LogicBio Announces Presentation of Retrospective Study of Disease Course in Pediatric Patients with Severe Methylmalonic Acidemia

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- Data presented at ACMG meeting evaluate disease progression in non-transplanted patients and children receiving liver transplantation

LEXINGTON, Mass., May 3, 2021 /PRNewswire/ -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC), a clinical-stage genetic medicine company pioneering gene delivery and gene editing platforms to address rare and serious diseases from infancy through adulthood, today announced results from a retrospective study of the disease course in pediatric patients living with severe methylmalonic acidemia (MMA) caused by a deficiency of the mitochondrial enzyme methylmalonyl-CoA mutase (MMUT). The study results were recently highlighted in a poster presentation at the American College of Medical Genetic and Genomics (ACMG) Annual Clinical Genetics Meeting.

MMA is a rare genetic disorder that can be caused by mutations in several genes, with the MMUT genetic mutation being the most common. Mutations in this gene, which provides instructions for making the MMUT enzyme, prevents the body from properly processing certain fats and proteins and can lead to the toxic buildup of methylmalonic acid and other disease-causing metabolites. Patients with severe MMA may present with symptoms as early as at birth including poor feeding, vomiting, hypotonia, hypothermia, respiratory distress and progressive encephalopathy. They are also at increased risk of neurological symptoms, failure to thrive, intellectual disability, severe infections and progressive renal insufficiency. There are currently no approved treatment options targeting the underlying cause of the disease. Current standard of care for MMA includes protein diet management and liver transplantation.

The retrospective non-interventional natural history study collected data via chart review from 18 patients diagnosed with severe MMA by newborn screening, including patients managed medically from birth to three years old (cohort 1) and pediatric-age recipients of liver transplant (cohort 2). Patients in cohort 2 were observed for the one-year period before liver transplantation and up to three years following the procedure. Results showed a significant and sustained decrease in methylmalonic acid levels post-transplantation compared to baseline levels. While levels did not approach normalization, there was an apparent reduction in variability, a potential indication of improved metabolic stability. Results indicate there were no significant differences in MMA- or transplant-related emergency room (ER) visits, stays or hospitalizations among patients pre- and post-transplantation up to three years. However, interpretation of the data on healthcare use was limited, as data for two and three years post-transplant were available for only one or two patients.

"Findings from this study are consistent with published clinical data supporting liver transplantation as an increasingly utilized therapeutic option for severe MMA patients while highlighting its limitations as an invasive and high-risk procedure that presents lifelong health considerations for patients," said Daniel Gruskin, M.D., senior vice president and head of clinical development at LogicBio. "These data help provide a better understanding of the natural progression and burden of this devastating rare disease and will help inform our clinical trial design and endpoint selection for future studies of our lead program LB-001. We also anticipate that results from a prospective natural history study we are initiating will provide important additional information about the disease course and position us to provide optimal support to families affected by MMA."

A link to the ACMG meeting study presentation is available under the "Events and Presentations" section of LogicBio's website, found here: https://investor.logicbio.com/events-and-presentations/events.

About Methylmalonic Acidemia (MMA)

Methylmalonic acidemia, or MMA, is a rare and life-threatening genetic disorder, affecting 1 in 25,000 to 50,000 newborns. In the most common form of MMA, a mutation in a gene called MMUT prevents the body from properly processing certain fats and proteins. As a result, toxic metabolites accumulate in the liver, in muscle tissue and in the brain. Symptoms include vomiting, lethargy, seizures, developmental delays and organ damage. There is no cure for MMA. To manage the symptoms, patients go on a severely restrictive, low-protein, high-calorie diet, often through a feeding tube. Even with aggressive management, these patients often experience life-threatening metabolic crises that can cause permanent neurocognitive damage.

About LogicBio Therapeutics, Inc.

LogicBio Therapeutics is a clinical-stage genetic medicine company pioneering gene delivery and gene editing platforms to address rare and serious diseases from infancy through adulthood. The company's proprietary GeneRide™ platform is a new approach to precise gene insertion that harnesses a cell's natural DNA repair process leading to durable therapeutic protein expression levels. LogicBio's cutting-edge sAAVy™ capsid development platform is designed to support development of treatments in a broad range of indications and tissues. The company is based in Lexington, MA. For more information, visit https://www.logicbio.com/.

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View original content:http://www.prnewswire.com/news-releases/logicbio-announces-presentation-of-retrospective-study-of-disease-course-in-pediatric-patients-with-severe-methylmalonic-acidemia-301282494.html

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