LogicBio Therapeutics Provides Business Updates

December 22, 2021

- SUNRISE Phase 1/2 interim data expected to be announced in 2Q 2022 -
- New GeneRide™ development candidate nominated for the treatment of hereditary tyrosinemia type 1 -
- Susan R. Kahn, leading advocate for patients and families impacted by rare genetic diseases, appointed to Board of Directors -

LEXINGTON, Mass., Dec. 22, 2021 /PRNewswire/ -- LogicBio Therapeutics, Inc. (Nasdaq: LOGC), a clinical-stage genetic medicine company, today provided updates around its SUNRISE Phase 1/2 clinical trial and development pipeline, and announced the appointment of Susan R. Kahn to its Board of Directors.

SUNRISE Phase 1/2 Clinical Trial Update

SUNRISE is a first-in-human, open-label, multi-center, Phase 1/2 clinical trial designed to assess the safety and tolerability of an intravenous infusion of LB-001, the company's investigational, single-administration genome editing therapy, in pediatric patients with methylmalonic acidemia (MMA). SUNRISE is designed to enroll up to eight patients across two age groups (six months to two years old, and three to twelve years old) and evaluate LB-001 at two dose levels (5×10^{13} vg/kg and 1×10^{14} vg/kg). In October, LogicBio announced early data from the SUNRISE trial demonstrating the first-ever *in vivo* genome editing in children. These early data showed measurable levels of albumin-2A, a technology-related biomarker indicating site-specific gene insertion and protein expression. In November, the company disclosed that the third patient dosed in SUNRISE, who received 5×10^{13} vg/kg of LB-001 and was in the lower age group, experienced a serious adverse event (SAE). This SAE has been completely resolved and all laboratory parameters that were abnormal at the time the SAE was observed have returned to normal. The SUNRISE trial is continuing in accordance with the protocol and additional safety measures implemented following the occurrence of the SAE. The company plans to report additional interim data from the SUNRISE trial in the second quarter of 2022.

"We believe reporting interim data from SUNRISE in the second quarter of 2022 instead of by year-end 2021 will allow us to amass a more robust and meaningful data set," said Fred Chereau, president and chief executive officer of LogicBio. "Safety is our top priority, and we are pleased that the patient who experienced the SAE is now fully recovered. We expect to dose two additional patients in the near term, one patient in the older age group at the higher dose, and another patient in the lower age group at the lower dose."

Nomination of New GeneRide Development Candidate for the Treatment of Hereditary Tyrosinemia Type 1

In accordance with its previously announced guidance, LogicBio also announced the nomination of a new development candidate, LB-401, for the treatment of hereditary tyrosinemia type 1 (HT1). This development candidate is based on the company's GeneRide genome editing platform. HT1 is a rare, genetic disorder characterized by elevated blood levels of the amino acid tyrosine. This condition is caused by a shortage of the enzyme fumarylacetoacetate hydrolase (FAH), one of the enzymes required for the multi-step process that breaks down tyrosine.

In preclinical studies presented at the 2021 ESGCT Annual Meeting (ESGCT) in October, HT1 models with acute liver damage showed that GeneRide-edited hepatocytes substantially repopulated the entire liver within four weeks post-administration, replacing the diseased hepatocytes with corrected hepatocytes. HT1 mice that received the GeneRide-FAH vector were no longer reliant on the current standard of care for the disease, and demonstrated restored normal body growth, liver function, and undetectable succinyl acetone levels.

"We are excited to explore the potential of our GeneRide platform in HT1, a devastating rare disease that can present within the first months of patients' lives," said Mariana Nacht, Ph.D., chief scientific officer of LogicBio. "The preclinical data presented at ESGCT for this indication validate our confidence in leveraging the selective advantage observed in our GeneRide platform to deliver corrected hepatocytes that drive improved disease markers."

Susan R. Kahn Appointed to Board of Directors

The company also announced today the appointment of Susan R. Kahn to its Board of Directors. From September 2007 to November 2021, Ms. Kahn was the executive director of the National Tay-Sachs & Allied Diseases Association (NTSAD), a highly regarded patient advocacy group for children and adults affected by rare genetic diseases. Previously, she was at Genzyme Genetics, where she led initiatives to develop and execute new business opportunities, acquisition and partnering strategies, new product and technology assessments, and technology licensing. Before that, Ms. Kahn worked at Chiron Diagnostics in roles of increasing responsibility, including roles in business development and finance. She earned an A.B. in applied mathematics-economics from Brown University and an M.B.A. from the Tuck School of Business at Dartmouth**.**

"Sue is a leading advocate for those impacted by rare genetic diseases. Her passion for these patients and their families aligns perfectly with our mission at LogicBio," said Mr. Chereau. "I am thrilled to welcome Sue to our Board and am excited to leverage her expertise and thought leadership in the advocacy space as we continue to advance our mission to deliver the hope of genetic medicine to people impacted by devastating, early onset diseases."

"I am excited to join the LogicBio Board at what I believe is a pivotal time for the company," said Ms. Kahn. "Having spent the last fourteen years supporting patients and families affected by rare genetic diseases, I look forward to helping LogicBio deliver much-needed treatment options to patients."

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.

About the SUNRISE Trial

The SUNRISE trial is an open-label, multi-center, Phase 1/2 clinical trial designed to assess the safety, tolerability and preliminary efficacy 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 in the trial. With the aim of evaluating LB-001 at an early age, the SUNRISE trial is designed to enroll up to eight patients with ages ranging from six months to twelve years and evaluate a single administration of LB-001 at two dose levels (5×10^{13} vg/kg and 1×10^{14} vg/kg).

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 Methylmalonic Acidemia (MMA)

Methylmalonic acidemia (MMA) is a rare and life-threatening genetic disorder affecting approximately 1 in 50,000 newborns in the United States. 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 require recurrent hospitalizations and cause permanent neurocognitive damage. Because of this risk for irreversible damage, early intervention is critical and newborns are screened for MMA in every state in the United States.

About Hereditary Tyrosinemia Type 1 (HT1)

Hereditary tyrosinemia type 1 (HT1) affects 1 in 100,000 to 120,000 newborns worldwide. In the most common form, it is characterized by elevated blood levels of the amino acid tyrosine, a building block of most proteins. This condition is caused by a shortage of the enzyme fumarylacetoacetate hydrolase (FAH), one of the enzymes required for the multi-step process that breaks down tyrosine. This enzyme shortage is caused by mutations in the FAH gene. Symptoms usually appear in the first few months of life and include failure to thrive, diarrhea, vomiting, jaundice, cabbage-like odor, and increased tendency to bleed (particularly nosebleeds). HT1 can lead to liver and kidney failure, softening and weakening of the bones, problems affecting the nervous system, and an increased risk of liver cancer.

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 validation of previous research; the potential of the GeneRide™ platform in hereditary tyrosinemia type 1 or generally; the anticipated timing of announcing interim clinical data; the anticipated number and ages of patients that we expect to enroll and/or dose in the near term; and delivering treatment options to patients. The terms "believe," "expect," "look forward," "plans," "potential" 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 risk that existing preclinical data may not be predictive of the results of ongoing or later preclinical and/or clinical results; the risk that we will not be able to generate sufficient clinical data or enroll our clinical trial in a timely manner or at all; 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 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 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 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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