First evidence of clinical stabilization in Tay-Sachs

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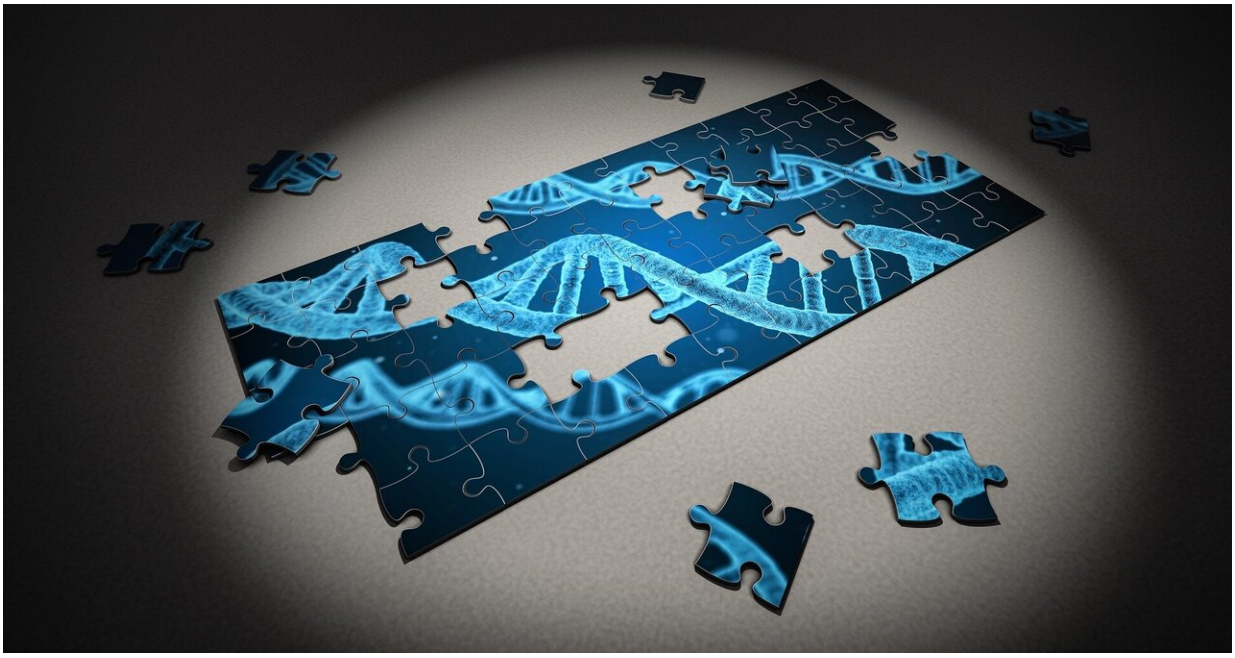

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Preliminary data from an expanded access study of an investigational gene therapy in two patients with infantile Tay-Sachs disease indicates the potential to modify the rate of disease progression, according to an upcoming report at the European Society of Gene and Cell Therapy Annual Congress in Barcelona by Terence R. Flotte, MD, the Celia and Isaac Haidak Professor of Medical Education, executive deputy

chancellor, provost and dean of the School of Medicine. Tay-Sachs is a rapidly progressive and fatal pediatric neurodegenerative genetic disorder that has a median life expectancy of approximately three to four years.

"Today's exciting clinical results from the trial are the first reported evidence for potential disease modification in Tay-Sachs disease, and suggest an opportunity for gene replacement therapy to improve outcomes for children with this devastating condition," said Gavin Corcoran, MD, chief research and development officer at Axovant Gene Therapies, a clinical-stage company developing innovative gene therapies that licensed rights to develop the therapy based on research discoveries at UMass Medical School. "Myelination is an important component of healthy brain development in infants and is often abnormal in children with Tay-Sachs disease. We were encouraged to see MRI evidence of preserved brain architecture and improved myelination in the early symptomatic child treated at 10 months of age."

In 2018, Axovant licensed exclusive worldwide rights from UMass Medical School for the development and commercialization of gene therapy programs for GM1 gangliosidosis and GM2 gangliosidosis, including Tay-Sachs and Sandhoff diseases. Research into potential therapies for lysosomal storage diseases such as Tay-Sachs, Sandhoff disease and GM1 gangliosidosis at UMass Medical School and Auburn University has led to significant advances in the field.

Miguel Sena-Esteves, Ph.D., associate professor of neurology at UMass Medical School; Heather Gray-Edwards, Ph.D., DVM, formerly of Auburn University and currently assistant professor of radiology at UMass Medical School; and Douglas Martin, Ph.D., professor of anatomy, physiology and pharmacology in the College of Veterinary Medicine and the Scott-Ritchey Research Center at Auburn University, have worked collaboratively for more than a decade on animal models

and therapeutic approaches for these and similar disorders.

AXO-AAV-GM2 was successfully administered in both patients and has been generally well tolerated to date, with no serious adverse events or clinically relevant laboratory abnormalities related to therapy.

"Bilateral intrathalamic and intrathecal delivery of rAAV gene therapy may surmount the obstacle of providing widespread distribution of therapeutic enzyme throughout the brain and CN," said Dr. Flotte.

"This innovative delivery could overcome one of the primary challenges for developing treatments for Tay-Sachs, Sandhoff and many other severe pediatric genetic disorders, providing much needed hope for these families."

Flotte is presenting the report at the European Society of Gene and Cell Therapy 27th Annual Congress on Wednesday, Oct. 23.

Provided by University of Massachusetts Medical School