LogicBio Therapeutics Announces Clinical and Corporate Updates

April 27, 2021

- First patient for Phase I/II SUNRISE trial expected to be enrolled by mid-year 2021
- Company also entered into strategic collaboration with CANbridge Pharmaceuticals and research partnership with Daiichi Sankyo

LEXINGTON, Mass., April 27, 2021 /PRNewswire/ -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC), a clinical-stage genetic medicine company pioneering gene delivery and gene editing platforms to address rare and serious diseases from infancy through adulthood, today announced an update on screening and enrollment activities for the SUNRISE Phase I/II clinical trial of LB-001, the company's investigational treatment for methylmalonic acidemia (MMA) based on LogicBio's proprietary gene insertion platform, GeneRide™.

"The first two patients who entered pre-dosing screening activities did not meet all of the enrollment criteria for the SUNRISE trial, each for different reasons, and we will continue to screen additional patients in the coming weeks," said Daniel Gruskin, M.D., senior vice president and head of clinical development at LogicBio. "With COVID-19 restrictions being lifted in some U.S. states, additional clinical trial sites have recently been activated and have begun recruiting and screening patients."

"We expect to enroll our first patient in the SUNRISE trial by mid-year 2021, which represents a slight delay from our earlier communications," said Frederic Chereau, president and chief executive officer of LogicBio. "We are also pleased to report that we have pre-identified enough patients to fully enroll SUNRISE, assuming a certain number of screening failures."

Today, LogicBio also announced a strategic collaboration with CANbridge Pharmaceuticals and research partnership with Daiichi Sankyo.

LogicBio entered into a strategic collaboration with CANbridge Pharmaceuticals for an exclusive option to license LB-001 in Greater China (China, Taiwan, Hong Kong and Macau). The deal also grants CANbridge a worldwide license with respect to AAV sL65, the first capsid based on LogicBio's proprietary sAAVy™ platform, to support development and commercialization of CANbridge's gene therapy programs for Fabry disease and Pompe disease, with options for two additional indications. Under the terms of the agreement, LogicBio is eligible to receive an upfront payment of $10 million in addition to up to $581 million in option payments and milestones payments, as well as up to double-digit royalties. A separate press release with more details can be found on LogicBio's investor relations website at investor.logicbio.com

LogicBio also entered into a research collaboration with Daiichi Sankyo for the development of treatments for two indications based on GeneRide™. The agreement also grants Daiichi Sankyo an option to negotiate and acquire worldwide licenses to LogicBio's development programs in these indications. A separate press release with more details can be found on LogicBio's investor relations website at investor.logicbio.com.

About LogicBio Therapeutics, Inc.

LogicBio Therapeutics is a clinical-stage genetic medicine company pioneering gene delivery and gene editing platforms to address rare and serious diseases from infancy through adulthood. The company's proprietary GeneRide™ platform is a new approach to precise gene insertion that harnesses a cell's natural DNA repair process leading to durable therapeutic protein expression levels. LogicBio's cutting-edge sAAVy™ capsid development platform is designed to support development of treatments in a broad range of indications and tissues. The company is based in Lexington, MA. For more information, visit https://www.logicbio.com/.

About the SUNRISE Trial

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 methylmalonic acidemia (MMA) characterized by methylmalonyl-CoA mutase gene (MMUT) mutations. Seven leading centers in the United States and one in Saudi Arabia are expected to participate. With the aim of evaluating LB-001 at an early age, before irreversible tissue damage has occurred, the SUNRISE trial is initially enrolling pediatric patients from 3 -12 years old, with the possibility to include patients as young as 6 months old after meeting certain safety and biomarker parameters. The SUNRISE trial will evaluate two doses of LB-001. Patients will participate in a pre-dosing observational period, will be administered a prophylactic steroid regimen, and will be followed for 52 weeks. Secondary and exploratory 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.

About LB-001

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 are associated with an increased risk of immune response and cancer. LB-001 is a first-in-class, single-dose gene-editing therapy for early intervention in methylmalonic acidemia (MMA) designed using the GeneRide™ platform to insert a corrective copy of the MMUT gene into the ALB 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 via an engineered rAAV vector utilizing the highly efficient and selective liver-targeted LK03 capsid (rAAV-LK03). Preclinical assessment in human hepatocytes demonstrated that LB-001 is capable of site-specific insertion. Preclinical in vivo studies found LB-001 to be safe and demonstrated transduction of hepatocytes, genomic integration, and liver 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.

About Methylmalonic Acidemia (MMA)

Methylmalonic acidemia, or MMA, is a rare and life-threatening genetic disorder, affecting 1 in 25,000 to 50,000 newborns. In the most common form of MMA, a mutation in a gene called MMUT prevents the body from properly processing certain fats and proteins. As a result, toxic metabolites accumulate in the liver, in muscle tissue and in the brain. Symptoms include vomiting, lethargy, seizures, developmental delays and organ damage. There is no cure for MMA. To manage the symptoms, patients go on a severely restrictive, low-protein, high-calorie diet, often through a feeding tube. Even with aggressive management, these patients often experience life-threatening metabolic crises that can cause permanent neurocognitive damage.

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 expectation regarding patient enrollment in the SUNRISE trial, its strategic collaboration with CANbridge Pharmaceuticals, its research partnership with Daiichi Sankyo and the potential of the GeneRide™ platform. 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 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 March 15, 2021 and the Company's subsequent 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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