axovant
GENE THERAPIES
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United States

Axovant Gene Therapies Announces US FDA Clearance of IND for Registrational Study of AXO-AAV-GM2 Gene Therapy in Tay-Sachs and Sandhoff Diseases

Dear Families and Members of the Patient Advocacy Community:

On behalf of Axovant Gene Therapies, it is our privilege to share this letter with an exciting update for our AXO-AAV-GM2 program targeting GM2 gangliosidosis.

Today, we are delighted to announce that the U.S. Food and Drug Administration (FDA) has completed their review of the Investigational New Drug (IND) Application for AXO-AAV-GM2, our investigational gene therapy for Tay-Sachs disease (TSD) and Sandhoff disease. This IND clearance will allow us to proceed to clinical trials in the United States. At Axovant, we are tremendously encouraged by this regulatory milestone as it brings us one-step closer towards bringing a new treatment option for children with Tay-Sachs and Sandhoff disease.

The study will enroll both infantile and juvenile patients with GM2 gangliosidosis in the U.S. at our clinical trial site, Massachusetts General Hospital (MGH), in Boston, MA. The two-part study will consist of (1) a dose ranging cohort evaluating the safety and efficacy of various doses of the gene therapy, followed by (2) an efficacy cohort with the optimal safe and effective dose. Terence R. Flotte, M.D., Professor of Pediatrics and Dean at the University of Massachusetts Medical School, will serve as principal investigator on the clinical trial and will coordinate closely with the staff at MGH.

Information about the study, including inclusion/exclusion criteria, will be posted on clinicaltrials.gov. To learn more about this study, you or your doctor may contact the Clinical Research Coordinator at MGH, Haley Andonian (handonian@partners.org).

All GM2 families who have contacted us are receiving this email simultaneously. As with all clinical trials, enrollment is dependent upon the study investigator's medical assessment of the patient meeting the inclusion criteria for the study, and as such, Axovant will play no role in that medical assessment. We realize many patients may not qualify for this study, and we are committed to providing timely information and stay abreast of the latest developments for this program. As always, your treating physician will serve as the best source of information for ongoing medical treatment.

To provide additional information and answer questions, Axovant will coordinate informational updates with a number of patient advocacy organizations, including the National Tay-Sachs & Allied Diseases Association (NTSAD), CATS Foundation, and the Cure Tay-Sachs Foundation. If you would like to reach out directly with any questions or feedback, you can contact us at GM1GM2@axovant.com.

This is an important milestone for the entire GM2 community made possible by the many families, researchers and patient organizations committed to finding new treatments for Tay-Sachs and Sandhoff disease, and we look forward to enrollment of patients in this program.

Sincerely,

Gavin Corcoran
Chief R&D Officer

Erika De Boever
VP, Clinical Development