Taysha Gene Therapies Launches with $30 Million Seed Financing and Unrivaled Strategic Partnership with the UT Southwestern Gene Therapy Program

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- Current pipeline of 15 AAV gene therapy programs with options to an additional 4 programs targeting severe and life-threatening monogenic CNS diseases in both rare and large-market indications
- Four INDs expected to be filed by the end of 2021, with first gene therapy candidate for GM2-Gangliosidosis anticipated to enter clinical studies later this year
- UT Southwestern Gene Therapy Program alliance led by Drs. Steven Gray and Berge Minassian established to accelerate R&D, with integration of translational research, clinical development and GMP manufacturing
- Key leadership team members and investors previously led the development and commercialization of Zolgensma®, the first FDA-approved gene therapy for CNS disease

DALLAS--(BUSINESS WIRE)--Taysha Gene Therapies today announced it has launched with a mission to eradicate severe and life-threatening monogenic diseases of the central nervous system (CNS) by advancing its pipeline of 15 AAV (adeno-associated virus) gene therapy programs, with options to an additional four programs. The company reunites former investors and executives from AveXis, while accelerating its discovery and development efforts with the UT Southwestern Gene Therapy Program and Department of Pediatrics, one of the largest pediatric neurology residency programs in the U.S.

To advance its mission and extensive pipeline, Taysha has raised $30 million in seed financing co-led by PBM Capital, the first institutional investor in AveXis, and Nolan Capital, the investment fund of former AveXis CEO Sean Nolan. Taysha's Board of Directors played an integral role in the formation of the company and is comprised of Sean Nolan (Chairman of the Board), Paul Manning of PBM Capital, Claire Aldridge, Ph.D., of UT Southwestern, and RA Session II, President, CEO and Founder of Taysha.

"Gene therapies have proven to deliver transformational benefit to patients who suffer from devastating diseases with significant, unmet medical need. Our mission at Taysha is to build upon these advancements to eradicate monogenic CNS diseases for the thousands affected," said RA Session II. "In joining forces with UT Southwestern—home to some of the brightest minds in gene therapy—we will advance our deep pipeline of potentially curative medicines with speed and scale. At Taysha, we are ushering in a new era of gene therapy drug development, one in which we can rapidly translate early discoveries into the clinic and beyond."

An Unrivaled Alliance with the UT Southwestern Gene Therapy Program

Taysha and UT Southwestern have entered into a strategic partnership to rapidly and efficiently translate novel AAV gene therapies from bench to bedside. The UT Southwestern Gene Therapy Program is led by Steven Gray, Ph.D., Director of the Viral Vector Core and Assistant Professor in the Department of Pediatrics, and Berge Minassian, M.D., Division Chief of Child Neurology. UT Southwestern has developed a state-of-the-art, GMP viral vector manufacturing facility with the capacity to support Taysha's broad preclinical and clinical development programs.

Under the partnership, UT Southwestern will conduct discovery and preclinical research, lead IND-enabling studies, provide clinical GMP manufacturing, as well as execute natural history studies. Taysha will lead all clinical development, regulatory strategy, commercial manufacturing and commercialization activities. The collaboration will be governed by a joint steering committee composed of key leadership members from Taysha and UT Southwestern.

"Together with Taysha, we are merging cutting-edge translational research, hands-on clinical care, and proven regulatory and commercial expertise – ultimately creating an engine for new cures," said Claire Aldridge, Ph.D., Taysha Board Member and Associate Vice President of Commercialization and Business Development at UT Southwestern Medical Center. "In a short amount of time, I've already witnessed how quickly and efficiently we can leverage our collective expertise and resources to advance new gene therapies to the patients who so desperately need them."

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. TGTX-101, a gene replacement therapy for GM2-Gangliosidosis, is expected to advance into clinical development later this year. In total, Taysha is planning to file four Initial New Drug (IND) applications by the end of 2021, including indications for SURF1 deficiency, SLC6A1 haploinsufficiency and Rett syndrome.

"Taysha is leveraging the proven safety and heritage of AAV technology—combining it with an experienced management team and the world-class capabilities of UT Southwestern—to rapidly accelerate the research and development of multiple new therapies at a scope we haven't seen before," said Sean Nolan, Chairman of Taysha and former CEO of AveXis. "This is an exciting time for gene therapy, and Taysha's approach brings promise to patients suffering from many devastating CNS diseases."

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—tc dramatically improve patients' lives. More information is available at www.tayshagtx.com.

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