Taysha Gene Therapies Announces Positive Recommendation from Independent Data Monitoring Committee of REVEAL Phase 1/2 Trial in Rett Syndrome

Independent Data Monitoring Committee recommended REVEAL Phase 1/2 trial continuation and proceeding with dosing of second patient based on encouraging initial clinical data from the first adult with Rett syndrome dosed with investigational gene therapy TSHA-102

Initial clinical update from the first patient dosed with TSHA-102 planned for forthcoming quarterly earnings call

Dosing of second patient expected in the third quarter of 2023

DALLAS, July 31, 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), announced today that the Independent Data Monitoring Committee (IDMC) recommended the continuation of the REVEAL Phase 1/2 trial and that dosing of the second patient in the first cohort can proceed. The decision follows a pre-specified IDMC review of initial clinical data from the first patient dosed with TSHA-102 following the 42-day evaluation period.

“We thank the IDMC members for their guidance and are pleased with their recommendation to continue the REVEAL Phase 1/2 trial,” said Sukumar Nagendran, M.D., President and Head of R&D of Taysha. “This recommendation was based on the analysis of initial clinical data from the first adult patient with Rett syndrome to receive TSHA-102. A second patient is expected to be dosed in the third quarter of this year. We are highly encouraged by the initial clinical observations, which support the transformative potential of TSHA-102 and mark important progress in our efforts to bring a gene therapy to patients and families living with Rett syndrome. We look forward to providing an initial clinical update on the first patient at our second quarter corporate update conference call in mid-August."

The REVEAL Phase 1/2 trial is a first-in-human, open-label, randomized, dose-escalation and dose-expansion study evaluating the safety and preliminary efficacy of TSHA-102 in adult females with Rett syndrome due to *MECP2* loss-of-function mutation. TSHA-102 is administered as a single lumbar intrathecal injection. Dose escalation will evaluate two dose levels of TSHA-102 sequentially. The maximum tolerated dose (MTD) or maximum administered dose (MAD) established will then be administered during dose expansion.

About TSHA-102
TSHA-102 is an investigational self-complementary intrathecally delivered AAV9 gene transfer therapy in clinical evaluation for Rett syndrome, a rare genetic neurodevelopmental disorder caused by mutations in the X-linked *MECP2* gene. TSHA-102 utilizes a novel miRNA-Responsive Auto-Regulatory Element (miRARE) platform designed to regulate cellular *MECP2* expression. TSHA-102 has received Orphan Drug and Rare Pediatric Disease designations from the FDA and has been granted Orphan Drug designation from the European Commission. TSHA-102 is being evaluated in the first-in-human, open label, randomized, dose escalation and dose-expansion REVEAL Phase 1/2 trial for adult female patients with Rett syndrome.

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 U.S., EU and UK.

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 UT 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.

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 administration, timing, progress and results of our preclinical studies and clinical trials of our product candidates, including TSHA-102, including statements regarding the dosing of additional patients in REVEAL Phase 1/2 trial, the timing of initiation and completion of studies or trials and related preparatory work and the period during which the results of the trials will become available, the potential of our product candidates, including TSHA-120 and TSHA-102, 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, our plans for regulatory applications submissions, the potential for these 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 these product candidates. 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, 2022, which is available on the SEC’s website at www.sec.gov. Additional information will be made available in other filings that we make from time to time with the SEC. Such risks may be amplified by the impacts of the COVID-19 pandemic. 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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