LogicBio Therapeutics Announces Collaboration with Takeda to Develop New Genome Editing Candidate LB-301 for the Treatment of Crigler-Najjar Syndrome

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– LB-301 is a recombinant AAV vector with a *UGT1A1* gene leveraging GeneRide™ genome editing platform for the treatment of Crigler-Najjar syndrome –

– The collaboration agreement grants Takeda an exclusive option to negotiate an exclusive, worldwide license to LogicBio's LB-301 program –

– Crigler-Najjar syndrome is the second indication to be pursued using GeneRide™ platform –

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 a research collaboration with Takeda Pharmaceutical Company Limited (Takeda) to further develop LB-301, an investigational therapy using LogicBio's proprietary, promoterless, nuclease-free genome editing technology, GeneRide™, for the treatment of Crigler-Najjar syndrome. LB-301 is a recombinant adeno-associated viral (AAV) vector with a uridine disphosphate-glucuronosyltransferase-1 (*UGT1A1*) gene. The collaboration will bring together LogicBio's propriety platform for genome editing and Takeda's expertise in researching and developing gene therapies.

"LogicBio's innovative, site-specific, genome editing platform has the potential to overcome the limitations that make it challenging to apply conventional gene editing and gene transfer in pediatric patients," said Dan Curran, Head, Rare Diseases Therapeutic Area Unit at Takeda. "We see GeneRide™ as a promising approach to explore as part of our aspiration to develop transformative – or even potentially curative – therapies to patients living with rare diseases."

"We are thrilled to be working with Takeda to advance our GeneRide™ platform in a second indication," said Fred Chereau, CEO of LogicBio. "Their insights and expertise in rare diseases drug development is expected to significantly accelerate the development of a much-needed therapy for this devastating pediatric disease. This collaboration recognizes GeneRide™ as a promising approach for bringing the transformational power of genome editing to children with an array of relentless, progressive pediatric diseases."

Under the agreement, LogicBio and Takeda will collaborate to further research and develop LB-301. Takeda will provide funding for the research program under the collaboration agreement and will have an exclusive option to negotiate an exclusive, worldwide license to LogicBio's LB-301 program.

Crigler-Najjar syndrome is a rare monogenic pediatric disease caused by a deficiency in the liver-specific *UGT1A1* gene, resulting in severely high levels of unconjugated bilirubin in the blood starting at birth, with lifelong risk of permanent neurological damage and death. Current clinical practice consists of daily, intense phototherapy treatment for approximately 12 hours, but this treatment becomes less effective with age, ultimately leaving liver transplantation as the only therapeutic option for survival.

LogicBio has demonstrated that a murine GeneRide™ construct of LB-301 can correct the gene deficiency in an animal model of Crigler-Najjar syndrome. The introduction of a *UGT1A1* gene into the albumin locus in mouse liver cells resulted in normalization of bilirubin levels and long-term survival of mice deficient in *UGT1A1* from fewer than 20 days to at least one year. The results from this research were published in *EMBO Molecular Medicine* (Porro et al., 2017).

About LB-301
LB-301 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-301 is designed to incorporate a functioning version of the faulty uridine disphosphate-glucuronosyltransferase-1 (*UGT1A1*) gene into the genome of Crigler-Najjar patients. LogicBio has demonstrated preclinical proof-of-concept of GeneRide™ in multiple animal models of the disease, improving survival and reversing disease pathology.

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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