Taysha Gene Therapies Provides Update on TSHA-120 Program in Giant Axonal Neuropathy (GAN)

Following Type C meeting feedback from the U.S. FDA, Taysha is discontinuing development of TSHA-120 in GAN due to challenges with study design feasibility for potential Biologics License Application (BLA) submission

Taysha will pursue external strategic options for the TSHA-120 program to potentially enable further program development

Strategic program prioritization will reduce operating expenses and is anticipated to extend cash runway into the fourth quarter of 2025 to support the continued development of TSHA-102 in evaluation for Rett syndrome

DALLAS, Sept. 19, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a clinical-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), today announced that subsequent to the receipt of Type C meeting feedback from the United States (U.S.) Food and Drug Administration (FDA) regarding a registrational path for TSHA-120, the Company will discontinue the development of its TSHA-120 program in evaluation for the treatment of giant axonal neuropathy (GAN). Further, Taysha announced that Astellas Gene Therapies, Inc. (f/k/a Audentes Therapeutics, Inc. (d/b/a Astellas Gene Therapy)) (Astellas) has elected not to exercise its option to obtain an exclusive license to TSHA-120 under the Option Agreement between Astellas and Taysha.

“We believe we have made significant progress in demonstrating the therapeutic potential of TSHA-120 and identifying a potential registrational path. Following FDA feedback, we have made the decision to discontinue further development of the program due to challenges related to the feasibility of the study designs to support a potential BLA submission in this ultra-rare neurodegenerative disease,” said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. “I want to express our gratitude to the patients and families who participated in the trial, the GAN community, and the National Institutes of Health (NIH) for their partnership in establishing the foundation for a potential treatment option in GAN. We plan to pursue external strategic options for TSHA-120 that may enable further development of TSHA-120 and help patients with this devastating disease.”

“This strategic program prioritization is expected to extend our cash runway into the fourth quarter of 2025 to support the continued clinical development of TSHA-102 in Rett syndrome, a rare neurodevelopmental disorder with no approved treatments that target the genetic root cause of the disease. We remain focused on continuing to evaluate the therapeutic potential of TSHA-102 in our ongoing REVEAL Phase 1/2 trial in adults and our planned pediatric trial,” concluded Mr. Nolan.

Richard Wilson, Senior Vice President, Primary Focus Lead of Genetic Regulation of Astellas, added, “While Astellas has declined to exercise its option for the GAN program, we remain focused on the needs of patients impacted by devastating diseases and look forward to continuing our relationship with Taysha.”

In 2022, Taysha submitted and reviewed with the FDA in a Type B end-of-Phase 2 meeting, a subset of available evidence from a Phase 1/2 clinical trial investigating TSHA-120 for the treatment of GAN, which was initiated by the NIH. FDA feedback included the need to address the heterogeneity of disease progression in GAN and the effort-dependent nature of MFM32 as a primary endpoint in an unblinded study. To further discuss a potential regulatory path forward for TSHA-120, Taysha submitted a new comprehensive analysis of the totality of data from the natural history and interventional trial comparing functional and biological measurements against a Disease Progression Model (DPM) as part of a Type C meeting request to the FDA in June 2023.

FDA Type C meeting feedback indicated that the FDA continues to recommend a randomized, double-blind, placebo-controlled trial as the optimal path to demonstrate efficacy in TSHA-120. Among other areas of feedback, the FDA also provided a potential path for a single-arm trial with an external control group matched with to-be treated patients by multiple prognostic factors and recommended longer term follow up to account for potential bias.

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. Together, we leverage our fully integrated platform 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,” “plans,” 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, 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, our ability to pursue strategic options for TSHA-120, anticipated cost savings due to the discontinuation of development of TSHA-120, our expected cash runway, the potential benefits of Taysha’s collaboration with Astellas and the potential for Astellas to exercise any of the options granted to it by Taysha. 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, 2022, 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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