



## Taysha Gene Therapies Announces Regenerative Medicine Advanced Therapy (RMAT) Designation Granted by U.S. FDA for TSHA-102 in Rett Syndrome

*RMAT designation follows FDA's review of available safety and efficacy data from the first three patients dosed with the low dose of TSHA-102 across both REVEAL Phase 1/2 trials (adolescent/adult and pediatric)*

*RMAT designation enables increased dialogue with the FDA to support the potential expedited development and review of TSHA-102 in clinical evaluation for Rett syndrome*

DALLAS, May 02, 2024 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a clinical-stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of severe monogenic diseases of the central nervous system (CNS), today announced the United States Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to TSHA-102, a self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome. RMAT designation was granted following the FDA's review of clinical data supporting the potential of TSHA-102 to address the unmet medical need for patients with Rett syndrome.

RMAT designation was designed to expedite the development and review of regenerative medicine therapies. A regenerative medicine therapy is eligible for RMAT designation if it is intended to treat, modify, reverse or cure a serious condition, and preliminary clinical evidence indicates the therapy has the potential to address unmet medical needs for such condition. Sponsor companies receiving RMAT designation can benefit from increased interactions with the FDA involving senior managers, with the goal of expediting drug development. RMAT designation follows the FDA's review of available safety and efficacy data from the first three patients with Rett syndrome dosed with the low dose of TSHA-102 ( $5.7 \times 10^{14}$  total vg) across the REVEAL Phase 1/2 adolescent and adult trial and the REVEAL Phase 1/2 pediatric trial.

"We believe receiving RMAT designation reinforces the high unmet medical need in Rett syndrome and the therapeutic potential of TSHA-102 to change the treatment paradigm," said Sukumar Nagendran, M.D., President and Head of R&D of Taysha. "Importantly, RMAT designation was granted following the FDA's review of safety and efficacy data from the first three patients dosed with the low dose of TSHA-102 across both of our REVEAL Phase 1/2 trials. We believe this important recognition from the FDA further supports the potential of our gene therapy candidate to bring meaningful change to patients and families living with Rett syndrome."

Rumana Haque-Ahmed, Chief Regulatory Officer of Taysha, added, "We remain focused on advancing the development TSHA-102 to bring a potentially disease-modifying therapy being evaluated to address the genetic root cause of Rett syndrome to all patients and families living with this devastating disease. Receiving RMAT designation helps facilitate this goal by enabling increased dialogue with the FDA to expedite our development plan for TSHA-102. We look forward to working closely with the FDA and other regulatory agencies as we continue to advance our TSHA-102 program."

TSHA-102 is being evaluated in the [REVEAL Phase 1/2 adolescent and adult trial](#) taking place in Canada and the U.S., and in the [REVEAL Phase 1/2 pediatric trial](#) taking place in the U.S. and cleared in the U.K.

### 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 our first patients dosed in the REVEAL trials, the potential for TSHA-102 to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and the potential benefits of Regenerative Medicine Advanced Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for TSHA-102. 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. 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.

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