



## **LogicBio Therapeutics Announces SUNRISE Phase 1/2 Clinical Design for LB-001 for the Treatment of Methylmalonic Acidemia in Pediatric Patients**

August 10, 2020

**– LogicBio’s First IND Clearance Leveraging GeneRide, an In Vivo Homologous Recombination-based Genome Editing Platform –**

**– Enrollment to Start with Patients as Young as 3 Years Old, De-escalating Down to 6 Months old –**

**– Webcast and Conference Call Today at 8 a.m. ET to Discuss LB-001 Update –**

LEXINGTON, Mass., Aug. 10, 2020 (GLOBE NEWSWIRE) -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC) (LogicBio or the Company), a company dedicated to extending the reach of genetic medicine with pioneering targeted delivery platforms, today announced the clinical trial design for the planned Phase 1/2 clinical trial for LB-001 in pediatric patients with methylmalonic acidemia (MMA) .

“We’re pleased to announce our plans for developing LB-001 in pediatric patients with MMA. This is an important milestone for patients, their families, our company and for the genetic medicines space more broadly as we believe this is the first Investigational New Drug application (IND) clearance for an *in vivo* gene editing program harnessing homologous recombination,” said Fred Chereau, CEO of LogicBio. “We have maintained a steadfast commitment to execution and the IND clearance is the first of several near-term milestones we expect to reach in our determined efforts to leverage cutting-edge medicines to address high unmet medical needs. I look forward to sharing those updates as we advance our LB-001 program.”

Daniel Gruskin, M.D., SVP, head of clinical development, commented, “The effects of MMA usually appear shortly after birth and can quickly become severe and life-threatening. Early intervention in this vulnerable population is critical to combat the manifestation of irreversible clinical disease pathologies including neurological damage. That’s the reason MMA is on the newborn screening panel in the United States. Our protocol allows for the first cohort to enroll patients as young as three years old and, once certain safety parameters are met, we can age de-escalate to as young as six months old. Our goal is to provide a safe and durable therapeutic with a single administration at an age when we can make a difference for these patients.”

### **SUNRISE - Phase 1/2 Clinical Trial of LB-001 in Pediatric Methylmalonic Acidemia (MMA)**

The SUNRISE trial is a multi-center, open-label, Phase 1/2 clinical trial designed to assess the safety and tolerability of a single intravenous infusion of LB-001 in pediatric patients with MMA characterized by methylmalonyl-CoA mutase gene (*MMUT*) mutations. Six leading centers in the United States are expected to participate in the SUNRISE Phase 1/2 trial. The trial is expected to enroll eight pediatric patients with ages ranging from 6 months to 12 years, initially starting with 3 to 12 year-old patients and then adding patients aged 6 months to 2 years. The SUNRISE Phase 1/2 trial will evaluate two doses of LB-001. Patients will participate in a pre-dosing observational period and will be administered a prophylactic steroid regimen. The primary endpoint of the SUNRISE trial is to assess the safety and tolerability of LB-001 at 52 weeks after a single infusion. Additional endpoints include changes in disease-related biomarkers, including serum methylmalonic acid, clinical outcomes such as growth and healthcare utilization, and the pharmacodynamic marker albumin-2A. The Company expects to enroll the first patient in early 2021.

### **LB-001 Webcast and Conference Call Information**

LogicBio will host a webcast and conference call on Monday, August 10, 2020 at 8:00 a.m. ET. The webcast will feature an overview of recently generated preclinical data using LB-001 and the planned clinical development plan for LB-001.

The event will be broadcast live and available under the investor relations section of LogicBio’s website at <https://investor.logicbio.com/>. The dial-in details for the call are +1 833-519-1335 or +1 602-585-9978, Conference ID: 1151546. A replay of the webcast will be available on the LogicBio website for one month following the call.

### **About LogicBio Therapeutics**

LogicBio Therapeutics is dedicated to extending the reach of genetic medicine with pioneering targeted delivery platforms.

LogicBio’s proprietary genome editing technology platform, GeneRide, enables the site-specific integration of a therapeutic transgene without nucleases or exogenous promoters by harnessing the native process of homologous recombination. LogicBio has received FDA clearance for the first-in-human clinical trial of LB-001, a wholly owned genome editing program leveraging GeneRide for the treatment of methylmalonic acidemia. Patient enrollment is expected to begin in early 2021. In addition, LogicBio has a collaboration with Takeda to research and develop LB-301, an investigational therapy leveraging GeneRide for the treatment of the rare pediatric disease Crigler-Najjar syndrome.

LogicBio is also developing a Next Generation Capsid platform for use in gene editing and gene therapies. Data presented have shown that the capsids deliver highly efficient functional transduction of human hepatocytes with improved manufacturability with low levels of pre-existing neutralizing antibodies in human samples. Top-tier capsid candidates from this effort demonstrated significant improvements over benchmark AAVs currently in clinical development. LogicBio is developing these highly potent vectors for internal development candidates and potentially for business development collaborations.

LogicBio is headquartered in Lexington, Mass. For more information, please visit [www.logicbio.com](http://www.logicbio.com).

## Forward Looking Statements

This press release contains “forward-looking” statements within the meaning of the federal securities laws, including those related to the Company’s plans to initiate, advance and complete its planned SUNRISE Phase 1/2 clinical trial of LB-001 in MMA; the timing, progress and results of the Company’s research and development activities, including those related to the GeneRide technology platform and Next Generation Capsid Program; and its plans for LB-301 in Crigler-Najjar. 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. In particular, the impact of the COVID-19 pandemic on the Company’s ability to progress with its research, development, manufacturing and regulatory efforts, including the Company’s plans to initiate, advance and complete its Phase 1/2 clinical trial for LB-001 in MMA, and the value of and market for the Company’s common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the United States and in other countries, and the effectiveness of actions taken globally to contain and treat the disease. 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 March 16, 2020 with the SEC, the Company’s Quarterly Report on Form 10-Q filed on May 11, 2020, 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.

### Contacts:

#### Investors:

Brian Luque  
Associate Director, Investor Relations  
[bluque@logicbio.com](mailto:bluque@logicbio.com)  
951-206-1200

#### Media:

Stephanie Simon  
Ten Bridge Communications  
[Stephanie@tenbridgecommunications.com](mailto:Stephanie@tenbridgecommunications.com)  
617-581-9333



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