



October 14, 2020

Dear Rett Syndrome Community,

Taysha Gene Therapies is a new gene therapy company focused on monogenic diseases of the central nervous system (CNS). You may have heard about us, as we have shared in several announcements that Rett syndrome is one of our more advanced programs. In our most recent announcement, we were proud to share Taysha Gene Therapies has been granted Rare Pediatric Disease Designation (RPD) and Orphan Drug Designation (ODD) from the Food and Drug Administration (FDA) for TSHA-102, an innovative AAV9-based gene therapy for the treatment of Rett syndrome. Therefore, it is our pleasure to introduce the Rett Syndrome Community to Taysha Gene Therapies.

You may be wondering what these designations mean for TSHA-102. Through these designations, the FDA recognizes Rett syndrome as a rare disease and in turn, we have the potential to smooth the development path and accelerate timelines with the FDA. With ODD, Taysha will receive exclusive marketing and development rights to recover the costs of researching and developing this treatment. With that said, it's important to note that these designations do not represent any regulatory approval. However, it does validate the importance of our work in Rett syndrome and may lead to a faster and more efficient development path for TSHA-102.

A word about Taysha. In the Caddo Native American language, *Taysha* means “ally” or “friend,” and when translated, also means “Texas.” Between our UT Southwestern collaboration and patient advocacy partnerships, **we hope to be an ally to the rare disease community, including the Rett syndrome community.**

“I am proud to be part of the Rett syndrome community. We are working diligently to push forward an innovative approach to treat children with this condition and are committed to understanding the unique challenges your community faces.”

– RA Session II, Founder, President & CEO, Taysha

Our focus is gene therapy, which is a highly innovative way to treat monogenetic diseases. We are working hand-in-hand with our collaborators at UT Southwestern, who are well-known gene therapy experts. We are also committed to engaging key stakeholder groups, including



medical experts in Rett syndrome, regulatory agencies and, importantly, patient groups. We are truly embracing an integrated approach—one that will help get new medicines in the hands of physicians treating children affected by Rett syndrome in the most efficient, effective and safest way possible.

TSHA-102 is designed to deliver a healthy version of the MECP2 gene as well as the miRNA-Responsive Auto-Regulatory Element, miRARE. miRARE is Taysha's novel platform technology that is designed to control the level of MECP2 expression and enable safe transgene expression in the brain. We hope to file an Investigational New Drug (IND) application with the FDA by the end of 2021 for Rett syndrome. If the IND is approved, we will then have permission from the FDA to initiate clinical trials in patients. We are currently developing details of the first clinical trial in children living with Rett syndrome and are committed to keeping you up to date on our progress through advocacy partners.

We are grateful for your support and are happy to share this news with you. Working together, we can help bring gene therapy to the Rett syndrome communities in the most efficient, effective and safest way possible.

Warm regards,

The Taysha Team