Taysha Gene Therapies Reports First Quarter 2023 Financial Results and Provides Corporate Update

Screening completed and dosing scheduled for first potential subject in the Phase 1/2 REVEAL trial in Rett syndrome; dosing of first adult patient with TSHA-102 expected in Q2 2023; initial available Phase 1/2 clinical data, primarily on safety, expected in Q2 2023

Clinical Trial Application (CTA) submission to United Kingdom (UK) MHRA for TSHA-102 in pediatric patients with Rett syndrome expected in mid-2023; Investigational New Drug (IND) application to United States (U.S.) Food and Drug Administration (FDA) in Rett syndrome anticipated in H2 2023

New preclinical data for TSHA-102 in Rett syndrome to be presented during a poster presentation at the upcoming American Society of Gene and Cell Therapy (ASGCT) 26th Annual Meeting

R&D Day in June 2023 will overview new findings from totality of data evaluation, including comprehensive analyses of functional, biological, and electrophysiological assessments of TSHA-120 in giant axonal neuropathy (GAN), and provide an update on TSHA-102 in Rett syndrome

Formal FDA meeting request submission to discuss regulatory path forward for TSHA-120 in GAN expected in Q2 2023; formal meeting anticipated in Q3 2023

Conference call and live webcast today at 4:30 PM Eastern Time

DALLAS, May 11, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a clinical-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), today reported financial results for the first quarter ended March 31, 2023, and provided a corporate update.

"We continue to make significant progress with our two lead clinical programs and remain on track to deliver on multiple key milestones, including the generation of first-in-human clinical data for TSHA-102 in adult patients with Rett syndrome, the submission of a CTA to the MHRA to initiate expansion of TSHA-102 in pediatric patients, the submission of an IND application to the FDA for TSHA-102, and obtaining further clarity from the FDA on the regulatory path forward for TSHA-120 in GAN," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "Screening is completed, and dosing is now scheduled for our first potential patient in the adult Rett syndrome study. For GAN, our comprehensive analyses of the totality of data for TSHA-120 continues to be encouraging and includes compelling findings with potential to further support a regulatory path forward."

Sukumar Nagendran, M.D., President, and Head of R&D added, "We plan to seek a formal meeting with the FDA to discuss the totality of findings from the functional, biological, and electrophysiological assessments of TSHA-120 in GAN, anticipated in the third quarter of this year. In the near term, we look forward to hosting an R&D Day in June where we will overview the GAN disease state and share the comprehensive analyses, as well as provide an update on our Rett program. For TSHA-102, new preclinical data supporting the efficacy and safety of TSHA-102 and the miRARE technology in Rett syndrome will be presented as part of a poster presentation at the upcoming ASGCT conference. We believe that the clinical and preclinical data generated to date across our Rett syndrome and GAN programs reinforce our gene therapy approach, and the therapeutic potential to address severe unmet needs in monogenic central nervous system disease."

Recent Corporate Highlights

TSHA-102 in Rett syndrome: a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare genetic neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression. 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.

- Phase 1/2 REVEAL trial in adult patients with Rett syndrome
  - Completed screening and scheduled dosing for first potential adult patient with dosing anticipated in Q2 2023
  - Initial available Phase 1/2 clinical data, primarily on safety, expected in Q2 2023, with planned quarterly updates on available clinical data thereafter
  - Continued dosing of adult patients with Rett syndrome in the REVEAL trial in H2 2023
- CTA submission to UK MHRA for TSHA-102 in pediatric patients with Rett syndrome anticipated in mid-2023
- IND application submission to U.S. FDA for Rett syndrome expected in H2 2023
- New preclinical data for TSHA-102 in Rett syndrome to be presented as a poster presentation at the upcoming ASGCT 26th Annual Meeting on Friday, May 19 at 12-2 PM PT; these data and available clinical data from Phase 1/2 REVEAL trial will be presented in upcoming R&D Day in June

TSHA-120 for giant axonal neuropathy (GAN): a self-complimentary intrathecally delivered AAV9 gene therapy in clinical evaluation for GAN, an ultra-rare inherited genetic neurodegenerative disorder with no approved treatments. TSHA-120 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission.

- Completed CMC module 3 amendment submission detailing commercial process product manufacturing and drug

comparability analysis; awaiting FDA feedback

- R&D Day in June 2023 to overview new findings from totality of data evaluation, including comprehensive analyses of functional, biological, and electrophysiological assessments of TSHA-120 in GAN
- Submission of a formal meeting request to the FDA in Q2 2023 to discuss alternative study designs, additional objective measures and regulatory path forward; formal meeting anticipated in Q3 2023

First Quarter 2023 Financial Highlights

Research and Development Expenses: Research and development expenses were $12.5 million for the three months ended March 31, 2023, compared to $38.2 million for the three months ending March 31, 2022. The $25.7 million decrease was due to reduced research and development compensation as a result of lower headcount of $10.7 million. The decrease was also due to reduced manufacturing and other raw material purchases of $7.1 million. We also incurred $6.4 million reduced expense in non-clinical studies related to translational and toxicology studies and $1.5 million lower expense in other research and development activities.

General and Administrative Expenses: General and administrative expenses were $8.8 million for the three months ended March 31, 2023, compared to $11.5 million for the three months ended March 31, 2022. The decrease of $2.7 million was due to reduced general and administrative compensation as a result of lower headcount and reduced consulting and professional fees.

Net loss: Net loss for the three months ended March 31, 2023 was $17.6 million or $0.28 per share, as compared to a net loss of $50.3 million, or $1.32 per share, for the three months ended March 31, 2022. The net loss for the three months ended March 31, 2023 was partially offset by revenue of $4.7 million recognized related to the Astellas Transactions.

Cash and cash equivalents: As of March 31, 2023, Taysha had $63.4 million in cash and cash equivalents. Taysha continues to expect that its current cash resources will support planned operating expenses and capital requirements into the first quarter of 2024.

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 4:30 pm ET to review its financial and operating results and to provide a corporate update. The dial-in number for the conference call is 855-327-6837 (U.S./Canada) or 631-891-4304 (international). The conference ID for all callers is 10021767. 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. Together, we leverage our fully integrated platform 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, 2022, which is 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, 2023	For the three months ended March 31, 2022
Revenue:		
Service Revenue	$ 4,706	$ -
Operating expenses:		

Research and development	12,514	38,182
General and administrative	8,751	11,469
Total operating expenses	21,265	49,651
Loss from operations	(16,559)	(49,651)
Other income (expense):		
Interest Income	319	14
Interest expense	(1,374)	(672)
Other expense	(8)	(8)
Total other expense	(1,063)	(666)
Net loss	**$ (17,622)**	**$ (50,317)**
Net loss per common share, basic and diluted	$ (0.28)	$ (1.32)
Weighted average common shares outstanding, basic and diluted	63,260,905	38,174,717

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

	March 31, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 63,425	$ 87,880
Prepaid expenses and other current liabilities	8,933	8,537
Total current assets	72,358	96,417
Restricted cash	2,637	2,637
Property, plant and equipment, net	14,642	14,963
Operating lease right-of-use assets	10,647	10,943
Other noncurrent assets	1,316	1,316
Total assets	**$ 101,600**	**$ 126,276**
LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' (DEFICIT) EQUITY		
Current liabilities:		
Accounts payable	$ 9,002	$ 10,946
Accrued expenses and other current liabilities	16,602	18,287
Deferred revenue	28,851	33,557
Total current liabilities	54,455	62,790
Term loan, net	38,161	37,967
Operating lease liability, net of current portion	19,928	20,440
Other noncurrent liabilities	4,004	4,130
Total liabilities	116,548	125,327
Preferred stock, $0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of March 31, 2023 and December 31, 2022	-	-
Stockholders' (deficit) equity		
Common stock, $0.00001 par value per share; 200,000,000 shares authorized and 63,473,349 and 63,207,507 issued and outstanding as of March 31, 2023 and December 31, 2022, respectively	1	1
Additional paid-in capital	404,114	402,389
Accumulated deficit	(419,063)	(401,441)
Total stockholders' (deficit) equity	(14,948)	949
Total liabilities, convertible preferred stock, and stockholders' (deficit) equity	**$ 101,600**	**$ 126,276**

Company Contact:
Hayleigh Collins

Director, Head of Corporate Communications
Taysha Gene Therapies, Inc.
hcollins@tayshagtx.com

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

Source: Taysha Gene Therapies, Inc.