



Taysha Gene Therapies Announces Presentations Highlighting Programs and Platform Technologies at the 2020 Annual Meeting of the American Society of Gene and Cell Therapy

May 7, 2020

Seven abstracts, including three oral presentations, highlight Taysha's extensive pipeline and focus on monogenic CNS diseases

DALLAS--([BUSINESS WIRE](#))--[Taysha Gene Therapies](#), a gene therapy company focused on eradicating monogenic CNS disease for rare and large market indications, today announced that seven abstracts, including three oral presentations, highlighting programs and platform technologies will be featured at the 23rd Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), taking place virtually from May 12-15, 2020. Abstracts are available on the [ASGCT Annual Meeting website](#).

Oral Presentations and Abstracts

Title: A New Approach for Designing a Feedback-Enabled AAV Genome Improves Therapeutic Outcomes of MiniMeCP2 Gene Transfer in Mice Modeling Rett Syndrome (RTT)

Presenter: Sarah E. Sinnett, Ph.D., Assistant Professor, Pediatrics, UT Southwestern Medical Center

Session: AAV Gene Delivery for CNS Disorders (Abstract #92)

Presentation Date & Time: Tuesday, May 12, 3:45 PM - 4:00 PM

Title: Intrathecal Delivery of Human Bicistronic Hexosaminidase Vector (TGTX-101) to Correct Sandhoff Disease in a Murine Model: A Dosage Study

Presenter: Alex E. Ryckman, Centre for Neuroscience Studies, Queen's University

Session: Main session, AAV Gene Delivery for CNS Disorders (Abstract #97)

Presentation Date & Time: Tuesday, May 12, 5:00 PM - 5:15 PM

Title: Direct Vagus Nerve Injection of AAV9 as a Treatment Approach for Autonomic Dysfunction in Giant Axonal Neuropathy

Presenter: Rachel M. Bailey, Ph.D., Assistant Professor, Neuroscience, UT Southwestern Medical Center

Session: New Techniques in Gene Therapy for Neurological Disorders (Abstract #936)

Presentation Date & Time: Thursday, May 14, 5:15 PM - 5:30 PM

*Taysha has exclusive rights to the novel vagus nerve route of administration redosing platform in select indications. Hannah's Hope Foundation owns the rights to the Giant Axonal Neuropathy program.

Poster Presentations and Abstracts

Title: SMRT Sequencing Allows High-Throughput Analysis of a Whole Capsid Shuffled AAV Capsid Library Following CNS Selection in Mice and NHPs

Presenter: Widler Casy, Ph.D., Postdoctoral Researcher, UT Southwestern Medical Center

Session: AAV Vectors - Virology and Vectorology (Abstract #156)

Presentation Date & Time: Tuesday, May 12, 5:30 PM - 6:30 PM

Title: Gene Replacement Therapy for SURF1-Related Leigh Syndrome Using AAV9

Presenter: Qinglan Ling, Ph.D., Postdoctoral Researcher, UT Southwestern Medical Center

Session: Neurologic Diseases (Abstract #299)

Presentation Date & Time: Tuesday, May 12, 5:30 PM - 6:30 PM

Title: A Dosage Study to Assess the Long-Term Effects of Gene Therapy for AB-Variant GM2 Gangliosidosis in a Mouse Model Using Adeno-Associated Virus Serotype 9

Presenter: Natalie M. Deschenes, Centre for Neuroscience Studies, Queen's University

Session: Main Session: Neurologic Diseases (Abstract #731)

Presentation Date & Time: Wednesday, May 13, 5:30 PM - 6:30 PM

Title: Preclinical Safety and Efficacy of AAV9 Gene Replacement Therapy for SLC6A1 Disorder

Presenter: Frances Shaffo, Ph.D., Postdoctoral Researcher, UT Southwestern Medical Center

Session: Neurologic Diseases (Abstract #718)

Presentation Date & Time: Wednesday, May 13, 5:30 PM - 6:30 PM

The Taysha Approach

Taysha's approach is to combine the speed, scale and expertise of the UT Southwestern Gene Therapy Program with the experience of a proven management team in the gene therapy space. The company is developing an extensive pipeline of potentially curative therapies for monogenic CNS diseases in both rare and large-market indications that are centered on proven AAV technology. In addition, Taysha is developing a novel AAV capsid platform that utilizes machine learning, DNA shuffling and directed evolution to improve targeted delivery. The company is also developing an AAV redosing platform that facilitates redosing by subverting the humoral immune response through delivery to the vagus nerve.

Taysha currently has 15 AAV gene therapy programs in its pipeline with options to an additional four programs.

About Taysha Gene Therapies

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. With a singular focus on developing curative medicines, we are able 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 quickly and efficiently 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 new cures—to dramatically improve patients' lives. More information is available at www.tayshqtx.com.

Contacts

Company Contact:

Niren Shah, PharmD, MBA

Taysha Gene Therapies

Nshah@tayshqtx.com

Media Contact:

Jason Spark

Canale Communications

Jason@canalecomm.com