LogicBio Therapeutics Receives Rare Pediatric Disease Designation for LB-001 for the Treatment of Methylmalonic Acidemia

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CAMBRIDGE, Mass., July 16, 2019 (GLOBE NEWSWIRE) -- LogicBio Therapeutics, Inc. (NASDAQ:LOGC), a genome editing company focused on developing medicines to durably treat rare diseases in pediatric patients, today announced the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation to LB-001, a recombinant adeno-associated viral vector with human methylmalonyl-COA mutase (MUT) gene for the treatment of methylmalonic acidemia (MMA). In addition to rare pediatric disease designation, LogicBio previously received orphan drug designation from the FDA for LB-001.

“MMA is a life-threatening rare disease that presents as early as the first week of life and has no approved therapies. The urgency of our work with LB-001, our lead program, is underscored by both this new rare pediatric disease designation and the previously granted orphan drug designation. These designations signal the clear unmet need for transformative treatments for MMA patients,” said Fred Chereau, CEO of LogicBio. “We look forward to advancing LB-001 and expect to file an Investigational New Drug (IND) application in the fourth quarter of 2019.”

The FDA grants rare pediatric disease designation for serious and life-threatening diseases that primarily affect children through age 18 and affect fewer than 200,000 people in the United States. Under the FDA's rare pediatric disease priority review voucher program, a sponsor who receives approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for a subsequent marketing application for a different product. For more information about rare pediatric disease 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 potential 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.

Forward-Looking Statements

This press release contains “forward-looking” statements within the meaning of the federal securities laws. These are not statements of historical facts and are based on management’s beliefs and assumptions and on information currently available. They are subject to risks and uncertainties that could cause the actual results and the implementation of the Company’s plans to vary materially, including the risks associated with the initiation, cost, timing, progress and results of the Company’s current and future research and development activities and preclinical studies and potential future clinical trials. These risks are discussed in the Company’s filings with the U.S. Securities and Exchange Commission (SEC), including, without limitation, the Company’s Annual Report on Form 10-K filed on April 1, 2019 with the SEC, and the Company’s subsequent Quarterly Reports on Form 10-Q and other filings with the SEC. Except as required by law, the Company assumes no obligation to update these forward-looking statements publicly, even if new information becomes available in the future.

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