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

TSHA-102 granted Breakthrough Therapy designation by FDA

Finalized FDA alignment on REVEAL pivotal trial protocol and SAP, including 6-month interim analysis that may expedite BLA submission, which was enabled by the rigorous developmental milestone evaluation in Part A REVEAL Phase 1/2 trials showing an unprecedented response rate

Dosing of first patient in REVEAL pivotal trial scheduled for Q4 2025, with enrollment of additional patients expected to continue at multiple sites this quarter

Presented new supplemental analysis of Part A REVEAL data reinforcing the broad and consistent, multi-domain impact of TSHA-102 on activities of daily living at the CNS Annual Meeting

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

Regained full unencumbered rights to TSHA-102 Rett syndrome program, enabling Taysha to focus on driving long-term value with full strategic flexibility and optionality

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

DALLAS, Nov. 04, 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 third quarter ended September 30, 2025, and provided a corporate update.

"The progress we've made in the third quarter of 2025 sets the stage for a potentially transformative period ahead for Taysha. We recently received FDA Breakthrough Therapy designation, which reflects the FDA's recognition of the therapeutic potential of TSHA-102 for individuals with Rett syndrome, who face a profound unmet need. Additionally, we're pleased to have finalized alignment with the FDA on our pivotal trial protocol and SAP, including a six-month interim analysis, which we believe provides a clear opportunity to expedite our BLA submission by at least two quarters," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "With Breakthrough Therapy designation and finalized FDA alignment, together with our strong balance sheet and regained global rights to TSHA-102, we believe we are strongly positioned to initiate our REVEAL pivotal trial and accelerate execution toward BLA submission. We remain on track to dose the first patient in the REVEAL pivotal trial this quarter and expect additional enrollment to continue at multiple sites this quarter. With an estimated 15,000 to 20,000 patients affected by Rett syndrome across the U.S., EU and U.K. and compelling clinical data from Part A of our REVEAL trials, we see a significant opportunity to bring an innovative therapy with disease-modifying potential to patients."

Recent Corporate and TSHA-102 Program Highlights

- **FDA Breakthrough Therapy Designation Granted to TSHA-102.** The U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy designation to TSHA-102 for the treatment of Rett syndrome based on the FDA's review of positive clinical evidence from Part A of the REVEAL Phase 1/2 adolescent/adult and pediatric trials (N=12).

- **Finalized FDA Alignment on REVEAL Pivotal Trial Protocol and SAP.** Taysha finalized alignment with the FDA on the REVEAL pivotal trial protocol and statistical analysis plan (SAP) that are intended to support the planned Biologics License Application (BLA) submission for TSHA-102, following the resolution of remaining clinical and statistical queries. Previously aligned upon key trial design elements remain unchanged, including the primary endpoint of response rate, defined as the percentage of patients in the developmental plateau population of Rett syndrome who gain/regain ≥ one of the 28 natural history defined developmental milestones, with each patient serving as their own control.
    - Inclusion of a 6-month interim analysis that may serve as the basis for BLA submission was enabled by the rigorous developmental milestone evaluation in Part A of the REVEAL Phase 1/2 trials that demonstrated an unprecedented response rate at 6 months that deepened over time.
    - Response rate of 33% (5 out of 15 patients) is the minimum threshold for success sufficient to reject the natural history established null hypothesis of 6.7%.

- **Presented New Supplemental Data Analysis Supporting TSHA-102 Clinical Program.** A poster presentation, which is available on the Company's website , was delivered at the 54th Child Neurology Society (CNS) Annual Meeting to highlight results from a new supplemental analysis of data from Part A of the REVEAL Phase 1/2 trials (May 2025 data cutoff). Results provide supportive evidence of broad and consistent functional skill gains/improvements outside of the natural history defined developmental milestones that further reinforce TSHA-102's consistent, multi-domain impact on activities of daily living.
    - In addition to the consistent achievement of natural history defined developmental milestones, 100% of patients

(N=10) achieved multiple additional skills/improvements derived from validated, structured efficacy scales, with a total of 22 developmental milestones and 165 additional skills/improvements achieved across the 10 patients post-TSHA-102.

- **TSHA-102 Continues 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 in Part A of the REVEAL Phase 1/2 trials (October 2025 data cutoff). This includes eight patients in the high dose cohort and four patients in the low dose cohort.

- **Regained Full Rights to TSHA-102 Rett Syndrome Program.** Taysha regained full rights to its lead TSHA-102 program in October 2025 following expiration of the 2022 Option Agreement between Astellas and Taysha, which had granted Astellas the exclusive option to enter a negotiation period to obtain an exclusive license to TSHA-102 and certain rights with respect to change in control transactions involving Taysha. Taysha now holds unencumbered rights to TSHA-102, which enables the Company to focus on driving long-term value with full strategic flexibility and optionality.

- **Strengthened Commercial Leadership.** David McNinch was appointed as Taysha's Chief Commercial Officer in September 2025, responsible for the Company's commercial function. Mr. McNinch brings over two decades of global commercialization and strategic market development experience across multiple therapeutic areas. Most recently, he served as Chief Business Officer of Encoded Therapeutics, where he led the commercial and partnering strategy across the company's gene therapy portfolio. Mr. McNinch previously held senior commercial leadership roles at Prothena Corp. as well as InterMune, where he led the launch of Esbriet, the first approved treatment for IPF, and supported the company's acquisition by Roche. In his role at Taysha, Mr. McNinch reports to Sean McAuliffe, Taysha's Chief Business Officer, who led the development and execution of the commercial launch of Zolgensma for spinal muscular atrophy, the first approved gene therapy for a monogenic CNS disease.

Anticipated Milestones

- Dosing of the first patient in the REVEAL pivotal trial is scheduled for the fourth quarter of 2025, with enrollment of additional patients expected to continue at multiple sites during the quarter.
- Update on longer-term safety and efficacy data from Part A of REVEAL Phase 1/2 trials expected in the first half of 2026

Third Quarter 2025 Financial Highlights

- **Research and Development Expenses:** Research and development expenses were $25.7 million for the three months ended September 30, 2025, compared to $14.9 million for the three months ended September 30, 2024. The increase was driven by BLA-enabling process performance qualification manufacturing initiatives, REVEAL clinical trial activities and higher compensation expenses as a result of increased headcount during the three months ended September 30, 2025.

- **General and Administrative Expenses:** General and administrative expenses were $8.3 million for the three months ended September 30, 2025, compared to $7.9 million for the three months ended September 30, 2024. The increase of $0.4 million was primarily due to debt issuance costs incurred in connection with the refinancing of the Company's existing loan and security agreement with Trinity Capital that are recorded in general and administrative expense under the fair value option and was partially offset by lower legal and professional fees.

- **Net Loss:** Net loss for the three months ended September 30, 2025, was $32.7 million, or $0.09 per share, compared to a net loss of $25.5 million, or $0.10 per share, for the three months ended September 30, 2024.

- **Cash and Cash Equivalents:** As of September 30, 2025, Taysha had $297.3 million in cash and cash equivalents. 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 800-245-3047 (U.S./Canada) or 203-518-9765 (international). The conference ID for all callers is TAYSHA. 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 Breakthrough Therapy, 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 enrolling and dosing patients, initiating additional trials, reporting data from Taysha's clinical trials and making regulatory submissions, communications with feedback from the FDA 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; Taysha's ability to realize the benefits of Breakthrough Therapy Designation; Taysha's ability to drive long-term value for stockholders; 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 September 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 September 30,		For the Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Revenue	$ —	$ 1,788	$ 4,288	$ 6,311
Operating expenses:				
Research and development	25,745	14,946	61,451	50,676
General and administrative	8,279	7,902	25,035	22,324
Impairment of long-lived assets	—	4,838	—	4,838
Total operating expenses	34,024	27,686	86,486	77,838
Loss from operations	(34,024)	(25,898)	(82,198)	(71,527)
Other income (expense):				
Change in fair value of warrant liability	(292)	75	(463)	(67)
Change in fair value of term loan	(1,534)	(1,703)	(4,525)	(4,035)
Interest income	3,169	2,107	6,354	5,240
Interest expense	(15)	(24)	(51)	(80)
Other expense	(37)	(81)	(261)	(44)
Total other income, net	1,291	374	1,054	1,014
Net loss	**$ (32,733)**	**$ (25,524)**	**$ (81,144)**	**$ (70,513)**
Net loss per common share, basic and diluted	$ (0.09)	$ (0.10)	$ (0.26)	$ (0.29)
Weighted average common shares outstanding, basic and diluted	353,309,524	267,824,045	307,175,982	244,052,057

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

	September 30, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	$ 297,344	$ 139,036
Restricted cash	449	449
Prepaid expenses and other current assets	2,158	2,645
Total current assets	299,951	142,130
Restricted cash	2,151	2,151
Property, plant and equipment, net	6,805	7,485
Operating lease right-of-use assets	7,463	8,381
Other non-current assets	184	217
Total assets	**$ 316,554**	**$ 160,364**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 5,438	$ 3,592
Accrued expenses and other current liabilities	17,708	12,862
Deferred revenue	5,485	9,773
Total current liabilities	28,631	26,227
Term loan, net	50,852	43,942
Operating lease liability, net of current portion	16,506	17,361
Other non-current liabilities	1,576	1,309
Total liabilities	97,565	88,839
Stockholders' equity		
Common stock, $0.00001 par value per share; 700,000,000 shares authorized and 273,915,373 issued and outstanding as of September 30, 2025, and 400,000,000 shares authorized and 204,943,306 issued and outstanding as of December 31, 2024	3	2
Additional paid-in capital	903,578	677,859
Accumulated other comprehensive loss	(1,143)	(4,031)
Accumulated deficit	(683,449)	(602,305)
Total stockholders' equity	218,989	71,525
Total liabilities and stockholders' equity	**$ 316,554**	**$ 160,364**

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