**Sales Milestone**"	5.3
"**Suspension IND**"	5.2
"**Term**"	10.1
"**University**"	1.46

ARTICLE 2
LICENSES AND OTHER RIGHTS

2.1 **Grant of License to Company**. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Company and its Affiliates an exclusive (even as to Licensor), worldwide, royalty-bearing right and license (with the right to sublicense, subject to the provisions of Section 2.2) under the Licensed Technology to research, Develop, manufacture, have manufactured, use and Commercialize Licensed Product in the Field in the Territory.

2.2 **Grant of Sublicense by Company**. Company shall have the right, in its sole discretion, to grant sublicenses, in whole or in part, under the licenses granted in Section 2.1; provided, however, that (a) the terms and conditions of each sublicense shall be consistent with the terms and conditions of this Agreement and the UNC Agreement and (b) the granting by Company of a sublicense shall not relieve Company of any of its obligations hereunder.

2.3 **Technology Transfer**. As soon as reasonably practicable after the Effective Date, but in no event later than [***] following the Effective Date (provided that Company shall have the right to reasonably extend such time period of time to the extent reasonably necessary to enable Company to receive the foregoing transfer), Licensor will transfer to Company, at Licensor's cost and expense, the Licensed Know-How set forth on Schedule 1.24 and Licensed Materials (except for Licensed Know-How and Licensed Materials relating to the manufacture of Licensed Product, which are the subject of Section 2.4).

2.4 **Manufacturing Technology Transfer**. The technology transfer described in this Section 2.4 shall be referred to as the "**Manufacturing Technology Transfer**" and shall be conducted in accordance with a manufacturing technology transfer plan to be mutually agreed upon in writing by the Parties within [***] after the Effective Date (the "**Manufacturing Technology Transfer Plan**"). The Manufacturing Technology Transfer Plan will: (a) specify goals and estimated time lines for the achievement of the Manufacturing Technology Transfer; (b) identify certain of the technology to be transferred; (c) specify the activities related to implementation of such technology in Company's facilities; and (d) set forth those obligations assigned to each Party with respect to such technology transfer. Manufacturing Technology Transfer Plan shall also include an obligation for Licensor to transfer to Company a copy of all Licensed Know-How and all Licensed Materials, including such Know-How as is specifically identified in the Manufacturing Technology Transfer Plan, including, without limitation, toxicity data related to Licensed Product, as is reasonably necessary to complete

part of the Chemistry, Manufacturing and Controls section of a regulatory submission document included in a BLA (collectively, "**CM&C Know-How**"). Each Party will bear its costs and expenses associated with performing the Manufacturing Technology Transfer. In conjunction with the execution of this Agreement, Licensor and Company are entering into a Purchase and Reimbursement Agreement, dated on or about the Effective Date (the form of which is attached hereto as Schedule 2.4), pursuant to which (i) Licensor shall sell, and Company shall purchase, the cGMP Inventory as set forth therein for a total consideration of [***] as set forth in the Purchase and Reimbursement Agreement.

2.5 **Regulatory Technology Transfer**. Upon Company's written request, Licensor shall, at Licensor's cost and expense, assign to Company all applications and filings made by or on behalf of Licensor with any Regulatory Authority with respect to Licensed Product, including any IND (including the IND existing as of the Effective Date (the "**Existing IND**")), BLA or orphan drug designations or any other application for regulatory consultations or consideration, including sponsorship thereof, as more fully detailed in Schedule 2.5. Notwithstanding the foregoing, the Parties acknowledge the assignment and transfer of all regulatory materials pursuant to this Section 2.5 is subject to the acceptance of the applicable Regulatory Authority. Accordingly, until such acceptance by each applicable Regulatory Authority, Licensor shall remain responsible for performing any tasks required by a Regulatory Authority, including, but not limited to, responding to any requests or communications with a Regulatory Authority; provided, however, that Licensor shall consult with Company regarding such obligations prior to taking any actions and Company shall have the final decision-making authority with respect to any such actions to be taken by Licensor.

2.6 **Procedures for Technology Transfer**. The technology transfers set forth in Sections 2.3, 2.4 and 2.5 shall occur in an orderly fashion and in a manner such that the value, usefulness and confidentiality of the transferred Licensed Know-How, Licensed Materials and regulatory documentation are preserved in all material respects. During the Term, Licensor shall, upon Company's written request, provide full and prompt disclosure of, and shall license, and hereby does license, to Company as a part of Licensed Know-How, any Know-How (including CM&C Know-How) that becomes Controlled by Licensor or any of its Affiliates after the Effective Date that is reasonably necessary for Company to manufacture Licensed Product or complete the Chemistry, Manufacturing and Controls section of a regulatory submission document included in a BLA.

2.7 **UNC Agreement**. Company hereby (a) acknowledges the [***].

2.8 **No Implied Licenses**. Except as explicitly set forth in this Agreement, neither Party shall be deemed by estoppel or implication to have granted the other Party any license or other right to any intellectual property of such Party. For clarity, notwithstanding anything contained in this Agreement to the contrary, Licensor retains all rights under the Licensed Technology for any and all uses outside of the Field.

2.9 **Negative Covenant**. Company covenants that it will not, and will not permit any of its Affiliates or Sublicensees to, use or practice any Licensed Technology outside the scope of the license granted to it under Section 2.1.

ARTICLE 3
DEVELOPMENT, MANUFACTURE AND COMMERCIALIZATION OF PRODUCT

3.1 **Development of the Licensed Product by Company**. Company shall have the exclusive right, and sole responsibility and decision-making authority, to research and Develop Licensed Product and to conduct (either itself or through its Affiliates, agents, subcontractors and/or Sublicensees) all Clinical Trials and non-clinical studies Company believes appropriate to obtain Regulatory Approval for Licensed Product in the Field.

3.2 **Licensor Support in Development**. Until the [***], Licensor shall make its employees, consultants, contractors, advisors and agents ("**Representatives**") that are knowledgeable regarding the Licensed Technology or Licensed Product (including the properties and functions thereof), available to Company for scientific and technical explanations, and advice that may reasonably be required by Company relating to the Development of Licensed Products (the "**Development Support**"). The Development Support shall be provided by Licensor free-of-charge up to [***]. Thereafter, any Development Support will be reimbursed by Company at a mutually agreed upon (in writing) hourly rate.

3.3 **Commercialization**. Company shall have the exclusive right, and sole responsibility and decision-making authority, to Commercialize Licensed Product itself or through one or more Affiliates or Sublicensees or other Third Parties selected by Company and shall have the sole decision-making authority and responsibility in all matters relating to the Commercialization of the Licensed Product.

3.4 **Clinical and Commercial Manufacturing**. Company shall have the exclusive right to manufacture Licensed Product itself or through one or more Affiliates or Sublicensees or other Third Parties selected by Company.

3.5 **Diligence by Company**. Subject to Licensor's fulfillment of its obligations under this Agreement, Company shall use Commercially Reasonable Efforts to (a) Develop at least one Licensed Product and (b) Commercialize at least one (1) Licensed Product in the United States. Activities by Company's Affiliates and Sublicensees will be considered as Company's activities under this Agreement for purposes of determining whether Company has complied with its obligation to use Commercially Reasonably Efforts.

3.6 **Trademarks**. As between Licensor and Company, Company shall have the sole authority to select trademarks for the Licensed Product and shall own all such trademarks.

3.7 **Reporting**. Company shall, within plus or minus [***] of each anniversary of the Effective Date, provide Licensor with a written report summarizing in reasonable detail its major Development and, as applicable, Commercialization activities conducted during the prior Calendar Year. All information and reports provided to Licensor pursuant to this Section 3.7 shall be without any commitment from Company and shall be treated as Confidential Information of Company hereunder. Notwithstanding the foregoing, Company's obligation to provide reports under this Section 3.7 shall expire upon First Commercial Sale in the United States.

ARTICLE 4
REGULATORY MATTERS

4.1 **Regulatory Filings**. As between Company and Licensor, Company shall own and maintain all regulatory filings and Regulatory Approvals for the Licensed Product, including all INDs and BLAs.

4.2 **Communications with Authorities**. Company (or one of its Affiliates or Sublicensees) shall be responsible, and act as the sole point of contact, for communications with Regulatory Authorities in connection with the Development, Commercialization, and manufacturing of Licensed Product.

Following the Effective Date, Licensor shall not initiate, with respect to Licensed Product, any meetings or contact with Regulatory Authorities without Company's prior written consent. To the extent Licensor receives any written or oral communication from any Regulatory Authority relating to Licensed Product, Licensor shall (a) refer such Regulatory Authority to Company, and (b) as soon as reasonably practicable (but in any event within [***] after becoming aware of such communication), notify Company and provide Company with a copy of any written communication received by Licensor or, if applicable, complete and accurate minutes of such oral communication.

4.3 **Recalls**. Company shall have the sole right to determine whether and how to implement a recall or other market withdrawal of the Licensed Product.

ARTICLE 5
FINANCIAL PROVISIONS

5.1 **Initial Fee**. Company shall pay, or cause to be paid, to Licensor a fee of Three Million USD (USD 3,000,000), within [***] following the Effective Date. Payment of the initial fee shall be subject to any withholding tax obligations set forth in Section 5.11.

5.2 **Milestone Payments**. Company shall pay to Licensor the following milestone payments upon the first achievement of the milestone events described below for each Licensed Product, whether achieved by Company, its Affiliates or their respective Sublicensees. Company shall notify Licensor in writing of the achievement of any such milestone event, within [***] of such achievement, and Licensor shall issue Company an invoice for the amount of the corresponding milestone payment, which invoice Company shall pay within [***] following receipt of such invoice.

Milestone event for each Licensed Product	Milestone Payment USD
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

For the avoidance of doubt, the total foregoing milestones shall be payable once per Licensed Product.

With respect to the IND Milestone set forth in the table above, the Parties acknowledge and agree that (a) as of the Effective Date, Licensor is transferring the Existing IND to Company, and (b) Company shall elect to either Develop and Commercialize Licensed Product under the Existing IND or submit its own IND for a Licensed Product based on a suspension re-formulation of such Licensed Product (the "**Suspension IND**"). In the event that Company elects to Develop and Commercialize Licensed Product under the Existing IND by providing express written notice to Licensor of such election, the process for payment of the milestone payment for the IND Milestone shall be triggered as of the date of such notice from Company. In the event that Company elects to file the Suspension IND, the process for the milestone payment for the IND Milestone shall be triggered upon the date of filing of the Suspension IND.

5.3 **Commercial Event Payments**. Company shall pay Licensor the following amounts for the first and only the first achievement of the following sales event milestones (each, a "**Sales Milestone**").

Sales Milestones for each Licensed Product	Milestone Payment USD
[***]	[***]
[***]	[***]

Company shall deliver written notice to Licensor within [***] of the end of the Calendar Year in which a Sales Milestone occurs and Licensor shall issue Company an invoice for the amount of the corresponding Sales Milestone payment, which invoice Company shall pay within [***] following receipt of such invoice.

For the avoidance of doubt, each aforementioned Sales Milestone payment shall be made only once for each Licensed Product that achieves such Sales Milestone.

5.4 **Royalty Payments for Licensed Product**.

(a) **Royalty Rate**. During the Royalty Term, Company shall pay to Licensor a [***] royalty on worldwide aggregate annual Net Sales of Licensed Product for each Calendar Year (subject to Section 5.5 below):

(b) **Net Sales Subject to Royalty Payments**. For purposes of determining whether a royalty threshold or a Sales Milestone has been attained, only Net Sales that are subject to a royalty payment shall be included in the total amount of Net Sales and any Net Sales that are not subject to a royalty payment shall be excluded. For clarity, Company's obligation to pay royalties to Licensor under this ARTICLE 5 is imposed only once with respect to the same unit of Licensed Product regardless of the number of Licensed Patents pertaining thereto.

5.5 **Existing Third-Party Agreements**. Licensor shall be responsible for the timely payment of any amounts due under any Existing Third-Party Agreement, and in the event that Licensor shall fail to make any payment when due under such Existing Third-Party Agreement, Company shall have the right to make such payment on behalf of Licensor. In such event, Licensor shall promptly reimburse Company any such amounts paid by Company or, at Company's election, Company may offset such amounts paid by Company against any amounts payable to Licensor hereunder.

5.6 **Timing of Payment**. Royalties payable under Section 5.4(a) shall be payable on actual Net Sales and shall accrue at the time the invoice for the sale of Licensed Product is delivered. Royalty obligations that have accrued during a particular Calendar Quarter shall be paid, on a Calendar Quarter basis, within [***] after the end of each Calendar Quarter during which the royalty obligation accrued.

5.7 **Mode of Payment and Currency; Invoices**.

(a) **Currency**. All payments to Licensor hereunder shall be made by deposit of USD in the requisite amount to such bank account as Licensor may from time to time designate by written notice to Company. With respect to sales not denominated in USD, Company shall convert applicable sales in foreign currency into USD by using the then current and reasonable standard exchange rate methodology applied to its external reporting. Based on the resulting sales in USD, the then applicable royalties shall be calculated. The Parties may vary the method of payment set forth herein at any time upon mutual written agreement, and any change shall be consistent with the local Law at the place of payment or remittance.

(b) **Invoices**. Licensor shall submit its invoices to accountspayable@pbmcap.com.

5.8 **Royalty Reports**. Within [***] after the end of each Calendar Quarter during which Licensed Product has been sold, Company shall deliver to Licensor, together with the applicable royalty payment due for such Calendar Quarter, a written report, on a Licensed Product-by-Licensed Product basis, of Net Sales subject to royalty payments for such Calendar Quarter. Such report shall be deemed "Confidential Information" of Company subject to the obligations of ARTICLE 8 of this Agreement.

5.9 **Late Payments**. Any failure by Company to make a payment within [***] after the date when due shall obligate Company to pay computed interest, the interest period commencing on the due date and ending on the actual payment date, to Licensor at a rate of [***] per month, or the highest rate allowed by Law, whichever is lower.

5.10 **Audits**.

(a) **Audits Generally**. During the Royalty Term and for [***] thereafter, and not more than once in each Calendar Year, Company shall permit, and shall cause its Affiliates or Sublicensees to permit, an independent certified public accounting firm of nationally recognized standing selected by Licensor, and reasonably acceptable to Company or such Affiliate or Sublicensee, to have access to and to review, during normal business hours upon reasonable prior written notice, the applicable records of Company and its Affiliates or Sublicensees to verify the accuracy of the royalty reports and payments under this ARTICLE 5. Such review may cover the records for sales made in any Calendar Year ending not more than [***] prior to the date of such request. The accounting firm shall disclose to Licensor and Company only whether the royalty reports are correct or incorrect and the specific details concerning any discrepancies. No other information shall be provided to Licensor.

(b) **Audit-Based Reconciliation**. If such accounting firm concludes that additional royalties were owed during such period, and Company agrees with such calculation, Company shall pay the additional undisputed royalties within [***] after the date Licensor delivers to Company such accounting firm's written report, together with any interest payable under Section 5.9. If such accounting firm concludes that an overpayment was made, such overpayment shall be fully creditable against amounts payable in subsequent payment periods or, at Company's request, shall be promptly reimbursed to Company. If Company disagrees with such calculation, it may retain its own independent certified public accounting firm of recognized standing and reasonably acceptable to Licensor, to conduct a review, and if such firm concurs with the other accounting firm, Company shall make the required payment within [***] after the date Company receives the report of its accounting firm. If Company's accounting firm does not concur, Company and Licensor shall meet and negotiate in good faith a resolution of the discrepancies between the two firms. Licensor shall pay for the cost of any audit, unless Company has underpaid Licensor by more than [***], in which case Company shall pay for the costs of audit.

(c) **Audit Confidentiality**. Each Party shall treat all information that it receives under this Section 5.10 in accordance with the confidentiality provisions of ARTICLE 7 of this Agreement, and shall cause its accounting firm to enter into an acceptable confidentiality agreement with the other Party obligating such firm to retain all such financial information in confidence pursuant to such confidentiality agreement, except to the extent necessary for such Party to enforce its rights under this Agreement. The terms of this Section 5.10 shall apply *mutatis mutandis* with respect to Company's right to audit Licensor's records related to those Out-of-Pocket Expenses for which Licensor seeks reimbursement hereunder.

5.11 **Taxes**. Licensor shall be responsible for the payment of any and all Taxes levied on account of the royalties and other payments paid to Licensor by Company or its Affiliates or Sublicensees under this Agreement. If Law requires that Taxes be deducted and withheld from royalties or other payments paid under this Agreement, Company shall (a) deduct those Taxes and interests and penalties assessed thereon from the payment or from any other payment owed by Company hereunder; (b) pay the Taxes to the proper Governmental Body; (c) send evidence of the obligation together with proof of Tax payment to Licensor within [***] following such payment; (d) remit the net amount, after deductions or withholding made under this Section 5.11; and (e) cooperate with Licensor in any way reasonably requested by Licensor, to obtain available reductions, credits or refunds of such Taxes; provided, however, that Licensor shall reimburse Company for Company's Out-of-Pocket Expenses incurred in providing such assistance.

ARTICLE 6
INVENTIONS AND PATENTS

6.1 **Further Assurances**. Licensor shall require all of its employees, and use its best efforts to require its contractors and agents, and any Affiliates and Third Parties working on its behalf under this Agreement (and their respective employees, contractors and agents), to assign to Licensor any Licensed Technology.

6.2 **Patent Prosecution and Maintenance**.

(a) **Licensed Patents**. As between the Parties, and subject to the UNC Agreement, Licensor shall have the first right, and the obligation, to file, prosecute and maintain Licensed Patents in Licensor's name. Licensor shall keep Company informed of the status of the filing and prosecution of Licensed Patents or related proceedings (e.g., interferences, oppositions, reexaminations, reissues, revocations or nullifications) in a timely manner, and will take into consideration the advice and recommendations of Company.

(b) **Election Not to file and Prosecute Licensed Patents**. As between the Parties, and subject to the UNC Agreement, if Licensor elects not to file or to continue to prosecute or maintain a Licensed Patent in Licensor's name, then it shall notify Company in writing at least [***] before any deadline applicable to the filing, prosecution or maintenance of such Licensed Patent, as the case may be, or any other date by which an action must be taken to establish or preserve such Licensed Patent in such country or possession. In such case, Company shall have the right to pursue the filing or support the continued prosecution or maintenance of such Licensed Patent. If Company elects to continue prosecution or maintenance of any of the Licensed Patents, Licensor shall provide Company with all relevant information, documentation and assistance in this respect as may reasonably be requested by Company. If Company fails to continue prosecution or maintenance of any of the Licensed Patents, then such abandoned Licensed Patents shall not extend the Royalty Term (i.e., no royalty payments shall be due under this Agreement on account of such abandoned Licensed Patents).

(c) **Patent Term Extension**. As between the Parties, and subject to the UNC Agreement, Company shall be responsible for obtaining patent term extensions wherever available for Licensed Patents, but Company has no obligation to seek any patent term extension. Licensor shall provide Company with all relevant information, documentation and assistance in this respect as may reasonably be requested by Company. Any such assistance, supply of information and consultation shall be provided promptly and in a manner that will ensure that all patent term extensions for Licensed Patents are obtained wherever legally permissible, and to the maximum extent available.

(d) **Company Patents**. Company shall own any inventions, discoveries, Know-How and intellectual property rights developed by Company or any of its Affiliates or a Third Party on behalf of Company and shall have the right, but not the obligation, to file, prosecute and maintain Patent Rights covering or claiming any such inventions, discoveries, Know-How or intellectual property ("**Company Patent**"). Company shall bear all costs and expenses of filing, prosecuting and maintaining Company Patents and Licensor shall have no particular rights with respect thereto.

6.3 **Enforcement and Defense**.

(a) **Notice**.

(i) If either Party believes that an infringement, unauthorized use, misappropriation or ownership claim or threatened infringement or other such activity by a Third Party with respect to any Licensed Technology, or if a Third Party claims that any Licensed Patent is invalid or unenforceable, the Party possessing such knowledge or belief shall notify the other Party and provide it with details of such infringement or claim that are known by such Party.

(ii) In the event that Licensor believes that a Company Patent, if any, is being infringed by a Third Party or if a Third Party claims that any Company Patent is invalid or unenforceable, Licensor shall notify Company and provide it with details of such infringement or claim.

(b) **Actions**.

(i) As between the Parties, and subject to the UNC Agreement, Licensor shall have the first right to attempt to resolve any infringement or claim, including by filing an infringement suit, defending against such claim or taking other similar action, with respect to a Licensed Patent (each, an "**Action**") and to compromise or settle any such infringement or claim subject to Section 6.3(c). Company shall provide reasonable assistance to Licensor at Licensor's cost and expense, including by providing access to relevant documents and other evidence and making its employees reasonably available. All amounts recovered by Licensor shall be allocated, first, to the costs and expenses of the Parties incurred to enforce the Licensed Patents and, second, to Company (provided that such remaining amounts shall be deemed Net Sales for royalty and Sales Milestone calculation purposes).

(ii) As between the Parties, and subject to the UNC Agreement, if Licensor elects not to exercise its first right with respect to an Action as set forth in Section 6.3(b)(i), then it shall promptly notify Company in writing (but in any event with at least [***] before any deadline applicable to such Action or any other date by which an action must be taken to establish or preserve such right bring or defend such Action). In such case, Company shall have the right to pursue the Action to enforce or defend such Licensed Patent. Licensor shall provide Company with all relevant information, documentation and assistance in this respect as may reasonably be requested by Company. Licensor shall join Company in such Action upon Company's written request. All amounts recovered by Company shall be retained by Company.

(c) **No Settlement without Consent**. Neither Party shall settle or otherwise compromise any Action by admitting that any Licensed Patent is invalid or unenforceable without the other Party's prior written consent, and, in the case of Licensor, Licensor may not settle or otherwise compromise an Action without Company's prior written consent.

(d) **Company Patents**. Company shall have the sole right and authority, but not the obligation, to enforce Company Patents against any Third Party infringer and defend Company Patents against any Third Party; provided, that Licensor shall provide reasonable assistance to Company with respect thereto, including providing access to relevant documents and other evidence and making its employees available, subject to Company's reimbursement of any Out-of-Pocket Expenses incurred on an on-going basis in providing such assistance.

ARTICLE 7
CONFIDENTIALITY

7.1 **Confidentiality Obligations**. Each Party agrees that, for the Term and for [***] thereafter, such Party shall, and shall ensure that its Representatives hold in confidence all Confidential Information disclosed to it by the other Party pursuant to this Agreement, unless such information, as demonstrated by competent evidence of the recipient:

(i) is or becomes generally available to the public other than as a result of disclosure by the recipient;

(ii) is already known by or in the possession of the recipient at the time of disclosure by the disclosing Party;

(iii) is independently developed by recipient without use of or reference to the disclosing Party's Confidential Information; or

(iv) is obtained by recipient from a Third Party that has not breached any obligations of confidentiality.

The recipient shall not disclose any of the Confidential Information, except to Representatives of the recipient who need to know the Confidential Information for the purpose of performing the recipient's obligations, or exercising its rights, under this Agreement and who are bound by obligations of non-use and non-disclosure substantially similar to those set forth herein. The recipient shall be responsible for any disclosure or use of the Confidential Information by such Representatives. The recipient shall protect Confidential Information using not less than the same care with which it treats its own confidential information, but at all times shall use at least reasonable care. Each Party shall: (a) implement and maintain appropriate security measures to prevent unauthorized access to, or disclosure of, the other Party's Confidential Information; (b) promptly notify the other Party of any unauthorized access or disclosure of such other Party's Confidential Information; and (c) cooperate with such other Party in the investigation and remediation of any such unauthorized access or disclosure.

7.2 **Use**. Notwithstanding Section 7.1, a Party may use the Confidential Information of the other Party for the purpose of performing its obligations, or exercising its rights, under this Agreement, including for purposes of:

(i) filing or prosecuting patent applications, subject to the terms of Section 6.2;

(ii) prosecuting or defending litigation;

(iii) conducting pre-clinical studies or Clinical Trials pursuant to this Agreement;

(iv) seeking or maintaining Regulatory Approval of the Licensed Product; or

(v) complying with Law, including securities Law and the rules of any securities exchange or market on which a Party's securities are listed or traded.

In addition to the foregoing, Company may, in furtherance of its rights under this Agreement, disclose Confidential Information of Licensor to any Third Party, provided that such Third Party is bound by obligations of confidentiality at least as stringent as the ones herein.

In making any disclosures set forth in clauses (i) through (v) above, the disclosing Party shall, where reasonably practicable, give such advance notice to the other Party of such disclosure requirement as is reasonable under the circumstances and will use its reasonable efforts to cooperate with the other Party in order to secure confidential treatment of such Confidential Information required to be disclosed. In addition, in connection with any permitted filing by either Party of this Agreement with any Governmental Body the filing Party shall endeavor to obtain confidential treatment of economic, trade secret information and such other information as may be requested by the other Party, and shall provide the other Party with the proposed confidential treatment request with reasonable time for such other Party to provide comments, and shall include in such confidential treatment request all reasonable comments of the other Party.

7.3 **Required Disclosure**. The recipient may disclose the Confidential Information to the extent required by Law or court order; provided, however, that the recipient promptly provides to the disclosing party, to the extent legally permissible, prior written notice of such disclosure and provides reasonable assistance in obtaining an order or other remedy protecting the Confidential Information from public disclosure.

7.4 **Publications**. Licensor shall not publish any information relating to Licensed Product without the prior written consent of Company (which consent may be withheld or given in Company's sole discretion), unless such information has already been publicly disclosed either prior to the Effective Date or after the Effective Date through no fault of Licensor or otherwise not in violation of this Agreement. Company shall have the right to make such publications as it chooses, in its sole discretion, without the approval of Licensor. Licensor shall submit to Company for Company's written approval (which approval be granted or denied in Company's sole discretion) any publication or presentation (including in any seminars, symposia or otherwise) of information related directly or indirectly to the Licensed Product for review and approval at least [***] prior to submission for the proposed date of publication or presentation.

7.5 **Press Releases and Disclosure**.

(a) **Initial Press Release**. Following the Effective Date, the Parties shall work in good faith to issue a mutually agreeable joint public announcement of the execution of this Agreement.

(b) **Public Disclosures By Licensor**. Except as provided in Section 7.5(d), Licensor may not make any subsequent press release or public announcement regarding the terms of this Agreement or any matter covered by this Agreement, including the Development or Commercialization of Licensed Products, without the prior written consent of Company.

(c) Public Disclosures by Company. Except as provided in Section 7.5(d), Company may not make any subsequent press release or public announcement regarding the terms of this Agreement; provided, however, that Company shall have the right to make such press releases as it chooses, in its sole discretion, regarding the status of its Development or Commercialization of Licensed Products without the approval of Licensor.

(d) Securities Filings and Regulatory Communications. Notwithstanding anything in this Agreement, either Party shall have the right without the approval of the other Party to make disclosures related to this Agreement or the Licensed Product, including (i) securities filings that such Party determines are required under applicable securities laws and regulations (provided, that it provides the text of such planned disclosure to the non-disclosing Party no less than [***] prior to disclosure, and has used reasonable efforts to incorporate all reasonable comments of the non-disclosing Party regarding such disclosure) and (ii) regulatory communications to the extent such Party determines that such disclosures are necessary or useful in connection with such regulatory communication (including, but not limited to, regulatory filings).

ARTICLE 8
REPRESENTATIONS, WARRANTIES AND COVENANTS

8.1 **Representations and Warranties**. Each Party represents and warrants to the other Party that, as of the Effective Date:

(i) such Party is duly organized and validly existing under the Laws of the jurisdiction of its incorporation or organization;

(ii) such Party has taken all action necessary to authorize the execution and delivery of this Agreement and the performance of its obligations under this Agreement;

(iii) this Agreement is a legal and valid obligation of such Party, binding upon such Party and enforceable against such Party in accordance with the terms of this Agreement, except as enforcement may be limited by applicable bankruptcy, fraudulent conveyance, insolvency, reorganization, moratorium and other laws relating to or affecting creditors' rights generally and by general equitable principles. The execution, delivery and performance of this Agreement by such Party does not conflict with, breach or create in any Third Party the right to accelerate, terminate or modify any agreement or instrument to which such Party is a party or by which such Party is bound, and does not violate any Law of any Governmental Body having authority over such Party;

(iv) no consent by any Third Party or Governmental Body is required with respect to the execution and delivery of this Agreement by such Party or the consummation by such Party of the transactions contemplated hereby; and

(v) such Party has all right, power and authority to enter into this Agreement, to perform its obligations under this Agreement.

8.2 **Additional Representations and Warranties of Licensor**. Licensor represents and warrants to Company that, as of the Effective Date:

(i) no claims have been asserted, or, to Licensor's knowledge, threatened by any Person, nor, to Licensor's knowledge, are there any valid grounds for any claim of any such kind (a) challenging the validity, effectiveness, or ownership of Licensed Technology, and/or (b) to the effect that the use, reproduction, modification, manufacturing, distribution, licensing, sublicensing, sale or any other exercise of rights in any of Licensed Technology infringes or will infringe on any intellectual property right of any Person;

(ii) to the knowledge of Licensor, there is no unauthorized use, infringement or misappropriation of any of Licensed Technology by any employee or former employee of Licensor, or any other Third Party;

(iii) the Licensed Patents are subsisting and are not the subject of any litigation procedure, discovery process, interference, reissue, reexamination, opposition, appeal proceedings or any other legal dispute;

(iv) the Licensed Patents constitute all Patents Controlled by Licensor as of the Effective Date that are directly related to, or are necessary or useful for, the research, Development, manufacture, use or Commercialization of Licensed Product;

(v) the Licensed Know-How (a) constitutes all Know-How Controlled by Licensor as of the Effective Date that is reasonably necessary for the research, Development, manufacture, use or Commercialization of Licensed Product and (b) constitutes all Know-How that is, to Licensor's knowledge, reasonably necessary for the research, Development, manufacture, use or Commercialization of Licensed Product;

(vi) it has the full right to provide the Licensed Materials to Company;

(vii) all Representatives of Licensor who have performed any activities on its behalf in connection with research regarding Licensed Product have assigned to Licensor in all material respects their rights in any intellectual property made, discovered or developed by them as a result of such research, and no Third Party has any rights to any such intellectual property;

(viii) Licensor has the right, power and authority to grant to Company the rights granted to Company hereunder, including, but not limited to, with respect to the Existing Third-Party Agreements. In particular, the grant of such sublicense requires no consent, waiver or other action by any party to the Existing Third-Party Agreements and the rights and obligations of Company set forth in this Agreement do not contravene nor are they inconsistent with or in conflict with the terms of any Existing Third-Party Agreement;

(ix) The Existing Third-Party Agreements constitute all agreements with Third Parties pursuant to which Licensor has in-licensed, or otherwise obtained rights, with respect to Licensed Product. Licensor has provided to Company an accurate, true and complete copy of each of the Existing Third Party Agreements, as amended to-date and each of the Existing Third Party Agreements is in full force and effect and Licensor is not in breach or default in the performance of its obligations under any of the Existing Third Party Agreements. Licensor has not received any notice from any Third Party of any breach, default or non-compliance of Licensor under the terms of any of the Existing Third-Party Agreements. There have been no amendments or other modification to any Existing Third-Party Agreements, except as have been disclosed to Company in writing;

(x) Licensor has not received any communication from University regarding any failure to achieve any milestone set forth on Appendix B of the UNC Agreement, including any failure to achieve any milestone within the time period set forth therein;

(xi) all tangible information and data provided by or on behalf of Licensor to Company on or before the Effective Date in contemplation of this Agreement was and is true, accurate and complete in all material respects, and Licensor has not failed to disclose, or cause to be disclosed, any information or data that would cause the information and data that has been disclosed to be misleading in any material respect;

(xii) Licensor (and its Affiliates) has not employed or otherwise used in any capacity the services of any Person debarred under United States law, including under 21 USC §335a or any foreign equivalent thereof, with respect to Licensed Product;

(xiii) all research and development (including non-clinical studies and Clinical Trials) related to Licensed Product prior to the Effective Date has been conducted in accordance with all Laws; and

(xiv) the cGMP Inventory to be provided to Company in connection herewith was (and at all times up until delivery of such materials hereunder shall remain) stored and handled in accordance with cGMP and all Laws and specifications (including, to the extent applicable, release specifications as provided by Licensor to Company in writing prior to the Effective Date) during the period in which such cGMP Inventory was in the possession or control of Licensor. Such cGMP Inventory is not adulterated or misbranded within the meaning of any Law and is not articles that could not, under the provisions of Law, be introduced into interstate commerce. All such cGMP Inventory is free and clear of all encumbrances (including through lien, charge, security interest, mortgage, encumbrance or otherwise).

8.3 **Licensor Covenants**. Licensor covenants to Company that:

(i) Licensor shall fulfill all of its obligations, (other than any obligations that Licensor cannot fulfill itself without breaching its obligations, or infringing the license and rights granted, to Company pursuant to this Agreement), including but not limited to its payment obligations, under any Existing Third-Party Agreement or any Third-Party License Agreement;

(ii) Licensor shall not amend or waive, or take any action or omit to taking any action that would alter, any of Licensor's rights under any Existing Third-Party Agreement or any Third-Party License Agreement in any manner that adversely affects, or would reasonably be expected to adversely affect, Company's rights and benefits under this Agreement. Licensor shall promptly notify Company of any default under, termination or amendment of, any Existing Third-Party Agreement or Third-Party License Agreement; and

(iii) [***].

ARTICLE 9
INDEMNIFICATION AND INSURANCE

9.1 **Indemnification by Company**. Company shall indemnify, defend and hold harmless Licensor and its Affiliates and each of their respective agents, employees, officers and directors (the "**Licensor Indemnitees**") from and against any and all liability, damage, loss, cost or expense (including reasonable attorneys' fees) to the extent arising out of Third Party claims or suits related to: (a) Company's negligence or willful misconduct; (b) Company's exploitation, including Development or Commercialization, of Licensed Products; or (c) breach by Company of this Agreement; provided, however, that Company's obligations pursuant to this Section 9.1 shall not apply (i) to the extent such claims or suits result from the negligence or willful misconduct of any of the Licensor Indemnitees, or (ii) with respect to claims or suits arising out of breach by Licensor of this Agreement.

9.2 **Indemnification by Licensor**. Licensor shall indemnify, defend and hold harmless Company and its Affiliates and each of their respective agents, employees, officers and directors (the "**Company Indemnitees**") from and against any and all liability, damage, loss, cost or expense (including reasonable attorney's fees) to the extent arising out of Third Party claims or suits (including Actions) related to: (a) Licensor's negligence or willful misconduct; (b) Licensor's provision of the cGMP Inventory (provided, that Licensor's indemnification obligations with respect to the manufacture of such cGMP Inventory by the Third Party manufacturer shall be limited to such recoveries that Licensor is entitled to obtain from the Third Party manufacturer that manufactured such cGMP Inventory); or (c) breach by Licensor of this Agreement; provided, however, that Licensor's obligations pursuant to this Section 9.2 shall not apply (i) to the extent that such claims or suits result from the negligence or willful misconduct of any of Company Indemnitees or (ii) with respect to claims or suits arising out of a breach by Company of this Agreement.

9.3 **No Consequential Damages**. EXCEPT WITH RESPECT TO A PARTY'S GROSS NEGLIGENCE OR WILLFUL MISCONDUCT OR EACH PARTY'S INDEMNIFICATION OBLIGATIONS UNDER SECTION 9.1 OR SECTION 9.2, AS APPLICABLE, IN NO EVENT SHALL EITHER PARTY OR ANY OF ITS AFFILIATES BE LIABLE TO THE OTHER PARTY OR ANY OF ITS AFFILIATES FOR SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES, INCLUDING LOSS OF PROFITS, WHETHER IN CONTRACT, WARRANTY, TORT, NEGLIGENCE, STRICT LIABILITY OR OTHERWISE ARISING OUT OF OR RELATING TO THIS AGREEMENT, THE TRANSACTIONS CONTEMPLATED HEREIN OR ANY BREACH HEREOF. NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS AGREEMENT SHALL LIMIT EITHER PARTY FROM SEEKING OR OBTAINING ANY REMEDY AVAILABLE UNDER LAW FOR ANY BREACH OF BY THE OTHER PARTY OF ITS CONFIDENTIALITY AND NON-USE OBLIGATIONS UNDER ARTICLE 8.

9.4 **Notification of Claims; Conditions to Indemnification Obligations**. As a condition to a Party's right to receive indemnification under this ARTICLE 9, it shall: (a) promptly notify the other Party as soon as it becomes aware of a claim or suit for which indemnification may be sought pursuant hereto; (b) cooperate, and cause the individual indemnitees to cooperate, with the indemnifying Party in the defense, settlement or compromise of such claim or suit; and (c) permit the indemnifying Party to control the defense, settlement or compromise of such claim or suit, including the right to select defense counsel. In no event, however, may the indemnifying Party compromise or settle any claim or suit in a manner which admits fault or negligence on the part of the indemnified Party or any indemnitee without the prior written consent of the indemnified Party. Each Party shall reasonably cooperate with the other Party and its counsel in the course of the defense of any such suit, claim or demand, such cooperation to include without limitation using reasonable efforts to provide or make available documents, information and witnesses. The indemnifying Party shall have no liability under this ARTICLE 9 with respect to claims or suits settled or compromised without its prior written consent.

9.5 **Insurance**. During the Term, each Party shall obtain and maintain, at its sole cost and expense, insurance (including any self-insured arrangements) in types and amounts, that are reasonable and customary in the United States pharmaceutical and biotechnology industry for companies engaged in comparable activities. It is understood and agreed that this insurance shall not be construed to limit either Party's liability with respect to its indemnification obligations hereunder. Each Party will, except to the extent self-insured, provide to the other Party upon request a certificate evidencing the insurance such Party is required to obtain and keep in force under this Section 9.5.

ARTICLE 10
TERM AND TERMINATION

10.1 **Term and Expiration**. The term of this Agreement (the "**Term**") shall commence on the Effective Date and, unless earlier terminated as provided in this ARTICLE 10, shall continue in full force and effect, on a country-by-country and Licensed Product-by-Licensed Product basis until the date on which the Royalty Term in such country with respect to such Licensed Product expires, at which time this Agreement shall expire in its entirety with respect to such Licensed Product in such country and the terms of Section 10.4(b)(i) shall apply.

10.2 **Termination of the Agreement for Convenience**. At any time during the Term, Company may, at its convenience, upon [***] prior written notice to Licensor, terminate this Agreement in its entirety.

10.3 **Termination upon Material Breach**.

10.3.1 If a Party breaches any of its material obligations under this Agreement, the Party not in default may give to the breaching Party written notice specifying the nature of the default, requiring it to cure such breach, and stating its intention to terminate this Agreement if such breach is not cured within [***]. If such breach is not cured within [***] after the receipt of such notice, the Party not in default shall be entitled to terminate this Agreement immediately by written notice to the other Party. Any dispute regarding an alleged material breach of this Agreement shall be resolved in accordance with ARTICLE 11 hereof.

10.3.2 Either Party may terminate this Agreement if, at any time, (a) voluntary or involuntary proceedings by or against the other Party are instituted in bankruptcy under any insolvency Law, which proceedings, if involuntary, shall not have been dismissed within [***] after the date of

filing; (b) a receiver or custodian is appointed for the other Party; (c) proceedings are instituted by or against the other Party for corporate reorganization, dissolution, liquidation or winding-up of the other Party, which proceedings, if involuntary, shall not have been dismissed within [***] after the date of filing; or (d) substantially all of the assets of the other Party are seized or attached and not released within [***] thereafter (each individual event set forth in clauses (a) through (d), an "**Insolvency Event**").

10.4 **Effects of Termination**.

(a) **Survival**.

(i) Notwithstanding the expiration or termination of this Agreement, the following provisions shall survive: Articles 1, 4, 7 and 12; and Sections 2.7(a)-(c), 5.10, 5.11, 6.3(d), 9.1, 9.2, 9.3, 9.4, 10.4, 10.5 and 10.6.

(ii) Expiration or termination of this Agreement shall not relieve the Parties of any liability that accrued hereunder prior to the effective date of such termination. In addition, except for the termination events addressed in Section 10.3, termination of this Agreement shall not preclude either Party from pursuing all rights and remedies it may have hereunder or at Law or in equity with respect to any breach of this Agreement nor prejudice either Party's right to obtain performance of any obligation.

(b) **Licenses**.

(i) As of the effective date of expiration of the Royalty Term with respect to a given Licensed Product and country, the license from Licensor to Company under Section 2.1 shall convert to a fully paid, royalty free, irrevocable, perpetual, exclusive, and sublicensable license under the Licensed Technology to research, Develop, manufacture, have manufactured, use and Commercialize such Licensed Product in the Field in such country.

(ii) Upon termination of this Agreement by Company pursuant to Section 10.2 or by Licensor pursuant to Section 10.3, the following terms and conditions shall apply:

(1) all licenses granted to Company under Section 2.1 shall terminate;

(2) Company shall, upon written request by Licensor and subject to Licensor assuming legal responsibility for any Clinical Trials of such Licensed Product(s) then ongoing, transfer to Licensor all regulatory documentation and Regulatory Approvals prepared or obtained by or on behalf of Company prior to the date of such termination, solely to the extent (A) Controlled by Company as of the effective date of termination and (B) related to such Licensed Product(s) and country(ies) and transferable, and Company shall have the right to retain one copy of such transferred documentation and Regulatory Approvals for solely record-keeping purposes;

(3) Company shall, upon written request of Licensor, return to Licensor or, at Licensor's option, destroy, all relevant records and materials in its possession or control containing or comprising the Licensed Know-How and the Licensed Materials, or such other Confidential Information of Licensor; provided, however, that Company shall have the right to retain one sample of Licensed Materials for regulatory purposes and one copy of such Licensed Know-How and such other Confidential Information of Licensor solely for record-keeping purposes;

(4) To the extent not prohibited by Law, Company shall wind down any ongoing Clinical Trials with respect to such Licensed Product(s), or at Licensor's option, transfer such Clinical Trials to Licensor at Licensor's cost and expense;

(5) Licensor shall have the right, but not the obligation, to purchase any and all of the inventory of Licensed Product held by Company as of the date of termination, at a price equal to the cost of goods of such inventory, together with any applicable external costs of transportation, storage and insurance, and import and export taxes and fees, plus [***]. If Licensor does not elect to purchase such inventory from Company, Company shall have the right to continue to sell such inventory in the Territory for [***] after the date of termination of this Agreement, subject to Company's continued payment of royalties on Net Sales of Licensed Product during such period; and

(6) Upon Licensor's written request, Company and Licensor may enter into a negotiation for Company to license to Licensor, (A) Know-How Controlled by Company, its Affiliates, or Sublicensees as of the effective date of termination of this Agreement that has been generated by or on behalf of Company, its Affiliates or Sublicensees that is specific solely to the Licensed Product, (B) any inventions generated by or on behalf of Company, its Affiliates or Sublicensees that is specific to the Licensed Product, and (C) Patents Controlled by Company or its Affiliates that Cover the Licensed Product in the Territory, in each case that are necessary or reasonably useful to enable Licensor to Develop and Commercialize Licensed Product (collectively, the "**Company IP**"). The Parties shall negotiate the terms of such license in good faith for a period not to exceed [***].

(iii) Upon termination of this Agreement by Company pursuant to Section 10.3, the following terms and conditions shall apply with respect to such Licensed Product(s) and country(ies) as are the subject of such termination:

(1) all licenses granted to Company under Section 2.1 shall terminate;

(2) Company shall, upon written request of Licensor, return to Licensor or, at Licensor's option and cost and expense, destroy, all relevant records and materials in its possession or control containing or comprising the Licensed Know-How and the Licensed Materials, or such other Confidential Information of Licensor; provided, however, that Company shall have the right to retain one copy of such Licensed Know-How and such other Confidential Information of Licensor solely for record-keeping purposes;

(3) To the extent not prohibited by Law, Company shall, at Licensor's sole cost and expense, wind down any ongoing Clinical Trials with respect to such Licensed Product(s), or at Licensor's option, transfer such Clinical Trials to Licensor; and

(4) Company shall have the right to continue to sell in the Territory any inventory of Licensed Products for [***] after the date of termination of this Agreement, without any obligation to continue payment of royalties on Net Sales of Licensed Product during such period (provided, that Company shall be obligated to pay any and all amounts payable to University by Licensor pursuant to Section 3.4 of the UNC Agreement as a result of such sale).

(iv) Upon any termination of this Agreement and subject, to the extent applicable, to compliance with Section 6.5 of the UNC Agreement, Company, and each of Company's Sublicensees, shall continue to have the rights set forth in Section 6.5 of the UNC Agreement; provided, however, that such Sublicensee is not then in breach of any of its material obligations under its sublicense agreements.

(v) Immediately following Company's notification of termination to Licensor pursuant to Sections 10.2 or 10.3, the diligence obligations in Section 3.5 shall no longer apply and Company shall have the right to wind-down all then on-going Development, manufacturing and/or Commercialization activities.

10.5 **Termination on Bankruptcy or Insolvency**. All rights and licenses granted under or pursuant to this Agreement by Licensor are, and shall otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code, if applicable, licenses of right to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The Parties agree that Company, as licensor of such rights under this Agreement, shall retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code. The Parties further agree that, in the event of an Insolvency Event with respect to Licensor, Company shall be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property, which, if not already in Company's possession, shall be promptly delivered to it (i) following any such commencement of a bankruptcy proceeding upon Company's written request therefor, unless Licensor elects to continue to perform all of its obligations under this Agreement or (ii) if not delivered under clause (a), following the rejection of this Agreement by Licensor upon written request therefor by Company.

10.6 **Other Remedies**. Termination of this Agreement for any reason shall not release either Party from any liability or obligation that already has accrued prior to such termination. Termination of this Agreement for any reason shall not constitute a waiver or release of, or otherwise be deemed to prejudice or adversely affect or limit, any rights or remedies that otherwise may be available at Law or in equity.

ARTICLE 11
DISPUTE RESOLUTION

11.1 **Escalation to Executive Officers**. Either Party may, by written notice to the other Party, request that a dispute arising between the Parties in connection with this Agreement, or a dispute relating to material breach, be resolved by the Executive Officers, within [***] after referral of such dispute to them. If the Executive Officers cannot resolve such dispute within [***] after referral of such dispute to them, then, at any time after such [***] period, either Party may proceed to enforce any and all of its rights with respect to such dispute.

11.2 **Injunctive Relief**. Subject to Section 10.3, no provision herein shall be construed as precluding a Party from bringing an action for injunctive relief or other equitable relief prior to the initiation or completion of the above procedure.

ARTICLE 12
MISCELLANEOUS PROVISIONS

12.1 **Relationship of the Parties**. Nothing in this Agreement is intended or shall be deemed, for financial, tax, legal or other purposes, to constitute a partnership, agency, joint venture or employer-employee relationship between the Parties.

12.2 **Assignment**. Except as expressly provided herein, neither this Agreement nor any interest hereunder shall be assignable, nor any other obligation delegable, by a Party without the prior written consent of the other Party, not to be unreasonably withheld or delayed; provided, however, that either Party may assign this Agreement, in whole or in part, without the consent of the other Party to (a) any Affiliate or (b) a Third Party in connection with a merger, acquisition of all or substantially all of the business or assets of such Party to which this Agreement relates, consolidation, change of control or other similar transaction. This Agreement shall be binding upon the successors and permitted assigns of the Parties. Any assignment not in accordance with this Section 12.2 shall be void. Licensor shall have the right to assign or transfer all, but not less than all, Licensed Technology to an Affiliate without the prior written consent of Company solely to the extent that Licensor also assigns and transfers this Agreement to such Affiliate.

12.3 **Performance and Exercise by Affiliates**. Company shall have the right to have any of its obligations hereunder performed, or its rights hereunder exercised, by, any of its Affiliates and the performance of such obligations by any such Affiliate shall be deemed to be performance by Company; provided, however, that Company shall be responsible for ensuring the performance of its obligations under this Agreement and that any failure of any Affiliate performing obligations of Company hereunder shall be deemed to be a failure by Company to perform such obligations. For clarity, the foregoing means that Company may designate an Affiliate to perform its obligations hereunder or to be the recipient of Licensor's performance obligations hereunder.

12.4 **Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments and to do all such other acts as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

12.5 **Force Majeure**. Neither Party shall be liable to the other Party or be deemed to have breached or defaulted under this Agreement for failure or delay in the performance of any of its obligations under this Agreement for the time and to the extent such failure or delay is caused by or results from acts of God, pandemic, earthquake, riot, civil commotion, terrorism, war, strikes or other labor disputes, fire, flood, failure or delay of transportation, omissions or delays in acting by a governmental authority, acts of a government or an agency thereof or judicial orders or decrees or restrictions or any other reason which is beyond the control of the respective Party. The Party affected by force majeure shall provide the other Party with full particulars thereof as soon as it becomes aware of the same (including its best estimate of the likely extent and duration of the interference with its activities), and will use Commercially Reasonable Efforts to overcome the difficulties created thereby and to resume performance of its obligations hereunder as soon as practicable.

12.6 **No Trademark Rights**. No right, express or implied, is granted by this Agreement to a Party to use in any manner the name or any other trade name or trademark of the other Party in connection with the performance of this Agreement or otherwise.

12.7 **Entire Agreement of the Parties; Amendments**. This Agreement and the Schedules hereto constitute and contain the entire understanding and agreement of the Parties respecting the subject matter hereof and cancel and supersede any and all prior negotiations, correspondence, understandings and agreements between the Parties, whether oral or written, regarding such subject matter. No waiver, modification or amendment of any provision of this Agreement shall be valid or effective unless made in a writing referencing this Agreement and signed by a duly authorized officer of each Party.

12.8 **Captions**. The captions to this Agreement are for convenience only, and are to be of no force or effect in construing or interpreting any of the provisions of this Agreement.

12.9 **Governing Law**. This Agreement shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding application of any conflict of laws principles that would require application of the Law of a jurisdiction outside of the State of Delaware, and will be subject to the exclusive jurisdiction of the courts of competent jurisdiction located in the State of Delaware.

12.10 **Notices and Deliveries**. Any notice, request, approval or consent required or permitted to be given under this Agreement shall be in writing and shall be deemed to have been sufficiently given if delivered in person, transmitted by facsimile (receipt verified) or by express courier service (signature required) to the Party to which it is directed at its address or facsimile number shown below or such other address or facsimile number as such Party shall have last given by notice to the other Party.

If to Company, addressed to:

Taysha Gene Therapies
[***]

With a copy, which shall not constitute notice, to:

[***]

If to Licensor, addressed to:

Abeona Therapeutics Inc.
[***]

12.11 **Waiver**. A waiver by either Party of any of the terms and conditions of this Agreement in any instance shall not be deemed or construed to be a waiver of such term or condition for the future, or of any other term or condition hereof. All rights, remedies, undertakings, obligations and agreements contained in this Agreement shall be cumulative and none of them shall be in limitation of any other remedy, right, undertaking, obligation or agreement of either Party.

12.12 **Severability**. When possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under Law, but if any provision of this Agreement is held to be prohibited by or invalid under Law, such provision will be ineffective only to the extent of such prohibition or invalidity, without invalidating the remainder of this Agreement. The Parties shall make a good faith effort to replace the invalid or unenforceable provision with a valid one which in its economic effect is most consistent with the invalid or unenforceable provision.

12.13 **No Implied License**. No right or license is granted to Licensor hereunder by implication, estoppel, or otherwise to any know-how, patent or other intellectual property right owned or controlled by Company or its Affiliates.

12.14 **Interpretation**. The words “include,” “includes” and “including” shall be deemed to be followed by the phrase “without limitation.” All references herein to Articles, Sections, and Schedules shall be deemed references to Articles and Sections of, and Schedules to, this Agreement unless the context shall otherwise require. Unless the context otherwise requires, countries shall include territories.

12.15 **Counterparts**. This Agreement may be executed in counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. A facsimile or a portable document format (PDF) copy of this Agreement, including the signature pages, will be deemed an original.

[**SIGNATURE PAGE FOLLOWS**]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have executed this Agreement as of the date first above written.

ABEONA THERAPEUTICS INC.

Signature: */s/ João Siffert*
Printed Name: João Siffert, M.D.
Title: Chief Executive Officer

TAYSHA GENE THERAPIES, INC.

Signature: */s/ RA Session II*
Printed Name: RA Session II
Title: President & CEO

[Signature Page to License Agreement]

TAYSHA GENE THERAPIES, INC.

2020 EQUITY INCENTIVE PLAN

ADOPTED BY THE BOARD OF DIRECTORS: July 1, 2020
APPROVED BY THE STOCKHOLDERS: July 1, 2020
TERMINATION DATE: July 1, 2030

1. General.

(a) Eligible Stock Award Recipients. Employees, Directors and Consultants are eligible to receive Stock Awards.

(b) Available Stock Awards. The Plan provides for the grant of the following types of Stock Awards: (i) Incentive Stock Options, (ii) Nonstatutory Stock Options, (iii) Stock Appreciation Rights, (iv) Restricted Stock Awards, (v) Restricted Stock Unit Awards and (vi) Other Stock Awards.

(c) Purpose. The Plan, through the grant of Stock Awards, is intended to help the Company secure and retain the services of eligible award recipients, provide incentives for such persons to exert maximum efforts for the success of the Company and any Affiliate and provide a means by which the eligible recipients may benefit from increases in value of the Common Stock.

2. Administration.

(a) Administration by the Board. The Board will administer the Plan. The Board may delegate administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) Powers of the Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine (A) who will be granted Stock Awards; (B) when and how each Stock Award will be granted; (C) what type of Stock Award will be granted; (D) the provisions of each Stock Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Stock Award; (E) the number of shares of Common Stock subject to, or the cash value of, a Stock Award; and (F) the Fair Market Value applicable to a Stock Award.

(ii) To construe and interpret the Plan and Stock Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Stock Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Stock Award Agreement, in a manner and to the extent it will deem necessary or expedient to make the Plan or Stock Award fully effective.

(iii) To settle all controversies regarding the Plan and Stock Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which a Stock Award may be exercised or vest (or the time at which cash or shares of Common Stock may be issued in settlement thereof).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or a Stock Award Agreement, suspension or termination of the Plan will not impair a Participant's rights under the Participant's then-outstanding Stock Award without the Participant's written consent except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, by adopting amendments relating to Incentive Stock Options and certain nonqualified deferred compensation under Section 409A of the Code and/or bringing the Plan or Stock Awards granted under the Plan into compliance with the requirements for Incentive Stock Options or ensuring that they are exempt from, or compliant with, the requirements for nonqualified deferred compensation under Section 409A of the Code, subject to the limitations, if any, of applicable law. If required by applicable law or listing requirements, and except as provided in Section 9(a) relating to Capitalization Adjustments, the Company will seek stockholder approval of any amendment of the Plan that (A) materially increases the number of shares of Common Stock available for issuance under the Plan, (B) materially expands the class of individuals eligible to receive Stock Awards under the Plan, (C) materially increases the benefits accruing to Participants under the Plan, (D) materially reduces the price at which shares of Common Stock may be issued or purchased under the Plan, (E) materially extends the term of the Plan, or (F) materially expands the types of Stock Awards available for issuance under the Plan. Except as otherwise provided in the Plan or a Stock Award Agreement, no amendment of the Plan will materially impair a Participant's rights under an outstanding Stock Award without the Participant's written consent.

(vii) To submit any amendment to the Plan for stockholder approval, including, but not limited to, amendments to the Plan intended to satisfy the requirements of Section 422 of the Code regarding Incentive Stock Options.

(viii) To approve forms of Stock Award Agreements for use under the Plan and to amend the terms of any one or more Stock Awards, including, but not limited to, amendments to provide terms more favorable to the Participant than previously provided in the Stock Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion; *provided however,* that a Participant's rights under any Stock Award will not be impaired by any such amendment unless (A) the Company requests the consent of the affected Participant, and (B) such Participant consents in writing. Notwithstanding the foregoing, (1) a Participant's rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant's rights, and (2) subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Stock Awards without the affected Participant's consent (A) to maintain the qualified status of the Stock Award as an Incentive Stock Option under Section 422 of the Code; (B) to change the terms of an Incentive Stock Option, if such change results in impairment of the Stock Award solely because it impairs the qualified status of the Stock Award as an Incentive Stock Option under Section 422 of the Code; (C) to clarify the manner of exemption from, or to bring the Stock Award into compliance with, Section 409A of the Code; or (D) to comply with other applicable laws.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan or Stock Awards.

(x) To adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Employees, Directors or Consultants who are foreign nationals or employed

outside the United States (provided that Board approval will not be necessary for immaterial modifications to the Plan or any Stock Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction).

(xi) To effect, with the consent of any adversely affected Participant, (A) the reduction of the exercise, purchase or strike price of any outstanding Stock Award; (B) the cancellation of any outstanding Stock Award and the grant in substitution therefor of a new (1) Option or SAR, (2) Restricted Stock Award, (3) Restricted Stock Unit Award, (4) Other Stock Award, (5) cash and/or (6) other valuable consideration determined by the Board, in its sole discretion, with any such substituted award (x) covering the same or a different number of shares of Common Stock as the cancelled Stock Award and (y) granted under the Plan or another equity or compensatory plan of the Company; or (C) any other action that is treated as a repricing under generally accepted accounting principles.

(c) Delegation to Committee. The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee, as applicable). Any delegation of administrative powers will be reflected in resolutions, not inconsistent with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(d) Delegation to an Officer. The Board may delegate to one or more Officers the authority to do one or both of the following: (i) designate Employees who are not Officers to be recipients of Options and SARs (and, to the extent permitted by applicable law, other Stock Awards) and, to the extent permitted by applicable law, the terms of such Stock Awards, and (ii) determine the number of shares of Common Stock to be subject to such Stock Awards granted to such Employees; *provided*, *however*, that the Board resolutions regarding such delegation will specify the total number of shares of Common Stock that may be subject to the Stock Awards granted by such Officer and that such Officer may not grant a Stock Award to himself or herself. Any such Stock Awards will be granted on the form of Stock Award Agreement most recently approved for use by the Committee or the Board, unless otherwise provided in the resolutions approving the delegation authority. The Board may not delegate authority to an Officer who is acting solely in the capacity of an Officer (and not also as a Director) to determine the Fair Market Value pursuant to Section 13(t) below.

(e) Effect of Board's Decision. All determinations, interpretations and constructions made by the Board in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

3. Shares Subject to the Plan.

(a) Share Reserve.

(i) Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards from and after the Effective Date will not exceed 3,529,412 shares (the ***"Share Reserve"***).

(ii) For clarity, the Share Reserve in this Section 3(a) is a limitation on the number of shares of Common Stock that may be issued pursuant to the Plan. Accordingly, this Section 3(a) does not limit the granting of Stock Awards except as provided in Section 7(a).

(b) Reversion of Shares to the Share Reserve. If a Stock Award or any portion thereof (i) expires or otherwise terminates without all of the shares covered by such Stock Award having been issued or (ii) is settled in cash (*i.e.*, the Participant receives cash rather than stock), such expiration, termination or settlement will not reduce (or otherwise offset) the number of shares of Common Stock that may be available for issuance under the Plan. If any shares of Common Stock issued pursuant to a Stock Award are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required to vest such shares in the Participant, then the shares that are forfeited or repurchased will revert to and again become available for issuance under the Plan. Any shares reacquired by the Company in satisfaction of tax withholding obligations on a Stock Award or as consideration for the exercise or purchase price of a Stock Award will again become available for issuance under the Plan.

(c) Incentive Stock Option Limit. Subject to the Share Reserve and Section 9(a) relating to Capitalization Adjustments, the aggregate maximum number of shares of Common Stock that may be issued pursuant to the exercise of Incentive Stock Options will be a number of shares of Common Stock equal to three multiplied by the Share Reserve.

(d) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise.

4. Eligibility.

(a) Eligibility for Specific Stock Awards. Incentive Stock Options may be granted only to employees of the Company or a "parent corporation" or "subsidiary corporation" thereof (as such terms are defined in Sections 424(e) and 424(f) of the Code). Stock Awards other than Incentive Stock Options may be granted to Employees, Directors and Consultants; *provided*, *however*, that Stock Awards may not be granted to Employees, Directors and Consultants who are providing Continuous Service only to any "parent" of the Company, as such term is defined in Rule 405, unless (i) the stock underlying such Stock Awards is treated as "service recipient stock" under Section 409A of the Code (for example, because the Stock Awards are granted pursuant to a corporate transaction such as a spin off transaction), (ii) the Company, in consultation with its legal counsel, has determined that such Stock Awards are otherwise exempt from Section 409A of the Code, or (iii) the Company, in consultation with its legal counsel, has determined that such Stock Awards comply with the distribution requirements of Section 409A of the Code.

(b) Ten Percent Stockholders. A Ten Percent Stockholder will not be granted an Incentive Stock Option unless the exercise price of such Option is at least 110% of the Fair Market Value on the date of grant and the Option is not exercisable after the expiration of five years from the date of grant.

(c) Consultants. A Consultant will not be eligible for the grant of a Stock Award if, at the time of grant, either the offer or sale of the Company's securities to such Consultant is not exempt under Rule 701 because of the nature of the services that the Consultant is providing to the Company, because the Consultant is not a natural person, or because of any other provision of Rule 701, unless the Company determines that such grant need not comply with the requirements of Rule 701 and will satisfy another exemption under the Securities Act as well as comply with the securities laws of all other relevant jurisdictions.

5. Provisions Relating to Options and Stock Appreciation Rights.

Each Option or SAR will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be separately designated Incentive Stock Options or Nonstatutory Stock Options at the time of grant, and, if certificates are issued, a separate certificate or certificates will be issued for shares of Common Stock purchased on exercise of each type of Option. If an Option is not specifically designated as an Incentive Stock Option, or if an Option is designated as an Incentive Stock Option but some portion or all of the Option fails to qualify as an Incentive Stock Option under the applicable rules, then the Option (or portion thereof) will be a Nonstatutory Stock Option. The provisions of separate Options or SARs need not be identical; *provided*, *however*, that each Stock Award Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Stock Award Agreement or otherwise) the substance of each of the following provisions:

(a) Term. Subject to the provisions of Section 4(b) regarding Ten Percent Stockholders, no Option or SAR will be exercisable after the expiration of 10 years from the date of its grant or such shorter period specified in the Stock Award Agreement.

(b) Exercise Price. Subject to the provisions of Section 4(b) regarding Ten Percent Stockholders, the exercise or strike price of each Option or SAR will be not less than 100% of the Fair Market Value of the Common Stock subject to the Option or SAR on the date the Stock Award is granted. Notwithstanding the foregoing, an Option or SAR may be granted with an exercise or strike price lower than 100% of the Fair Market Value of the Common Stock subject to the Stock Award if such Stock Award is granted pursuant to an assumption of or substitution for another option or stock appreciation right pursuant to a Corporate Transaction and in a manner consistent with the provisions of Section 409A of the Code and, if applicable, Section 424(a) of the Code. Each SAR will be denominated in shares of Common Stock equivalents.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

(i) by cash, check, bank draft, electronic funds transfer or money order payable to the Company;

(ii) subject to Company and/or Board consent at the time of exercise and provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a “broker-assisted exercise”, “same day sale”, or “sell to cover”;

(iii) subject to Company and/or Board consent at the time of exercise and provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by

actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "Delivery" for these purposes, in the sole discretion of the Company and/or the Board, at the time Participant exercises their Option, will include delivery to the Company of Participant's attestation of ownership of such shares of Common Stock in a form approved by the Company. Participant may not exercise their option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock;

(iv) subject to Company and/or Board consent at the time of exercise, and provided that the Option is a Nonstatutory Stock Option, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of the Option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price plus, to the extent permitted by the Company and/or Board at the time of exercise, the aggregate withholding obligations in respect of the Option exercise; provided, further that Participant must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be subject to the Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are used to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations;

(v) according to a deferred payment or similar arrangement with the Optionholder; *provided*, *however*, that interest will compound at least annually and will be charged at the minimum rate of interest necessary to avoid (A) the imputation of interest income to the Company and compensation income to the Optionholder under any applicable provisions of the Code, and (B) the classification of the Option as a liability for financial accounting purposes; or

(vi) in any other form of legal consideration that may be acceptable to the Board.

(d) Exercise and Payment of a SAR. To exercise any outstanding SAR, the Participant must provide written notice of exercise to the Company in compliance with the provisions of the Stock Appreciation Right Agreement evidencing such SAR. The appreciation distribution payable on the exercise of a SAR will be not greater than an amount equal to the excess of (A) the aggregate Fair Market Value (on the date of the exercise of the SAR) of a number of shares of Common Stock equal to the number of Common Stock equivalents in which the Participant is vested under such SAR, and with respect to which the Participant is exercising the SAR on such date, over (B) the aggregate strike price of the number of Common Stock equivalents with respect to which the Participant is exercising the SAR on such date. The appreciation distribution may be paid in Common Stock, in cash, in any combination of the two or in any other form of consideration, as determined by the Board and contained in the Stock Award Agreement evidencing such SAR.

(e) Transferability of Options and SARs. The Board may, in its sole discretion, impose such limitations on the transferability of Options and SARs as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options and SARs will apply:

(i) Restrictions on Transfer. An Option or SAR will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option or SAR in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided in the Plan, neither an Option nor a SAR may be transferred for consideration.

(ii) Domestic Relations Orders. Subject to the approval of the Board or a duly authorized Officer, an Option or SAR may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument as permitted by Treasury Regulation 1.421-1(b)(2). If an Option is an Incentive Stock Option, such Option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

(iii) Beneficiary Designation. Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, upon the death of the Participant, will thereafter be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, upon the death of the Participant, the executor or administrator of the Participant's estate will be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. However, the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.

(f) Vesting Generally. The total number of shares of Common Stock subject to an Option or SAR may vest and therefore become exercisable in periodic installments that may or may not be equal. The Option or SAR may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of performance goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options or SARs may vary. The provisions of this Section 5(f) are subject to any Option or SAR provisions governing the minimum number of shares of Common Stock as to which an Option or SAR may be exercised.

(g) Termination of Continuous Service. Except as otherwise provided in the applicable Stock Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates (other than for Cause and other than upon the Participant's death or Disability), the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Stock Award as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date three months following the termination of the Participant's Continuous Service (or such longer or shorter period specified in the applicable Stock Award Agreement, which period will not be less than 30 days if necessary to comply with applicable laws unless such termination is for Cause) and (ii) the expiration of the term of the Option or SAR as set forth in the Stock Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR (as applicable) within the applicable time frame, the Option or SAR will terminate.

(h) Extension of Termination Date. If the exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause and other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option or SAR will terminate on the earlier of (i) the expiration of a total period of time (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the exercise of the Option or SAR would not be in violation of such registration requirements, and (ii) the expiration of the term of the Option or SAR as set forth in the applicable Stock Award Agreement. In addition, unless otherwise provided in a Participant's

Stock Award Agreement, if the sale of any Common Stock received upon exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause) would violate the Company's insider trading policy, then the Option or SAR will terminate on the earlier of (i) the expiration of the period of time (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would not be in violation of the Company's insider trading policy, and (ii) the expiration of the term of the Option or SAR as set forth in the applicable Stock Award Agreement.

(i) Disability of Participant. Except as otherwise provided in the applicable Stock Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates as a result of the Participant's Disability, the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Option or SAR as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date 12 months following such termination of Continuous Service (or such longer or shorter period specified in the Stock Award Agreement, which period will not be less than six months if necessary to comply with applicable laws unless such termination is for Cause), and (ii) the expiration of the term of the Option or SAR as set forth in the Stock Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR (as applicable) will terminate.

(j) Death of Participant. Except as otherwise provided in the applicable Stock Award Agreement or other agreement between the Participant and the Company, if (i) a Participant's Continuous Service terminates as a result of the Participant's death, or (ii) the Participant dies within the period (if any) specified in the Stock Award Agreement for exercisability after the termination of the Participant's Continuous Service (for a reason other than death), then the Option or SAR may be exercised (to the extent the Participant was entitled to exercise such Option or SAR as of the date of death) by the Participant's estate, by a person who acquired the right to exercise the Option or SAR by bequest or inheritance or by a person designated to exercise the Option or SAR upon the Participant's death, but only within the period ending on the earlier of (i) the date 18 months following the date of death (or such longer or shorter period specified in the Stock Award Agreement, which period will not be less than six months if necessary to comply with applicable laws unless such termination is for Cause), and (ii) the expiration of the term of such Option or SAR as set forth in the Stock Award Agreement. If, after the Participant's death, the Option or SAR is not exercised within the applicable time frame, the Option or SAR (as applicable) will terminate.

(k) Termination for Cause. Except as explicitly provided otherwise in a Participant's Stock Award Agreement or other individual written agreement between the Company or any Affiliate and the Participant, if a Participant's Continuous Service is terminated for Cause, the Option or SAR will terminate immediately upon such Participant's termination of Continuous Service, and the Participant will be prohibited from exercising his or her Option or SAR (whether vested or unvested) from and after the date of such termination of Continuous Service.

(l) Non-Exempt Employees. If an Option or SAR is granted to an Employee who is a non-exempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option or SAR will not be first exercisable for any shares of Common Stock until at least six months following the date of grant of the Option or SAR (although the Stock Award may vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option or SAR is not assumed, continued,

or substituted, (iii) upon a Change in Control, or (iv) upon the Participant's retirement (as such term may be defined in the Participant's Stock Award Agreement, in another agreement between the Participant and the Company, or, if no such definition, in accordance with the Company's then current employment policies and guidelines), the vested portion of any Options and SARs may be exercised earlier than six months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option or SAR will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt employee in connection with the exercise, vesting or issuance of any shares under any other Stock Award will be exempt from the employee's regular rate of pay, the provisions of this Section 5(l) will apply to all Stock Awards and are hereby incorporated by reference into such Stock Award Agreements.

(m) Early Exercise of Options. An Option may, but need not, include a provision whereby the Optionholder may elect at any time before the Optionholder's Continuous Service terminates to exercise the Option as to any part or all of the shares of Common Stock subject to the Option prior to the full vesting of the Option. Subject to the "Repurchase Limitation" in Section 8(l), any unvested shares of Common Stock so purchased may be subject to a repurchase right in favor of the Company or to any other restriction the Board determines to be appropriate. Provided that the "Repurchase Limitation" in Section 8(l) is not violated, the Company will not be required to exercise its repurchase right until at least six months (or such longer or shorter period of time required to avoid classification of the Option as a liability for financial accounting purposes) have elapsed following exercise of the Option unless the Board otherwise specifically provides in the Option Agreement.

(n) Right of Repurchase. Subject to the "Repurchase Limitation" in Section 8(l), the Option or SAR may include a provision whereby the Company may elect to repurchase all or any part of the vested shares of Common Stock acquired by the Participant pursuant to the exercise of the Option or SAR.

(o) Right of First Refusal. The Option or SAR may include a provision whereby the Company may elect to exercise a right of first refusal following receipt of notice from the Participant of the intent to transfer all or any part of the shares of Common Stock received upon the exercise of the Option or SAR. Such right of first refusal will be subject to the "Repurchase Limitation" in Section 8(l). Except as expressly provided in this Section 5(o) or in the Stock Award Agreement, such right of first refusal will otherwise comply with any applicable provisions of the bylaws of the Company.

6. Provisions of Stock Awards Other than Options and SARs.

(a) Restricted Stock Awards. Each Restricted Stock Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. To the extent consistent with the Company's bylaws, at the Board's election, shares of Common Stock underlying a Restricted Stock Award may be (i) held in book entry form subject to the Company's instructions until any restrictions relating to the Restricted Stock Award lapse; or (ii) evidenced by a certificate, which certificate will be held in such form and manner as determined by the Board. The terms and conditions of Restricted Stock Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Award Agreements need not be identical. Each Restricted Stock Award Agreement will conform to (through incorporation of the provisions hereof by reference in the agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. A Restricted Stock Award may be awarded in consideration for (A) cash, check, bank draft or money order payable to the Company, (B) past services to the Company or an Affiliate, or (C) any other form of legal consideration (including future services) that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. Subject to the "Repurchase Limitation" in Section 8(l), shares of Common Stock awarded under the Restricted Stock Award Agreement may be subject to forfeiture to the Company in accordance with a vesting schedule to be determined by the Board.

(iii) Termination of Participant's Continuous Service. If a Participant's Continuous Service terminates, the Company may receive through a forfeiture condition or a repurchase right, any or all of the shares of Common Stock held by the Participant that have not vested as of the date of termination of Continuous Service under the terms of the Restricted Stock Award Agreement.

(iv) Transferability. Rights to acquire shares of Common Stock under the Restricted Stock Award Agreement will be transferable by the Participant only upon such terms and conditions as are set forth in the Restricted Stock Award Agreement, as the Board will determine in its sole discretion, so long as Common Stock awarded under the Restricted Stock Award Agreement remains subject to the terms of the Restricted Stock Award Agreement.

(v) Dividends. A Restricted Stock Award Agreement may provide that any dividends paid on Restricted Stock will be subject to the same vesting and forfeiture restrictions as apply to the shares subject to the Restricted Stock Award to which they relate.

(b) Restricted Stock Unit Awards. Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(i) Consideration. At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) Vesting. At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(iii) Payment. A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(iv) Additional Restrictions. At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(v) Dividend Equivalents. Dividend equivalents may be credited in respect of shares of Common Stock covered by a Restricted Stock Unit Award, as determined by the Board and contained in the Restricted Stock Unit Award Agreement. At the sole discretion of the Board, such dividend equivalents may be converted into additional shares of Common Stock covered by the Restricted Stock Unit Award in such manner as determined by the Board. Any additional shares covered by the Restricted Stock Unit Award credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying Restricted Stock Unit Award Agreement to which they relate.

(vi) Termination of Participant's Continuous Service. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant's termination of Continuous Service.

(vii) Compliance with Section 409A of the Code. Notwithstanding anything to the contrary set forth herein, any Restricted Stock Unit Award granted under the Plan that is not exempt from the requirements of Section 409A of the Code will contain such provisions so that such Restricted Stock Unit Award will comply with the requirements of Section 409A of the Code. Such restrictions, if any, will be determined by the Board and contained in the Restricted Stock Unit Award Agreement evidencing such Restricted Stock Unit Award. For example, such restrictions may include, without limitation, a requirement that any Common Stock that is to be issued in a year following the year in which the Restricted Stock Unit Award vests must be issued in accordance with a fixed pre-determined schedule.

(c) Other Stock Awards. Other forms of Stock Awards valued in whole or in part by reference to, or otherwise based on, Common Stock, including the appreciation in value thereof (e.g., options or stock rights with an exercise price or strike price less than 100% of the Fair Market Value of the Common Stock at the time of grant) may be granted either alone or in addition to Stock Awards provided for under Section 5 and the preceding provisions of this Section 6. Subject to the provisions of the Plan, the Board will have sole and complete authority to determine the persons to whom and the time or times at which such Other Stock Awards will be granted, the number of shares of Common Stock (or the cash equivalent thereof) to be granted pursuant to such Other Stock Awards and all other terms and conditions of such Other Stock Awards.

7. Covenants of the Company.

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy then-outstanding Stock Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise of the Stock Awards; *provided, however,* that this undertaking will not require the Company to register under the Securities Act the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise of such Stock Awards unless and until such

authority is obtained. A Participant will not be eligible for the grant of a Stock Award or the subsequent issuance of cash or Common Stock pursuant to the Stock Award if such grant or issuance would be in violation of any applicable securities law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the time or manner of exercising such Stock Award. Furthermore, the Company will have no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of a Stock Award or a possible period in which the Stock Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of a Stock Award to the holder of such Stock Award.

8. Miscellaneous.

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Stock Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Stock Awards. Corporate action constituting a grant by the Company of a Stock Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Stock Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Stock Award Agreement or related grant documents as a result of a clerical error in the papering of the Stock Award Agreement or related grant documents, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Stock Award Agreement or related grant documents.

(c) Stockholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to a Stock Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares of Common Stock under, the Stock Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to the Stock Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Stock Award Agreement or any other instrument executed thereunder or in connection with any Stock Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Stock Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, (ii) the service of a Consultant pursuant to the terms of such Consultant's agreement with the Company or an Affiliate, or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state in which the Company or the Affiliate is incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant's regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee or takes an extended leave of absence) after the date of grant of any Stock Award to the Participant, the Board has the right in its sole discretion to (x) make a corresponding reduction in the number of shares subject to any portion of such

Stock Award that is scheduled to vest or become payable after the date of such change in time commitment, and (y) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Stock Award. In the event of any such reduction, the Participant will have no right with respect to any portion of the Stock Award that is so reduced or extended.

(f) Incentive Stock Option Limitations. To the extent that the aggregate Fair Market Value (determined at the time of grant) of Common Stock with respect to which Incentive Stock Options are exercisable for the first time by any Optionholder during any calendar year (under all plans of the Company and any Affiliates) exceeds $100,000 (or such other limit established in the Code) or otherwise does not comply with the rules governing Incentive Stock Options, the Options or portions thereof that exceed such limit (according to the order in which they were granted) or otherwise do not comply with such rules will be treated as Nonstatutory Stock Options, notwithstanding any contrary provision of the applicable Option Agreement(s).

(g) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Stock Award, (i) to give written assurances satisfactory to the Company as to the Participant's knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and business matters and that the Participant is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Stock Award; and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Stock Award for the Participant's own account and not with any present intention of selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (A) the issuance of the shares upon the exercise or acquisition of Common Stock under the Stock Award has been registered under a then currently effective registration statement under the Securities Act, or (B) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(h) Withholding Obligations. Unless prohibited by the terms of a Stock Award Agreement, the Company may, in its sole discretion, satisfy any federal, state or local tax withholding obligation relating to a Stock Award by any of the following means or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Stock Award; *provided*, *however*, that no shares of Common Stock are withheld with a value exceeding the maximum amount of tax required to be withheld by law (or such lesser amount as may be necessary to avoid classification of the Stock Award as a liability for financial accounting purposes); (iii) withholding cash from a Stock Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant; or (v) by such other method as may be set forth in the Stock Award Agreement.

(i) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically or posted on the Company's intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(j) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or

settlement of all or a portion of any Stock Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A of the Code. Consistent with Section 409A of the Code, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Stock Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant's termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(k) Compliance with Section 409A of the Code. To the extent that the Board determines that any Stock Award granted hereunder is subject to Section 409A of the Code, the Stock Award Agreement evidencing such Stock Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code. To the extent applicable, the Plan and Stock Award Agreements will be interpreted in accordance with Section 409A of the Code. Notwithstanding anything to the contrary in the Plan (and unless the Stock Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding a Stock Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) will be issued or paid before the date that is six months following the date of such Participant's "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) or, if earlier, the date of the Participant's death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six month period elapses, with the balance paid thereafter on the original schedule.

(l) Repurchase Limitation. The terms of any repurchase right will be specified in the Stock Award Agreement. The repurchase price for vested shares of Common Stock will be the Fair Market Value of the shares of Common Stock on the date of repurchase. The repurchase price for unvested shares of Common Stock will be the lower of (i) the Fair Market Value of the shares of Common Stock on the date of repurchase or (ii) their original purchase price. However, the Company will not exercise its repurchase right until at least six months (or such longer or shorter period of time necessary to avoid classification of the Stock Award as a liability for financial accounting purposes) have elapsed following delivery of shares of Common Stock subject to the Stock Award, unless otherwise specifically provided by the Board.

9. Adjustments upon Changes in Common Stock; Other Corporate Events.

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a), (ii) the class(es) and maximum number of securities that may be issued pursuant to the exercise of Incentive Stock Options pursuant to Section 3(c), and (iii) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution or Liquidation. Except as otherwise provided in the Stock Award Agreement, in the event of a dissolution or liquidation of the Company, all outstanding Stock Awards (other than Stock Awards consisting of vested and outstanding shares of Common Stock not subject to a forfeiture condition or the Company's right of repurchase) will terminate immediately prior to the

completion of such dissolution or liquidation, and the shares of Common Stock subject to the Company's repurchase rights or subject to a forfeiture condition may be repurchased or reacquired by the Company notwithstanding the fact that the holder of such Stock Award is providing Continuous Service, *provided*, *however,* that the Board may, in its sole discretion, cause some or all Stock Awards to become fully vested, exercisable and/or no longer subject to repurchase or forfeiture (to the extent such Stock Awards have not previously expired or terminated) before the dissolution or liquidation is completed but contingent on its completion.

(c) Corporate Transaction. The following provisions will apply to Stock Awards in the event of a Corporate Transaction unless otherwise provided in the instrument evidencing the Stock Award or any other written agreement between the Company or any Affiliate and the Participant or unless otherwise expressly provided by the Board at the time of grant of a Stock Award. In the event of a Corporate Transaction, then, notwithstanding any other provision of the Plan, the Board may take one or more of the following actions with respect to Stock Awards, contingent upon the closing or completion of the Corporate Transaction:

(i) arrange for the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company) to assume or continue the Stock Award or to substitute a similar stock award for the Stock Award (including, but not limited to, an award to acquire the same consideration paid to the stockholders of the Company pursuant to the Corporate Transaction);

(ii) arrange for the assignment of any reacquisition or repurchase rights held by the Company in respect of Common Stock issued pursuant to the Stock Award to the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company);

(iii) accelerate the vesting, in whole or in part, of the Stock Award (and, if applicable, the time at which the Stock Award may be exercised) to a date prior to the effective time of such Corporate Transaction as the Board determines (or, if the Board does not determine such a date, to the date that is five days prior to the effective date of the Corporate Transaction), with such Stock Award terminating if not exercised (if applicable) at or prior to the effective time of the Corporate Transaction; *provided*, *however*, that the Board may require Participants to complete and deliver to the Company a notice of exercise before the effective date of a Corporate Transaction, which exercise is contingent upon the effectiveness of such Corporate Transaction;

(iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by the Company with respect to the Stock Award;

(v) cancel or arrange for the cancellation of the Stock Award, to the extent not vested or not exercised prior to the effective time of the Corporate Transaction, in exchange for such cash consideration (including no consideration) as the Board, in its sole discretion, may consider appropriate; and

(vi) make a payment, in such form as may be determined by the Board equal to the excess, if any, of (A) the value of the property the Participant would have received upon the exercise of the Stock Award immediately prior to the effective time of the Corporate Transaction, over (B) any exercise price payable by such holder in connection with such exercise. For clarity, this payment may be zero ($0) if the value of the property is equal to or less than the exercise price. Payments under this provision may be delayed to the same extent that payment of consideration to the holders of the Company's Common Stock in connection with the Corporate Transaction is delayed as a result of escrows, earn outs, holdbacks or any other contingencies.

The Board need not take the same action or actions with respect to all Stock Awards or portions thereof or with respect to all Participants. The Board may take different actions with respect to the vested and unvested portions of a Stock Award.

(d) Change in Control. A Stock Award may be subject to additional acceleration of vesting and exercisability upon or after a Change in Control as may be provided in the Stock Award Agreement for such Stock Award or as may be provided in any other written agreement between the Company or any Affiliate and the Participant, but in the absence of such provision, no such acceleration will occur.

10. Plan Term; Earlier Termination or Suspension of the Plan.

(a) Plan Term. The Board may suspend or terminate the Plan at any time. Unless terminated sooner by the Board, the Plan will automatically terminate on the day before the 10th anniversary of the earlier of (i) the date the Plan is adopted by the Board, or (ii) the date the Plan is approved by the stockholders of the Company. No Stock Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

(b) No Impairment of Rights. Suspension or termination of the Plan will not impair rights and obligations under any Stock Award granted while the Plan is in effect except with the written consent of the affected Participant or as otherwise permitted in the Plan.

11. Effective Date of Plan.

This Plan will become effective on the Effective Date.

12. Choice of Law.

The laws of the State of Delaware will govern all questions concerning the construction, validity and interpretation of this Plan, without regard to that state's conflict of laws rules.

13. Definitions. As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "***Affiliate***" means, at the time of determination, any "parent" or "majority-owned subsidiary" of the Company, as such terms are defined in Rule 405. The Board will have the authority to determine the time or times at which "parent" or "majority-owned subsidiary" status is determined within the foregoing definition.

(b) "***Board***" means the Board of Directors of the Company.

(c) "***Capitalization Adjustment***" means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Effective Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure, or any similar equity restructuring transaction, as that term is used in Statement of Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(d) "***Cause***" will have the meaning ascribed to such term in any written agreement between the Participant and the Company defining such term and, in the absence of such agreement, such term means, with respect to a Participant, the occurrence of any of the following events: (i) such Participant's commission of any felony or any crime involving fraud, dishonesty or moral turpitude under the laws of the United States or any state thereof; (ii) such Participant's attempted commission of, or participation in, a fraud or act of dishonesty against the Company, or any of its employees or directors; (iii) such Participant's intentional, material violation of any contract or agreement between the Participant and the Company, the Company's employment policies, or of any statutory or other duty owed to the Company; (iv) such Participant's unauthorized use or disclosure of the Company's confidential information or trade secrets; or (v) such Participant's gross misconduct. The determination that a termination of the Participant's Continuous Service is either for Cause or without Cause will be made by the Company, in its sole discretion. Any determination by the Company that the Continuous Service of a Participant was terminated with or without Cause for the purposes of outstanding Stock Awards held by such Participant will have no effect upon any determination of the rights or obligations of the Company or such Participant for any other purpose.

(e) "***Change in Control***" means the occurrence, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) any Exchange Act Person becomes the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities other than by virtue of a merger, consolidation or similar transaction. Notwithstanding the foregoing, a Change in Control will not be deemed to occur (A) on account of the acquisition of securities of the Company directly from the Company, (B) on account of the acquisition of securities of the Company by an investor, any affiliate thereof or any other Exchange Act Person that acquires the Company's securities in a transaction or series of related transactions the primary purpose of which is to obtain financing for the Company through the issuance of equity securities or (C) solely because the level of Ownership held by any Exchange Act Person (the "***Subject Person***") exceeds the designated percentage threshold of the outstanding voting securities as a result of a repurchase or other acquisition of voting securities by the Company reducing the number of shares outstanding, provided that if a Change in Control would occur (but for the operation of this sentence) as a result of the acquisition of voting securities by the Company, and after such share acquisition, the Subject Person becomes the Owner of any additional voting securities that, assuming the repurchase or other acquisition had not occurred, increases the percentage of the then outstanding voting securities Owned by the Subject Person over the designated percentage threshold, then a Change in Control will be deemed to occur;

(ii) there is consummated a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the stockholders of the Company immediately prior thereto do not Own, directly or indirectly, either (A) outstanding voting securities representing more than 50% of the combined outstanding voting power of the surviving Entity in such merger, consolidation or similar transaction or (B) more than 50% of the combined outstanding voting power of the parent of the surviving Entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such transaction; or

(iii) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries, other than a sale, lease, license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries to an Entity, more than 50% of the combined voting power of the voting securities of which are Owned by stockholders of the Company in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such sale, lease, license or other disposition.

Notwithstanding the foregoing definition or any other provision of this Plan, (A) the term Change in Control will not include a sale of assets, merger or other transaction effected exclusively for the purpose of changing the domicile of the Company, (B) the definition of Change in Control (or any analogous term) in an individual written agreement between the Company or any Affiliate and the Participant will supersede the foregoing definition with respect to Stock Awards subject to such agreement; *provided, however*, that if no definition of Change in Control or any analogous term is set forth in such an individual written agreement, the definition set forth herein will apply, and (C) if at any time the Company's Certificate of Incorporation provides definitions of various analogous transactions that would be deemed a liquidation event for the Company, then such definition will apply as if it were the definition set forth herein except as is otherwise expressly provided in an individual written agreement between the Company or any Affiliate and the Participant.

(f) "***Code***" means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(g) "***Committee***" means a committee of one or more Directors to whom authority has been delegated by the Board in accordance with Section 2(c).

(h) "***Common Stock***" means the common stock of the Company.

(i) "***Company***" means Taysha Gene Therapies, Inc., a Delaware corporation.

(j) "***Consultant***" means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan.

(k) "***Continuous Service***" means that the Participant's service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Director or Consultant or a change in the Entity for which the Participant renders such service, provided that there is no interruption or termination of the Participant's service with the Company or an Affiliate, will not terminate a Participant's Continuous Service; *provided, however*, that if the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board in its sole discretion, such Participant's Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. For example, a change in status from an Employee of the Company to a Consultant of an Affiliate or to a Director will not constitute an interruption of Continuous Service. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party's sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive officer, including sick leave,

military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. Notwithstanding the foregoing, a leave of absence will be treated as Continuous Service for purposes of vesting in a Stock Award only to such extent as may be provided in the Company's leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law.

(l) "***Corporate Transaction***" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of more than 50% of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

(m) "***Director***" means a member of the Board.

(n) "***Disability***" means, with respect to a Participant, the inability of such Participant to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or that has lasted or can be expected to last for a continuous period of not less than twelve (12) months as provided in Sections 22(e)(3) and 409A(a)(2)(c)(i) of the Code, and will be determined by the Board on the basis of such medical evidence as the Board deems warranted under the circumstances.

(o) "***Effective Date***" means the effective date of this Plan, which is the earlier of (i) the date that this Plan is first approved by the Company's stockholders, and (ii) the date this Plan is adopted by the Board.

(p) "***Employee***" means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.

(q) "***Entity***" means a corporation, partnership, limited liability company or other entity.

(r) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(s) "***Exchange Act Person***" means any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act), except that "Exchange Act Person" will not include (i) the Company or any Subsidiary of the Company, (ii) any employee benefit plan of the Company or any Subsidiary of the Company or any trustee or other fiduciary holding securities under an

employee benefit plan of the Company or any Subsidiary of the Company, (iii) an underwriter temporarily holding securities pursuant to a registered public offering of such securities, (iv) an Entity Owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their Ownership of stock of the Company; or (v) any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act) that, as of the Effective Date, is the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities.

(t) "***Fair Market Value***" means, as of any date, the value of the Common Stock determined by the Board in compliance with Section 409A of the Code or, in the case of an Incentive Stock Option, in compliance with Section 422 of the Code.

(u) "***Incentive Stock Option***" means an option granted pursuant to Section 5 of the Plan that is intended to be, and that qualifies as, an "incentive stock option" within the meaning of Section 422 of the Code.

(v) "***Nonstatutory Stock Option***" means an option granted pursuant to Section 5 of the Plan that does not qualify as an Incentive Stock Option.

(w) "***Officer***" means any person designated by the Company as an officer.

(x) "***Option***" means an Incentive Stock Option or a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.

(y) "***Option Agreement***" means a written agreement between the Company and an Optionholder evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.

(z) "***Optionholder***" means a person to whom an Option is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Option.

(aa) "***Other Stock Award***" means an award based in whole or in part by reference to the Common Stock which is granted pursuant to the terms and conditions of Section 6(c).

(bb) "***Other Stock Award Agreement***" means a written agreement between the Company and a holder of an Other Stock Award evidencing the terms and conditions of an Other Stock Award grant. Each Other Stock Award Agreement will be subject to the terms and conditions of the Plan.

(cc) "***Own***," "***Owned***," "***Owner***," "***Ownership***" A person or Entity will be deemed to "Own," to have "Owned," to be the "Owner" of, or to have acquired "Ownership" of securities if such person or Entity, directly or indirectly, through any contract, arrangement, understanding, relationship or otherwise, has or shares voting power, which includes the power to vote or to direct the voting, with respect to such securities.

(dd) "***Participant***" means a person to whom a Stock Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.

(ee) "***Plan***" means this 2020 Equity Incentive Plan.

(ff) "***Restricted Stock Award***" means an award of shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(a).

(gg) "***Restricted Stock Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Award evidencing the terms and conditions of a Restricted Stock Award grant. Each Restricted Stock Award Agreement will be subject to the terms and conditions of the Plan.

(hh) "***Restricted Stock Unit Award***" means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).

(ii) "***Restricted Stock Unit Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(jj) "***Rule 405***" means Rule 405 promulgated under the Securities Act.

(kk) "***Rule 701***" means Rule 701 promulgated under the Securities Act.

(ll) "***Securities Act***" means the Securities Act of 1933, as amended.

(mm) "***Stock Appreciation Right***" or "***SAR***" means a right to receive the appreciation on Common Stock that is granted pursuant to the terms and conditions of Section 5.

(nn) "***Stock Appreciation Right Agreement***" means a written agreement between the Company and a holder of a Stock Appreciation Right evidencing the terms and conditions of a Stock Appreciation Right grant. Each Stock Appreciation Right Agreement will be subject to the terms and conditions of the Plan.

(oo) "***Stock Award***" means any right to receive Common Stock granted under the Plan, including an Incentive Stock Option, a Nonstatutory Stock Option, a Restricted Stock Award, a Restricted Stock Unit Award, a Stock Appreciation Right or any Other Stock Award.

(pp) "***Stock Award Agreement***" means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

(qq) "***Subsidiary***" means, with respect to the Company, (i) any corporation of which more than 50% of the outstanding capital stock having ordinary voting power to elect a majority of the board of directors of such corporation (irrespective of whether, at the time, stock of any other class or classes of such corporation will have or might have voting power by reason of the happening of any contingency) is at the time, directly or indirectly, Owned by the Company, and (ii) any partnership, limited liability company or other entity in which the Company has a direct or indirect interest (whether in the form of voting or participation in profits or capital contribution) of more than 50%.

(rr) "***Ten Percent Stockholder***" means a person who Owns (or is deemed to Own pursuant to Section 424(d) of the Code) stock possessing more than 10% of the total combined voting power of all classes of stock of the Company or any Affiliate.

TAYSHA GENE THERAPIES, INC.

STOCK OPTION GRANT NOTICE
(2020 EQUITY INCENTIVE PLAN)

Taysha Gene Therapies, Inc. (the "***Company***"), pursuant to its 2020 Equity Incentive Plan (as amended and/or restated as of the Date of Grant set forth below, the "***Plan***"), has granted to Optionholder an option to purchase the number of shares of the Common Stock set forth below (the "***Option***"). The Option is subject to all of the terms and conditions as set forth in this Stock Option Grant Notice (the "***Grant Notice***") and in the Plan, the Option Agreement, and the Notice of Exercise, all of which are attached to this Grant Notice and incorporated into this Grant Notice in their entirety. Capitalized terms not explicitly defined in this Grant Notice but defined in the Plan or the Option Agreement shall have the meanings set forth in the Plan or the Option Agreement, as applicable. If the Company uses an electronic capitalization table system (such as Carta or Shareworks) and the fields below are blank or the information is otherwise provided in a different format electronically, the blank fields and other information (such as exercise schedule and type of grant) shall be deemed to come from the electronic capitalization system and is considered part of this Grant Notice.

Optionholder:	
Date of Grant:	
Vesting Commencement Date:	
Number of Shares Subject to Option:	
Exercise Price (Per Share)[1]:	
Total Exercise Price:	
Expiration Date:	
Exercise Schedule:	[Same as Vesting Schedule] [Early Exercise Permitted]
Type of Grant[2]:	[Incentive Stock Option] [Nonstatutory Stock Option]

Vesting Schedule: [**Sample of standard vesting.** 12/48ths of the total shares will vest on the one-year anniversary of the Vesting Commencement Date, and 1/48th of the total shares will vest each month thereafter on the same day of the month as the Vesting Commencement Date (or if there is no corresponding day, on the last day of the month), subject to Optionholder's Continuous Service as of each such date.]

1 The exercise price may be paid by one or a combination of the methods permitted in the Option Agreement.

2 If this is an Incentive Stock Option, it (plus other outstanding Incentive Stock Options) cannot be first *exercisable* for more than $100,000 in value (measured by exercise price) in any calendar year. Any excess over $100,000 is a Nonstatutory Stock Option.

Optionholder Acknowledgements: By Optionholder's signature below or by electronic acceptance or authentication in a form authorized by the Company, Optionholder understands and agrees that the Option is governed by this Stock Option Grant Notice, and the provisions of the Plan and the Option Agreement and the Notice of Exercise, all of which are made a part of this document.

By accepting this Option, Optionholder consents to receive this Grant Notice, the Option Agreement, the Plan, and any other Plan-related documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company. Optionholder represents that he or she has read and is familiar with the provisions of the Plan and the Option Agreement. Optionholder acknowledges and agrees that this Grant Notice and the Option Agreement may not be modified, amended or revised except in writing signed by Optionholder and a duly authorized officer of the Company.

Optionholder further acknowledges that in the event of any conflict between the provisions in this Grant Notice, the Option Agreement, the Notice of Exercise and the terms of the Plan, the terms of the Plan shall control. Optionholder further acknowledges that the Option Agreement sets forth the entire understanding between Optionholder and the Company regarding the acquisition of Common Stock and supersedes all prior oral and written agreements, promises and/or representations on that subject with the exception of other equity awards previously granted to Optionholder and any written employment agreement, offer letter, severance agreement, written severance plan or policy, or other written agreement between the Company and Optionholder in each case that specifies the terms that should govern this Option.

Optionholder further acknowledges that this Grant Notice has been prepared on behalf of the Company by Cooley LLP, counsel to the Company and that Cooley LLP does not represent, and is not acting on behalf of, Optionholder in any capacity. Optionholder has been provided with an opportunity to consult with Optionholder's own counsel with respect to this Grant Notice.

This Grant Notice may be executed in one or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, Uniform Electronic Transactions Act or other applicable law) or other transmission method and any counterpart so delivered will be deemed to have been duly and validly delivered and be valid and effective for all purposes.

Taysha Gene Therapies, Inc.		**Optionholder:**	
By:	_____	By:	_____
	(Signature)		(Signature)
Title:	_____	Email:	_____
Date:	_____	Date:	_____

Attachments: Option Agreement, 2020 Equity Incentive Plan and Notice of Exercise

TAYSHA GENE THERAPIES, INC.

2020 Equity Incentive Plan

OPTION AGREEMENT
(INCENTIVE STOCK OPTION OR NONSTATUTORY STOCK OPTION)

Pursuant to your Stock Option Grant Notice ("***Grant Notice***") and this Option Agreement, **Taysha Gene Therapies, Inc.** (the "***Company***") has granted you an option under its 2020 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of the Company's Common Stock indicated in your Grant Notice at the exercise price indicated in your Grant Notice. The option is granted to you effective as of the date of grant set forth in the Grant Notice (the "***Date of Grant***"). If there is any conflict between the terms in this Option Agreement and the Plan, the terms of the Plan will control. Capitalized terms not explicitly defined in this Option Agreement or in the Grant Notice but defined in the Plan will have the same definitions as in the Plan.

The details of your option, in addition to those set forth in the Grant Notice and the Plan, are as follows:

1. Vesting. Your option will vest as provided in your Grant Notice. Vesting will cease upon the termination of your Continuous Service.

2. Number of Shares and Exercise Price. The number of shares of Common Stock subject to your option and your exercise price per share in your Grant Notice will be adjusted for Capitalization Adjustments.

3. Exercise Restriction for Non-Exempt Employees. If you are an Employee eligible for overtime compensation under the Fair Labor Standards Act of 1938, as amended (that is, a "***Non-Exempt Employee***"), and except as otherwise provided in the Plan, you may not exercise your option until you have completed at least six months of Continuous Service measured from the Date of Grant, even if you have already been an employee for more than six months. Consistent with the provisions of the Worker Economic Opportunity Act, you may exercise your option as to any vested portion prior to such six month anniversary in the case of (i) your death or disability, (ii) a Corporate Transaction in which your option is not assumed, continued or substituted, (iii) a Change in Control or (iv) your termination of Continuous Service on your "retirement" (as defined in the Company's benefit plans).

4. Exercise prior to Vesting ("Early Exercise"). If permitted in your Grant Notice (*i.e.*, the "Exercise Schedule" indicates "Early Exercise Permitted") and subject to the provisions of your option, you may elect at any time that is both (i) during the period of your Continuous Service and (ii) during the term of your option, to exercise all or part of your option, including the unvested portion of your option; *provided*, *however*, that:

a. a partial exercise of your option will be deemed to cover first vested shares of Common Stock and then the earliest vesting installment of unvested shares of Common Stock;

b. any shares of Common Stock so purchased from installments that have not vested as of the date of exercise will be subject to the purchase option in favor of the Company as described in the Company's form of Early Exercise Stock Purchase Agreement;

c. you will enter into the Company's form of Early Exercise Stock Purchase Agreement with a vesting schedule that will result in the same vesting as if no early exercise had occurred; and

d. if your option is an Incentive Stock Option, then, to the extent that the aggregate Fair Market Value (determined at the Date of Grant) of the shares of Common Stock with respect to which your option plus all other Incentive Stock Options you hold are exercisable for the first time by you during any calendar year (under all plans of the Company and its Affiliates) exceeds $100,000, your option(s) or portions thereof that exceed such limit (according to the order in which they were granted) will be treated as Nonstatutory Stock Options.

5. Method of Payment. You must pay the full amount of the exercise price for the shares you wish to exercise. The permitted methods of payment are as follows:

a. by cash, check, bank draft, electronic funds transfer or money order payable to the Company;

b. subject to Company and/or Board consent at the time of exercise and provided that at the time of exercise the Common Stock is publicly traded, pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of Common Stock, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds. This manner of payment is also known as a "broker-assisted exercise", "same day sale", or "sell to cover";

c. subject to Company and/or Board consent at the time of exercise and provided that at the time of exercise the Common Stock is publicly traded, by delivery to the Company (either by actual delivery or attestation) of already-owned shares of Common Stock that are owned free and clear of any liens, claims, encumbrances or security interests, and that are valued at Fair Market Value on the date of exercise. "Delivery" for these purposes, in the sole discretion of the Company at the time you exercise your option, will include delivery to the Company of your attestation of ownership of such shares of Common Stock in a form approved by the Company. You may not exercise your option by delivery to the Company of Common Stock if doing so would violate the provisions of any law, regulation or agreement restricting the redemption of the Company's stock;

d. subject to Company and/or Board consent at the time of exercise, and provided that the Option is a Nonstatutory Stock Option, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issued upon exercise of the Option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price plus, to the extent permitted by the Company and/or Board at the time of exercise, the aggregate withholding obligations in respect of the Option exercise; provided, further that you must pay any remaining balance of the aggregate exercise price not satisfied by the "net exercise" in cash or other permitted form of payment. Shares of Common Stock will no longer be subject to the Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are used to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to you as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations;

e. subject to the consent of the Company and/or Board at the time of exercise, according to a deferred payment or similar arrangement with you; *provided*, *however*, that interest will compound at least annually and will be charged at the minimum rate of interest necessary to avoid (A) the imputation of interest income to the Company and compensation income to the Optionholder under any applicable provisions of the Code, and (B) the classification of the Option as a liability for financial accounting purposes; or

f. in any other form of legal consideration that may be acceptable to the Board.

6. Whole Shares. You may exercise your option only for whole shares of Common Stock.

7. Securities Law Compliance. In no event may you exercise your option unless the shares of Common Stock issuable upon exercise are then registered under the Securities Act or, if not registered, the Company has determined that your exercise and the issuance of the shares would be exempt from the registration requirements of the Securities Act. The exercise of your option also must comply with all other applicable laws and regulations governing your option, and you may not exercise your option if the Company determines that such exercise would not be in material compliance with such laws and regulations (including any restrictions on exercise required for compliance with Treas. Reg. 1.401(k)-1(d)(3), if applicable).

8. Term. You may not exercise your option before the Date of Grant or after the expiration of the option's term. Except as set forth in your Grant Notice, the term of your option expires, subject to the provisions of Section 5(h) of the Plan, upon the earliest of the following:

a. immediately upon the termination of your Continuous Service for Cause;

b. three months after the termination of your Continuous Service for any reason other than Cause, your Disability or your death (except as otherwise provided in Section 8(d) below); *provided*, *however*, that if during any part of such three month period your option is not exercisable solely because of the condition set forth in the section above relating to "Securities Law Compliance," your option will not expire until the earlier of the Expiration Date or until it has been exercisable for an aggregate period of three months after the termination of your Continuous Service; *provided further*, that if (i) you are a Non-Exempt Employee, (ii) your Continuous Service terminates within six months after the Date of Grant, and (iii) you have vested in a portion of your option at the time of your termination of Continuous Service, your option will not expire until the earlier of (x) the later of (A) the date that is seven months after the Date of Grant, and (B) the date that is three months after the termination of your Continuous Service, and (y) the Expiration Date;

c. 12 months after the termination of your Continuous Service due to your Disability (except as otherwise provided in Section 8(d)) below;

d. 18 months after your death if you die either during your Continuous Service or within three (3) months after your Continuous Service terminates for any reason other than Cause;

e. the Expiration Date indicated in your Grant Notice; or

f. the day before the 10th anniversary of the Date of Grant.

If your option is an Incentive Stock Option, note that to obtain the federal income tax advantages associated with an Incentive Stock Option, the Code requires that at all times beginning on the Date of Grant and ending on the day three months before the date of your option's exercise, you must be an employee of the Company or an Affiliate, except in the event of your death or Disability. The Company has provided for extended exercisability of your option under certain circumstances for your benefit but cannot guarantee that your option will necessarily be treated as an Incentive Stock Option if you continue to provide services to the Company or an Affiliate as a Consultant or Director after your employment terminates or if you otherwise exercise your option more than three months after the date your employment with the Company or an Affiliate terminates.

9. Exercise.

a. You may exercise the vested portion of your option (and the unvested portion of your option if your Grant Notice so permits) during its term by delivering a Notice of Exercise (in a form designated by the Company) together with the exercise price to the Secretary of the Company, or to such other person as the Company may designate, during regular business hours. If required by the Company, your exercise may be made contingent on your execution of any additional documents specified by the Company (including, without limitation, any voting agreement or other agreement between the Company and some or all of its stockholders).

b. By exercising your option you agree that, as a condition to any exercise of your option, the Company may require you to enter into an arrangement providing for the payment by you to the Company of any tax withholding obligation of the Company arising by reason of (i) the exercise of your option, (ii) the lapse of any substantial risk of forfeiture to which the shares of Common Stock are subject at the time of exercise, or (iii) the disposition of shares of Common Stock acquired upon such exercise.

c. If your option is an Incentive Stock Option, by exercising your option you agree that you will notify the Company in writing within 15 days after the date of any disposition of any of the shares of the Common Stock issued upon exercise of your option that occurs within two years after the Date of Grant or within one year after such shares of Common Stock are transferred upon exercise of your option.

d. By exercising your option you agree that you will not sell, dispose of, transfer, make any short sale of, grant any option for the purchase of, or enter into any hedging or similar transaction with the same economic effect as a sale with respect to any shares of Common Stock or other securities of the Company held by you, for a period of 180 days following the effective date of a registration statement of the Company filed under the Securities Act or such longer period as the underwriters or the Company will request to facilitate compliance with applicable FINRA rules (the "***Lock-Up Period***"); *provided, however*, that nothing contained in this section will prevent the exercise of a repurchase option, if any, in favor of the Company during the Lock-Up Period. You further agree to execute and deliver such other agreements as may be reasonably requested by the Company or the underwriters that are consistent with the foregoing or that are necessary to give further effect thereto. In order to enforce the foregoing covenant, the Company may impose stop-transfer instructions with respect to your shares of Common Stock until the end of such period. You also agree that any transferee of any shares of Common Stock (or other securities) of the Company held by you will be bound by this Section 9(d). The underwriters of the Company's stock are intended third party beneficiaries of this Section 9(d) and will have the right, power and authority to enforce the provisions hereof as though they were a party hereto. You further agree that the obligations contained in this Section 9(d) shall also, if so determined by the Company's Board of Directors, apply in the Company's initial listing of its Common Stock on a national securities exchange by means of a registration statement on Form S-1 under the Securities Act (or any successor registration form under the Securities Act subsequently adopted by the Securities and Exchange Commission) filed by the Company with the Securities and Exchange Commission that registers shares of existing capital stock of the Company for resale (a "***Direct Listing***"), provided that all holders of at least 5% of the Company's outstanding Common Stock (after giving effect to the conversion into Common Stock of any outstanding Preferred Stock of the Company) are subject to substantially similar obligations with respect to such Direct Listing.

10. Transferability. Except as otherwise provided in this Section 10, your option is not transferable, except by will or by the laws of descent and distribution, and is exercisable during your life only by you.

a. Certain Trusts. Upon receiving written permission from the Board or its duly authorized designee, you may transfer your option to a trust if you are considered to be the sole beneficial owner (determined under Section 671 of the Code and applicable state law) while the option is held in the trust. You and the trustee must enter into transfer and other agreements required by the Company.

b. Domestic Relations Orders. Upon receiving written permission from the Board or its duly authorized designee, and provided that you and the designated transferee enter into transfer and other agreements required by the Company, you may transfer your option pursuant to the terms of a domestic relations order, official marital settlement agreement or other divorce or separation instrument as permitted by Treasury Regulation 1.421-1(b)(2) that contains the information required by the Company to effectuate the transfer. You are encouraged to discuss the proposed terms of any division of this option with the Company prior to finalizing the domestic relations order or marital settlement agreement to help ensure the required information is contained within the domestic relations order or marital settlement agreement. If this option is an Incentive Stock Option, this option may be deemed to be a Nonstatutory Stock Option as a result of such transfer.

c. Beneficiary Designation. Upon receiving written permission from the Board or its duly authorized designee, you may, by delivering written notice to the Company, in a form approved by the Company and any broker designated by the Company to handle option exercises, designate a third party who, on your death, will thereafter be entitled to exercise this option and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, your executor or administrator of your estate will be entitled to exercise this option and receive, on behalf of your estate, the Common Stock or other consideration resulting from such exercise.

11. Right of First Refusal. Shares of Common Stock that you acquire upon exercise of your option are subject to any right of first refusal that may be described in the Company's bylaws in effect at such time the Company elects to exercise its right; *provided*, *however,* that if there is no right of first refusal described in the Company's bylaws at such time, the right of first refusal described below will apply. The Company's right of first refusal will expire on the first date upon which any security of the Company is listed (or approved for listing) upon notice of issuance on a national securities exchange or quotation system (the "***Listing Date***").

a. Prior to the Listing Date, you may not validly Transfer (as defined below) any shares of Common Stock acquired upon exercise of your option, or any interest in such shares, unless such Transfer is made in compliance with the following provisions:

1) Before there can be a valid Transfer of any shares of Common Stock or any interest therein, the record holder of the shares of Common Stock to be transferred (the "***Offered Shares***") will give written notice (by registered or certified mail) to the Company. Such notice will specify the identity of the proposed transferee, the cash price offered for the Offered Shares by the proposed transferee (or, if the proposed Transfer is one in which the holder will not receive cash, such as an involuntary transfer, gift, donation or pledge, the holder will state that no purchase price is being proposed), and the other terms and conditions of the proposed Transfer. The date such notice is mailed will be hereinafter referred to as the "***Notice Date***" and the record holder of the Offered Shares will be hereinafter referred to as the "***Offeror***." If, from time to time, there is any stock dividend, stock split or other change in the character or amount of any of the outstanding Common Stock which is subject to the provisions of your option, then in such event any and all new, substituted or additional securities to which

you are entitled by reason of your ownership of the shares of Common Stock acquired upon exercise of your option will be immediately subject to the Company's Right of First Refusal (as defined below) with the same force and effect as the shares subject to the Right of First Refusal immediately before such event.

2) For a period of 30 calendar days after the Notice Date, or such longer period as may be required to avoid the classification of your option as a liability for financial accounting purposes, the Company will have the option to purchase all (but not less than all) of the Offered Shares at the purchase price and on the terms set forth in Section 11(a)(iii) (the Company's "***Right of First Refusal***"). In the event that the proposed Transfer is one involving no payment of a purchase price, the purchase price will be deemed to be the Fair Market Value of the Offered Shares as determined in good faith by the Board in its discretion. The Company may exercise its Right of First Refusal by mailing (by registered or certified mail) written notice of exercise of its Right of First Refusal to the Offeror prior to the end of said 30 days (including any extension required to avoid classification of the option as a liability for financial accounting purposes).

3) The price at which the Company may purchase the Offered Shares pursuant to the exercise of its Right of First Refusal will be the cash price offered for the Offered Shares by the proposed transferee (as set forth in the notice required under Section 11(a)(i)), or the Fair Market Value as determined by the Board in the event no purchase price is involved. To the extent consideration other than cash is offered by the proposed transferee, the Company will not be required to pay any additional amounts to the Offeror other than the cash price offered (or the Fair Market Value, if applicable). The Company's notice of exercise of its Right of First Refusal will be accompanied by full payment for the Offered Shares and, upon such payment by the Company, the Company will acquire full right, title and interest to all of the Offered Shares.

4) If, and only if, the option given pursuant to Section 11(a)(ii) is not exercised, the Transfer proposed in the notice given pursuant to Section 11(a)(i) may take place; *provided, however*, that such Transfer must, in all respects, be exactly as proposed in said notice except that such Transfer may not take place either before the 10th calendar day after the expiration of the 30 day option exercise period or after the ninetieth 90th calendar day after the expiration of the 30 day option exercise period, and if such Transfer has not taken place prior to said 90th day, such Transfer may not take place without once again complying with this Section 11(a). The option exercise periods in this Section 11(a)(iv) will be adjusted to include any extension required to avoid the classification of your option as a liability for financial accounting purposes.

b. As used in this Section 11, the term "***Transfer***" means any sale, encumbrance, pledge, gift or other form of disposition or transfer of shares of Common Stock or any legal or equitable interest therein; *provided, however*, that the term Transfer does not include a transfer of such shares or interests by will or intestacy to your Immediate Family (as defined below). In such case, the transferee or other recipient will receive and hold the shares of Common Stock so transferred subject to the provisions of this Section, and there will be no further transfer of such shares except in accordance with the terms of this Section 11. As used herein, the term "***Immediate Family***" will mean your spouse, the lineal descendant or antecedent, father, mother, brother or sister, child, adopted child, grandchild or adopted grandchild of you or your spouse, or the spouse of any child, adopted child, grandchild or adopted grandchild of you or your spouse.

c. None of the shares of Common Stock purchased on exercise of your option will be transferred on the Company's books nor will the Company recognize any such Transfer of any such shares or any interest therein unless and until all applicable provisions of this Section 11 have been complied with in all respects. The certificates of stock evidencing shares of Common Stock purchased on exercise of your option will bear an appropriate legend referring to the transfer restrictions imposed by this Section 11.

d. To ensure that the shares subject to the Company's Right of First Refusal will be available for repurchase by the Company, the Company may require you to deposit the certificates evidencing the shares that you purchase upon exercise of your option with an escrow agent designated by the Company under the terms and conditions of an escrow agreement approved by the Company. If the Company does not require such deposit as a condition of exercise of your option, the Company reserves the right at any time to require you to so deposit the certificates in escrow. As soon as practicable after the expiration of the Company's Right of First Refusal, the agent will deliver to you the shares and any other property no longer subject to such restriction. In the event the shares and any other property held in escrow are subject to the Company's exercise of its Right of First Refusal, the notices required to be given to you will be given to the escrow agent, and any payment required to be given to you will be given to the escrow agent. Within 30 days after payment by the Company for the Offered Shares, the escrow agent will deliver the Offered Shares that the Company has repurchased to the Company and will deliver the payment received from the Company to you.

12. Option not a Service Contract. Your option is not an employment or service contract, and nothing in your option will be deemed to create in any way whatsoever any obligation on your part to continue in the employ of the Company or an Affiliate, or of the Company or an Affiliate to continue your employment. In addition, nothing in your option will obligate the Company or an Affiliate, their respective stockholders, boards of directors, officers or employees to continue any relationship that you might have as a Director or Consultant for the Company or an Affiliate.

13. Withholding Obligations.

a. At the time you exercise your option, in whole or in part, and at any time thereafter as requested by the Company, you hereby authorize withholding from payroll and any other amounts payable to you, and otherwise agree to make adequate provision for (including by means of a "same day sale" pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board to the extent permitted by the Company), any sums required to satisfy the federal, state, local and foreign tax withholding obligations of the Company or an Affiliate, if any, which arise in connection with the exercise of your option.

b. If this option is a Nonstatutory Stock Option, then upon your request and subject to approval by the Company, and compliance with any applicable legal conditions or restrictions, the Company may withhold from fully vested shares of Common Stock otherwise issuable to you upon the exercise of your option a number of whole shares of Common Stock having a Fair Market Value, determined by the Company as of the date of exercise, not in excess of the minimum amount of tax required to be withheld by law (or such lower amount as may be necessary to avoid classification of your option as a liability for financial accounting purposes). If the date of determination of any tax withholding obligation is deferred to a date later than the date of exercise of your option, share withholding pursuant to the preceding sentence will not be permitted unless you make a proper and timely election under Section 83(b) of the Code, covering the aggregate number of shares of Common Stock acquired upon such exercise with respect to which such determination is otherwise deferred, to accelerate the determination of such tax withholding obligation to the date of exercise of your option. Notwithstanding the filing of such election, shares of Common Stock will be withheld solely from fully vested shares of Common Stock determined as of the date of exercise of your option that are otherwise issuable to you upon such exercise. Any adverse consequences to you arising in connection with such share withholding procedure will be your sole responsibility.

c. You may not exercise your option unless the tax withholding obligations of the Company and/or any Affiliate are satisfied. Accordingly, you may not be able to exercise your option when desired even though your option is vested, and the Company will have no obligation to issue a certificate for such shares of Common Stock or release such shares of Common Stock from any escrow provided for herein, if applicable, unless such obligations are satisfied.

14. Tax Consequences. You hereby agree that the Company does not have a duty to design or administer the Plan or its other compensation programs in a manner that minimizes your tax liabilities. You will not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates related to tax liabilities arising from your option or your other compensation. In particular, you acknowledge that this option is exempt from Section 409A of the Code only if the exercise price per share specified in the Grant Notice is at least equal to the "fair market value" per share of the Common Stock on the Date of Grant and there is no other impermissible deferral of compensation associated with the option. Because the Common Stock is not traded on an established securities market, the Fair Market Value is determined by the Board, perhaps in consultation with an independent valuation firm retained by the Company. You acknowledge that there is no guarantee that the Internal Revenue Service will agree with the valuation as determined by the Board, and you will not make any claim against the Company, or any of its Officers, Directors, Employees or Affiliates in the event that the Internal Revenue Service asserts that the valuation determined by the Board is less than the "fair market value" as subsequently determined by the Internal Revenue Service.

15. Notices. Any notices provided for in your option or the Plan will be given in writing (including electronically) and will be deemed effectively given upon receipt or, in the case of notices delivered by mail by the Company to you, five days after deposit in the United States mail, postage prepaid, addressed to you at the last address you provided to the Company. The Company may, in its sole discretion, decide to deliver any documents related to participation in the Plan and this option by electronic means or to request your consent to participate in the Plan by electronic means. By accepting this option, you consent to receive such documents by electronic delivery and to participate in the Plan through an on-line or electronic system established and maintained by the Company or another third party designated by the Company.

16. Governing Plan Document. Your option is subject to all the provisions of the Plan, the provisions of which are hereby made a part of your option, and is further subject to all interpretations, amendments, rules and regulations, which may from time to time be promulgated and adopted pursuant to the Plan. If there is any conflict between the provisions of your option and those of the Plan, the provisions of the Plan will control.

TAYSHA GENE THERAPIES, INC.
NOTICE OF EXERCISE

This constitutes notice to **Taysha Gene Therapies, Inc.** (the "***Company***") under my stock option that I elect to purchase the below number of shares of Common Stock of the Company (the "***Shares***") for the price set forth below. Use of certain payment methods is subject to Company and/or Board consent and certain additional requirements set forth in the Option Agreement and the Plan. If the Company uses an electronic capitalization table system (such as Carta or Shareworks) and the fields below are blank, the blank fields shall be deemed to come from the electronic capitalization system and is considered part of this Notice of Exercise.

Option Information

Type of option (check one):	Incentive ☐ Nonstatutory ☐
Stock option dated:	
Number of Shares as to which option is exercised:	
Certificates to be issued in name of:[3]	

Exercise Information

Date of Exercise:	
Total exercise price:	
Cash:[4]	
Regulation T Program (cashless exercise):[5]	
Value of _____ Shares delivered with this notice:[6]	
Value of _____ Shares pursuant to net exercise:[7]	

By this exercise, I agree (i) to provide such additional documents as you may require pursuant to the terms of the 2020 Equity Incentive Plan, (ii) to provide for the payment by me to you (in the manner designated by you) of your withholding obligation, if any, relating to the exercise of this option, and (iii) if this exercise relates to an incentive stock option, to notify you in writing within 15 days after the date of any disposition of any of the Shares issued upon exercise of this option that occurs within two years after the date of grant of this option or within one year after such Shares are issued upon exercise of this option. I further agree that this Notice of Exercise may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, Uniform Electronic Transactions Act or other applicable law) or other transmission method and will be deemed to have been duly and validly delivered and be valid and effective for all purposes.

I hereby make the following certifications and representations with respect to the number of Shares listed above, which are being acquired by me for my own account upon exercise of the option as set forth above:

I acknowledge that the Shares have not been registered under the Securities Act of 1933, as amended (the "***Securities Act***"), and are deemed to constitute "restricted securities" under Rule 701 and Rule 144 promulgated under the Securities Act. I warrant and represent to the Company that I have no present intention of distributing or selling said Shares, except as permitted under the Securities Act and any applicable state securities laws.

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3 If left blank, will be issued in the name of the option holder.
4 Cash may be in the form of cash, check, bank draft, electronic funds transfer or money order payment.
5 Subject to Company and/or Board consent and must meet the public trading and other requirements set forth in the Option Agreement.
6 Subject to Company and/or Board consent and must meet the public trading and other requirements set forth in the Option Agreement. Shares must be valued in accordance with the terms of the option being exercised, and must be owned free and clear of any liens, claims, encumbrances or security interests. Certificates must be endorsed or accompanied by an executed assignment separate from certificate.
7 Subject to Company and/or Board consent and must be a Nonstatutory Option.

I further acknowledge and agree that, except for such information as required to be delivered to me by the Company pursuant to the option or the Plan (if any), I will have no right to receive any information from the Company by virtue of the grant of the option or the purchase of shares of Common Stock through exercise of the option, ownership of such shares of Common Stock, or as a result of my being a holder of record of stock of the Company. Without limiting the foregoing, to the fullest extent permitted by law, I hereby waive all inspection rights under Section 220 of the Delaware General Corporation Law and all such similar information and/or inspection rights that may be provided under the law of any jurisdiction, or any federal, state or foreign regulation, that are, or may become, applicable to the Company or the Company's capital stock (the "***Inspection Rights***"). I hereby covenant and agree never to directly or indirectly commence, voluntarily aid in any way, prosecute, assign, transfer, or cause to be commenced any claim, action, cause of action, or other proceeding to pursue or exercise the Inspection Rights.

I further acknowledge that I will not be able to resell the Shares for at least 90 days after the stock of the Company becomes publicly traded (*i.e.*, subject to the reporting requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934) under Rule 701 and that more restrictive conditions apply to affiliates of the Company under Rule 144.

I further acknowledge that all certificates representing any of the Shares subject to the provisions of the option will have endorsed thereon appropriate legends reflecting the foregoing limitations, as well as any legends reflecting restrictions pursuant to the Company's Certificate of Incorporation, Bylaws and/or applicable securities laws.

I further agree that, if required by the Company (or a representative of the underwriters) in connection with the first underwritten registration of the offering of any securities of the Company under the Securities Act, I will not sell, dispose of, transfer, make any short sale of, grant any option for the purchase of, or enter into any hedging or similar transaction with the same economic effect as a sale with respect to any shares of Common Stock or other securities of the Company for a period of 180 days following the effective date of a registration statement of the Company filed under the Securities Act (or such longer period as the underwriters or the Company will request to facilitate compliance with applicable FINRA rules) (the "***Lock-Up Period***"). I further agree to execute and deliver such other agreements as may be reasonably requested by the Company or the underwriters that are consistent with the foregoing or that are necessary to give further effect thereto. In order to enforce the foregoing covenant, the Company may impose stop-transfer instructions with respect to securities subject to the foregoing restrictions until the end of such period. I further agree that the obligations contained in this paragraph shall also, if so determined by the Company's Board of Directors, apply in the Company's initial listing of its Common Stock on a national securities exchange by means of a registration statement on Form S-1 under the Securities Act (or any successor registration form under the Securities Act subsequently adopted by the Securities and Exchange Commission) filed by the Company with the Securities and Exchange Commission that registers shares of existing capital stock of the Company for resale (a "***Direct Listing***"), provided that all holders of at least 5% of the Company's outstanding Common Stock (after giving effect to the conversion into Common Stock of any outstanding Preferred Stock of the Company) are subject to substantially similar obligations with respect to such Direct Listing.

Very truly yours,

_____

(Signature)

_____

Name (Please Print)

Address of Record: _____

_____

_____

Email: _____

TAYSHA GENE THERAPIES, INC.

EARLY EXERCISE STOCK PURCHASE AGREEMENT
UNDER THE 2020 EQUITY INCENTIVE PLAN

This Agreement is made by and between **Taysha Gene Therapies, Inc.**, a Delaware corporation (the "***Company***"), and the individual designated on the signature page hereto as a Purchaser ("***Purchaser***").

Recitals:

A. Purchaser holds a stock option, granted on , to purchase shares of common stock ("***Common Stock***") of the Company (the "***Option***") pursuant to the Company's 2020 Equity Incentive Plan (as amended and/or restated, the "***Plan***").

B. The Option consists of a Stock Option Grant Notice and a Stock Option Agreement.

C. Purchaser desires to exercise the Option on the terms and conditions contained herein.

D. Purchaser wishes to take advantage of the early exercise provision of Purchaser's Option and therefore to enter into this Agreement.

The parties agree as follows:

17. Incorporation of Plan and Option by Reference. This Agreement is subject to all of the terms and conditions as set forth in the Plan and the Option. If there is a conflict between the terms of this Agreement and/or the Option and the terms of the Plan, the terms of the Plan will control. If there is a conflict between the terms of this Agreement and the terms of the Option, the terms of the Option will control. Defined terms not explicitly defined in this Agreement but defined in the Plan will have the same definitions as in the Plan. Defined terms not explicitly defined in this Agreement or the Plan but defined in the Option will have the same definitions as in the Option.

18. Purchase and Sale of Common Stock.

a. Agreement to purchase and sell Common Stock. Purchaser hereby agrees to purchase from the Company, and the Company hereby agrees to sell to Purchaser, shares of the Common Stock of the Company in accordance with the Notice of Exercise duly executed by Purchaser and attached hereto as Exhibit A.

b. Closing. The closing hereunder, including payment for and delivery of the Common Stock, will occur at the offices of the Company immediately following the execution of this Agreement, or at such other time and place as the parties may mutually agree; *provided, however,* that if stockholder approval of the Plan is required before the Option may be exercised, then the Option may not be exercised, and the closing will be delayed, until such stockholder approval is obtained. If such stockholder approval is not obtained within the time limit specified in the Plan, then this Agreement is null and void.

19. Unvested Share Repurchase Option.

a. Repurchase Option. In the event Purchaser's Continuous Service terminates, then the Company has an irrevocable option (the "***Repurchase Option***") for a period of six months after

said termination (or in the case of shares issued upon exercise of the Option after such date of termination, within six months after the date of the exercise), or such longer period as may be agreed to by the Company and Purchaser (the "***Repurchase Period***"), to repurchase from Purchaser or Purchaser's personal representative, as the case may be, those shares that Purchaser received pursuant to the exercise of the Option that have not as yet vested as of such termination date in accordance with the Vesting Schedule indicated on Purchaser's Stock Option Grant Notice (the "***Unvested Shares***").

b. Share Repurchase Price. The Company may repurchase all or any of the Unvested Shares at the lower of (i) the Fair Market Value of the such shares (as determined under the Plan) on the date of repurchase, or (ii) the price equal to Purchaser's Exercise Price for such shares as indicated on Purchaser's Stock Option Grant Notice.

20. Exercise of Repurchase Option. The Repurchase Option will be exercised by written notice signed by such person as designated by the Company, and delivered or mailed as provided herein. Such notice will identify the number of shares of Common Stock to be purchased and will notify Purchaser of the time, place and date for settlement of such purchase, which will be scheduled by the Company within the term of the Repurchase Option set forth above. In addition, the Company will be deemed to have exercised the Repurchase Option as of the last day of the Repurchase Period, unless an officer of the Company notifies the holder of the Unvested Shares during the Repurchase Period in writing (delivered or mailed as provided herein) that the Company expressly declines to exercise its Repurchase Option for some or all of the Unvested Shares. The Company will be entitled to pay for any shares of Common Stock purchased pursuant to its Repurchase Option at the Company's option in cash or by offset against any indebtedness owing to the Company by Purchaser (including without limitation any Promissory Note given in payment for the Common Stock), or by a combination of both. Upon exercise of the Repurchase Option and payment of the purchase price in any of the ways described above, the Company will become the legal and beneficial owner of the Common Stock being repurchased and all rights and interest therein or related thereto, and the Company will have the right to transfer to its own name the Common Stock being repurchased by the Company, without further action by Purchaser.

21. Capitalization Adjustments to Common Stock. In the event of a Capitalization Adjustment, then any and all new, substituted or additional securities or other property to which Purchaser is entitled by reason of Purchaser's ownership of Common Stock will be immediately subject to the Repurchase Option and be included in the word "Common Stock" for all purposes of the Repurchase Option with the same force and effect as the shares of the Common Stock presently subject to the Repurchase Option, but only to the extent the Common Stock is, at the time, covered by such Repurchase Option. While the total Option Price will remain the same after each such event, the Option Price per share of Common Stock upon exercise of the Repurchase Option will be appropriately adjusted.

22. Corporate Transactions. In the event of a Corporate Transaction, then the Repurchase Option may be assigned by the Company to the successor of the Company (or such successor's parent company), if any, in connection with such Corporate Transaction. To the extent the Repurchase Option remains in effect following such Corporate Transaction, it will apply to the new capital stock or other property received in exchange for the Common Stock in consummation of the Corporate Transaction, but only to the extent the Common Stock was at the time covered by such right. Appropriate adjustments will be made to the price per share payable upon exercise of the Repurchase Option to reflect the Corporate Transaction upon the Company's capital structure; *provided, however,* that the aggregate price payable upon exercise of the Repurchase Option remains the same.

23. Escrow of Unvested Common Stock. As security for Purchaser's faithful performance of the terms of this Agreement and to insure the availability for delivery of Purchaser's Common Stock upon exercise of the Repurchase Option herein provided for, Purchaser agrees, at the closing hereunder, to

deliver to and deposit with the Secretary of the Company or the Secretary's designee ("***Escrow Agent***"), as Escrow Agent in this transaction, three stock assignments duly endorsed (with date and number of shares blank) in the form attached hereto as Exhibit B, together with a certificate or certificates evidencing all of the Common Stock subject to the Repurchase Option; said documents are to be held by the Escrow Agent and delivered by said Escrow Agent pursuant to the Joint Escrow Instructions of the Company and Purchaser set forth in Exhibit C, attached hereto and incorporated by this reference, which instructions also will be delivered to the Escrow Agent at the closing hereunder.

24. Rights of Purchaser. Subject to the provisions of the Option, Purchaser will exercise all rights and privileges of a stockholder of the Company with respect to the shares deposited in escrow. Purchaser will be deemed to be the holder of the shares for purposes of receiving any dividends that may be paid with respect to such shares and for purposes of exercising any voting rights relating to such shares, even if some or all of such shares have not yet vested and been released from the Company's Repurchase Option.

25. Limitations on Transfer. In addition to any other limitation on transfer created by applicable securities laws, Purchaser will not sell, assign, hypothecate, donate, encumber or otherwise dispose of any interest in the Common Stock while the Common Stock is subject to the Repurchase Option. After any Common Stock has been released from the Repurchase Option, Purchaser will not sell, assign, hypothecate, donate, encumber or otherwise dispose of any interest in the Common Stock except in compliance with the provisions herein and applicable securities laws. Furthermore, the Common Stock is subject to any right of first refusal in favor of the Company or its assignees or other transfer restrictions that may be contained in the Company's Bylaws.

26. Restrictive Legends. All certificates representing the Common Stock will have endorsed thereon legends in substantially the following forms (in addition to any other legend which may be required by other agreements between the parties hereto):

a. "THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO AN OPTION SET FORTH IN AN AGREEMENT BETWEEN THE COMPANY AND THE REGISTERED HOLDER, OR SUCH HOLDER'S PREDECESSOR IN INTEREST, A COPY OF WHICH IS ON FILE AT THE PRINCIPAL OFFICE OF THIS COMPANY. ANY TRANSFER OR ATTEMPTED TRANSFER OF ANY SHARES SUBJECT TO SUCH OPTION IS VOID WITHOUT THE PRIOR EXPRESS WRITTEN CONSENT OF THE COMPANY."

b. "THE SHARES REPRESENTED BY THIS CERTIFICATE HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933 AS AMENDED. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF AN EFFECTIVE REGISTRATION STATEMENT AS TO THE SECURITIES UNDER SAID ACT OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY THAT SUCH REGISTRATION IS NOT REQUIRED."

c. "THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO A RIGHT OF FIRST REFUSAL OPTION IN FAVOR OF THE COMPANY AND/OR ITS ASSIGNEE(S) AS PROVIDED IN THE BYLAWS OF THE COMPANY AND IN AN AGREEMENT WITH THE COMPANY."

d. "THE SHARES REPRESENTED BY THIS CERTIFICATE WERE ISSUED PURSUANT TO THE EXERCISE OF **[AN INCENTIVE STOCK OPTION/ A NONSTATUTORY STOCK OPTION]**."

e. "THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO A TRANSFER RESTRICTION, AS PROVIDED IN THE BYLAWS OF THE COMPANY."

f. Any legend required by appropriate blue sky officials.

27. Investment Representations. In connection with the purchase of the Common Stock, Purchaser represents to the Company the following:

a. Purchaser is aware of the Company's business affairs and financial condition and has acquired sufficient information about the Company to reach an informed and knowledgeable decision to acquire the Common Stock. Purchaser is acquiring the Common Stock for investment for Purchaser's own account only and not with a view to, or for resale in connection with, any "distribution" thereof within the meaning of the Securities Act.

b. Purchaser understands that the Common Stock has not been registered under the Securities Act by reason of a specific exemption therefrom, which exemption depends upon, among other things, the bona fide nature of Purchaser's investment intent as expressed herein.

c. Purchaser further acknowledges and understands that the Common Stock must be held indefinitely unless the Common Stock is subsequently registered under the Securities Act or an exemption from such registration is available. Purchaser further acknowledges and understands that the Company is under no obligation to register the Common Stock. Purchaser understands that the certificate evidencing the Common Stock will be imprinted with a legend that prohibits the transfer of the Common Stock unless the Common Stock is registered or such registration is not required in the opinion of counsel for the Company.

d. Purchaser is familiar with the provisions of Rules 144 and 701, under the Securities Act, as in effect from time to time, which, in substance, permit limited public resale of "restricted securities" acquired, directly or indirectly, from the issuer thereof (or from an affiliate of such issuer), in a non-public offering subject to the satisfaction of certain conditions. Rule 701 provides that if the issuer qualifies under Rule 701 at the time of issuance of the securities, such issuance will be exempt from registration under the Securities Act. In the event the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the securities exempt under Rule 701 may be sold by Purchaser 90 days thereafter, subject to the satisfaction of certain of the conditions specified by Rule 144 and the market stand-off provision described in Purchaser's Stock Option Agreement.

e. In the event that the sale of the Common Stock does not qualify under Rule 701 at the time of purchase, then the Common Stock may be resold by Purchaser in certain limited circumstances subject to the provisions of Rule 144, which requires, among other things: (i) the availability of certain public information about the Company, and (ii) the resale occurring following the required holding period under Rule 144 after Purchaser has purchased, and made full payment of (within the meaning of Rule 144), the securities to be sold.

f. Purchaser further understands that at the time Purchaser wishes to sell the Common Stock there may be no public market upon which to make such a sale, and that, even if such a public market then exists, the Company may not be satisfying the current public current information requirements of Rule 144 or 701, and that, in such event, Purchaser would be precluded from selling the Common Stock under Rule 144 or 701 even if the minimum holding period requirement had been satisfied.

g. Purchaser further warrants and represents that Purchaser has either (i) preexisting personal or business relationships, with the Company or any of its officers, directors or controlling persons, or (ii) the capacity to protect his own interests in connection with the purchase of the Common Stock by virtue of the business or financial expertise of Purchaser or of professional advisors to Purchaser who are unaffiliated with and who are not compensated by the Company or any of its affiliates, directly or indirectly. Purchaser further warrants and represents that Purchaser's purchase the Common Stock was not accomplished by the publication of any advertisement.

28. Section 83(b) Election. Purchaser understands that Section 83(a) of the Code taxes as ordinary income the difference between the amount paid for the Common Stock and the fair market value of the Common Stock as of the date any restrictions on the Common Stock lapse. In this context, "restriction" includes the right of the Company to buy back the Common Stock pursuant to the Repurchase Option set forth above. Purchaser understands that Purchaser may elect to be taxed at the time the Common Stock is purchased, rather than when and as the Repurchase Option expires, by filing an election under Section 83(b) (an "***83(b) Election***") of the Code with the Internal Revenue Service within 30 days of the date of purchase, a copy of which is included as Exhibit D. Even if the fair market value of the Common Stock at the time of the execution of this Agreement equals the amount paid for the Common Stock, the 83(b) Election must be made to avoid income under Section 83(a) in the future. Purchaser understands that failure to file such an 83(b) Election in a timely manner may result in adverse tax consequences for Purchaser. Purchaser further understands that Purchaser must file an additional copy of such 83(b) Election with his or her federal income tax return for the calendar year in which the date of this Agreement falls. Purchaser acknowledges that the foregoing is only a summary of the effect of United States federal income taxation with respect to purchase of the Common Stock hereunder, and does not purport to be complete. Purchaser further acknowledges that the Company has directed Purchaser to seek independent advice regarding the applicable provisions of the Code, the income tax laws of any municipality, state or foreign country in which Purchaser may reside, and the tax consequences of Purchaser's death. Purchaser assumes all responsibility for filing an 83(b) Election and paying all taxes resulting from such election or the lapse of the restrictions on the Common Stock.

29. Refusal to Transfer. The Company is not required (a) to transfer on its books any shares of Common Stock of the Company which have been transferred in violation of any of the provisions set forth in this Agreement, or (b) to treat as owner of such shares or to accord the right to vote as such owner or to pay dividends to any transferee to whom such shares have been so transferred.

30. No Employment Rights. This Agreement is not an employment contract and nothing in this Agreement affects in any manner whatsoever the right or power of the Company or its Affiliates to terminate Purchaser's employment for any reason at any time, with or without cause and with or without notice.

31. Miscellaneous.

a. Notices. All notices required or permitted hereunder will be in writing and will be deemed effectively given: (i) upon personal delivery to the party to be notified, (ii) when sent by confirmed facsimile if sent during normal business hours of the recipient, and if not during normal business hours of the recipient, then on the next business day, (iii) five calendar days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (iv) one business day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All communications will be sent to the other party hereto at such party's address hereinafter set forth on the signature page hereof, or at such other address as such party may designate by 10 days advance written notice to the other party hereto.

b. Successors and Assigns. This Agreement will inure to the benefit of the successors and assigns of the Company and, subject to the restrictions on transfer herein set forth, be binding upon Purchaser, Purchaser's successors, and assigns. The Company may assign the Repurchase Option hereunder at any time or from time to time, in whole or in part.

c. Attorneys' Fees; Specific Performance. Purchaser will reimburse the Company for all costs incurred by the Company in enforcing the performance of, or protecting its rights under, any part of this Agreement, including reasonable costs of investigation and attorneys' fees. It is the intention of the parties that the Company, upon exercise of the Repurchase Option and payment for the shares repurchased, pursuant to the terms of this Agreement, will be entitled to receive the Common Stock, *in specie*, in order to have such Common Stock available for future issuance without dilution of the holdings of other stockholders. Furthermore, it is expressly agreed between the parties that money damages are inadequate to compensate the Company for the Common Stock and that the Company will, upon proper exercise of the Repurchase Option, be entitled to specific enforcement of its rights to purchase and receive said Common Stock.

d. Governing Law; Venue. This Agreement will be governed by and construed in accordance with the laws of the State of Delaware. The parties agree that any action brought by either party to interpret or enforce any provision of this Agreement will be brought in, and each party agrees to, and does hereby, submit to the jurisdiction and venue of, the appropriate state or federal court for the district encompassing the Company's principal place of business.

e. Further Execution. The parties agree to take all such further action(s) as may reasonably be necessary to carry out and consummate this Agreement as soon as practicable, and to take whatever steps may be necessary to obtain any governmental approval in connection with or otherwise qualify the issuance of the securities that are the subject of this Agreement.

f. Independent Counsel. Purchaser acknowledges that this Agreement has been prepared on behalf of the Company by Cooley LLP, counsel to the Company and that Cooley LLP does not represent, and is not acting on behalf of, Purchaser in any capacity. Purchaser has been provided with an opportunity to consult with Purchaser's own counsel with respect to this Agreement.

g. Entire Agreement; Amendment. This Agreement constitutes the entire agreement between the parties with respect to the subject matter hereof and supersedes and merges all prior agreements or understandings, whether written or oral. This Agreement may not be amended, modified or revoked, in whole or in part, except by an agreement in writing signed by each of the parties hereto.

h. Severability. If one or more provisions of this Agreement are held to be unenforceable under applicable law, the parties agree to renegotiate such provision in good faith. In the event that the parties cannot reach a mutually agreeable and enforceable replacement for such provision, then (i) such provision will be excluded from this Agreement, (ii) the balance of the Agreement will be interpreted as if such provision were so excluded and (iii) the balance of the Agreement will be enforceable in accordance with its terms.

i. Counterparts. This Agreement may be executed in two or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, Uniform Electronic Transactions Act or other applicable law) or other transmission method and any counterpart so delivered will be deemed to have been duly and validly delivered and be valid and effective for all purposes.

[Remainder of page intentionally left blank]

The parties hereto have executed this Agreement as of .

COMPANY:

TAYSHA GENE THERAPIES, INC.

By: _____

Name: _____

Title: _____

Email: _____

PURCHASER:

_____

(Signature)

_____

Name (Please Print)

_____

Email

TAYSHA GENE THERAPIES, INC.

RESTRICTED STOCK AWARD GRANT NOTICE
(2020 EQUITY INCENTIVE PLAN)

Taysha Gene Therapies, Inc. (the "***Company***"), pursuant to its 2020 Equity Incentive Plan (the "***Plan***"), hereby awards to Participant, in consideration for Participant's past or future services actually or to be rendered to the Company, the number of shares of Common Stock (the "***Shares***") set forth below (the "***Award***"). The Award is subject to all of the terms and conditions as set forth in this Restricted Stock Award Grant Notice (the "***Grant Notice***") and the attached Restricted Stock Award Terms and Conditions (together with the Grant Notice, the "***Award Agreement***"), and the Plan, all of which are attached to this Grant Notice and incorporated into this Grant Notice in their entirety. Capitalized terms not explicitly defined in the Award Agreement but defined in the Plan will have the meanings provided in the Plan. If the Company uses an electronic capitalization table system (such as Carta or Shareworks) and the fields below are blank or the information is otherwise provided in a different format electronically, the blank fields and other information (such as exercise schedule and type of grant) shall be deemed to come from the electronic capitalization system and is considered part of this Grant Notice.

Participant:	_____
Date of Grant:	_____
Vesting Commencement Date:	_____
Number of Shares Subject to Award:	_____
Consideration:	[Participant's services]

Vesting Schedule: [**Sample of standard vesting.** 12/48ths of the total shares will vest on the one-year anniversary of the Vesting Commencement Date, and 1/48th of the total shares will vest each month thereafter on the same day of the month as the Vesting Commencement Date (or if there is no corresponding day, on the last day of the month), subject to Participant's Continuous Service as of each such date].

Additional Terms/Acknowledgements: Participant acknowledges receipt of a copy of the Plan and represents that he or she is familiar with the terms and provisions thereof, and hereby accepts this Award subject to all of the terms and provisions of the Plan and this Award Agreement (including all attachments and exhibits) and has had an opportunity to obtain the advice of counsel prior to executing and accepting the Award. By accepting this Award, Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Board upon any questions arising under the Plan or this Award.

Participant further consents to receive any documents related to the Plan by electronic delivery and to participate in the Plan through an online or electronic system established and maintained by the Company or a third party designated by the Company.

Participant further acknowledges that as of the Date of Grant, this Award Agreement and the Plan set forth the entire understanding between Participant and the Company regarding the acquisition of stock in the Company and supersede all prior oral and written agreements on that subject, with the exception of (i) options, restricted stock awards or other compensatory stock awards previously granted and delivered to Participant, and (ii) any written employment or severance arrangement that would provide for vesting acceleration of this Award upon the terms and conditions set forth therein.

Participant further acknowledges that this Award Agreement has been prepared on behalf of the Company by Cooley LLP, counsel to the Company and that Cooley LLP does not represent, and is not acting on behalf of, Participant in any capacity. Participant has been provided with an opportunity to consult with Participant's own counsel with respect to this Award Agreement.

This Award may be executed in one or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, Uniform Electronic Transactions Act or other applicable law) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes.

Taysha Gene Therapies, Inc.

By: _____
Signature

Title: _____

Date: _____

Participant:

_____
Signature

Date: _____

EXECUTIVE EMPLOYMENT AGREEMENT

THIS EXECUTIVE EMPLOYMENT AGREEMENT ("Agreement") is signed and effective as of April 1, 2020 (the "Effective Date"), by and between Taysha Gene Therapies, Inc., a Delaware corporation (the "Company"), and R.A. Session II, an individual (the "Executive").

The Company desires to employ the Executive, and the Executive is willing to be employed by the Company, upon the terms and subject to the conditions contained herein.

NOW, THEREFORE, intending to be legally bound, the Company agrees to employ Executive, and Executive hereby agrees to be employed by the Company, upon the following terms and conditions:

1. Position. Until the Executive resigns from the Company or is terminated, the Executive shall serve as the Company's Chief Executive Officer and President. The Executive shall perform those duties generally required of persons in such position as well as such other duties assigned to Executive from time to time by the Company's Board of Directors (the "Board") or its designee. The Executive shall be subject to the authority of the Board and shall comply with all Company policies.

2. Scope of Services. During the Executive's employment with the Company, the Executive agrees to devote substantially all of Executive's business time, attention, skills and efforts to the business of the Company, subject to the terms set forth herein. The Company acknowledges that Executive will continue to serve on the following Boards of Directors: (i) ReCode Therapeutics, Inc.; (ii) Sandhill Therapeutics, Inc.; and (iii) Lung Therapeutics, Inc.

3. Salary, Compensation and Benefits.

3.1 Base Salary. The Company agrees to pay, and the Executive agrees to accept, as the Executive's salary for all services to be rendered by the Executive hereunder, an annual base salary of $450,000 ("Base Salary"), payable at the same time that the Company pays its executives generally, but no less frequently than monthly; provided, that the Base Salary shall be subject to adjustment in the discretion of the Board.

3.2 Performance Bonus. During the period the Executive is employed by the Company, the Executive will be eligible to earn, in the discretion of the Board, an annual performance bonus in an amount equal to fifty percent (50%) of the Executive's Base Salary for the applicable year (the "Performance Bonus"). Whether or not the Executive earns any Performance Bonus will be dependent upon (a) the Executive's continuous performance of services to the Company through the end of the period with respect to which performance is being reviewed; and (b) achievement of Company and individual performance goals (the "Performance Goals"). Any Performance Bonus with respect to a calendar year, if earned, shall be paid between January 1 and March 15 of the immediately following calendar year; and provided further that, except as provided in Section 4.3, in no event shall the Executive be eligible for a Performance Bonus with respect to a calendar year if the Executive separates from service with the Company prior to the date on which such Performance Bonus is paid. The Performance Goals will be set in

advance in writing and revised annually, semi-annually, or quarterly by the Board together with input from the Executive, and the Board will in its sole discretion assess the performance of the Executive and the Company against these Performance Goals in its determination of what amount, if any, of the Performance Bonus will be awarded. The Board will inform the Executive of the Performance Goals upon Executive's request.

3.3 Equity Incentives.

3.3.1 Upon approval of any incentive plan for Company employees by the Board, the Company will grant the Executive restricted shares equal to 3% of the Company's then outstanding Common Stock (the "Grant"). The Grant will vest one-third on the first anniversary of the date of this Agreement, with the remainder of the Grant vesting monthly over a period of two years, subject to the Executive's continuous service to the Company on each such date; provided, that, subject to the remainder of this Agreement, if (i) the Company undergoes a Change in Control (as defined below), (ii) the Company (or any parent, subsidiary or affiliate of the Company or successor in interest to this Agreement) terminates the Executive's employment with or engagement by the Company other than for Cause (as defined below), (iii) the Executive dies or becomes permanently disabled (as determined reasonably by his physician), or (iv) the Executive terminates his employment with or engagement by the Company (or any parent, subsidiary or affiliate of the Company or successor in interest to this Agreement) for Good Reason (as defined below), then vesting of all shares underlying the Grant, along with the vesting of any other Company stock or other equity incentives of the Company held by the Executive on the Date of Termination (as defined below) that remain unvested on the Date of Termination, shall accelerate in full (the "Acceleration Benefit").

3.3.2 "Cause" shall mean termination for one or more of the following reasons: (i) material failure to follow any proper and lawful directive of the Board that remains uncured more than thirty (30) days after a written demand is delivered to the Executive that specifically identifies the manner in which the Board believes that the Executive has failed to follow such instructions, provided, that failure to meet performance targets shall not, in and of itself, be deemed a failure to follow any such instructions; (ii) the Executive's commission of an act of (a) fraud, embezzlement, or theft or (b) dishonesty that injures the business, business reputation or business relationships of the Company; (iii) the Executive's conviction of, or pleading guilty or nolo contendere to, a felony; and (iv) material violation of any agreement between the Executive and Company that remains uncured more than thirty (30) days after written notice thereof is delivered to Executive that specifically identities such violation. "Good Reason" shall mean a termination by the Executive within thirty (30) days after the occurrence of one or more of the following events without the consent of the Executive: (A) a material breach of an agreement between the Company and the Executive by the Company that remains uncured more than thirty (30) days

after written notice thereof is delivered to the Company that specifically identities such breach; (B) the Company significantly reduces the Executive's Base Salary or the percentage eligibility established for the Executive's Performance Bonus, other than any Company-wide reduction in compensation of employees; (C) the Company significantly reduces the Executive's duties, authority or responsibilities relative to Executive's duties, authority or responsibilities in effect immediately prior to such reduction; or (D) the Company relocates the facility that is the Executive's principal place of business with the Company to a location more than fifty (50) miles from the immediately preceding location (excluding regular travel in the ordinary course of business). "Change of Control" shall mean the occurrence of any one or more of the following events: (x) any person (within the meaning of Section 13(d) or 14(d) of the Securities Exchange Act of 1934, as amended) becomes the owner, directly or indirectly, of securities of the Company representing more than fifty percent of the combined voting power of the Company's then outstanding securities (other than in connection with a transaction involving the issuance of securities by the Company the principal purpose of which is to raise capital for the Company); (y) there is consummated a merger, consolidation or similar transaction to which the Company is a party and the stockholders of the Company immediately prior thereto do not own outstanding voting securities representing more than fifty percent of the combined outstanding voting power of the surviving entity immediately following such merger, consolidation or similar transaction or more than fifty percent of the combined outstanding voting power of the parent of the surviving entity immediately following such merger, consolidation or similar transaction; or (z) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its subsidiaries, other than a sale, lease or other disposition of all or substantially all of the consolidated assets of the Company and its subsidiaries to an entity more than fifty percent (50%) of the combined voting power of which is owned immediately following such disposition by the stockholders of the Company immediately prior thereto.

3.4 Conflicts. In the event of any conflict between the terms of this Agreement and the terms of any Grant documentation, including without limitation vesting terms or the definition of "Cause," the terms of this Agreement shall govern.

3.5 Vacation. In addition to statutory holidays, the Executive shall be entitled to such number of days of paid vacation each calendar year that the Company provides to executive-level employees, which, if the Company's accrues vacation time, shall accrue ratably each month and be administered in accordance with the Company's policies in effect from time to time. No unused vacation time of the Executive may be carried forward to the next year unless the Company has a policy requiring or allowing otherwise.

3.6 Benefits. During the period the Executive is employed by the Company, the Executive shall be entitled to participate in such employee benefits that the Company makes

available from time to time to executive-level employees generally, on a basis no less favorable than such executive-level employees, subject to the terms and conditions applicable to such benefits or benefit plans.

3.7 Reimbursement. The Company shall reimburse the Executive for (or, in the Company's sole discretion, shall pay directly) reasonable out-of-pocket expenses incurred by the Executive in accordance with any expense reimbursement policy adopted by the Company relating to the business or affairs of the Company or the performance of the Executive's duties hereunder, including, without limitation, reasonable expenses with respect to entertainment, travel and similar items. All reimbursements shall be paid as soon as administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred.

3.8 Withholding. The Company will withhold from the Executive's compensation all applicable amounts required by law or authorized by the Executive.

4. Payments Upon Termination of Employment.

4.1 Termination for Any Reason. In the event that the Executive's employment with the Company terminates for any reason, the Company shall pay to the Executive any unpaid Base Salary earned through the Executive's last day of employment (the "Date of Termination") in accordance with this Agreement and expense reimbursements accrued but unpaid as of the Date of Termination (the "Accrued Benefit").

4.2 Benefits Upon Termination Without Cause.

4.2.1 If the Company terminates the Executive's employment for a reason other than for Cause, or the Executive terminates his employment with the Company for Good Reason, the Company will provide the Executive with the Acceleration Benefit and the following "Salary Continuation Payments" (collectively, the "Severance Benefit"), provided, that: (i) the Executive complies with the provisions set forth in Section 4.2.4, (ii) the Executive enters into a general release reasonably acceptable to the Company (the "Release"), and (iii) such general release becomes effective and irrevocable prior to the Executive's receipt of any Severance Benefits: continuation of the Executive's Base Salary for the twelve (12) month period that immediately follows the Date of Termination (the "Severance Period").

4.2.2 The Severance Benefit will be subject to standard payroll deductions and withholdings and will payable in equal installments over the Severance Period on the Company's ordinary payroll dates, beginning no later than the Company's second payroll date that occurs after the effective date of the Release, with the remaining installments occurring on the Company's regularly scheduled payroll dates thereafter, in cash or immediately available funds.

4.2.3 The Severance Benefit and Acceleration Benefit are further conditioned on the following: (i) if the Executive holds any other positions with the Company, including as member of the Board or any boards of directors of any subsidiaries, the Executive resigns such position(s) to be effective no later than the Date of Termination (or such other date as requested by the Board); (ii) the Executive returns all Company property; and (iii) the Executive complies with the terms of the Release, including without limitation any non-disparagement and confidentiality provisions contained therein.

4.2.4 In exchange for the Severance Benefit and the Acceleration Benefit, Executive agrees that Executive shall not, during and for a period of 12 months following the Date of Termination, or, if a court of competent jurisdiction determines that such period is unenforceable, during and for a period of 9 months following the Date of Termination, or, if a court of competent jurisdiction determines that such period is unenforceable, during and for a period of 6 months following the Date of Termination, or, if a court of competent jurisdiction determines that such period is unenforceable, during and for a period of 3 months following the Date of Termination, directly or indirectly, for himself or any third party other than the Company:

4.2.4.1 own any interest in, manage, control, participate in, render services for, be employed in an executive, managerial or administrative capacity by, or in any manner engage in any business, if such business's product or service (a) competes with any product or service sold or provided by the Company, (b) competes with any product or service intended to be sold or provided by the Company on the Date of Termination, or (c) competed with any product or service sold or provided by the Company at any time during Executive's employment with the Company, in each case, anywhere in the world where the Company does business;

4.2.4.2 solicit sales from any of the Company's customers for any product or service that (a) competes with any product or service sold or provided by the Company, (b) competes with any product or service intended to be sold or provided by the Company at the time of the termination of Executive's employment with the Company, or (c) competed with any product or service sold or provided by the Company at any time during Executive's employment with the Company;

4.2.4.3 entice any vendor, consultant, collaborator, agent, or contractor of the Company to cease its business relationship with the Company or engage in any activity that would cause them to cease their business relationship with the Company; or

4.2.4.4 solicit, induce, recruit, or encourage any of the Company's employees to leave their employment, or attempt to solicit, induce, recruit, encourage, or take away Company employees.

4.3 Death or Disability of the Executive. If the Executive dies or becomes permanently disabled (as determined reasonably by his physician), the Company will provide the Executive with the Acceleration Benefit.

4.4 Termination of Employment as a Result of Executive's Resignation without Good Reason, or a Termination by the Company for Cause. In the event the Executive's employment is terminated as a result of the Executive's (i) termination for Cause by the Company, or (ii) Executive's resignation without Good Reason, the Company's sole obligation shall be to pay Executive, within sixty (60) days of the Date of Termination, or an earlier date if required by law, the amount of the Accrued Benefit.

5. Section 409A.

5.1 Solely for purposes of Section 409A of the Internal Revenue Code of 1986, as amended (the "Code"), the Severance Benefit (and the separate payment of any portion thereof) is considered a separate payment. Anything in this Agreement to the contrary notwithstanding, if at the time of the Executive's separation from service within the meaning of Section 409A of the Code, the Company determines that the Executive is a "specified employee" within the meaning of Section 409A(a)(2)(B)(i) of the Code, then to the extent any payment or benefit that the Executive becomes entitled to under this Agreement on account of the Executive's separation from service would be considered deferred compensation subject to the 20 percent additional tax imposed pursuant to Section 409A(a) of the Code as a result of the application of Section 409A(a)(2)(B)(i) of the Code, such payment shall not be payable and such benefit shall not be provided until the date that is the earlier of (i) six months and one day after the Executive's separation from service, or (ii) the Executive's death. If any such delayed cash payment is otherwise payable on an installment basis, the first payment shall include a catch-up payment covering amounts that would otherwise have been paid during the six-month period but for the application of this provision, and the balance of the installments shall be payable in accordance with their original schedule. No interest shall accrue on any such delayed cash payment.

5.2 All in-kind benefits provided and expenses eligible for reimbursement under this Agreement shall be provided by the Company or incurred by the Executive during the time periods set forth in this Agreement. All reimbursements shall be paid as soon as administratively practicable, but in no event shall any reimbursement be paid after the last day of the taxable year following the taxable year in which the expense was incurred. The amount of in-kind benefits provided or reimbursable expenses incurred in one taxable year shall not affect the in-kind benefits to be provided or the expenses eligible for reimbursement in any other taxable year. Such right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit.

5.3 To the extent that any payment or benefit described in this Agreement constitutes "non-qualified deferred compensation" under Section 409A of the Code, and to the extent that such payment or benefit is payable upon the Executive's termination of employment,

then such payments or benefits shall be payable only upon the Executive's "separation from service." The determination of whether and when a separation from service has occurred shall be made in accordance with the presumptions set forth in Treasury Regulation Section 1.409A 1(h).

5.4 The parties intend that this Agreement will be administered in accordance with Section 409A of the Code. To the extent that any provision of this Agreement is ambiguous as to its compliance with Section 409A of the Code, the provision shall be read in such a manner so that all payments hereunder comply with Section 409A of the Code. The parties agree that this Agreement may be amended, as reasonably requested by either party, and as may be necessary to fully comply with Section 409A of the Code and all related rules and regulations in order to preserve the payments and benefits provided hereunder without additional cost to either party.

6. Executive's Representations and Warranties. The Executive represents and warrants that the Executive is not a party to any other employment, non-competition, or other agreement or restriction which could interfere with the Executive's employment by the Company or the Executive's or the Company's rights and obligations hereunder and that the Executive's acceptance of employment by the Company pursuant to the terms of this Agreement and the performance of the Executive's duties hereunder will not breach the provisions of any contract, agreement, or understanding to which the Executive is party or any duty owed by the Executive to any other person.

7. Waivers and Amendments. The respective rights and obligations of the Company and the Executive under this Agreement may be waived (either generally or in a particular instance, either retroactively or prospectively, and either for a specified period of time or indefinitely) or amended only with the written consent of a duly authorized representative of the Company and the Executive. The waiver by either party of a breach of any provision of this Agreement by the other party shall not operate or be construed as a waiver of any subsequent breach by such other party.

8. Successors and Assigns. The provisions hereof shall inure to the benefit of, and be binding upon, the Executive and the Company, along with the Company's successors and assigns. The Executive may not assign or delegate to any third person the Executive's obligations under this Agreement. The rights and benefits of the Executive under this Agreement are personal to the Executive and inure solely to the benefit of the Executive and to the Executive's estate, as applicable.

9. Entire Agreement. This Agreement, the Grant Documentation, and the other agreements referenced herein, constitute the full and entire understanding and agreement of the parties with regard to the subjects hereof and supersede in their entirety all other or prior agreements, whether oral or written, with respect thereto.

10. Notices. All demands, notices, requests, consents and other communications required or permitted under this Agreement shall be in writing and shall be personally delivered or sent by reputable commercial overnight delivery service (including Federal Express and U.S. Postal Service overnight delivery service) or deposited with the U.S. Postal Service mailed first class, registered or certified mail, postage prepaid, as set forth below:

If to the Company, addressed to:

Taysha Gene Therapies, Inc.
2280 Inwood Road
Dallas, TX 75235

and, if to the Executive, to the address set forth in the Company's records.

Notices shall be deemed given upon the earlier to occur of (i) receipt by the party to whom such notice is directed; (ii) on the first business day (other than a Saturday, Sunday or legal holiday in the jurisdiction to which such notice is directed) following the day the same is deposited with the commercial courier if sent for overnight delivery by commercial delivery service; or (iii) the fifth day (other than a Saturday, Sunday or legal holiday in the jurisdiction to which such notice is directed) following deposit thereof with the U.S. Postal Service as aforesaid. Each party, by notice duly given in accordance therewith, may specify a different address for the giving of any notice hereunder.

11. Governing Law. This Agreement shall be construed and enforced in accordance with and governed by the laws of the State of Texas (without giving effect to any conflicts or choice of laws provisions thereof that would cause the application of the domestic substantive laws of any other jurisdiction).

12. Employment at Will. The Executive and the Company understand and agree that the Executive is an employee at-will, and that the Executive may resign, or the Company may terminate the Executive's employment, at any time, with or without cause or advance notice. Nothing in this Agreement shall be construed to alter the at-will nature of the Executive's employment, nor shall anything in this Agreement be construed as providing the Executive with a definite term of employment. The provisions in Section 4 above govern the amount of compensation, if any, to be provided to the Executive upon termination of employment and do not alter this at-will status. The at-will nature of the Executive's employment with the Company may be changed only in an express written agreement signed by the Executive and an officer of the Company.

13. Severability; Titles and Subtitles; Gender; Singular and Plural; Counterparts; Facsimile.

(i). In case any provision of this Agreement shall be invalid, illegal or unenforceable, the validity, legality and enforceability of the remaining provisions of this Agreement shall not in any way be affected or impaired thereby.

(ii). The titles of the sections and subsections of this Agreement are for convenience of reference only and are not to be considered in construing this Agreement.

(iii). The use of any gender in this Agreement shall be deemed to include the other genders, and the use of the singular in this Agreement shall be deemed to include the plural (and vice versa), wherever appropriate.

(iv). This Agreement may be executed in any number of counterparts, each of which shall be an original, but all of which together constitute one instrument.

(v). Counterparts of this Agreement (or applicable signature pages hereof) that are manually signed and delivered by facsimile transmission shall be deemed to constitute signed original counterparts hereof and shall bind the parties signing and delivering in such manner.

[signature page follows]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the Effective Date.

TAYSHA GENE THERAPIES, INC.

By: /s/ R.A. Session II
Name: R.A. Session II
Title: President

R.A. SESSION II

/s/ R.A. Session II

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the use in this Registration Statement on Form S-1 of our report dated July 31, 2020, relating to the financial statements of Taysha Gene Therapies, Inc. We also consent to the reference to us under the heading “Experts” in such Registration Statement.

/s/ Deloitte & Touche LLP

Dallas, Texas
September 2, 2020