



## Taysha Gene Therapies Reports Second Quarter 2024 Financial Results and Provides Corporate Update

*Presented cohort one (low dose) data from both trials of TSHA-102 at 2024 IRSF Rett Syndrome Scientific Meeting that demonstrated durable improvements across consistent clinical domains and an encouraging safety profile in adult (up to 52 weeks) and pediatric (up to 22 weeks) patients*

*TSHA-102 was generally well tolerated with no SAEs or DLTs as of initial six-week assessment in first patient in cohort two (high dose) of adolescent/adult trial; IDMC approved dosing of second (adolescent/adult trial) and first (pediatric trial) patient in cohort two, which is scheduled for Q3 2024*

*Cohort two (n=3) and cohort one (n=2) data from both trials (adolescent/adult and pediatric) expected in 1H 2025*

*\$76.8 million net proceeds from June 2024 public follow-on offering extends anticipated cash runway into Q4 2026*

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

DALLAS, Aug. 12, 2024 (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, 2024, and provided a corporate update.

"In the second quarter, we made strong progress across our TSHA-102 clinical program, including reporting encouraging preliminary data in our pediatric trial and longer-term data in our adolescent and adult trial following the low dose of TSHA-102. We dosed the first patient in the high dose cohort of our adolescent and adult trial, and TSHA-102 was generally well tolerated as of the initial six-week assessment. Subsequently, we received IDMC approval to proceed with dosing the second adolescent/adult and first pediatric patient in the high dose cohort of our REVEAL trials. This progress enables us to build on the promising low dose data that demonstrated an encouraging safety profile and improvements across consistent clinical domains impacting daily activities in adult and pediatric patients suffering from Rett syndrome," said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. "We remain focused on clinical trial execution and data collection that will further inform discussions with regulatory authorities on the development plan for the next phase of our studies."

Mr. Nolan continued, "Dosing of the second patient in our adolescent and adult trial and the first patient in our pediatric trial in the high dose cohort is scheduled for the third quarter of 2024. We are moving toward reporting cohort-based updates with more mature data sets to provide more fulsome updates on our clinical data. In line with this decision, we plan to report clinical data from the high dose cohorts and an update on clinical data from the low dose cohorts in both REVEAL trials in the first half of 2025. With our balance sheet strengthened and runway extended, we believe we are in an excellent position to execute on key value-creating milestones."

### Recent Corporate and Program Highlights

- Completed public follow-on offering with total net proceeds of \$76.8 million with anticipated cash runway into the fourth quarter of 2026

**REVEAL Phase 1/2 Adolescent and Adult Trial (Canada and United States (U.S.)):** a first-in-human, open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in adolescent and adult females aged 12 years and older with Rett syndrome due to *MECP2* loss-of-function mutation.

- Presented longer-term data from cohort one (low dose, n=2) of  $5.7 \times 10^{14}$  total vector genomes (vg) at the 2024 International Rett Syndrome Foundation (IRSF) Rett Syndrome Scientific Meeting:
  - Generally well tolerated with no serious adverse events (SAEs) related to TSHA-102 or dose-limiting toxicities (DLTs) as of the 52- and 36-week assessment for patient one and two, respectively
  - Sustained and new improvements across multiple efficacy measures and clinical domains relative to baseline, including fine and gross motor skills, communication/socialization, autonomic function and seizure events, through 52- and 25-weeks post-treatment for patient one and two, respectively
- Dosed the first patient in cohort two (high dose, n=3) of  $1 \times 10^{15}$  total vg, and TSHA-102 was generally well tolerated with no SAEs or DLTs as of the initial six-week assessment
- Enrolled the second patient in cohort two and scheduled dosing for the current quarter, following Independent Data Monitoring Committee (IDMC) review of initial six-week clinical data from the first patient dosed in cohort two

**REVEAL Phase 1/2 Pediatric Trial (U.S., United Kingdom (U.K.) and Canada):** an open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in pediatric females aged 5 to 8 years old with Rett syndrome due to *MECP2* loss-of-function mutation.

- Health Canada cleared the pediatric clinical trial application (CTA), enabling expansion of the ongoing U.S. and U.K. REVEAL pediatric trial into Canada

- Presented preliminary data from cohort one (low dose, n=2) of  $5.7 \times 10^{14}$  total vg at the 2024 IRSF Rett Syndrome Scientific Meeting:
  - Generally well-tolerated with no SAEs related to TSHA-102 or DLTs as of the 22- and 11-week assessment for patient one and two, respectively
  - Initial improvements across multiple efficacy measures and clinical domains relative to baseline, including fine and gross motor skills, communication/socialization, autonomic function and seizure events, as of 12- and eight-weeks post-treatment for patient one and two, respectively
- Enrolled the first pediatric patient in cohort two (high dose, n=3) of  $1 \times 10^{15}$  total vg and scheduled dosing for the current quarter, following IDMC approval to proceed with the Company's request for early advancement to cohort two after review of the initial six-week safety data from the first patient treated with the high dose of TSHA-102 in the adolescent and adult trial

#### Anticipated Milestones

- **REVEAL Adolescent and Adult Trial**
  - Dosing of the second patient in cohort two (high dose) scheduled for the third quarter of 2024
  - Safety and efficacy data from cohort two (n=3) and an update on safety and efficacy data from cohort one (n=2) expected in the first half of 2025
- **REVEAL Pediatric Trial**
  - Dosing of the first patient in cohort two (high dose) scheduled for the third quarter of 2024
  - Safety and efficacy data from cohort two (n=3) and an update on safety and efficacy data from cohort one (n=2) expected in the first half of 2025

#### Second Quarter 2024 Financial Highlights

**Research and Development Expenses:** Research and development expenses were \$15.1 million for the three months ended June 30, 2024, compared to \$19.8 million for the three months ending June 30, 2023. The \$4.7 million decrease was primarily due to a milestone fee payable to Abeona Therapeutics Inc. during the three months ended June 30, 2023, following the dosing of the first patient in the REVEAL Phase 1/2 adolescent and adult trial.

**General and Administrative Expenses:** General and administrative expenses were \$7.3 million for the three months ended June 30, 2024, compared to \$6.0 million for the three months ended June 30, 2023. The increase of \$1.3 million was primarily due to \$0.9 million of higher stock-based compensation expenses and \$0.4 million of higher consulting, professional fees and other expenses.

**Net loss:** Net loss for the three months ended June 30, 2024, was \$20.9 million, or \$0.09 per share, compared to a net loss of \$24.6 million, or \$0.38 per share, for the three months ended June 30, 2023.

**Cash and cash equivalents:** As of June 30, 2024, Taysha had \$172.7 million in cash and cash equivalents. Taysha expects 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 13747741. 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.tayshaqtx.com](http://www.tayshaqtx.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, 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, 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, 2023, 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 Three Months Ended June 30,		For the Six Months Ended June 30,	
	2024	2023	2024	2023
<b>Revenue</b>	\$ 1,112	\$ 2,395	\$ 4,523	\$ 7,101
<b>Operating expenses:</b>				
Research and development	15,073	19,791	35,730	32,305
General and administrative	7,338	5,988	14,422	14,739
Total operating expenses	22,411	25,779	50,152	47,044
<b>Loss from operations</b>	(21,299)	(23,384)	(45,629)	(39,943)
<b>Other income (expense):</b>				
Change in fair value of warrant liability	195	-	(142)	-
Change in fair value of term loan	(1,279)	-	(2,332)	-
Interest income	1,440	223	3,133	542
Interest expense	(27)	(1,440)	(56)	(2,814)
Other (expense) income	42	3	37	(5)
<b>Total other income (expense), net</b>	371	(1,214)	640	(2,277)
<b>Net loss</b>	<b>\$ (20,928)</b>	<b>\$ (24,598)</b>	<b>\$ (44,989)</b>	<b>\$ (42,220)</b>
Net loss per common share, basic and diluted	\$ (0.09)	\$ (0.38)	\$ (0.19)	\$ (0.66)
Weighted average common shares outstanding, basic and diluted	232,821,553	64,244,531	232,035,448	63,755,435

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

	June 30, 2024	December 31, 2023
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 172,743	\$ 143,940
Restricted cash	449	449
Prepaid expenses and other current assets	3,278	3,479
Assets held for sale	2,000	2,000
Total current assets	178,470	149,868
Restricted cash	2,151	2,151
Property, plant and equipment, net	10,513	10,826

Operating lease right-of-use assets	8,971	9,582
Other non-current assets	288	304
<b>Total assets</b>	<b>\$ 200,393</b>	<b>\$ 172,731</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable	\$ 8,718	\$ 6,366
Accrued expenses and other current liabilities	11,875	12,284
Deferred revenue	13,583	18,106
Total current liabilities	34,176	36,756
Term loan, net	37,835	40,508
Operating lease liability, net of current portion	18,134	18,953
Other non-current liabilities	1,380	1,577
Total liabilities	91,525	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 June 30, 2024, and December 31, 2023	—	—
Common stock, \$0.00001 par value per share; 400,000,000 shares authorized and 201,381,450 and 186,960,193 issued and outstanding as of June 30, 2024, and December 31, 2023, respectively	2	2
Additional paid-in capital	664,457	587,942
Accumulated other comprehensive income	2,405	—
Accumulated deficit	(557,996)	(513,007)
Total stockholders' equity	108,868	74,937
<b>Total liabilities and stockholders' equity</b>	<b>\$ 200,393</b>	<b>\$ 172,731</b>

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