



## Taysha Gene Therapies Reports Full-Year 2024 Financial Results and Provides Corporate Update

*High dose and low dose of TSHA-102 continue to be generally well tolerated with no treatment-related SAEs or DLTs in all pediatric, adolescent and adult patients treated (high dose, n=6; low dose, n=4) across both REVEAL trials as of February 2025 data cutoff*

*Completed dosing of the 10 patients in Part A of both REVEAL trials; maturing dataset continues to support advancement toward pivotal Part B trial*

*Productive ongoing discussions with the FDA to solidify regulatory pathway for TSHA-102; update on pivotal trial design expected in H1 2025*

*Clinical data from cohort two (high dose) and cohort one (low dose) of both REVEAL trials expected in H1 2025*

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

DALLAS, Feb. 26, 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 full year ended December 31, 2024, and provided a corporate update.

"We are pleased with the pace at which our TSHA-102 clinical program is advancing across a broad range of ages and stages of patients with Rett syndrome. TSHA-102 continues to be well tolerated in the pediatric, adolescent and adult patients treated across the high dose and low dose cohorts of our two REVEAL trials. With dosing of the 10 patients in Part A of our REVEAL trials complete, we have a strong, maturing dataset in hand to further solidify the regulatory pathway for TSHA-102 with the U.S. Food and Drug Administration (FDA)," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "We remain encouraged by our productive, ongoing discussions with the FDA, and we look forward to providing an update on the pivotal trial design for our TSHA-102 program in the first half of 2025. We also expect to provide an update on the clinical data from Part A, including the low and high dose cohorts in our adolescent and adult trial, as well as our pediatric trial, in the first half of 2025. We remain confident in our differentiated gene therapy candidate, which we believe has potential to provide meaningful therapeutic benefit to a broad population of patients with Rett syndrome."

### Recent Corporate and TSHA-102 Program Highlights

- **Completed Dosing of the 10 Patients in Part A of the REVEAL Trials.** Dosing of the 10 patients with Rett syndrome in Part A, the dose escalation portion of the REVEAL Phase 1/2 adolescent/adult trial and the REVEAL Phase 1/2 pediatric trial, has been completed. The dataset includes six patients in cohort two (high dose,  $1 \times 10^{15}$  total vector genomes (vg)) and four patients in cohort one (low dose,  $5.7 \times 10^{14}$  total vg). The Company believes this maturing dataset continues to support advancement toward the pivotal Part B trial for TSHA-102.
- **High Dose ( $1 \times 10^{15}$  total vg) and Low Dose ( $5.7 \times 10^{14}$  total vg) of TSHA-102 Continue to be Generally Well Tolerated.** TSHA-102 was generally well tolerated with no treatment-related serious adverse events (SAEs) or dose-limiting toxicities (DLTs) in the 10 pediatric, adolescent and adult patients dosed across the two REVEAL Phase 1/2 trials as of the February 17, 2025, data cutoff. This includes six patients in the high dose cohort and four patients in the low dose cohort.

### Anticipated Milestones

#### Regulatory Update

- Update on the pivotal trial design for TSHA-102 expected in the first half of 2025

#### REVEAL Adolescent and Adult Trial

- Safety and efficacy data in cohort two (high dose; n=3) and an update on safety and efficacy data in cohort one (low dose; n=2) expected in the first half of 2025

#### REVEAL Pediatric Trial

- Safety and efficacy data in cohort two (high dose; n=3) and an update on safety and efficacy data in cohort one (low dose; n=2) expected in the first half of 2025

### Full-Year 2024 Financial Highlights

**Research and Development Expenses:** Research and development expenses were \$66.0 million for the full year ended December 31, 2024, compared to \$56.8 million for the full year ended December 31, 2023. The \$9.2 million increase was driven by Good Manufacturing Practices batch activities for the intended commercial manufacturing process for TSHA-102 and additional clinical trial activities across the two REVEAL Phase 1/2 clinical trials in the year ended December 31, 2024.

**General and Administrative Expenses:** General and administrative expenses were \$29.0 million for the full year ended December 31, 2024, compared to \$30.0 million for the full year ended December 31, 2023. The decrease of \$1.0 million was primarily due to the decrease in issuance costs allocated to the liability-classified 2023 pre-funded warrants associated with the August 2023 financing.

**Net Loss:** Net loss for the full year ended December 31, 2024, was \$89.3 million, or \$0.36 per share, compared to a net loss of \$111.6 million, or \$0.96 per share, for the full year ended December 31, 2023.

**Cash and Cash Equivalents:** As of December 31, 2024, Taysha had \$139.0 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 fourth quarter of 2026.

#### 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 13751800. 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](http://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, 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, and our other product candidates 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, including the timing of initiating additional trials and reporting data from our clinical trials, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, the clinical potential of intrathecal administration and our current cash resources supporting our planned operating expenses and capital requirements into the fourth quarter of 2026. 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, 2024, which is available on the SEC's website at [www.sec.gov](http://www.sec.gov). Additional information will be made available in other filings that we make from time to time with the SEC. 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)

|                                 | For the Year<br>Ended December 31, |           |
|---------------------------------|------------------------------------|-----------|
|                                 | 2024                               | 2023      |
| Revenue                         | \$ 8,333                           | \$ 15,451 |
| Operating expenses:             |                                    |           |
| Research and development        | 66,001                             | 56,778    |
| General and administrative      | 28,953                             | 30,047    |
| Impairment of long-lived assets | 4,838                              | 1,065     |
| Total operating expenses        | 99,792                             | 87,890    |
| Loss from operations            | (91,459)                           | (72,439)  |

**Other income (expense):**

|                                                               |                    |                     |
|---------------------------------------------------------------|--------------------|---------------------|
| Change in fair value of warrant liability                     | 16                 | (34,718)            |
| Change in fair value of term loan                             | (4,583)            | (1,538)             |
| Loss on debt extinguishment                                   | —                  | (1,398)             |
| Interest income                                               | 6,940              | 3,572               |
| Interest expense                                              | (102)              | (4,998)             |
| Other expense                                                 | (110)              | (47)                |
| Total other income (expense), net                             | 2,161              | (39,127)            |
| <b>Net loss</b>                                               | <b>\$ (89,298)</b> | <b>\$ (111,566)</b> |
| Net loss per common share, basic and diluted                  | \$ (0.36)          | \$ (0.96)           |
| Weighted average common shares outstanding, basic and diluted | 250,134,421        | 116,121,482         |

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

|                                                                                                                                                                                                | December 31,<br>2024 | December 31,<br>2023 |
|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|----------------------|----------------------|
| <b>ASSETS</b>                                                                                                                                                                                  |                      |                      |
| Current assets:                                                                                                                                                                                |                      |                      |
| Cash and cash equivalents                                                                                                                                                                      | \$ 139,036           | \$ 143,940           |
| Restricted cash                                                                                                                                                                                | 449                  | 449                  |
| Prepaid expenses and other current assets                                                                                                                                                      | 2,645                | 3,479                |
| Assets held for sale                                                                                                                                                                           | —                    | 2,000                |
| Total current assets                                                                                                                                                                           | 142,130              | 149,868              |
| Restricted cash                                                                                                                                                                                | 2,151                | 2,151                |
| Property, plant and equipment, net                                                                                                                                                             | 7,485                | 10,826               |
| Operating lease right-of-use assets                                                                                                                                                            | 8,381                | 9,582                |
| Other non-current assets                                                                                                                                                                       | 217                  | 304                  |
| <b>Total assets</b>                                                                                                                                                                            | <b>\$ 160,364</b>    | <b>\$ 172,731</b>    |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>                                                                                                                                                    |                      |                      |
| Current liabilities:                                                                                                                                                                           |                      |                      |
| Accounts payable                                                                                                                                                                               | \$ 3,592             | \$ 6,366             |
| Accrued expenses and other current liabilities                                                                                                                                                 | 12,862               | 12,284               |
| Deferred revenue                                                                                                                                                                               | 9,773                | 18,106               |
| Total current liabilities                                                                                                                                                                      | 26,227               | 36,756               |
| Term loan, net                                                                                                                                                                                 | 43,942               | 40,508               |
| Operating lease liability, net of current portion                                                                                                                                              | 17,361               | 18,953               |
| Other non-current liabilities                                                                                                                                                                  | 1,309                | 1,577                |
| Total liabilities                                                                                                                                                                              | 88,839               | 97,794               |
| <b>Stockholders' equity</b>                                                                                                                                                                    |                      |                      |
| Preferred stock, \$0.00001 par value per share; 10,000,000 shares authorized and no shares issued and outstanding as of December 31, 2024, and December 31, 2023                               | —                    | —                    |
| Common stock, \$0.00001 par value per share; 400,000,000 shares authorized and 204,943,306 and 186,960,193 issued and outstanding as of December 31, 2024, and December 31, 2023, respectively | 2                    | 2                    |
| Additional paid-in capital                                                                                                                                                                     | 677,859              | 587,942              |
| Accumulated other comprehensive loss                                                                                                                                                           | (4,031)              | —                    |
| Accumulated deficit                                                                                                                                                                            | (602,305)            | (513,007)            |
| Total stockholders' equity                                                                                                                                                                     | 71,525               | 74,937               |
| <b>Total liabilities and stockholders' equity</b>                                                                                                                                              | <b>\$ 160,364</b>    | <b>\$ 172,731</b>    |

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Source: Taysha Gene Therapies, Inc.