Taysha Gene Therapies Reports First Quarter 2022 Financial Results and Provides Corporate Update

Initiated clinical development of TSHA-102 for Rett Syndrome under recently approved Clinical Trial Application (CTA) with preliminary Phase 1/2 data expected by year-end 2022

Received Orphan Drug Designation from the European Commission for TSHA-120 for giant axonal neuropathy (GAN) and recently completed commercially representative GMP batch; regulatory update expected in mid-2022

Existing cash and cash equivalents, along with full access to the term loan facility, is expected to fund operating expenses and capital requirements into the fourth quarter of 2023

DALLAS, May 16, 2022 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric, pivotal-stage 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, today reported financial results for the first quarter ended March 31, 2022 and provided a corporate update.

"In 2022, we are focused on advancing our key programs in Rett syndrome and GAN. We initiated clinical development of TSHA-102 in Rett syndrome and expect preliminary clinical data from the REVEAL study by year-end," said RA Session II, President, Founder and CEO of Taysha. "Recently, the European Commission granted orphan drug designation for TSHA-120 for GAN, further highlighting the unmet need for treatment options for these patients and the important potential of TSHA-120. We have completed a commercially representative GMP batch for TSHA-120 with release testing currently underway. Our existing capital resources, along with full access to the term loan facility, should fund operating expenses and capital requirements into the fourth quarter of 2023."

Recent Corporate Highlights

TSHA-120 for giant axonal neuropathy (GAN): an intrathecally dosed AAV9 gene therapy currently being evaluated in a clinical trial for the treatment of GAN, a rare inherited genetic disorder that affects both the central and peripheral nervous systems and is caused by loss-of-function mutations in the gene coding for *gigaxonin.* TSHA-120 is designed to deliver a functional copy of the GAN gene to the CNS and PNS. TSHA-120 has already received orphan drug and rare pediatric disease designations from the U.S. Food and Drug Administration (FDA) and orphan drug designation from the European Commission.

- Received orphan drug designation from the European Commission for GAN in April 2022
- Reported positive clinical efficacy and safety data for high dose cohort and long-term durability data across all therapeutic dose cohorts for TSHA-120 in GAN
    - Efficacy data for high dose cohort demonstrated clinically meaningful and statistically significant improvement in MFM32 by Year 1 compared to natural history (n=3)
    - Long-term durability data across all therapeutic dose cohorts demonstrated a 10-point improvement in mean change in MFM32 by Year 3 compared to estimated natural history decline of 24 points (n=5)
    - Biopsy data in five of six patient samples analyzed to date confirmed active regeneration of nerve fibers following treatment with TSHA-120 (n=6)
    - 53 patient-years of clinical data support the safety and tolerability profile of TSHA-120
- Commercially representative GMP batch completed and release testing underway
- Regulatory feedback for TSHA-120 in GAN expected mid-2022

TSHA-102 in Rett syndrome: a self-complementary intrathecally delivered AAV9 gene replacement therapy under development for the treatment of Rett syndrome. TSHA-102 utilizes the novel miRARE platform to regulate transgene expression genotypically on a cell-by-cell basis. TSHA-102 is the first-and-only gene therapy in clinical development for Rett syndrome. TSHA-102 has received orphan drug and rare pediatric disease designations from the FDA and has been granted orphan drug designation from the European Commission.

- CTA approved by Health Canada in March 2022
- Published preclinical data for TSHA-102 in Rett syndrome presented at the International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting and the ASCEND Rett Syndrome National Summit in April 2022
- Initiation of clinical development with the REVEAL study, an open-label, dose escalation, randomized, multicenter Phase 1/2 clinical trial in adult female patients with Rett syndrome
    - Sainte-Justine Mother and Child University Hospital Center in Montreal, Quebec, Canada selected as initial clinical site under the direction of Dr. Elsa Rossignol, principal investigator
    - Key assessments to include Rett-specific and global assessments, quality of life, biomarkers and neurophysiology and imaging
- Preliminary clinical data for TSHA-102 in Rett syndrome expected by year-end 2022

AAV9 Gene Replacement for CLN7 Batten disease: an investigational AAV9 intrathecally dosed gene replacement therapy designed to deliver a full-length copy of the *CLN7* gene to potentially treat CLN7 disease, a rapidly progressing rare lysosomal storage disease with no approved

treatments. The clinical development of the CLN7 program is being funded by UT Southwestern (UTSW), Children's Health and Children's Medical Center Foundation.

- Reported positive preliminary clinical safety data for first-generation construct in CLN7 Batten disease from UTSW-sponsored clinical trial
  - Data from three patients dosed presented at the 18th Annual WORLDSymposium
  - Fourth patient with CLN7 disease dosed at 1.0 x 10^{15} total vg

Anticipated Milestones

- Regulatory update for TSHA-120 in GAN by mid-2022
- Preliminary clinical data from the REVEAL study for TSHA-102 in Rett syndrome by year-end 2022
- Initiation of clinical development for TSHA-105 in SLC13A5 deficiency
- Continued clinical development of the first-generation construct for CLN7 disease in 2022
- Continued clinical development for TSHA-118 in CLN1 disease

First Quarter 2022 Financial Highlights

Research and Development (R&D) Expenses: Research and development expenses were $37.8 million for the three months ended March 31, 2022, compared to $23.9 million for the three months ended March 31, 2021. The increase of approximately $13.9 million was primarily attributable to an increase of $9.3 million in employee compensation, which included $2.2 million of severance and one-time termination costs in connection with the strategic reprioritization of programs completed in March 2022 and $1.0 million of non-cash stock-based compensation. Additionally, in the three months ended March 31, 2022, we incurred an increase of $2.9 million of expenses in research and development manufacturing and other raw material purchases. We also incurred an increase of $1.7 million of third-party research and development consulting fees, primarily related to GLP toxicology studies and clinical study activities.

General and Administrative (G&A) Expenses: General and administrative expenses were $11.5 million for the three months ended March 31, 2022, compared to $8.2 million for the three months ended March 31, 2021. The increase of approximately $3.3 million was primarily attributable to $2.9 million of incremental compensation expense, which included $0.4 million of severance and one-time termination costs and $0.7 million of non-cash stock-based compensation. We also incurred an increase of $0.4 million in professional fees related to insurance, investor relations/communications, accounting, and market research.

Net loss: Net loss for the three months ended March 31, 2022 was $50.1 million, or $1.31 per share, as compared to a net loss of $32.0 million, or $0.87 per share, for the three months ended March 31, 2021.

Cash and cash equivalents: As of March 31, 2022, we had $96.6 million in cash and cash equivalents. This excludes approximately $12 million in gross proceeds generated from the sale of common stock, par value $0.00001 per share, under our existing at-the-market facility in April 2022.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 8:00 am ET / 7:00 am CT to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13729044. The live webcast and replay may be accessed by visiting Taysha's website at https://ir.tayshagtx.com/news-events/events-presentations. An archived version of the webcast will be available on the website for 30 days.

About Taysha Gene Therapies

Taysha Gene Therapies (Nasdaq: TSHA) is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class UT Southwestern Gene Therapy Program to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," "plans," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of our product candidates, including our preclinical product candidates, to positively impact quality of life and alter the course of disease in the patients we seek to treat, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and whether, if approved, these product candidates will be successfully distributed and marketed, the potential market opportunity for these product candidates, our corporate growth plans, the forecast of our cash runway and the implementation and potential impacts of our strategic pipeline prioritization initiatives. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission ("SEC") filings, including in our Annual Report on Form 10-K for the full-year ended December 31, 2021 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2022, both of which are available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and we disclaim any obligation to update these statements except as may be required by law.

Taysha Gene Therapies, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share data)
(Unaudited)

	For the Three Months Ended March 31,	
	2022	2021
Operating expenses:		
Research and development	$ 37,799	$ 23,854
General and administrative	11,469	8,236
Total operating expenses	49,268	32,090
Loss from operations	(49,268)	(32,090)
Other income (expense):		
Interest income	14	66
Interest expense	(849)	-
Other expense	(8)	-
Total other expense, net	(843)	66
Net loss	**$ (50,111)**	**$ (32,024)**
Net loss per common share, basic and diluted	$ (1.31)	$ (0.87)
Weighted average common shares outstanding, basic and diluted	38,174,717	36,992,377

Taysha Gene Therapies, Inc.
Condensed Consolidated Balance Sheet Data
(in thousands, except share and per share data)
(Unaudited)

	March 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 96,630	$ 149,103
Prepaid expenses and other current assets	10,261	10,499
Total current assets	106,891	159,602
Restricted cash	2,637	2,637
Deferred lease asset	655	667
Property, plant and equipment, net	55,120	50,610
Other non-current assets	673	440
Total assets	**$ 165,976**	**$ 213,956**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 21,997	$ 21,763
Accrued expenses and other current liabilities	26,620	29,983
Total current liabilities	48,617	51,746
Build-to-suit lease liability	25,752	25,900
Term Loan, net	37,386	37,192
Other non-current liabilities	3,496	3,735
Total liabilities	115,251	118,573
Stockholders' equity		
Preferred stock, $0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of March 31, 2022 and December 31, 2021	-	-
Common stock, $0.00001 par value per share; 200,000,000 shares authorized and 38,473,945 issued and outstanding as of March 31, 2022 and December 31, 2021	-	-
Additional paid-in capital	336,485	331,032
Accumulated deficit	(285,760)	(235,649)
Total stockholders' equity	50,725	95,383
Total liabilities and stockholders' equity	**$ 165,976**	**$ 213,956**

Company Contact:
Kimberly Lee, D.O.
Chief Corporate Affairs Officer
Taysha Gene Therapies
klee@tayshagtx.com

Media Contact:
Carolyn Hawley
Canale Communications
carolyn.hawley@canalecomm.com

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