



Taysha Gene Therapies Reports First Quarter 2026 Financial Results and Provides Corporate Update

Reaffirmed FDA alignment on TSHA-102 BLA pathway, including pivotal trial design, endpoints and potential to submit for approval based on REVEAL pivotal trial 6-month interim analysis, following recent breakthrough therapy Type B meeting

Further advanced dosing in the REVEAL pivotal trial, with enrollment in the ASPIRE trial ongoing across multiple sites; on track to complete dosing in REVEAL and ASPIRE 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 May 2026 data cutoff; plan to present longer-term safety and efficacy data from Part A of REVEAL Phase 1/2 trials in Q2 2026

Initiated BLA-enabling PPQ campaign for TSHA-102 in April 2026; completion expected in Q4 2026

Conference call and webcast today at 8:30 AM ET

DALLAS, May 06, 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 first quarter ended March 31, 2026, and provided a corporate update.

"We continued to execute our clinical development strategy for TSHA-102 and recently reaffirmed alignment with the FDA on our pathway to a BLA filing, including trial design, endpoints and the potential to submit for approval based on the six-month interim analysis from the REVEAL pivotal trial," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "We further advanced dosing in the REVEAL pivotal trial, with enrollment in the ASPIRE trial ongoing across multiple sites, and we remain on track to complete dosing in both trials this quarter. In parallel, we plan to report longer-term safety and efficacy data from Part A of our REVEAL Phase 1/2 trials in the second quarter of this year."

Mr. Nolan continued, "Our pivotal development strategy is grounded in the rigor of our natural history analysis and Part A data collection and evaluation, with trial design, endpoints and statistical analyses developed based on discussions with the FDA. In our upcoming Part A data readout, we expect to report longer-term follow-up, including at least 12-month data from all 12 pediatric, adolescent and adult patients treated with TSHA-102. These results will include functional gains based on natural history-defined developmental milestones and additional skills and improvements that impact activities of daily living. We look forward to sharing this data as we continue to demonstrate the differentiated opportunity for TSHA-102 to deliver meaningful benefit to a broad population of patients with Rett syndrome who continue to face high unmet medical need."

Recent Corporate and TSHA-102 Program Highlights

- **Reaffirmed FDA Alignment on Planned BLA Submission Pathway for TSHA-102.** Following a recent initial breakthrough therapy Type B multidisciplinary meeting with the U.S. Food and Drug Administration (FDA), Taysha reaffirmed alignment on the planned pathway to a Biologics License Application (BLA) submission for TSHA-102, including:
 - Pivotal trial design and endpoints
 - BLA submission scenarios, including the potential to submit for approval based on the six-month interim analysis from the REVEAL pivotal trial
- **Further Advanced Dosing in REVEAL Pivotal Trial, with Multiple Patients Dosed 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. Standardized milestone assessments will be administered and captured on video at pre- and post-treatment timepoints, with determination of milestone gain/regain upon video-evidence review by independent, blinded central raters based on prespecified definitions of achievement for each milestone. The study includes a six-month interim analysis that may serve as the basis for BLA submission.
- **Enrollment in ASPIRE Trial is Ongoing Across Multiple Clinical Trial Sites.** The ASPIRE safety-focused trial is designed to support a planned BLA submission to enable broad labeling of TSHA-102 for patients aged ≥ 2 years with Rett syndrome. 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 all patients treated in the REVEAL Phase 1/2 and REVEAL pivotal trials as of the May 2026 data cutoff.

- Initiated BLA-Enabling PPQ Campaign for TSHA-102 Using Commercial Manufacturing Process in April 2026.**
 Following a Type C meeting in January 2026, the FDA endorsed the Company's proposed Process Performance Qualification (PPQ) campaign strategy in support of the BLA submission, enabling the execution of the BLA-enabling PPQ campaign using the commercial manufacturing process. Initiating the PPQ campaign ensures Chemistry Manufacturing and Controls (CMC) activities remain aligned with clinical development timelines for the planned BLA submission.
- Peer-Reviewed Publication Further Validates Lumbar IT Dosing as a Safe, Effective and Minimally Invasive Approach to Deliver a Gene Therapy to the CNS.** The publication titled "*rAAV9 Vector Biodistribution in Nonhuman Primate Brain and Spinal Cord Following Lumbar Intrathecal Infusion*" was published in *Frontiers in Medicine – Gene and Cell Therapy* (DOI: 10.3389/fmed.2026.1819594) on April 28, 2026. The publication includes preclinical data previously presented at the 2025 International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting, demonstrating IT and direct-to-brain intra-cisterna magna (ICM) administration showed comparable, consistent and widespread biodistribution of AAV9 vector throughout the brain and spinal cord regions in non-human primates.
- Abstract Accepted for Presentation at Upcoming American Society of Gene and Cell Therapy (ASGCT) 2026 Annual Meeting.** The poster titled "*Superior expression of self-complementary AAV and comparable functionality of mini and full-length MECP2 support the design of TSHA-102 for Rett syndrome*" will be presented on May 14, 2026, and highlights new preclinical in vitro data demonstrating that self-complementary AAV9 achieved significantly higher MeCP2 expression compared to single-stranded AAV9 in neuronal mouse cell models, and the comparable functionality of mini and full-length *MECP2*.

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 the REVEAL Phase 1/2 trials (n=12) is expected in the second quarter of 2026
- Completion of BLA-enabling PPQ campaign for TSHA-102 is expected in the fourth quarter of 2026

First Quarter 2026 Financial Highlights

Research and Development Expenses: Research and development expenses were \$33.8 million for the three months ended March 31, 2026, compared to \$15.6 million for the three months ended March 31, 2025. The \$18.2 million increase was primarily driven by BLA-enabling PPQ manufacturing initiatives performed during the three months ended March 31, 2026, and higher clinical expenses from the REVEAL and ASPIRE trials. Compensation expenses, including non-cash stock-based compensation, also increased as a result of additional research and development headcount.

General and Administrative Expenses: General and administrative expenses were \$9.7 million for the three months ended March 31, 2026, compared to \$8.2 million for the three months ended March 31, 2025. The increase of \$1.5 million was primarily due to higher compensation expenses, including non-cash stock-based compensation expense, and increases in consulting and professional fees, including commercial launch-readiness initiatives.

Net Loss: Net loss for the three months ended March 31, 2026, was \$42.4 million, or \$0.12 per share, compared to a net loss of \$21.5 million, or \$0.08 per share, for the three months ended March 31, 2025.

Cash and Cash Equivalents: As of March 31, 2026, Taysha had \$276.6 million in cash and cash equivalents. 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, regulatory and manufacturing 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, and the ability of Taysha's current cash resources to support planned operating expenses into 2028. 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 Three Months Ended March 31,	
	2026	2025
Revenue	\$ —	\$ 2,302
Operating expenses:		
Research and development	33,809	15,565
General and administrative	9,677	8,158
Total operating expenses	43,486	23,723
Loss from operations	(43,486)	(21,421)
Other income (expense):		
Change in fair value of warrant liability	—	102
Change in fair value of term loan	(1,470)	(1,530)
Interest income	2,586	1,326
Interest expense	(9)	(19)
Other income (expense)	(31)	13
Total other income (expense), net	1,076	(108)
Net loss	\$ (42,410)	\$ (21,529)
Net loss per common share, basic and diluted	\$ (0.12)	\$ (0.08)
Weighted average common shares outstanding, basic and diluted	366,632,827	269,306,331

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

	March 31, 2026	December 31, 2025
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 276,576	\$ 319,767
Restricted cash	449	449
Prepaid expenses and other current assets	5,225	4,431
Total current assets	282,250	324,647
Restricted cash	2,315	2,315

Property, plant and equipment, net	6,450	6,736
Operating lease right-of-use assets	9,155	9,439
Other non-current assets	181	183
Total assets	\$ 300,351	\$ 343,320
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 4,564	\$ 6,275
Accrued expenses and other current liabilities	15,534	20,277
Total current liabilities	20,098	26,552
Term loan, net	48,961	50,106
Operating lease liability, net of current portion	17,766	18,172
Other non-current liabilities	1,582	1,552
Total liabilities	88,407	96,382
Stockholders' equity		
Common stock, \$0.00001 par value per share; 700,000,000 shares authorized and 287,276,885 and 285,051,648 issued and outstanding as of March 31, 2026 and December 31, 2025, respectively	3	3
Additional paid-in capital	964,666	958,427
Accumulated other comprehensive income (loss)	985	(192)
Accumulated deficit	(753,710)	(711,300)
Total stockholders' equity	211,944	246,938
Total liabilities and stockholders' equity	\$ 300,351	\$ 343,320

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