Taysha Gene Therapies to Host Virtual R&D Day

June 17, 2021

Two-day R&D day on June 28 and 29, 2021 at 10:00 am ET will highlight progress across its pipeline and will feature presentations from key opinion leaders

DALLAS--(BUSINESS WIRE)--Jun. 17, 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 will host its first two-day virtual research and development (R&D) day for analysts and investors. The event will be webcast live on June 28 and June 29, 2021 from 10:00 a.m. to 1:00 p.m. ET each day.

The event will highlight the company's R&D progress, focused on advancement of its early- and late-stage investigational programs. Topics of discussion will include:

Day 1 – June 28, 2021

- TSHA-120 (GAN): Pivotal-stage AAV9 gene replacement therapy program for the treatment of giant axonal neuropathy (GAN), a rare autosomal recessive disease of the central and peripheral nervous systems caused by loss-of-function gigaxonin gene mutations.
- TSHA-101 (GM2 gangliosidosis): The first bicistronic AAV9 gene therapy in clinical development designed to deliver two genes, HEXA and HEXB, comprising the alpha and beta sub-units of beta hexosaminidase A, intrathecally for the treatment of GM2 gangliosidosis, also called Tay-Sachs or Sandhoff disease. TSHA-101 is currently in Phase 1/2 development.
- TSHA-118 (CLN1 disease): AAV9-based gene therapy designed to express a human codon-optimized CLN1 transgene to potentially treat CLN1 disease, a rapidly progressing rare lysosomal storage disease with no currently approved treatments. This gene replacement therapy program is currently under an open IND, with initiation of a Phase 1/2 trial expected in the second half of 2021.
- TSHA-102 (Rett syndrome): AAV9-based gene therapy in development for Rett syndrome, a severe neurodevelopmental disorder, designed to deliver MECP2, as well as a novel miRARE platform that regulates transgene expression on a cell-by-cell basis. This regulated gene replacement therapy is currently in IND/CTA-enabling studies, with an IND/CTA filing expected in the second half of 2021.

Day 2 – June 29, 2021

- TSHA-104 (SURF1-associated Leigh syndrome): AAV9-based gene therapy with a transgene encoding the human SURF1 protein to potentially treat SURF1-associated Leigh syndrome, a monogenic mitochondrial disorder with no currently approved treatments. This gene replacement therapy program is currently in IND/CTA-enabling studies, with an IND/CTA filing expected in the second half of 2021.
- TSHA-105 (SLC13A5 deficiency): AAV9-based gene therapy expressing human SLC13A5 protein to potentially treat SLC13A5 deficiency, a rare autosomal recessive epileptic encephalopathy characterized by the onset of seizures within the first few days of life. This gene replacement therapy program is currently in IND/CTA-enabling studies.
- TSHA-103 (SLC6A1 haploinsufficiency disorder): Gene replacement therapy constructed from a codon-optimized version of the human SLC6A1 gene packaged within an AAV9 viral vector for the treatment of SLC6A1 haploinsufficiency disorder. This program is currently in IND/CTA-enabling studies.
- TSHA-111-LAFORIN and TSHA-111-MALIN (Lafora disease): Recombinant AAV9 viral vectors with miRNA targeting the knockdown of GYS1 for the treatment of Lafora disease. These programs are currently in IND/CTA-enabling studies.
- TSHA-112 (APBD): Recombinant AAV9 viral vector with miRNA targeting the knockdown of GYS1 for the treatment of Adult Polyglucosan Body Disease (APBD). This program is currently in IND/CTA-enabling studies.
- Additional preclinical programs, including tauopathies (TSHA-113, an AAV9 viral vector that utilizes AAV-mediated gene silencing to potentially deliver life-long reduction of tau protein levels in neurons following a single dose) and Angelman syndrome (TSHA-106, an AAV9 viral vector designed for shRNA-mediated knockdown of UBE3A-ATS).

The event will feature presentations from Taysha senior leaders and the following key opinion leaders:

- Steven Gray, Ph.D.
  Chief Scientific Advisor, Taysha Gene Therapies
  Associate Professor, Department of Pediatrics at UT Southwestern

- Berge Minassian, M.D.
  Chief Medical Advisor, Taysha Gene Therapies
  Division Chief, Pediatric Neurology at UT Southwestern

- Rachel Bailey, Ph.D.
  ssistant Professor, Department of Pediatrics at UT Southwestern

- Kimberly Goodspeed, M.D.
  Assistant Professor, Department of Pediatrics, Neurology and Psychiatry at UT Southwestern

Registration for this event is available through LifeSci Events. A live video webcast will be available in the "Events & Media" section of the Taysha corporate website. An archived version of the event will be available on the website for 60 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," 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 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 our plans to establish a commercial-scale cGMP manufacturing facility to provide preclinical, clinical and commercial supply. 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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