# LogicBio Therapeutics Announces First Patient Dosed in Groundbreaking Phase 1/2 SUNRISE Clinical Trial for the Treatment of Pediatric Patients Suffering from Methylmalonic Acidemia

June 2, 2021

**- Company believes dosing represents first in vivo gene editing therapy delivered systemically to a pediatric patient**
**- Clinical trial designed to treat patients as young as 6 months old with LB-001 based on proprietary GeneRide™ platform**
**- Company remains on track to announce update on enrollment, dose escalation and age de-escalation in late 2021 and interim data by year-end 2021**

LEXINGTON, Mass., June 2, 2021 /PRNewswire/ -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC), a clinical-stage genetic medicine company pioneering gene editing and gene delivery platforms to address rare and serious diseases from infancy through adulthood, today announced that the first patient has been dosed with LB-001, the Company's investigational single-administration gene editing therapy based on its proprietary GeneRide™ platform, in the SUNRISE Phase 1/2 clinical trial in pediatric patients with methylmalonic acidemia (MMA). The child received the intravenous infusion of LB-001 at Monroe Carell Jr. Children's Hospital at Vanderbilt.

"Today's landmark announcement represents a significant step forward in gene editing for children suffering from early onset genetic diseases," said Frederic Chereau, president and chief executive officer of LogicBio. "In addition to dosing the first patient, we have now opened several trial sites and identified enough additional patients to fully enroll SUNRISE, subject to screening clearances. We look forward to providing an update on enrollment, dose escalation and age de-escalation in late 2021. In addition, we continue to expect to announce interim clinical data by the end of the year."

"This moment represents the next step in a potential new era for the treatment of MMA," said Kathy Stagni, executive director of the Organic Acidemia Association (OAA). "The OAA community of patients and caregivers are grateful to the researchers at LogicBio who have been dedicated to advancing this promising research and it is exciting to see that we are one step closer to a more hopeful future."

MMA is a rare and life-threatening genetic disorder for which there are currently no treatments addressing the underlying cause of the disease. To manage the symptoms and detrimental effects of MMA, patients must maintain a severely restrictive diet. Even with aggressive management, MMA patients often experience metabolic crises that can cause permanent neurocognitive damage.

The SUNRISE trial is initially enrolling patients 3-12 years old and will potentially enroll infants as young as 6 months once the first two patients meet certain safety parameters and a biomarker indicating genome integration and protein expression is detected.

"Many genetic medicines are unable to target pediatric indications such as MMA, but an early and durable intervention in this vulnerable population has the potential to prevent disease progression and irreversible symptoms, including neurological damage," said Daniel Gruskin, MD, chief medical officer of LogicBio. "Our goal is to provide a safe and durable therapeutic option to treat MMA early enough to make a meaningful difference in patients' lives and eliminate the need for invasive liver transplantation, which is increasingly performed in children suffering from this disease."

"This milestone takes us one step closer to bringing a much-needed treatment option to patients living with MMA. I look forward to helping advance the SUNRISE trial and seeing the results," said Thomas M. Morgan, MD, the principal investigator of the SUNRISE trial at Monroe Carell Jr. Children's Hospital at Vanderbilt.

**About the SUNRISE Trial**

The SUNRISE trial is an open-label, multi-center, 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. On June 2, 2021, the Company announced that the first patient was dosed. Seven leading centers in the United States and one in Saudi Arabia are expected to participate in the trial. With the aim of evaluating LB-001 at an early age, before irreversible damage has occurred, the SUNRISE trial is initially enrolling 3-12 year old patients with the potential to include infants as young as 6 months old after meeting certain safety parameters and biomarker detection. The SUNRISE trial will enroll up to 8 patients and evaluate a single administration of LB-001 at two dose levels.

**About LB-001**

LB-001 is an investigational, first-in-class, single-administration, gene editing therapy for early intervention in methylmalonic acidemia (MMA) using the GeneRide™ 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 are 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 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 for LB-001 for the treatment of MMA. In addition, the Company has received rare pediatric disease designation and orphan drug designation from the FDA for LB-001.

**About Methylmalonic Acidemia (MMA)**

Methylmalonic acidemia (MMA) is a rare and life-threatening genetic disorder affecting approximately 1 in 50,000 newborns. In the most common form of MMA, a mutation in a gene called methylmalonyl-CoA mutase (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 approved medical therapy addressing the underlying cause of the disease. 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. Because of the need for early intervention, newborns are screened for MMA in every state in the United States.

**About LogicBio Therapeutics**

LogicBio Therapeutics is a clinical-stage genetic medicine company pioneering gene editing and gene delivery platforms to address rare and serious diseases from infancy through adulthood. The Company's first 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 second 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 dosing representing the first in vivo gene editing therapy delivered systemically to a pediatric patient; the expected timing of providing an update on enrollment, dose escalation and age de-escalation in the SUNRISE trial; the expected timing of announcing interim clinical data in the SUNRISE trial; dosing representing a potential new era for the treatment of MMA; enrolling patients as young as 6 months old in the SUNRISE trial; the potential benefits of LB-001; and the sites expected to participate in the SUNRISE trial. The terms "believe," "expect," "goal," "look forward," "on track," "potential," "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 the potential direct or indirect impact of the COVID-19 pandemic on our business, operations, and the markets and communities in which we and our partners, collaborators, vendors and customers operate; the risk that existing preclinical data may not be predictive of the results of ongoing or later clinical trials; the risks that clinical trials may not be successful or may be discontinued or delayed for any reason; 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 timing and content of decisions made by regulatory authorities; the actual time it takes to initiate and complete preclinical and clinical studies; 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 Annual Report on Form 10-K for the year ended December 31, 2020 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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