# Taysha Gene Therapies Receives Rare Pediatric Disease Designation and Orphan Drug Designation for TSHA-102 as a Treatment for Rett Syndrome

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*Program leverages novel miRARE platform technology used to control transgene expression on a cellular basis*

*TSHA-102 anticipated to submit Investigational New Drug application in 2021*

DALLAS--(BUSINESS WIRE)--Oct. 14, 2020-- Taysha Gene Therapies Inc. (Nasdaq: TSHA), a patient-centric gene therapy company focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the central nervous system in both rare and large patient populations, today announced that it has received rare pediatric disease designation and orphan drug designation from the U.S. Food and Drug Administration (FDA) for TSHA-102, an AAV9-based gene therapy in development for the treatment of Rett syndrome. Taysha anticipates that it will submit an Investigational New Drug (IND) application for TSHA-102 to the FDA in 2021.

Rett syndrome is one of the most common genetic causes of severe intellectual disability worldwide, with a prevalence of over 25,000 cases in the U.S. and European Union (EU). It is an X-linked disease that primarily occurs in females, but it can be seen very rarely in males. It is usually recognized in children between six to 18 months of age as they begin to miss developmental milestones or lose abilities they had developed. Individuals with Rett syndrome also show symptoms that include loss of speech, loss of purposeful use of hands, loss of mobility, seizures, cardiac impairments, breathing issues and sleep disturbances.

“Patients with Rett syndrome are currently managed with symptomatic treatments as there are no therapies approved to treat the underlying cause of disease,” said Berge Minassian, M.D., Chief Medical Advisor of Taysha and Chief of Pediatric Neurology at the University of Texas Southwestern Medical Center (UT Southwestern). Dr. Minassian is credited with describing the CNS isoform of the MECP2 gene which is responsible for neuronal and synaptic function throughout the brain. “Gene therapy offers a potentially curative option for patients suffering with Rett syndrome.”

Rett syndrome is caused by mutations in the MECP2 gene. TSHA-102 is designed to deliver a healthy version of the MECP2 gene as well as the miRNA-Responsive Auto-Regulatory Element, miRARE, platform technology to control the level of MECP2 expression. “TSHA-102 represents an important step forward in the field of gene therapy, where we are leveraging a novel regulatory platform called miRARE to prevent the overexpression of MECP2,” said Steven Gray, Ph.D., Chief Scientific Advisor of Taysha and Associate Professor in the Department of Pediatrics at UT Southwestern. “In collaboration with Sarah Sinnett, Ph.D. to develop miRARE, our goal was to design a regulated construct that allowed us to control MECP2 expression to potentially avoid adverse events that are typically seen with unregulated gene therapies.”

The FDA defines a rare pediatric disease as a serious or life-threatening disease in which the disease manifestations primarily affect individuals aged from birth to 18 years. Pediatric diseases recognized as "rare" affect under 200,000 people in the U.S. The Rare Pediatric Disease Priority Review Voucher Program is intended to address the challenges that drug companies face when developing treatments for these unique patient populations. Under this program, companies are eligible to receive a priority review voucher following approval of a product with rare pediatric disease designation if the marketing application submitted for the product satisfies certain conditions. If issued, a sponsor may redeem a priority review voucher for priority review of a subsequent marketing application for a different product candidate, or the priority review voucher could be sold or transferred to another sponsor.

Orphan drug designation is granted by the FDA Office of Orphan Products Development to investigational treatments that are intended for the treatment of rare diseases affecting fewer than 200,000 people in the U.S.

“Obtaining these designations is a validation of decades-long work to identify and optimize a potential gene therapy treatment for this devastating disease,” said RA Session II, President, CEO and Founder of Taysha. “We are also excited to advance our miRARE platform whereby regulated expression of a transgene is possible on a cellular basis. The miRARE platform has broad applicability across a wide range of monogenic CNS disorders where there is a need to control transgene expression.”

**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 goa of dramatically improving patients’ lives. More information is available at www.tayshagtx.com.

**About miRARE**

For disorders that require replacement of dose-sensitive genes, we have combined high-throughput microRNA, or miRNA, profiling and genome mining to create miRNA-Responsive Auto-Regulatory Element, or miRARE, our novel miRNA target panel. This approach is designed to enable our product candidates to maintain safe transgene expression levels in the brain. This built-in regulation system is fully endogenous, and does not require any additional exogenous drug application. Instead, the miRARE system utilizes endogenous transgene-responsive miRNA to downregulate transgene expression in the event that overexpression occurs. miRARE may be applicable to a range of diseases where overexpression of a therapeutic transgene is a concern.

**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 or implying the potential of our product candidates, including TSHA-102, to positively impact quality of life and alter the course of disease in the patients we seek to treat, the benefits of, and our ability to develop product candidates using, miRARE, our research, development and regulatory plans for our product candidates, the potential benefits of rare pediatric disease designation and orphan drug designation to 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. 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 filings, including in our prospectus dated September 23, 2020, as filed with the Securities and Exchange Commission (“SEC”) on September 24, 2020, pursuant to Rule 424(b) under the Securities Act of 1933, as amended, 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:**
Niren Shah, PharmD, MBA
Taysha Gene Therapies
Nshah@tayshagtx.com

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

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