Taysha Gene Therapies Receives Orphan Drug Designation for TSHA-105 for the Treatment of Epilepsy Caused by SLC13A5 Deficiency From the European Commission

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Designation supports unmet need for treatment options for patients with rare form of genetic epilepsy

TSHA-105 is first program in Taysha's pipeline to receive designation from European Commission

No approved treatments for the underlying cause of the disease

DALLAS--(BUSINESS WIRE)--Aug. 25, 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 that it has been granted orphan drug designation from the European Commission for TSHA-105, an AAV9-based gene therapy in development for SLC13A5-related epilepsy.

"SLC13A5 deficiency leads to a debilitating form of genetic epilepsy in children that results in persistent seizures and developmental delays, requiring constant supervision and care. With no available disease modifying treatments for this disease, we are pleased that TSHA-105 has been granted orphan drug designation from both the FDA and European Commission, highlighting the global need and important potential of TSHA-105 in helping treat this form of epilepsy," said RA Session II, President, Founder and CEO of Taysha. "We look forward to working with regulatory agencies to advance this promising gene replacement strategy as expeditiously as possible."

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 are anti-seizure medications which only target the symptoms and do not address the underlying cause of the disease.

The European Commission grants orphan drug designation for medicines being developed for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions that affect fewer than 5 in 10,000 people in the European Union. Orphan designation in the European Union includes benefits such as protocol assistance, reduced regulatory fees and market exclusivity.

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 June 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.

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