Taysha Gene Therapies Reports Fourth Quarter and Full Year 2022 Financial Results and Provides Corporate Update

Initiated screening of first potential subject in Phase 1/2 REVEAL trial in Rett syndrome; dosing of first adult patient with TSHA-102 expected in H1 2023; submitted protocol amendment to allow for younger patients; initial available Phase 1/2 clinical data, primarily on safety, expected in H1 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

FDA feedback for TSHA-120 in giant axonal neuropathy (GAN) suggests consideration of alternative clinical trial designs for clinically meaningful and objectively measured treatment effects; Company plans to request a formal meeting with FDA to discuss final findings from currently ongoing comprehensive data analyses and potential regulatory path forward in Q2 2023

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

DALLAS, March 28, 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 fourth quarter and full-year ended December 31, 2022, and provided a corporate update.

“The actions taken early this year to improve execution and expedite progress on our two lead clinical programs in Rett syndrome and GAN are having a positive effect,” said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. “We recently initiated screening of the first potential adult subject for the REVEAL Rett syndrome trial and remain on track to dose the first patient and deliver initial available first-in-human adult data, primarily on safety, for TSHA-102 in the first half of the year. Additionally, we recently submitted a protocol amendment to allow patients as young as 15 years old to be included in the study, which we believe will further expedite enrollment. We remain on track to submit a CTA to the MHRA in mid-2023 to conduct a pediatric Rett syndrome trial, and plan to submit an IND to the FDA for Rett syndrome in the second half of 2023. For TSHA-120 in GAN, based on the constructive feedback recently received from the FDA in response to our follow up questions to the formal Type B end-of-Phase 2 meeting minutes, coupled with the positive preliminary assessment of the ongoing comprehensive data analyses, we plan to submit a formal meeting request to the Agency in the second quarter of 2023 to discuss the potential regulatory pathway forward for this ultra-rare disease with no approved treatment.”

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 inherited genetic neurodevelopmental disorder. 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.

- Screening initiated for first potential adult patient with Rett syndrome for the Phase 1/2 REVEAL trial
- Submitted protocol amendment expanding enrollment eligibility to include subjects ≥ 15 years
- Dosing of the first adult patient with Rett syndrome anticipated in H1 2023
- Initial available Phase 1/2 clinical data, primarily on safety, for TSHA-102 in adult patients with Rett syndrome expected in H1 2023, with planned quarterly updates on available clinical data thereafter
- 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
- Continued dosing of adult patients with Rett syndrome in the REVEAL trial in H2 2023

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 to FDA detailing commercial process product manufacturing and drug comparability analysis
- Receipt of FDA’s response to Taysha’s follow up questions to the formal Type B end-of-Phase 2 meeting minutes
- FDA clarified MFM32, the primary efficacy scale discussed at the FDA Type B end-of-Phase 2 meeting, as a relevant primary endpoint only in the setting of a randomized double blind placebo controlled trial and acknowledged Taysha's challenge in executing and enrolling such a study design due to the ultra-rare nature of GAN
- FDA is open to regulatory flexibility in a controlled trial setting and willing to consider alternative study designs utilizing

objective measurements to demonstrate a relatively large treatment effect that is self-evident and clinically meaningful

- Ongoing comprehensive analyses of functional, biological and electrophysiological assessments as part of totality of data evaluation to inform future interactions with the FDA
- Submission of a formal meeting request to the FDA planned in Q2 2023

Fourth Quarter and Full-Year 2022 Financial Highlights

Research and Development Expenses: Research and development expenses were $13.9 million for the three months ended December 31, 2022, compared to $37.9 million for the three months ended December 31, 2021. Research and development expenses were $91.2 million for the full year ended December 31, 2022, compared to $131.9 million for the full year ended December 31, 2021. The $40.7 million decrease was primarily attributable to a decrease of $20.3 million in research and development manufacturing and other raw material purchases and a $9.0 million decrease in license fees. The decrease in research and development expenses for the year ended December 31, 2022 was also attributable to a $12.0 million decrease in third-party research and development fees, mainly related to non-clinical studies and toxicology studies and a $4.7 million decrease in compensation expense as a result of lower headcount. Overall, lower research and development expenses for the year ended December 31, 2022 were partially offset by higher clinical trial expenses of $2.4 million and higher severance expense of $2.9 million in 2022.

General and Administrative Expenses: General and administrative expenses were $7.3 million for the three months ended December 31, 2022, compared to $11.8 million for the three months ended December 31, 2021. General and administrative expenses were $37.4 million for the year ended December 31, 2022, compared to $41.3 million for the year ended December 31, 2021. The decrease of approximately $3.9 million was primarily attributable to $5.0 million of lower consulting professional fees and lower compensation expenses driven by lower headcount in 2022. Lower general and administrative expenses were partially offset by $1.1 million of severance expense.

Net loss: Net loss for the three months ended December 31, 2022 was $55.7 million, or $0.99 per share, as compared to a net loss of $50.4 million, or $1.32 per share, for the three months ended December 31, 2021. In November 2022, we recorded a $36.4 million non-cash, non-recurring impairment charge related to the North Carolina manufacturing facility. The net loss for the three months ended December 31, 2022 was partially offset by revenue of $2.5 million recognized related to the Astellas Transactions. Net loss for the full year ended December 31, 2022 was $166.0 million or $3.78 per share, as compared to a net loss of $174.5 million, or $4.64 per share, for the full year ended December 31, 2021.

Cash and cash equivalents: As of December 31, 2022, Taysha had $87.9 million in cash and cash equivalents. The Company 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 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13736479. 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—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 TSHA-102 and TSHA-120 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. 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		For the twelve months ended	
	December 31 2022	December 31 2021	December 31 2022	December 31 2021

Revenue:				
Service Revenue	$ 2,502	-	$ 2,502	$ -
Operating expenses:				
Research and development	13,861	37,918	91,169	131,943
General and administrative	7,341	11,806	37,360	41,324
Impairment of long-lived assets	36,420	-	36,420	-
Total operating expenses	57,622	49,724	164,949	173,267
Loss from operations	(55,120)	(49,724)	(162,447)	(173,267)
Other income (expense):				
Interest Income	199	29	249	172
Interest expense	(796)	(691)	(3,798)	(1,428)
Other	(6)	-	(18)	-
Total other income (expense)	(603)	(662)	(3,567)	(1,256)
Net loss	$ (55,723)	$ (50,386)	$ (166,014)	$ (174,523)
Net loss per common share, basic and diluted	$ (0.99)	$ (1.32)	$ (3.78)	$ (4.64)
Weighted average common shares outstanding, basic and diluted	56,386,130	38,110,597	43,952,015	37,650,566

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

	December 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 87,880	$ 149,103
Prepaid expenses and other current assets	8,537	10,499
Total current assets	96,417	159,602
Restricted cash	2,637	2,637
Property, plant and equipment, net	14,963	50,610
Operating lease right-of-use assets	10,943	-
Other noncurrent assets	1,316	1,107
Total assets	**$ 126,276**	**$ 213,956**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 10,946	$ 21,763
Accrued expenses and other current liabilities	18,287	29,983
Deferred revenue	33,557	-
Total current liabilities	62,790	51,746
Build-to-suit lease liability	-	25,900
Term loan, net	37,967	37,192
Operating lease liability, net of current portion	20,440	-
Other noncurrent liabilities	4,130	3,735
Total liabilities	125,327	118,573
Stockholders' equity		
Common stock, $0.00001 par value per share; 200,000,000 shares authorized and 63,207,507 issued and outstanding as of December 31, 2022 and 38,473,945 outstanding as of December 31, 2021	1	-
Additional paid-in capital	402,389	331,032
Accumulated deficit	(401,441)	(235,649)
Total stockholders' equity	949	95,383
Total liabilities and stockholders' equity	**$ 126,276**	**$ 213,956**

Company Contact:
Hayleigh Collins
Director, Head of Corporate Communications
Taysha Gene Therapies
hcollins@tayshagtx.com

Media Contact:
Carolyn Hawley
Evoke Canale
carolyn.hawley@evokegroup.com

Source: Taysha Gene Therapies, Inc.