



LogicBio Therapeutics Reports Second Quarter 2022 Financial Results and Provides Corporate Update

August 15, 2022

- Interim data from SUNRISE trial in four pediatric patients showed detectable levels of ALB-2A biomarker demonstrating site-specific gene insertion and protein expression
- Two of four patients demonstrated increasing ALB-2A levels over time, indicating expansion of edited hepatocytes carrying the corrective gene
- Proprietary manufacturing process, mAAVRx, has shown 15- to 30-fold yield increase over standard transfection processes

LEXINGTON, Mass., Aug. 15, 2022 /PRNewswire/ -- LogicBio® Therapeutics, Inc. (NASDAQ: LOGC), a clinical-stage genetic medicine company, today reported financial results for the second quarter ended June 30, 2022, and provided business updates, including early clinical data from its ongoing SUNRISE trial evaluating LB-001 in pediatric patients with severe methylmalonic acidemia (MMA).

"We are excited to continue to see GeneRide® demonstrating its ability to precisely knock in the correct version of a gene with a single intravenous infusion—a significant milestone in the field of genetic medicine," said Fred Chereau, president and chief executive officer of LogicBio. "Additionally, LogicBio's proprietary mAAVRx™ process has continued to show significant improvement in production yields. As quality and cost of goods in genetic medicine manufacturing remain a key priority, we intend to leverage mAAVRx for our development candidates and as a potential source for business development collaborations."

Interim SUNRISE Phase 1/2 Results

SUNRISE is a first-in-human, 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 MMA. LB-001 is designed to non-disruptively knock-in 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. LB-001 is based on the company's proprietary GeneRide technology, which uses homologous recombination, a natural DNA repair process, to enable 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. Detection of the technology-related biomarker albumin-2A (ALB-2A) in the serum indicates MMUT gene integration and MUT protein expression. Increasing levels of ALB-2A suggest the expansion of the edited cells over time.

The Phase 1/2 interim results include safety and efficacy data from four patients treated with a single intravenous infusion of LB-001 at dose level 5e¹³ vg/kg. The first two patients dosed were in the three to 12 years old age group and experienced no drug-related serious adverse events (SAEs). As previously disclosed, the third and fourth patients, who were in the six months to two years old age group, each experienced a drug-related SAE, categorized as thrombotic microangiopathy (TMA). The TMA events have resolved, and both patients remain in the study. Based on dialogue with the U.S. Food and Drug Administration (FDA), the SUNRISE protocol was amended to include enhanced monitoring measures, including frequent testing for complement activation, a characteristic of TMA, as well as the use of a complement inhibitor in the event there are laboratory findings indicating a potential or imminent TMA. Additionally, prior to the TMA event, the fourth patient experienced a grade 1 drug-related SAE that was categorized as cytokine release syndrome and necessitated an additional day in the hospital post-dosing.

In addition to safety and tolerability, SUNRISE is designed to evaluate preliminary efficacy through biomarkers such as ALB-2A, a technology-related biomarker, as well as several others related to the disease itself, including methylmalonic acid, methylcitric acid, fibroblast growth factor 21 (FGF21) and propionate oxidation. ALB-2A has been detected in the serum of all four patients, which indicates site-specific integration of the MMUT gene. In two of the four patients, increasing levels of ALB-2A were seen over time, indicating selective advantage. Selective advantage enables edited hepatocytes carrying the corrective gene to survive and reproduce better than the endogenous mutated hepatocytes and to ultimately repopulate a part or whole of the diseased liver. The disease-related biomarkers, including methylmalonic acid, methylcitric acid, propionate oxidation and FGF-21, were variable and do not show a clear trend to date.

"These early results from the first four pediatric patients treated with LB-001 validate the proof of mechanism for our novel GeneRide genome editing technology," said Dr. Daniel Gruskin, chief medical officer of LogicBio. "The interim data suggest that a single systemic administration of LB-001 can lead to precise insertion of a corrective copy of the MMUT gene in the patient's hepatocytes. While the increase in ALB-2A is a promising sign, based on an analysis of preclinical and clinical data generated to date, we believe that significant additional time would be needed to determine clinical efficacy. I would like to thank the patients, their families, and the investigators who are participating in this ground-breaking trial. We look forward to continuing to better understand the biochemical and clinical effect of our genome editing therapy."

The company plans to continue observing the four patients through the long-term follow-up study to the SUNRISE trial where efficacy parameters will continue to be measured per protocol. As previously disclosed, the company expects to dose the next patient in the SUNRISE trial in the third quarter.

Recent Business Highlights:

- In May, the FDA lifted the clinical hold on LogicBio's Investigational New Drug Application (IND) for LB-001, allowing patient enrollment to resume in the Phase 1/2 SUNRISE trial in pediatric patients with MMA.

- In May, LogicBio presented four abstracts highlighting the company's GeneRide® technology in preclinical hereditary tyrosinemia type 1 (HT1) models and optimized adeno-associated virus (AAV) manufacturing processes at the American Society of Gene & Cell Therapy (ASGCT) 2022 Annual Meeting in Washington D.C.
- As part of the ASGCT presentations, LogicBio highlighted mAAVRx, its new proprietary manufacturing process. mAAVRx is an improved transient transfection of suspension cells, which has shown a 15- to 30-fold increase in vector yields compared to standard upstream processes.

Second Quarter 2022 Financial Results:

Three Months Ended June 30, 2022 and 2021

- **Revenue:** Revenue for the quarter ended June 30, 2022 consisted of \$3.2 million in collaboration and service revenue recognized under our April 2021 agreements with CANbridge Care Pharma Hong Kong Limited (CANbridge) and Daiichi Sankyo Company, Limited (Daiichi Sankyo). Revenue for the quarter ended June 30, 2021 consisted of \$0.8 million in collaboration and service revenue related to our arrangements with CANbridge, Daiichi, and our agreement with Takeda Pharmaceutical Company Limited (Takeda).
- **R&D Expenses:** Research and development expenses for the quarter ended June 30, 2022 were \$4.8 million, compared to \$7.3 million for the quarter ended June 30, 2021. The decrease of approximately \$2.4 million was primarily due to a decrease of \$1.2 million in LB-001 external development and manufacturing costs incurred during second quarter 2021 to start up the LB-001 SUNRISE clinical trial and a \$1.0 million decrease in other research and development costs primarily related to one-time intellectual property costs that occurred as a result of entering into the April 2021 collaboration agreement with CANbridge.
- **G&A Expenses:** General and administrative expenses were \$3.3 million for the quarter ended June 30, 2022, compared to \$3.8 million for the quarter ended June 30, 2021. The decrease of approximately \$0.5 million was primarily driven by a decrease of approximately \$0.5 million in professional service fees as we brought more professional work in-house through key hires made during 2021.
- **Net Loss:** Net loss for the quarter ended June 30, 2022 was \$5.0 million or \$0.15 per share, compared to a net loss of \$10.5 million, or \$0.33 per share, for the quarter ended June 30, 2021.
- **Cash Position:** As of June 30, 2022, we had cash and cash equivalents of \$38.8 million as compared to \$53.5 million as of December 31, 2021. As of June 30, 2022, we had 32,962,733 shares outstanding.
- **Financial Guidance:** Based upon our current operating plan, we believe that our \$38.8 million in cash and cash equivalents as of June 30, 2022 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2023.

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, sAAV™, 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's proprietary manufacturing process, mAAVRx™, aims to overcome one of the current limitations of AAV manufacturing by improving yields and product quality. The company is based in Lexington, MA. For more information, visit www.logicbio.com, which does not form a part of this release.

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.

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 the potential of the GeneRide[®] platform; the potential of LB-001, including its ability to lead to precise insertion; our ability to leverage mAAVRx[™] for our development candidates or any potential business development collaborations; the potential expansion of edited cells and timing thereof; the amount of time necessary to demonstrate clinical efficacy; and the anticipated timing of when we expect to dose the next patient. The terms "believe," "look forward," "future," "intend," "designed," "potential," "suggests," "plans," "expects" 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 and/or clinical data may not be predictive of the results of ongoing or later preclinical and/or clinical results; the risk that we may not be successful in efforts to leverage our technologies for business development or otherwise; 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, including the actual time it takes to demonstrate clinical efficacy; the competitive landscape; changes in the economic and financial conditions of LogicBio. 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, 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.

LogicBio Therapeutics, Inc.
Condensed Consolidated Statements of Operations
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
REVENUE				
Collaboration and service revenue	\$ 3,199	\$ 802	\$ 6,015	\$ 1,263
Total revenue	3,199	802	6,015	1,263
OPERATING EXPENSES				
Research and development	4,832	7,257	10,473	13,676
General and administrative	3,259	3,765	6,883	7,824
Total operating expenses	8,091	11,022	17,356	21,500
LOSS FROM OPERATIONS	(4,892)	(10,220)	(11,341)	(20,237)
OTHER INCOME (EXPENSE):				
Interest income	45	4	50	10
Interest expense	(197)	(283)	(415)	(554)
Total other expense, net	(152)	(279)	(365)	(544)
Net loss	\$ (5,044)	\$ (10,499)	\$ (11,706)	\$ (20,781)
Net loss per share—basic and diluted	\$ (0.15)	\$ (0.33)	\$ (0.36)	\$ (0.65)
Weighted-average common stock outstanding—basic and diluted	32,962,733	32,162,375	32,961,961	32,048,716

LogicBio Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(In thousands)

	As of	
	June 30, 2022 (Unaudited)	December 31, 2021
Cash and cash equivalents	\$ 38,846	\$ 53,480
Other assets	8,171	9,290
TOTAL ASSETS	\$ 47,017	\$ 62,770
Total liabilities	\$ 26,301	\$ 32,043
Stockholders' equity	20,716	30,727
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	\$ 47,017	\$ 62,770

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