



Taysha Gene Therapies Reports Full-Year 2025 Financial Results and Provides Corporate Update

Dosed multiple Rett syndrome patients in REVEAL pivotal trial of TSHA-102, with enrollment advancing across multiple sites; on track to complete dosing in Q2 2026

Received FDA clearance to initiate ASPIRE trial in three patients aged 2 to <4 years with inclusion of ≥ 3 months of safety data in planned BLA submission to support potential for broad label; on track to complete dosing in Q2 2026

Maintained favorable tolerability profile with no treatment-related SAEs or DLTs in REVEAL Phase 1/2 and REVEAL pivotal trials as of March 2026 data cutoff; longer-term safety and efficacy data from Part A of REVEAL Phase 1/2 trials expected in Q2 2026

Reached written alignment with FDA on proposed PPQ and comparability strategy for commercial TSHA-102 manufacturing to support planned BLA submission package

Conference call and webcast today at 8:30 AM ET

DALLAS, March 19, 2026 (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 full year ended December 31, 2025, and provided a corporate update.

"2025 was a year of significant execution for Taysha, setting the stage for what we expect to be a transformative year ahead. We are focused on completing the pivotal development of TSHA-102 and bolstering our commercial readiness efforts as we advance toward potential registration. Multiple patients have been dosed in our REVEAL pivotal trial, and we remain on track to complete dosing in the REVEAL pivotal and ASPIRE trials in the second quarter of this year," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha.

Mr. Nolan continued, "Importantly, we have maintained ongoing, constructive dialogue with the FDA over the past two years that continues to support a streamlined path to registration. We recently received written FDA alignment on our proposed PPQ and comparability strategy, including the ability to leverage data across the REVEAL and ASPIRE trials to support our planned BLA submission. In parallel, recent market research reinforces the strong commercial opportunity for TSHA-102, demonstrating high anticipated demand from both clinicians and caregivers in the U.S. The research also indicates a clear preference for intrathecal administration, which is viewed as familiar, accessible and scalable, enabling the potential to safely and efficiently treat patients across multiple institutions. With a favorable tolerability profile demonstrated to date, continued patient enrollment and a well defined regulatory and commercial path, we believe TSHA 102 has the potential to meaningfully address the genetic root cause of this devastating disease with high unmet need."

Recent Corporate and TSHA-102 Program Highlights

- **Dosed Multiple Patients in REVEAL Pivotal Trial with Additional Enrollment Advancing Across Multiple Clinical Trial Sites.** The single-arm, open-label trial is evaluating a single intrathecal (IT) administration of high dose TSHA-102 (1×10^{15} total vector genomes (vg)) in 15 females between the ages of 6 and <22 years in the developmental plateau population of Rett syndrome. The primary endpoint will assess response rate, defined as the percentage of patients who gain or regain $\geq$ one of the 28 natural history-defined developmental milestones, with each patient serving as their own control. The study includes a six-month interim analysis that may serve as the basis for Biologics License Application (BLA) submission.
- **Received FDA Clearance to Initiate the ASPIRE Trial and Written Alignment on Data for Inclusion in BLA Submission to Enable Broad Label.** In January 2026, Taysha announced written alignment with the U.S. Food and Drug Administration (FDA) on the ASPIRE safety-focused trial and the data for inclusion in the planned BLA submission to enable broad labeling of TSHA-102 for patients aged ≥ 2 years with Rett syndrome. Subsequently, the Company received FDA clearance to initiate the ASPIRE trial. Taysha is enrolling three females with Rett syndrome, aged 2 to <4 years, to evaluate the safety and preliminary efficacy of a single IT administration of high dose TSHA-102 (1×10^{15} total vg), scaled to account for the lower brain volume in 2 to <4-year-olds.
 - A minimum of three months of ASPIRE safety data will be included in the planned BLA submission, while efficacy in the 2 to <6-year-old population will be extrapolated from data collected in the REVEAL pivotal trial.
- **TSHA-102 Continues to be Generally Well Tolerated.** High dose (1×10^{15} total vg) and low dose (5.7×10^{14} total vg) TSHA-102 continue to be generally well tolerated with no treatment-related serious adverse events (SAEs) or dose-limiting toxicities (DLTs) in the patients treated in the REVEAL Phase 1/2 trials and the REVEAL pivotal trial as of the March 2026 data cutoff.
- **Reached Written FDA Alignment on CMC Requirements for TSHA-102 Supporting Planned BLA Submission Package.** Following a Type C Meeting with the FDA in the first quarter of 2026, Taysha further aligned on the following Chemistry Manufacturing and Controls (CMC) requirements to support the planned BLA submission in parallel with its

clinical development timelines:

- Alignment on the proposed comparability approach between TSHA-102 material derived from the clinical and final commercial manufacturing processes. The FDA agreed that the approach may support pooling data from the REVEAL Phase 1/2 trials with data from the ongoing REVEAL pivotal and ASPIRE trials for the planned BLA submission.
- Endorsement of the Company's proposed Process Performance Qualification (PPQ) campaign strategy to support process validation for the BLA submission, including:
 - Stability data package
 - Potency assay strategy
 - Execution of BLA-enabling PPQ lots using the commercial manufacturing process, with initiation expected in Q2 2026

- **Advanced Commercial Readiness Activities:**

- Further strengthened commercial leadership team with the appointment of Brad Martin as Senior Vice President, Market Access and Value in February 2026. Mr. Martin brings over two decades of market access and commercial strategy experience in gene therapy, having held senior roles at Neurotech Pharmaceuticals, Sarepta Therapeutics and AveXis. At AveXis, he played a crucial role in securing market access for the blockbuster gene therapy Zolgensma for the treatment of spinal muscular atrophy.
- Completed market research demonstrating strong clinician and caregiver demand and anticipated broad adoption of TSHA-102 for the 6,000 to 9,000 pediatric, adolescent and adult patients with Rett syndrome in the U.S., with a clear preference for intrathecal administration, reinforcing the significant commercial potential of TSHA-102.

Anticipated Milestones

- Completion of dosing in the REVEAL pivotal trial is expected in the second quarter of 2026
- Completion of dosing in the ASPIRE trial is expected in the second quarter of 2026
- Update on longer-term safety and efficacy data from Part A of REVEAL Phase 1/2 trials expected in the second quarter of 2026

Full-Year 2025 Financial Highlights

Research and Development Expenses: Research and development expenses were \$86.4 million for the year ended December 31, 2025, compared to \$66.0 million for the year ended December 31, 2024. The \$20.4 million increase was primarily driven by higher compensation expenses due to increased research and development headcount. Clinical trial and GMP expenses also increased during the year ended December 31, 2025, due to clinical trial activities in the REVEAL studies and BLA-enabling PPQ manufacturing initiatives.

General and Administrative Expenses: General and administrative expenses were \$33.9 million for the year ended December 31, 2025, compared to \$29.0 million for the year ended December 31, 2024. The increase of \$4.9 million was primarily due to higher compensation expenses and legal and professional fees as well as debt issuance costs incurred in connection with the 2025 Trinity Term Loan that are recorded in general and administrative expense under the fair value option.

Net Loss: Net loss for the year ended December 31, 2025, was \$109.0 million, or \$0.34 per share, compared to a net loss of \$89.3 million, or \$0.36 per share, for the year ended December 31, 2024.

Cash and Cash Equivalents: As of December 31, 2025, Taysha had \$319.8 million in cash and cash equivalents. During the fourth quarter, the Company raised an additional \$50.0 million in gross proceeds by utilizing its at-the-market equity offering program, with proceeds intended to support a potential commercial inventory build in 2027. The Company expects that its current cash resources will be sufficient to fund planned operating expenses into 2028.

Conference Call and Webcast Information

Taysha management will host a live conference call and webcast today at 8:30 a.m. ET to review its financial and operating results and provide a corporate update. Participants may access the live webcast of the conference call 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 and Taysha's other product candidates 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 its product candidates, communications with the FDA, including with respect to the BLA for TSHA-102, the potential for Taysha's 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 and the potential market opportunity for Taysha's product candidates, including anticipated clinician and caregiver demand. 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 our Annual Report on Form 10-K for the full-year ended December 31, 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 Year Ended December 31,	
	2025	2024
Revenue	\$ 9,773	\$ 8,333
Operating expenses:		
Research and development	86,403	66,001
General and administrative	33,868	28,953
Impairment of long-lived assets	—	4,838
Total operating expenses	120,271	99,792
Loss from operations	(110,498)	(91,459)
Other income (expense):		
Change in fair value of warrant liability	(1,199)	16
Change in fair value of term loan	(6,168)	(4,583)
Interest income	9,224	6,940
Interest expense	(63)	(102)
Other expense	(291)	(110)
Total other income, net	1,503	2,161
Net loss	\$ (108,995)	\$ (89,298)
Net loss per common share, basic and diluted	\$ (0.34)	\$ (0.36)
Weighted average common shares outstanding, basic and diluted	319,711,972	250,134,421

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

	December 31, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 319,767	\$ 139,036

Restricted cash	449	449
Prepaid expenses and other current assets	4,431	2,645
Total current assets	324,647	142,130
Restricted cash	2,315	2,151
Property, plant and equipment, net	6,736	7,485
Operating lease right-of-use assets	9,439	8,381
Other non-current assets	183	217
Total assets	\$ 343,320	\$ 160,364
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 6,275	\$ 3,592
Accrued expenses and other current liabilities	20,277	12,862
Deferred revenue	—	9,773
Total current liabilities	26,552	26,227
Term loan, net	50,106	43,942
Operating lease liability, net of current portion	18,172	17,361
Other non-current liabilities	1,552	1,309
Total liabilities	96,382	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 December 31, 2025, and December 31, 2024	—	—
Common stock, \$0.00001 par value per share; 700,000,000 shares authorized and 285,051,648 issued and outstanding as of December 31, 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	958,427	677,859
Accumulated other comprehensive loss	(192)	(4,031)
Accumulated deficit	(711,300)	(602,305)
Total stockholders' equity	246,938	71,525
Total liabilities and stockholders' equity	\$ 343,320	\$ 160,364

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