Taysha Gene Therapies Announces Late-Breaking Abstract and Poster Presentation on Positive Preclinical Data For TSHA-105 Demonstrating Therapeutic Potential for the Treatment of Epilepsy Caused by SLCA13A5 Deficiency

Data presented at the American Epilepsy Society Annual Meeting

TSHA-105 normalized electroencephalogram (EEG) and citrate levels and improved survival in both early treated juvenile and later treated adult preclinical mouse models of SLC13A5 deficiency

No adverse findings detected following CSF delivery of TSHA-105

TSHA-105 granted orphan drug designation from the U.S. FDA and the European Commission

IND/CTA filing expected in 2022

DALLAS--(BUSINESS WIRE)--Dec. 6, 2021-- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric, pivotal-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) in both rare and large patient populations, today announced a late-breaking abstract and poster presentation by Dr. Rachel Bailey, Assistant Professor at UT Southwestern Medical Center on positive preclinical data for TSHA-105, an AAV9-based gene therapy in development for SLC13A5-related epilepsy at the American Epilepsy Society Annual Meeting on December 6th 2021.

“SLC13A5-related epilepsy results from a mutation in the *SLC13A5* gene that prevents citrate from being taken up into neurons in the brain. Affected children present with seizures beginning within a few days of birth and as they continue to grow, have motor progression and difficulty with speech and language development. Some children never achieve walking independently,” said Rachel M. Bailey, Ph.D., Assistant Professor with the Center for Alzheimer's and Neurodegenerative Diseases and Pediatrics at UT Southwestern. “Currently there are no treatments for SLC13A5 deficiency that target the underlying cause of disease. In knockout mouse models of SLC13A5 deficiency, treatment with TSHA-105 resulted in normalized citrate levels, reduced seizure activity and improved survival regardless of age. We are highly encouraged by the positive therapeutic response and absence of toxicity in these preclinical models and look forward to further exploring the possible utility of TSHA-105 as a treatment for SLC13A5 deficiency.”

Dr. Bailey, whose research is supported in part by Taysha, and UT Southwestern maintain financial interests in Taysha Gene Therapies due to their development of the intellectual property that serves as the basis for TSHA-105.

“These preclinical results suggest that TSHA-105 can demonstrate functional improvements with intervention at any age in a potentially safe and tolerable manner which would be expected to translate into a meaningful benefit to patients with SCL13A5 deficiency,” said Suyash Prasad, MBBS, M.Sc., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha. “These highly encouraging preclinical data further support our plan to submit an IND/CTA filing in 2022.”

TSHA-105 is a self-complementary vector encoding a codon-optimized human *SLC13A5* gene that was evaluated in an SLC13A5 knockout (KO) mouse model recapitulating the increased plasma citrate levels, electroencephalogram (EEG) abnormalities and an increased susceptibility to seizure induction seen in patients. CSF-delivered TSHA-105 significantly decreased plasma citrate levels in SLC13A5 KO mice and reduced epileptic activity. Increased seizure susceptibility in SLC13A5 KO mice measured by the Racine scale, a well-established methodology for assessing seizure severity in preclinical models, was attenuated with TSHA-105 treatment in both age groups. No adverse findings were detected following CSF delivery of TSHA-105. An IND/CTA filing for TSHA-105 for the treatment of SLC13A5-related epilepsy is expected in 2022. TSHA-105 previously received orphan drug designation from the U.S. Food and Drug Administration and from the European Commission.

SLC13A5 deficiency is a form of infantile epilepsy caused by mutations in the *SLC13A5* gene. As an autosomal recessive disorder, two copies of the mutated gene must be inherited for an infant to be affected. This type of epilepsy manifests as developmental delay, and seizures beginning within the first few days of life. SLC13A5 deficiency is a rare disorder, with an estimated prevalence of 1,900 patients in the United States and in Europe. Current standards of care include anti-seizure medications which only target the symptoms and do not address the underlying cause of the disease.

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 to build an extensive, AAV gene therapy pipeline focused on both rare and large-market indications. Together, we leverage our fully integrated platform—an engine for potential new cures—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,” and “future” or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning the potential of our product candidates, including TSHA-105, 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, TSHA-105’s eligibility for accelerated approval in the United States and Europe, 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, 2020 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, both of which are 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20211206005635/en/

Company Contact:
Kimberly Lee, D.O.
SVP, Corporate Communications and Investor Relations
Taysha Gene Therapies
klee@tayshagtx.com

Media Contact:
Carolyn Hawley
Canale Communications
carolyn.hawley@canalecomm.com

Source: Taysha Gene Therapies, Inc.