LogicBio Therapeutics Announces Submission of Investigational New Drug Application (IND) for LB-001 for Methylmalonic Acidemia (MMA) and Highlights Key Corporate Milestones

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– Filed IND for lead GeneRide™ candidate LB-001 in pediatric MMA patients –
– Phase 1/2 trial initiation planned for H1 2020, with preliminary data in H2 2020 –
– Established research collaboration with Takeda for GeneRide in Crigler-Najjar Syndrome (CN) –

CAMBRIDGE, Mass., Jan. 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 it has submitted an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) to initiate a Phase 1/2 trial of LB-001, a recombinant adeno-associated viral vector with human methylmalonyl-COA mutase (*MMUT)* gene for the treatment of methylmalonic acidemia (MMA). LB-001 leverages LogicBio's proprietary, promoterless, nuclease-free genome editing technology, GeneRide™, and has previously received both orphan drug and rare pediatric disease designations from the FDA.

LogicBio intends to disclose additional details regarding the planned Phase 1/2 trial, including trial size, endpoints, and timelines, once the FDA accepts the IND. LogicBio plans to initiate a Phase 1/2 trial in pediatric MMA patients in the first half of 2020, with preliminary data expected in the second half of 2020.

"We founded LogicBio with the mission of bringing genetic medicines to children with rare diseases. Both the IND submission and the nomination of our second indication represent significant steps in advancing our goal," said Fred Chereau, CEO of LogicBio. "MMA and CN are devastating early onset diseases with no approved pharmacological therapies, and we are committed to developing novel medicines based on our GeneRide platform for pediatric patients. We look forward to a transformational year for LogicBio as we work to advance our programs, validate our platform, and expand our pipeline."

Today, LogicBio also highlighted key recent and upcoming milestones.

- **Established collaboration with Takeda to leverage the GeneRide platform in a second indication, Crigler-Najjar Syndrome.** LogicBio and Takeda will further research and develop LB-301, an investigational pediatric genome editing therapy based on LogicBio's GeneRide technology for the treatment of CN. The LB-301 construct, utilizing the modularity of GeneRide, is expected to share several components with LB-001 to facilitate development. Those components include: LK-03 as the capsid; the albumin gene as the target genetic locus for integration; and a 2A peptide sequence to facilitate polycistronic expression and serving as a circulating biomarker. A separate press release with more details can be found on LogicBio's investor relations website at investor.logicbio.com.
- **Initiated Retrospective Natural History Study in MMA.** This study is designed to evaluate disease progression in pediatric patients (born since 2010) with severe MMA, with the aim of informing LogicBio's future development in MMA and its discussions with regulatory agencies.
- **Reported positive data on Next Generation Capsid Development program at European Society of Gene and Cell Therapy Annual Meeting.** Data were presented from a set of novel, synthetic adeno-associated virus (AAV) capsid candidates tested against references AAV2, AAV8, and LK-03. All capsids showed selective tropism and more potent transduction and gene expression than the reference capsids in human hepatocytes of a chimeric FRG mouse model. LogicBio, working in partnership with the Children's Medical Research Institute, intends to advance this research and present additional findings at a scientific conference in 2020.
- **Doubling available lab and office space to support GeneRide platform development and capabilities expansion.** LogicBio expects to move into new headquarters in Lexington, Mass. in the spring of 2020. LogicBio will be adding vivarium space, internal development capabilities, and will increase its capacity for in-house manufacturing of preclinical material. The expanded lab space will support the continued advancement of a robust pipeline that builds on the modular GeneRide construct. The new facilities will also support continued growth of the capsid program, which aims to develop and license new state-of-the-art viral vectors.

About Methylmalonic Acidemia
Primarily caused by mutations in the *MMUT* gene, methylmalonic acidemia is a rare, life-threatening, autosomal recessive disease for which there are no approved therapies. The disease, which starts in the first month of life, 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. The incidence of MMA in the United States is reported to be 1 in 50,000 births. LogicBio estimates the number of MMA patients with the genetic deficiency targeted by LB-001 to be 3,400 to 5,100 patients in key global markets, of which 1,000 to 1,500 patients are in the United States.

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 *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. 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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