LogicBio Therapeutics Provides Update on LB-001 Clinical Development Program

February 2, 2022

- Company to host conference call and webcast today at 8:30 a.m. ET

LEXINGTON, Mass., Feb. 2, 2022 /PRNewswire/ -- LogicBio Therapeutics, Inc. (Nasdaq: LOGC), a clinical-stage genetic medicine company, today provided an update on the LB-001 clinical development program. The U.S. Food and Drug Administration (FDA) has notified the company that its Phase 1/2 SUNRISE clinical trial of LB-001 in pediatric patients with methylmalonic acidemia (MMA) has been placed on clinical hold. The company will host a conference call and webcast this morning to discuss this update.

To date, four patients have been dosed in the SUNRISE trial with LB-001, LogicBio's investigational, single-administration, adeno-associated virus (AAV) genome editing therapy. In accordance with the FDA-cleared protocol, the first two patients dosed were in the older age group (3 to 12 years old) and received 5 x 10^{13} vg/kg of LB-001. These first two patients are doing well, have not experienced drug-related serious adverse events (SAEs), and are being monitored in accordance with the protocol.

As previously disclosed, the third patient dosed in the SUNRISE trial, who received 5 x 10^{13} vg/kg of LB-001 and is in the younger age group (6 months to 2 years old), experienced a drug-related SAE, which was categorized as a case of thrombotic microangiopathy (TMA). TMA has been previously reported in association with other AAV genetic therapies. The patient was hospitalized and responded well to intravenous fluids and parenteral nutrition. Following this SAE, the company implemented additional safety measures in the SUNRISE trial, and reported the SAE to the FDA and the Data Safety Monitoring Board for the trial (DSMB). In December 2021, the company announced that the SAE experienced by the third patient had resolved.

In January 2022, the fourth patient dosed in the SUNRISE trial, who received 5 x 10^{13} vg/kg of LB-001 and is in the younger age group, experienced a drug-related SAE, which was categorized as a case of TMA. The patient is being closely followed by the patient's care team and has been steadily improving. The company reported the SAE to the FDA and the DSMB. The FDA subsequently notified the company that the SUNRISE trial has been placed on clinical hold. LogicBio will be working closely with the FDA and the DSMB to determine the next steps for the SUNRISE trial and the LB-001 program.

"Patient safety is our first priority. I would like to thank the patient and the patient's family for participating in our trial as well as the on-site team for the excellent care they are providing," said Fred Chereau, president and chief executive officer of LogicBio. "We look forward to working closely with the FDA and the DSMB to determine the next steps for the trial and the program."

Until the company has more clarity regarding the impact of the clinical hold, LogicBio is suspending guidance on the timing of announcing interim data for the SUNRISE trial.

Conference Call and Webcast Details

LogicBio will host a conference call and webcast today, Wednesday, February 2, 2022, at 8:30 a.m. ET to discuss the program update. To listen to the conference call, please dial +1 (833) 519-1335 (domestic) or +1 (602) 585-9978 (international) using conference ID number 7545016. A live webcast of the call can be accessed via the Investors section of the company's website at https://investor.logicbio.com. A webcast replay will be available following the call and archived for approximately 30 days.

About LB-001

LB-001 is an investigational, first-in-class, single-administration, genome editing therapy for early intervention in methylmalonic acidemia (MMA) using LogicBio's proprietary GeneRide™ drug development platform. GeneRide technology utilizes a natural DNA repair process called homologous recombination that enables precise editing of the genome without the need for exogenous nucleases and promoters that have been associated with an increased risk of immune response and cancer. LB-001 is designed to non-disruptively insert a corrective copy of the methylmalonyl-CoA mutase (MMUT) gene into the albumin locus to drive lifelong therapeutic levels of MMUT expression in the liver, the main site of MMUT expression and activity. LB-001 is delivered to hepatocytes intravenously via liver-targeted, engineered recombinant adeno-associated virus vector (rAAV-LK03). Preclinical studies found that LB-001 was safe and demonstrated transduction of hepatocytes, site-specific genomic integration, and transgene expression. LB-001–corrected hepatocytes in a mouse model of MMA demonstrated preferential survival and expansion (selective advantage), thus contributing to a progressive increase in hepatic MMUT expression over time. LB-001 resulted in improved growth, metabolic stability, and survival in MMA mice. The U.S. Food and Drug Administration (FDA) granted fast track designation, rare pediatric disease designation and orphan drug designation for LB-001 for the treatment of MMA. In addition, the European Medicines Agency (EMA) granted orphan drug designation for LB-001 for the treatment of MMA.

About LogicBio Therapeutics

LogicBio Therapeutics is a clinical-stage genetic medicine company pioneering genome editing and gene delivery platforms to address rare and serious diseases from infancy through adulthood. The company's genome editing platform, GeneRide™, is a new approach to precise gene insertion harnessing a cell's natural DNA repair process potentially leading to durable therapeutic protein expression levels. The company's gene delivery platform, sAAVy™, is an adeno-associated virus (AAV) capsid engineering platform designed to optimize gene delivery for treatments in a broad range of indications and tissues. The company is based in Lexington, MA. For more information, visit www.logicbio.com, which does not form a part of this release.

Forward-Looking Statements

Statements in this press release regarding LogicBio's strategy, plans, prospects, expectations, beliefs, intentions and goals are forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, as amended, including but not limited to statements regarding interactions with the FDA and the DSMB to determine next steps for the SUNRISE trial and the LB-001 development program. The terms "look forward," "will" and similar references are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including risks associated with continuing to advance the LB-001 development program, including the SUNRISE trial; the risk that clinical trials may not be successful or may be discontinued or delayed for any reason; the risk that existing preclinical and clinical data, including early clinical data from a trial, may not be predictive of the results of ongoing or later clinical trials; the timing and content of decisions made by regulatory authorities, including the FDA, and safety monitoring boards, including the DSMB; the actual time it takes to initiate and complete preclinical and clinical studies; the potential direct or indirect impact of the COVID-19 pandemic on LogicBio's business, operations, and the markets and communities in which LogicBio and its partners, collaborators and vendors operate; manufacturing risks; risks associated with management and key personnel changes and transitional periods; the actual funding required to develop and commercialize product candidates, including for safety, tolerability, enrollment, manufacturing or economic reasons; the competitive landscape; changes in the economic and financial conditions of LogicBio; and LogicBio's ability to obtain, maintain and enforce patent and other intellectual property protection for LB-001 and any other product candidates. Other risks and uncertainties include those identified under the heading "Risk Factors" in LogicBio's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021 and other filings that LogicBio may make with the U.S. Securities and Exchange Commission in the future. These forward-looking statements (except as otherwise noted) speak only as of the date of this press release, and LogicBio does not undertake, and specifically disclaims, any obligation to update any forward-looking statements contained in this press release.

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