Taysha Gene Therapies Receives Orphan Drug Designation from the European Commission for TSHA-120 for the Treatment of Giant Axonal Neuropathy (GAN)

Clinical efficacy data for TSHA-120 provide quantitative evidence of long-term durability across all therapeutic dose cohorts with a 10-point improvement in mean change in MFM32 by Year 3 compared to estimated natural history decline of 24 points

Biopsy data in five of six patient samples analyzed to date confirmed active regeneration of nerve fibers following treatment with TSHA-120

53 patient-years of clinical data support favorable safety and tolerability profile of TSHA-120

Estimated addressable patient population of 5,000 worldwide represents a multi-billion dollar commercial opportunity

No approved treatments for the underlying cause of the disease

DALLAS, May 03, 2022 (GLOBE NEWSWIRE) -- 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-120, an intrathecally dosed AAV9 gene therapy currently in ongoing clinical evaluation for the treatment of giant axonal neuropathy (GAN).

“GAN is a progressive and devastating neurodegenerative disease that has an estimated addressable patient population of 5,000 worldwide. The disease impacts a broad range of patients, with an early onset form of the disease affecting young infants, while a late onset can affect patients into adulthood,” said Suyash Prasad, MBBS, M.SC., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha. “In January we announced promising data for TSHA-120, our most advanced program, demonstrating long-term durability for all three therapeutic dose cohorts and clinically significant improvements in MFM32 over time compared to decline in patients observed in natural history studies. The long-term safety and tolerability of TSHA-120 was supported by 53-patient years of data, and importantly, biopsy data confirmed active nerve fiber regeneration following treatment with TSHA-120. We are pleased to receive orphan drug designation by the European commission which can help facilitate rapid clinical advancement and subsequent access to patients as we further approach regulatory approval.”

GAN is a rare inherited genetic disorder that is a progressive neurodegenerative disease that affects both the central and peripheral nervous systems. The disease is caused by loss-of-function mutations in the gene coding for *gigaxonin*, which results in dysregulation of intermediate filament turnover, an important structural component of the cell. Children with GAN present before the age of five with symptoms including unsteady gait, frequent falls, motor weakness. Currently, there are no approved treatments for GAN, which results in death for patients in their late teens or early twenties.

TSHA-120, an intrathecally dosed AAV9 gene replacement therapy delivering the gene gigaxonin for the treatment of GAN is currently being evaluated in an ongoing clinical trial conducted by the National Institute of Neurological Disorders and Stroke (NINDS) division of the National Institutes of Health (NIH) under the leadership of principal investigator, Carsten Bönneman, M.D. Taysha has partnered with GeneDx to support inclusion of the genetic marker for GAN in the GeneDx hereditary neuropathy panel at no cost to individuals at risk for or suspected of having GAN, and with the Hereditary Neuropathy Foundation and Charcot-Marie-Tooth Association Centers of Excellence to increase GAN disease awareness and access to testing. TSHA-120 has previously received Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA).

The European Commission grants orphan drug designation for medicines being developed for the diagnosis, prevention or treatment of treat 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 goa 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-120, 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-120’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, 2021, 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.

Company Contact:
Kimberly Lee, D.O.
Chief Corporate Affairs Officer
Taysha Gene Therapies
klee@tayshagtx.com

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

Source: Taysha Gene Therapies, Inc.