Taysha Gene Therapies Announces Two Poster Presentations on TSHA-102 in Rett Syndrome at Upcoming European Society of Gene & Cell Therapy (ESGCT) 30th Annual Congress

DALLAS, Oct. 10, 2023 (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 monogenic diseases of the central nervous system (CNS), today announced that it will present data on its TSHA-102 program in evaluation for Rett syndrome during two poster presentations at the European Society of Gene & Cell Therapy (ESGCT) 30th Annual Congress, taking place in Brussels, Belgium from October 24-27, 2023.

TSHA-102 is a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy that 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. The Company will present new preclinical *in vitro* data supporting the miRARE technology, as well as initial clinical data from the first adult patient dosed with TSHA-102 in the REVEAL Phase 1/2 adult trial.

Poster presentation details are as follows:

Abstract Title: The microRNA-responsive autoregulatory element from TSHA-102 for Rett Syndrome modulates therapeutic transgene expression in response to cellular MECP2 in mouse and human cell lines
Presenters: Emdadul Haque, Ph.D., Director, Translational Sciences, and Fred Porter, Ph.D., Chief of Staff and Technical Operations Officer, Taysha Gene Therapies
Poster Session Date/Time: Wednesday, October 25 at 17:00-18:15 CET and Thursday, October 26 at 20:30-21:30 CET
Poster Session: CNS & Sensory Diseases
Poster Number: P435

Abstract Title: Early safety and efficacy observations following the first use of TSHA-102 gene therapy in a patient with Rett Syndrome
Presenter: Benit Maru, MBChB, Ph.D., Chief Medical Officer and Head of Clinical Development, Taysha Gene Therapies
Poster Session Date/Time: Wednesday, October 25 at 18:15-19:30 CET and Thursday, October 26 at 19:30-20:30 CET
Poster Session: Accessibility of Gene Therapy
Poster Number: P302

Additional details on the meeting can be found at the ESGCT 30th Annual Congress website.

About TSHA-102
TSHA-102 is a self-complementary intrathecally delivered AAV9 investigational gene transfer therapy in clinical evaluation for Rett syndrome. 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 Fast Track designation and Orphan Drug and Rare Pediatric Disease designations from the Food and Drug Administration (FDA) and has been granted Orphan Drug designation from the European Commission.

About Rett Syndrome
Rett syndrome is a rare neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene, which is a gene that's essential for neuronal and synaptic function in the brain. The disorder is characterized by intellectual disabilities, loss of communication, seizures, slowing and/or regression of development, motor and respiratory impairment, and shortened life expectancy. 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 Unites States, European Union and the United Kingdom.

About Taysha Gene Therapies
Taysha Gene Therapies (Nasdaq: TSHA) is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we aim to rapidly translate our treatments from bench to bedside. We have combined our team's proven experience in gene therapy drug development and commercialization with the world-class University of Texas Southwestern Gene Therapy Program. Together, we leverage our fully integrated platform with a goal of dramatically improving patients' lives. More information is available at www.tayshagtx.com.

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