Taysha Gene Therapies Receives Orphan Drug Designation and Rare Pediatric Disease Designation for TSHA-101 for GM2 Gangliosidosis

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DALLAS--(BUSINESS WIRE)--Taysha Gene Therapies, a patient-centric gene therapy company with a mission to eradicate monogenic CNS diseases, today announced that it has received Orphan Drug Designation and Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) for TSHA-101, an AAV9-based gene therapy in development for GM2 Gangliosidosis. The company anticipates TSHA-101 to enter the clinic by the end of 2020.

“Receiving both Orphan Drug Designation and Rare Pediatric Disease Designation by the FDA speaks to the strength of the translational data package supporting TSHA-101 for GM2 Gangliosidosis,” said RA Session II, President, CEO and Founder of Taysha. “Furthermore, these designations also highlight the FDA’s recognition that GM2 Gangliosidosis is a devastating rare disease, and we believe this is an important milestone for the GM2 Gangliosidosis community.”

GM2 Gangliosidosis, also known as Tay-Sachs disease and Sandhoff disease, is a rare, neurodegenerative disease that causes progressive dysfunction of the central nervous system. There are no therapies for the treatment of the disease, and current treatment is limited to supportive care. Patients with GM2 Gangliosidosis typically succumb to disease in early childhood.

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 United States. A Rare Pediatric Disease Designation may make the company eligible for a Priority Review Voucher to be redeemed to receive priority review of a subsequent marketing application for a different product.

About Taysha Gene Therapies

Taysha Gene Therapies is a patient-centric gene therapy company with a mission to eradicate monogenic CNS disease. We are focused on developing and commercializing AAV-based gene therapies for the treatment of monogenic diseases of the CNS in both rare and large patient populations. We were founded in partnership with The University of Texas Southwestern Medical Center, or UT Southwestern, to develop and commercialize transformative gene therapy treatments. Together with UT Southwestern, we are advancing a deep and sustainable product portfolio of 18 gene therapy product candidates, with exclusive options to acquire four additional development programs. By combining our management team’s proven experience in gene therapy drug development and commercialization with UT Southwestern’s world-class gene therapy research capabilities, we believe we have created a powerful engine to develop transformative therapies to dramatically improve patients’ lives. More information is available at www.tayshagtx.com.

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