Taysha Gene Therapies Reports Second Quarter 2025 Financial Results and Provides Corporate Update

Commenced site activation for REVEAL pivotal trial in accordance with previously aligned upon key design elements, following receipt of No Objection Letter (NOL) from Health Canada and feedback from the FDA; patient enrollment anticipated to begin in Q4 2025

High dose and low dose TSHA-102 continue to be generally well tolerated with no treatment-related SAEs or DLTs in the 12 patients treated in Part A of REVEAL Phase 1/2 trials as of August 2025 data cutoff

Part A data from REVEAL Phase 1/2 trials presented at IRSF Scientific Meeting showed 100% response rate for pivotal trial primary endpoint of gain/regain of ≥ one developmental milestone, corroborated by improvements in key secondary endpoints post-TSHA-102

Company anticipates reporting new supplemental REVEAL Part A clinical data supporting TSHA-102 therapeutic impact in Q4 2025

Gross proceeds of $230 million public follow-on offering, including full exercise of the underwriters' option to purchase additional shares, strengthen balance sheet and extend cash runway into 2028

Conference call and webcast today at 8:30 AM Eastern Time

DALLAS, Aug. 12, 2025 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA) (Taysha or the Company), a clinical-stage biotechnology company focused on advancing adeno-associated virus (AAV)-based gene therapies for severe monogenic diseases of the central nervous system (CNS), today reported financial results for the second quarter ended June 30, 2025, and provided a corporate update.

"We have made strong progress across the Company supporting the advancement of our TSHA-102 development program toward potential registration for females two years and older with Rett syndrome. In May, we announced alignment with the FDA on key design elements of our REVEAL pivotal trial, and subsequently, submitted our IND application and CTA amendments to both the FDA and Health Canada. I'm pleased to share we have commenced site activation in accordance with the key design elements previously aligned on with the FDA, based on feedback recently received from these regulatory agencies. As a result, we anticipate beginning patient enrollment in the fourth quarter of this year," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "Our frequent and constructive dialogue with the FDA through the Regenerative Medicine Advanced Therapy (RMAT) mechanism has been instrumental in enabling us to navigate this novel regulatory pathway. With a strengthened balance sheet, our pivotal trial underway and a clear path to registration, we believe we are well positioned to advance TSHA-102 to benefit patients living with this devastating disease."

Elsa Rossignol, M.D., FRCP, FAAP, Associate Professor in Neuroscience and Pediatrics at the Université de Montréal, Director of the Rett Multidisciplinary Clinic of the CHU Sainte-Justine and a Principal Investigator of the REVEAL trials, added, "The enthusiasm generated by the REVEAL Phase 1/2 clinical data presented at the IRSF Scientific Meeting was inspiring and highlights the therapeutic potential of TSHA-102. The developmental milestones achieved across all pediatric, adolescent and adult patients treated with TSHA-102, combined with the positive safety profile to date and minimally invasive intrathecal delivery, reinforces my belief in the potential of TSHA-102 to redefine the treatment landscape for Rett syndrome. I'm pleased to participate in the REVEAL pivotal trial and to contribute to the advancement of a potential therapy for individuals living with Rett syndrome, who continue to face profound unmet need."

Recent Corporate and TSHA-102 Program Highlights

- **Commenced Site Activation for REVEAL Pivotal Trial Following Regulatory Feedback.** In May 2025, Taysha announced alignment with the U.S. Food and Drug Administration (FDA) on key elements of the REVEAL pivotal Part B trial design and next steps for enabling study initiation. Subsequently, the Company submitted its Investigational New Drug (IND) application amendment to the FDA and Clinical Trial Application Amendment (CTA-A) to Health Canada. Taysha commenced site activation for the REVEAL pivotal trial in accordance with previously aligned upon key design elements, following receipt of a NOL from Health Canada and written feedback from the FDA
    - **Trial Design:** Single-arm, open-label trial, with each patient serving as their own control
    - **Dose and Administration:** Single administration of high dose of TSHA-102 ($1x10^{15}$ total vector genomes (vg)) delivered by lumbar intrathecal (IT) injection
    - **Patient Population:** Enrolling 15 females aged 6 to <22 years in the developmental plateau population of Rett syndrome; these patients have a low likelihood (0% to <6.7%) of gaining new or regaining developmental milestones that were lost after a defined number of years based on Taysha's natural history data analysis (N = ~1100 females with Rett syndrome, with up to 14 years follow-up)
    - **Primary Endpoint:** Will assess response rate, defined as the percentage of patients who gain or regain ≥ one developmental milestone from a list of 28 defined milestones across the core functional domains of communication, fine motor and gross motor, following TSHA-102
        - Selected milestones reflect meaningful functional gains and improvements in activities of daily living identified through caregiver research, and have a documented likelihood of spontaneous gain/regain of <6.7% in the untreated Rett syndrome population aged ≥ 6 years
        - Standardized milestone assessments will be administered and captured on video at pre- and post-treatment

timepoints; video-evidenced determination of milestone gain/regain will be determined by independent, blinded central raters based on prespecified definitions of achievement for each milestone
    - The Company plans to perform a 12-month primary analysis and a 6-month interim analysis
        - Previously disclosed clinical data from Part A of REVEAL Phase 1/2 Adolescent/Adult and Pediatric trials (N=10, 6-21 years) demonstrating a 100% response rate indicate the pivotal trial is well powered to establish efficacy of TSHA-102 (May 2025 data cutoff)
    - **Key Secondary Endpoints:** Include the average number of total developmental milestones gained/regained per patient following TSHA-102, as well as clinician-assessed outcome measures: Revised Motor Behavior Assessment (R-MBA) and Clinician Global Impression – Improvement (CGI-I)

- **Obtained Written Alignment from FDA on Extrapolation Approach in a Safety-Focused Study in Females 2 to <6 Years of Age.** Planned safety-focused study will evaluate the safety and preliminary efficacy of TSHA-102 in the pre-developmental plateau population (aged 2 to <6 years) of females with Rett syndrome. Efficacy will be extrapolated from the REVEAL pivotal trial. This approach is intended to enable access across a broad population of patients with Rett syndrome

- **High and Low Dose of TSHA-102 Continue to be Generally Well Tolerated.** High dose ($1x10^{15}$ total vg) and low dose ($5.7x10^{14}$ total vg) of TSHA-102 continue to be generally well tolerated with no treatment-related serious adverse events (SAEs) or dose-limiting toxicities (DLTs) in the 12 pediatric, adolescent and adult patients dosed across Part A of the REVEAL Phase 1/2 trials as of the August 2025 data cutoff. This includes eight patients in the high dose cohort and four patients in the low dose cohort.

- **Presented Data Supporting TSHA-102 Clinical Program at IRSF.** Three oral presentations were delivered at the 2025 International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting. The presentations, which are available on the Company's website , highlighted the following:
    - Clinical data from Part A of REVEAL Phase 1/2 trials (May 2025 data cutoff)
    - Caregiver research on meaningful improvements in gene therapy for Rett syndrome supporting the primary endpoint for the REVEAL pivotal trial
    - Preclinical data demonstrating broad biodistribution of AAV9 gene therapy vectors across the brain and spinal cord regions following lumbar IT administration in non-human primates

- **Completed Public Follow-on Offering with Total Gross Proceeds of $230 Million**. Proceeds included full exercise of the underwriters' option to purchase additional shares; anticipated cash runway extends into 2028

Anticipated Milestones

- REVEAL pivotal trial patient enrollment expected to begin in the fourth quarter of 2025

- New supplemental clinical data from Part A of REVEAL Phase 1/2 trials supporting the broad therapeutic impact of TSHA-102 anticipated in the fourth quarter of 2025

Second Quarter 2025 Financial Highlights

Research and Development Expenses: Research and development expenses were $20.1 million for the three months ended June 30, 2025, compared to $15.1 million for the three months ending June 30, 2024. The $5.0 million increase was driven by BLA-enabling process performance qualification (PPQ) manufacturing initiatives, REVEAL clinical trial activities and higher compensation expenses as a result of increased headcount during the three months ended June 30, 2025.

General and Administrative Expenses: General and administrative expenses were $8.6 million for the three months ended June 30, 2025, compared to $7.3 million for the three months ended June 30, 2024. The increase of $1.3 million was primarily due to higher legal and professional fees.

Net Loss: Net loss for the three months ended June 30, 2025, was $26.9 million, or $0.09 per share, compared to a net loss of $20.9 million, or $0.09 per share, for the three months ended June 30, 2024.

Cash and Cash Equivalents: As of June 30, 2025, Taysha had $312.8 million in cash and cash equivalents. This reflects the gross proceeds of $230.0 million from the May 2025 follow-on financing, including full exercise of the underwriters' option to purchase additional shares. The Company expects that its current cash resources will support planned operating expenses and capital requirements into 2028.

Conference Call and Webcast Information
Taysha management will hold a conference call and webcast today at 8:30 a.m. ET to review its financial and operating results and 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 13754869. The live webcast and replay may be accessed by visiting Taysha's website.

About TSHA-102
TSHA-102 is a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy in clinical evaluation for Rett syndrome. Designed as a one-time treatment, TSHA-102 aims to address the genetic root cause of the disease by delivering a functional form of *MECP2* to cells

in the CNS. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) technology designed to mediate levels of *MECP2* in the CNS on a cell-by-cell basis without risk of overexpression. TSHA-102 has received Regenerative Medicine Advanced Therapy, Fast Track and Orphan Drug and Rare Pediatric Disease designations from the FDA, Orphan Drug designation from the European Commission and Innovative Licensing and Access Pathway designation from the Medicines and Healthcare products Regulatory Agency.

About Rett Syndrome

Rett syndrome is a rare neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene encoding methyl CpG-binding protein 2 (MeCP2), which is essential for regulating neuronal and synaptic function in the brain. The disorder is characterized by loss of communication and hand function, slowing and/or regression of development, motor and respiratory impairment, seizures, intellectual disabilities and shortened life expectancy. Rett syndrome progression is divided into four key stages, beginning with early onset stagnation at 6 to 18 months of age followed by rapid regression, plateau and late motor deterioration. Rett syndrome primarily occurs in females and is one of the most common genetic causes of severe intellectual disability. Currently, there are no approved disease-modifying therapies that treat the genetic root cause of the disease. Rett syndrome caused by a pathogenic/likely pathogenic *MECP2* mutation is estimated to affect between 15,000 and 20,000 patients in the U.S., EU, and U.K.

About Taysha Gene Therapies

Taysha Gene Therapies (Nasdaq: TSHA) is a clinical-stage biotechnology company focused on advancing adeno-associated virus (AAV)-based gene therapies for severe monogenic diseases of the central nervous system. Its lead clinical program TSHA-102 is in development for Rett syndrome, a rare neurodevelopmental disorder with no approved disease-modifying therapies that address the genetic root cause of the disease. With a singular focus on developing transformative medicines, Taysha aims to address severe unmet medical needs and dramatically improve the lives of patients and their caregivers. The Company's management team has proven experience in gene therapy development and commercialization. Taysha leverages this experience, its manufacturing process and a clinically and commercially proven AAV9 capsid in an effort to rapidly translate treatments from bench to bedside. For more information, please visit 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, but are not limited to, statements concerning the potential of TSHA-102, including the reproducibility and durability of any favorable results initially seen in patients dosed to date in clinical trials, including with respect to functional milestones, to positively impact quality of life and alter the course of disease in the patients Taysha seeks to treat, Taysha's research, development and regulatory plans for TSHA-102, including the timing of initiating additional trials, reporting data from Taysha's clinical trials and making regulatory submissions, communications with and feedback from the FDA and Health Canada on the regulatory pathway for TSHA-102, the potential for TSHA-102 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 and the potential market opportunity for Taysha's product candidates and Taysha's anticipated 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 Taysha's business are described in detail in Taysha's Securities and Exchange Commission ("SEC") filings, including in Taysha's Annual Report on Form 10-K for the full-year ended December 31, 2024 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, which are available on the SEC's website at www.sec.gov. Additional information will be made available in other filings that Taysha makes from time to time with the SEC. These forward-looking statements speak only as of the date hereof, and Taysha disclaims 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)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Revenue	$ 1,986	$ 1,112	$ 4,288	$ 4,523
Operating expenses:				
Research and development	20,141	15,073	35,706	35,730
General and administrative	8,598	7,338	16,756	14,422
Total operating expenses	28,739	22,411	52,462	50,152
Loss from operations	(26,753)	(21,299)	(48,174)	(45,629)
Other income (expense):				
Change in fair value of warrant liability	(273)	195	(171)	(142)
Change in fair value of term loan	(1,461)	(1,279)	(2,991)	(2,332)
Interest income	1,859	1,440	3,185	3,133
Interest expense	(17)	(27)	(36)	(56)
Other (expense), income	(237)	42	(224)	37
Total other (expense) income, net	(129)	371	(237)	640
Net loss	**$ (26,882)**	**$ (20,928)**	**$ (48,411)**	**$ (44,989)**
Net loss per common share, basic and diluted	$ (0.09)	$ (0.09)	$ (0.17)	$ (0.19)
Weighted average common shares outstanding, basic and diluted	297,988,978	232,821,553	283,726,888	232,035,448

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

	June 30, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	$ 312,761	$ 139,036
Restricted cash	449	449
Prepaid expenses and other current assets	3,054	2,645
Total current assets	316,264	142,130
Restricted cash	2,151	2,151
Property, plant and equipment, net	6,957	7,485
Operating lease right-of-use assets	7,773	8,381
Other non-current assets	186	217
Total assets	**$ 333,331**	**$ 160,364**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 7,713	$ 3,592
Accrued expenses and other current liabilities	12,151	12,862
Deferred revenue	5,485	9,773
Total current liabilities	25,349	26,227
Term loan, net	41,051	43,942
Operating lease liability, net of current portion	16,808	17,361
Other non-current liabilities	1,396	1,309
Total liabilities	84,604	88,839
Stockholders' equity		
Preferred stock, $0.00001 par value per share; 10,000,000 shares authorized, and no shares issued and outstanding as of June 30, 2025, and December 31, 2024	—	—
Common stock, $0.00001 par value per share; 700,000,000 shares authorized and 272,730,060 issued and outstanding as of June 30, 2025 and 400,000,000 shares authorized and 204,943,306 issued and outstanding as of December 31, 2024	3	2
Additional paid-in capital	900,139	677,859
Accumulated other comprehensive loss	(699)	(4,031)
Accumulated deficit	(650,716)	(602,305)
Total stockholders' equity	248,727	71,525
Total liabilities and stockholders' equity	**$ 333,331**	**$ 160,364**

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