LogicBio Therapeutics Receives FDA Orphan Drug Designation for LB-001 for the Treatment of Methylmalonic Acidemia

Cambridge, Mass., April 29, 2019 – LogicBio Therapeutics, Inc. (NASDAQ:LOGC), a genome editing company focused on developing medicines to durably treat rare diseases in pediatric patients, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to LB-001, a recombinant adeno-associated viral vector with human methylmalonyl-COA mutase (MUT) gene for the treatment of methylmalonic acidemia (MMA).

"We are pleased to receive orphan drug designation for LB-001, our lead program in development for patients with methylmalonic acidemia. This regulatory distinction underscores the need for revolutionary treatments for MMA patients," said Fred Chereau, CEO of LogicBio. "We believe that LB-001 has potential to transform the treatment of this devastating disease, and receiving this designation represents a step forward for our program."

Orphan drug designation is granted by the FDA Office of Orphan Products Development to drugs and biologics which are intended for the treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. Under the Orphan Drug Act, the FDA may provide grant funding toward clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States following marketing approval by the FDA. For more information about orphan designation, please visit the FDA website at www.fda.gov.

About LB-001
LB-001 is an investigational pediatric genome editing therapy based on LogicBio's GeneRide™ technology. GeneRide™ enables site-specific integration and lifelong expression of therapeutic transgenes, without the use of exogenous promoters or nucleases. LB-001 is designed to incorporate a functioning version of the faulty MUT gene into the genome of MMA patients. LogicBio has demonstrated preclinical proof-of-concept of GeneRide™ in multiple animal models of the disease, improving survival and reversing disease pathology. In preclinical MMA models, LogicBio has shown that cells into which GeneRide™ has inserted a transgene demonstrate a selective survival advantage over cells not expressing the transgene.

About MMA
Primarily caused by mutations in the MUT gene, MMA is a rare, life-threatening, autosomal recessive disease that starts in early childhood for which there are no approved therapies. The disease prevents the body from properly processing certain fats and proteins, resulting in a toxic accumulation of metabolites that can cause life threatening decompensations in infants and children. This buildup can lead to significant morbidity and mortality, including infections, neurodevelopmental disabilities and chronic kidney disease.

About LogicBio Therapeutics

LogicBio Therapeutics is a genome editing company focused on developing medicines to durably treat rare diseases in pediatric patients with significant unmet medical needs using GeneRide™, its proprietary technology platform. GeneRide enables the site-specific integration of a therapeutic transgene in a nuclease-free and promoterless approach by relying on the native process of homologous recombination to drive lifelong expression. Headquartered in Cambridge, Mass., LogicBio is committed to developing medicines that will transform the lives of pediatric patients and their families.

For more information, please visit www.logicbio.com.

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