Taysha Gene Therapies Announces Multiple Data Presentations and Workshop Presentations at the 24th Annual Meeting of the American Society of Gene & Cell Therapy

May 5, 2021

TSHA-104 increased COX1 activity in brain and muscle and restored elevation of blood lactate on exhaustive exercise in dose-dependent manner in SURF1 knockout mice

TSHA-105 significantly reduced plasma citrate levels, normalized EEG brain activity, and reduced the number of seizures and seizure susceptibility in SLC13A5 knockout mice

On track to file IND/CTA for TSHA-104 in SURF1-associated Leigh syndrome in second half of 2021

IND/CTA-enabling studies for TSHA-105 in SLC13A5 deficiency are ongoing

DALLAS--(BUSINESS WIRE)--May 5, 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 preclinical data from its investigational gene therapy programs will be presented at the 24th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT), which will be held virtually May 11-14, 2021.

“Presentations at this year’s ASGCT will highlight the positive preclinical results for TSHA-104 in SURF1-associated Leigh syndrome and TSHA-105 in SLC13A5 deficiency that support our advancement of these programs,” said RA Session II, President, Founder and Chief Executive Officer of Taysha. “Among the compelling data, we have shown that TSHA-104 as a single intrathecally administered gene replacement therapy was effective in improving SURF1 deficiency-related dysfunctions, such as diminished COX1 activity in brain and muscle and blood lactate on exhaustive exercise in a dose-dependent manner in SURF1 knockout mice. In SLC13A5 deficiency, CSF-delivered TSHA-105 resulted in improved EEG activity and reduced seizure susceptibility in SLC13A5 knockout mice. We remain on track to file an IND/CTA in SURF1-associated Leigh syndrome in the second half of this year and continue to advance TSHA-105 in SLC13A5 deficiency towards the clinic.”

Summary of Abstracts and Posters (all times in Eastern Time)

Thursday, May 13, 2021 at 7:00 – 7:15 pm

- Abstract Presentation title: *Gene Replacement Therapy for SURF1-Related Leigh Syndrome Using AAV9*
- Session title: Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases
- Abstract number: 165
- Authors: Qinglan Ling, Matthew Rioux, Steven Gray
- Presenter: Qinglan Ling, Ph.D., of UT Southwestern Medical Center
    - COX activity was partially and significantly rescued in all tested tissues of AAV9/h*SURF1*-treated mice via intrathecal (IT) delivery
    - AAV9/hSURF1-treated mice demonstrated a dose-dependent increase in h*SURF1* mRNA expression, restoration of MT-CO1 protein expression in the brain
    - Gene replacement treatment also mitigated the lactic acidosis upon exhaustive exercise at mid-age

Thursday, May 13, 2021 at 7:00 – 7:15 pm

- Abstract Presentation title: *scAAV9 Gene Replacement Therapy for Epileptic SLC13A5 Deficiency*
- Session title: AAV Therapies for Neurological and Sensory Diseases
- Abstract number: 137
- Authors: Rachel Marion Bailey, Lauren Bailey, Morgan Schackmuth, Irvin Garza
- Presenter: Rachel Bailey, Ph.D., of UT Southwestern Medical Center
    - TSHA-105-treated knockout mice demonstrated significantly decreased plasma citrate levels compared to knockout mice treated with vehicle
    - TSHA-105-treated knockout mice had reduced spike train activity and seizure frequency on electroencephalogram (EEG)

Tuesday, May 11, 2021 at 8:00 – 10:00 am

- Poster title: *Novel AAV Capsids for Enhanced Gene Transfer to the Cerebellum, Spinal Cord, and Schwann Cells*
- Session: AAV Vectors – Virology and Vectorology
- Abstract number: 314
- Authors: Xin Chen, Thomas Dong, Widler Casy, Yuhui Hu, Daphne Chen, Thomas McCown, Steven Gray
    - A capsid DNA shuffling and directed evolution process was pursued to generate new AAV variants for nervous system gene transfer. In the IT cohort, more than 15 variants had biodistribution values at least ten times greater than AAV9 with reduced biodistribution to the liver
    - Results demonstrated that some variants could be used to treat cerebellar diseases with Purkinje cell involvement and other variants could be used to treat peripheral demyelinating neuropathies with Schwann cell involvement

The ASGCT abstracts are now available at https://www.cell.com/molecular-therapy-family/molecular-therapy/issue?pii=S1525-0016(21)X0002-0.

ASGCT-Sponsored Pre-Meeting Workshops (all times in Eastern Time)

Monday, May 10, 2021 at 12:55 – 1:05 pm

- Workshop title: Transitioning From Academics to Industry
- Session 2: Learning From Experience: Case Studies of Transitions from Academia to Industry
- Topic: Moving from Academic Vector Production to Commercial Scale Manufacturing
- Speaker: Frederick Porter, Ph.D., Chief Technical Officer of Taysha Gene Therapies

Monday, May 10, 2021 at 3:00 – 3:15 pm

- Workshop title: Emerging Issues in Market Access
- Session 1: Gene Therapy Investment and Capital
- Keynote: Attracting Capital and Building a Company in the Gene Therapy Space
- Speaker: RA Session II, President, Founder and Chief Executive Officer of Taysha Gene Therapies

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, 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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Company Contact:
Kimberly Lee, D.O.
SVP, Corporate Communications and Investor Relations
Taysha Gene Therapies
klee@tayshagtx.com

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

Source: Taysha Gene Therapies, Inc.