Cure GM1 Foundation and TEGA Therapeutics Establish Collaboration to Develop GM1 Gangliosidosis Therapy

Albany, CA – The Cure GM1 Foundation and TEGA Therapeutics, Inc. announced a strategic collaboration to develop an Enzyme Replacement Therapy (ERT) as a potential treatment of neurodegeneration associated with GM1 gangliosidosis. The scope of the collaboration includes multiple key activities required for clinical development, the first of which is to establish a cell line for GMP manufacture and evaluate the safety, efficacy and dosing in the mouse model for GM1 gangliosidosis in collaboration with the Jackson Laboratory.

"Enzyme replacement therapy has been successfully developed for other lysosomal storage disorders and we are hopeful that it may also prove efficacious in treating neurodegeneration associated GM1 gangliosidosis. GM1 is a complex and truly devastating disease," said Christine Waggoner, President of Cure GM1.

Additional activities under the collaboration include manufacture of the ERT for IND-enabling studies, and execution of toxicology studies as a prerequisite for clinical development. All activities will be conducted by TEGA Therapeutics with funding from the Cure GM1 Foundation. The collaboration is expected to move a clinical candidate toward IND enabling studies by 2023.

"We are excited to be working with the Cure GM1 Foundation, a leader in patient advocacy and research in GM1 gangliosidosis', said Tim Scott, CEO of TEGA. "Cure GM1 has been at the forefront of developing newborn screening assays and animal models for this disease. We look forward to this collaboration to rapidly move forward the development of an enzyme replacement therapy for this terrible disease."

Recently, an article published in Journal of Biological Chemistry by Chen and colleagues[a] demonstrated that intracerebroventricular (ICV) injection of recombinant human β-galactosidase (rhBeta-Gal) into the brain effectively treated the CNS symptoms in a GM1 gangliosidosis mouse model. Weekly ICV injections significantly reduced GM1 ganglioside levels in the brain, reversed secondary neuropathology and normalized Beta-Gal activity in the liver and bone marrow, demonstrating systemic exposure from the ICV-delivered ERT. Taken together, these results highlight the need for further development of ICV-ERT as a safe and effective therapeutic approach for GM1 gangliosidosis. Safety and efficacy of ICV delivery of ERT has been demonstrated in other lysosomal storage disorders.

[a] *Chen et al., Intracerebroventricular enzyme replacement therapy with beta-galactosidase reverses brain pathologies due to GM1 gangliosidosis in mice. 2020 J Biol Chem 295, 13532-13555.*

About GM1 Gangliosidosis

GM1 gangliosidosis is a rare, autosomal recessive genetic disorder caused by mutations in the structural GLB1 gene encoding the lysosomal hydrolase enzyme, β-galactosidase (Beta-Gal). Mutations in Beta-Gal result in accumulation of GM1 gangliosides in the brain, heart, liver and bones, leading to progressive central nervous system (CNS) dysfunction. The disease is commonly associated with seizures and rapid developmental regression resulting in dramatic morbidity and premature death, most often in young children. Currently there is no approved therapy to treat this devastating disease.

About Cure GM1 Foundation

The Cure GM1 Foundation is dedicated to hope and to directly funding research for a cure for a GM1 Gangliosidosis, a fatal monogenic lysosomal storage disease. Cure GM1 is distinguished as the only 501(c)(3) nonprofit dedicated entirely to GM1 Gangliosidosis drug development, research, and patient advocacy.

About TEGA Therapeutics

TEGA was founded to utilize glycoscience-related technologies for the treatment of life-threatening diseases. Glycans, or complex carbohydrates, plate important roles in many physiological and pathological processes. TEGA specializes in the science of glycans, and is working to uncover their value as pharmaceutical targets and therapies.

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