



LogicBio Therapeutics Provides Update on FDA Review of Investigational New Drug Application for LB-001 for Methylmalonic Acidemia

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CAMBRIDGE, Mass., Feb. 10, 2020 (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 that the U.S. Food and Drug Administration (FDA) has placed a clinical hold on the Investigational New Drug (IND) submission for LB-001 for the treatment of methylmalonic acidemia (MMA) pending the resolution of certain clinical and nonclinical questions. The Company submitted the IND in January 2020 to support the initiation of a Phase 1/2 clinical trial in patients with MMA. LogicBio expects that the FDA questions will be provided in writing within 30 days. LogicBio plans to work closely with the FDA to resolve these questions as quickly as possible.

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 human methylmalonyl-COA mutase (*MMUT*) 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. LB-001 has received both orphan drug and rare pediatric disease designations from the U.S. Food and Drug Administration.

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, including with respect to the Company's plans to resolve the FDA's clinical hold on the IND for LB-001. 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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