Taysha Gene Therapies Receives Rare Pediatric Disease and Orphan Drug Designations for TSHA-105 for the Treatment of Epilepsy Caused by SLC13A5 Deficiency

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Designations reinforce unmet need for treatment options for patients with rare form of genetic epilepsy

TSHA-105 is the second program from Taysha's genetic epilepsy franchise to receive dual designations

TSHA-105 joins a portfolio of rare pediatric disease and orphan drug designations obtained in multiple pipeline programs, including TSHA-101 for GM2 gangliosidosis, TSHA-118 for CLN1, TSHA-102 for Rett syndrome, TSHA-104 for SURF1-associated Leigh syndrome and TSHA-103 for SLC6A1-related epilepsy

DALLAS--(BUSINESS WIRE)--Jan. 19, 2021-- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations, today announced that it has received both rare pediatric disease and orphan drug designations from the U.S. Food and Drug Administration (FDA) for TSHA-105, an AAV9-based gene therapy in development for SLC13A5-related epilepsy.

"There are no approved therapies for epilepsy caused by SLC13A5 that address the underlying cause of this disease," said RA Session II, President, Founder and CEO of Taysha. "We are encouraged by the early evidence of TSHA-105's disease-modifying approach and believe these designations will help us potentially accelerate the development of this exciting program. We look forward to working with the FDA to make TSHA-105 available to patients as expeditiously as possible."

SLC13A5 is a form of infantile epilepsy caused by mutations in the *SLC13A5* gene. The disorder is an autosomal recessive disorder, so two copies of the mutated gene must be inherited to affect an infant. This rare form of epilepsy manifests as developmental delay, and seizures beginning within the first few days of life.

"We are pleased that the FDA recognizes TSHA-105's potential as an innovative therapeutic option for SLC13A5 deficiency," said Rachel Bailey, Ph.D., Assistant Professor in Pediatric Neurology at UT Southwestern. "This disease is a debilitating form of genetic epilepsy in children that significantly impacts movement, motor control, cognition and quality of life, and there remains a need to alter the course of this disease early in life."

"As a mother of two children with SLC13A5 deficiency, I have witnessed firsthand the devastating impact that numerous seizures and comorbidities accompanying the disease has on those affected by this disease," said Kim Nye, Founder of TESS Research Foundation. "Taysha's commitment to developing a potentially life-changing gene therapy for SLC13A5 deficiency is greatly welcomed by our patient community."

The FDA grants rare pediatric disease designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the United States. The Rare Pediatric Disease Priority Review Voucher Program is intended to address the challenges that drug companies face when developing treatments for these unique patient populations. Under this program, companies are eligible to receive a priority review voucher following approval of a product with rare pediatric disease designation if the marketing application submitted for the product satisfies certain conditions, including approval prior to September 30, 2026 unless changed by legislation. If issued, a sponsor may redeem a priority review voucher for priority review of a subsequent marketing application for a different product candidate, or the priority review voucher could be sold or transferred to another sponsor.

Orphan drug designation is granted by the FDA Office of Orphan Products Development to investigational treatments that are intended for the treatment of rare diseases affecting fewer than 200,000 people in the United States. The program was developed to encourage the development of medicines for rare diseases, and benefits include tax credits and application fee waivers designed to offset some development costs, as well as eligibility for market exclusivity for seven years post approval.

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 or implying 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, the potential benefits of rare pediatric disease designation and orphan drug designation to our product candidates, 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. 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, 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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