Taysha Gene Therapies Announces Positive Safety Data from UT Southwestern-Sponsored Clinical Trial for the Treatment of CLN7 Batten Disease at 18th Annual WORLDSymposium

February 9, 2022

Data demonstrated favorable safety and tolerability profile at 1.0x10^{15} total vg, the highest dose ever delivered intrathecally in humans

Stabilization in nerve conduction demonstrated absence of dorsal root ganglia inflammation

Taysha holds exclusive option to license CLN7 Batten disease program from UT Southwestern

DALLAS--(BUSINESS WIRE)--Feb. 9, 2022-- 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 the presentation of clinical safety data supporting the first-generation construct at the highest dose of 1.0x10^{15} total vg for the treatment of CLN7 Batten disease at the 18th Annual WORLD *Symposium*, held February 7-11, 2022, in San Diego, CA by investigators from UT Southwestern.

“This is the first ever gene therapy trial for the treatment of CLN7 Batten disease, and we are encouraged that the first-generation construct was well tolerated by all patients treated at the highest dose ever delivered intrathecally in humans,” said Suyash Prasad, MBBS, M.Sc., MRCP, MRCPCH, FFPM, Chief Medical Officer and Head of Research and Development of Taysha. “Importantly, the absence of evidence of dorsal root ganglia inflammation following gene transfer supports the therapy’s safety profile and provides positive readthrough across our platform.”

Safety data for the first-generation construct for the treatment of CLN7 Batten disease from the ongoing clinical trial following intrathecal administration further demonstrated that the first-generation construct was well-tolerated at multiple doses including 1.0x10^{15} total vg, which is the highest dose administered in humans ever for a gene therapy product. No adverse immune responses have been noted, including no evidence of dorsal root ganglion toxicity or brain inflammation across all subjects. Moreover, stabilization in sural nerve conduction supported the absence of dorsal root ganglia inflammation. The ongoing trial includes three patients dosed to date, with two patients treated at the highest dose of 1.0x10^{15}. Complete blood counts revealed no signs of bone marrow suppression or clinically significant bone marrow reactivity, and cerebral spinal fluid (CSF) analysis revealed no signs of pleocytosis.

The clinical trial is a UTSW-sponsored, first-in-human, single center, open-label, dose escalation study enrolling patients at Children’s Health. The primary endpoint of the trial is safety and tolerability by incidence and severity of treatment related serious adverse events. Secondary efficacy endpoints include the Clinical Global Impression, neuropsychological, ataxia and motor function assessments and quality of life. Taysha holds the exclusive option to license the CLN7 Batten disease program from UT Southwestern.

CLN7 Batten disease is a rare, fatal, and rapidly progressive neurodegenerative disease that is a form of Batten disease. CLN7 is caused by autosomal recessive mutations in the *MFSD8* gene that results in lysosomal dysfunction. Disease onset occurs around two to five years of age, with death often ensuing in young adolescence. Patients experience gradual nerve cell loss in certain parts of the brain and typically present with seizures, vision loss, speech impairment, and mental and motor regression. Currently, there are no approved therapies to treat CLN7 Batten disease, which impacts an estimated 4,000 patients globally.

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 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, 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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