Taysha Gene Therapies Provides Update on TSHA-120 Program in Giant Axonal Neuropathy and a 2023 Corporate Outlook

Type B end-of-Phase 2 meeting with U.S. Food and Drug Administration (FDA) provided additional clarity for TSHA-120 for the treatment of giant axonal neuropathy (GAN) ultra-rare disease program
- FDA acknowledged MFM32 as an acceptable endpoint with a recommendation to dose additional patients in a double-blind, placebo-controlled design to support Biologics License Application (BLA) submission

Organizational and business review by new management with operational, structural and personnel changes implemented to enhance execution

Dosing of first adult patient with Rett syndrome from ongoing trial in Canada expected in H1 2023; update of initial available clinical data anticipated in H1 2023 with quarterly updates primarily on safety thereafter

Submission of Clinical Trial Application (CTA) to United Kingdom (UK) MHRA for TSHA-102 in pediatric patients with Rett syndrome expected in mid-2023

Submission of an Investigational New Drug (IND) application for TSHA-102 for Rett syndrome to FDA planned in H2 2023

Conference call and live webcast today at 4:30 PM Eastern Time

DALLAS, Jan. 31, 2023 (GLOBE NEWSWIRE) -- Taysha Gene Therapies, Inc. (Nasdaq: TSHA), a patient-centric, clinical -stage gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic rare diseases of the central nervous system (CNS), today provided an update on the TSHA-120 program in giant axonal neuropathy (GAN) and a corporate outlook for 2023.

“We expect to deliver on several key milestones in 2023, including the generation of first-in-human adult clinical data in Rett syndrome, CTA submission to MHRA to enable initiation of our pediatric Rett syndrome program and submission of an IND for Rett syndrome in the U.S. to further expand our clinical study footprint. For our GAN program, we received the formal FDA meeting minutes and recently submitted follow up questions to clarify some of their recommendations including the feasibility of a proposed study design and the totality of evidence required for BLA submission. Their feedback will help inform next steps for the program in this ultra-rare indication with no approved treatments,” said Sean P. Nolan, Chairman and Chief Executive Officer of Taysha. “I believe that the operational, structural and personnel actions recently implemented position us well to execute across our near-term milestones and deliver on our commitments to key stakeholders, especially patients.”

Clinical Program Updates

TSHA-120 in GAN:

- Receipt of formal written meeting minutes from FDA in January 2023 following completion of Type B end-of-Phase 2 meeting
    - Overall approach to manufacturing of pivotal/to-be marketed product deemed appropriate pending review of a planned submission of Chemistry, Manufacturing, and Controls (CMC) data package for TSHA-120
    - FDA acknowledged MFM32 as an acceptable endpoint with a recommendation to dose additional patients in a double-blind, placebo-controlled design to support BLA submission
- Awaiting response from FDA on follow up questions the Company submitted on recommended design and totality of evidence required for BLA submission

TSHA-102 in Rett syndrome:

- Dosing of the first adult patient with Rett syndrome anticipated in H1 2023
- Initial available clinical data for TSHA-102 in the adult Rett syndrome study expected in H1 2023 with planned quarterly updates on available clinical data primarily on safety from the adult study thereafter
- Company anticipates submission of a CTA to UK MHRA for TSHA-102 in pediatric patients with Rett syndrome in mid-2023
- Company plans to submit an IND application for Rett syndrome to FDA in H2 2023

Corporate Updates

- Operational, structural and personnel changes implemented following thorough business review to enhance execution

Conference Call and Webcast Information

Taysha management will hold a conference call and webcast today at 4:30 pm ET to provide regulatory feedback from FDA on the GAN program and a corporate update. The dial-in number for the conference call is 877-407-0792 (U.S./Canada) or 201-689-8263 (international). The conference ID for all callers is 13736009. The live webcast and replay may be accessed by visiting Taysha's website at https://ir.tayshagtx.com/news-events/events-presentations. An archived version of the webcast will be available on the website for 30 days.

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," "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, such as TSHA-120 and TSHA-102 and including our preclinical 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, the potential market opportunity for these product candidates, our corporate growth plans and the impacts of our corporate operational, structural and personnel changes. 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 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, 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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