



## LogicBio Therapeutics Reports Third Quarter 2021 Financial Results and Highlights Recent Company Milestones

November 15, 2021

- **Early clinical results from SUNRISE trial demonstrated first-ever *in vivo* genome editing in children**
- **New preclinical data highlighting successful repopulation of diseased livers in mice with healthy corrected hepatocytes in two new indications**

LEXINGTON, Mass., Nov. 15, 2021 /PRNewswire/ -- LogicBio Therapeutics, Inc. (Nasdaq: LOGC), a clinical-stage genetic medicine company, today reported financial results for the third quarter ended September 30, 2021 and highlighted recent corporate milestones.

"The recent progress we have made provides further validation of the potential of our GeneRide™ technology and brings us closer to delivering the hope of genetic medicine to people impacted by devastating diseases," said Frederic Chereau, president and chief executive officer of LogicBio Therapeutics. "We remain on track to provide interim data from our Phase 1/2 SUNRISE clinical trial in pediatric patients with methylmalonic acidemia by the end of the year. Additionally, the compelling preclinical data we recently presented at ESGCT showcase the potential applicability of our GeneRide technology in additional indications. We look forward to nominating our next development candidate by year-end."

### Anticipated Upcoming Milestones:

#### *Ph 1/2 SUNRISE Trial for LB-001 in Pediatric Patients with Methylmalonic Acidemia*

- **Year-end 2021:** Announce interim clinical data

#### *Pipeline*

- **Year-end 2021:** Nomination of next development candidate

### Recent Business Highlights

#### *Announced early clinical trial results demonstrating first-ever *in vivo* genome editing in children*

- In October, LogicBio announced clinical trial results demonstrating the first-ever *in vivo* genome editing in children. Early data from the company's Phase 1/2 SUNRISE clinical trial in pediatric patients with methylmalonic acidemia (MMA) showed measurable levels of albumin-2A, a technology-related biomarker indicating site-specific gene insertion and protein expression.

#### *Preclinical data presented at ESGCT demonstrated successful repopulation of diseased livers in mice with healthy corrected hepatocytes in two new liver indications*

- In October, the company presented new preclinical data highlighting GeneRide genome editing technology at the European Society of Gene and Cell Therapy (ESGCT) Virtual Congress 2021. The newly presented preclinical data further validated previous research in MMA and highlighted selective advantage, a key feature of the GeneRide technology, in two additional indications characterized by intrinsic liver damage, hereditary tyrosinemia type 1 (HT1) and Wilson disease.

### Third Quarter 2021 Financial Results

- **Revenue:** Revenue for the three months ended September 30, 2021 was \$2.1 million, compared to \$0.9 million for the three months ended September 30, 2020. The increase of approximately \$1.2 million was related to collaboration and service revenue recognized under our April 2021 agreements with CANbridge Care Pharma Hong Kong Limited and Daiichi Sankyo Company, Limited, and partially offset by winding down activities under our January 2020 agreement with Takeda Pharmaceutical Company Limited.
- **R&D Expenses:** Research and development expenses for the three months ended September 30, 2021 were \$7.8 million, compared to \$5.5 million for the three months ended September 30, 2020. The increase of approximately \$2.3 million was primarily due to increases of \$1.2 million in personnel-related costs related to an increase in headcount associated with the progress of both our partnered and internal programs and a corresponding increase of \$0.4 million in lab supplies. In addition, LB-001 external development and manufacturing costs increased \$0.5 million mainly driven by an increase in activities supporting the LB-001 development program.
- **G&A Expenses:** General and administrative expenses were \$4.3 million for the three months ended September 30, 2021, compared to \$3.2 million for the three months ended September 30, 2020. The increase of approximately \$1.1 million was primarily driven by an increase of \$0.4 million in personnel expenses, as we increased our headcount to support our continued research and development activities and build our corporate and administrative functions, as well as an increase of \$0.4 million in fees associated with professional services due to an increase in general corporate activities.
- **Net Loss:** Net loss for the three months ended September 30, 2021 was \$10.2 million or \$0.31 per share, compared to a net loss of \$8.0 million, or \$0.34 per share, for the three months ended September 30, 2020.
- **Cash Position:** As of September 30, 2021, we had cash and cash equivalents of \$59.6 million as compared to \$68.1 million as of June 30, 2021. As of September 30, 2021, we had 32,945,616 shares outstanding.
- **Financial Guidance:** Based upon our current operating plan, we believe that our \$59.6 million in cash and cash equivalents as of September 30, 2021 will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2022.

## 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 ( $5 \times 10^{13}$  vg/kg and  $1 \times 10^{14}$  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 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 is based in Lexington, MA. For more information, visit [www.logicbio.com](http://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 validation of previous research; the potential of the GeneRide™ platform; and the anticipated timing of announcing interim clinical data and the company's next development candidate. The terms "anticipate," "look forward," "on track," "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 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 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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	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
REVENUE				
Collaboration and service revenue	\$ 2,120	\$ 926	\$ 3,383	\$ 2,912
Total revenue	2,120	926	3,383	2,912
OPERATING EXPENSES				
Research and development	7,806	5,492	21,482	18,560
General and administrative	4,257	3,200	12,081	9,421
Total operating expenses	12,063	8,692	33,563	27,981
LOSS FROM OPERATIONS	(9,943)	(7,766)	(30,180)	(25,069)
OTHER INCOME (EXPENSE):				
Interest income	3	2	13	179
Interest expense	(270)	(276)	(824)	(821)
Other (expense) income, net	(3)	1	(3)	(10)
Total other expense, net	(270)	(273)	(814)	(652)
Loss before income taxes	(10,213)	(8,039)	(30,994)	(25,721)
Income tax benefit	28	—	28	—
Net loss	\$ (10,185)	\$ (8,039)	\$ (30,966)	\$ (25,721)
Net loss per share—basic and diluted	\$ (0.31)	\$ (0.34)	\$ (0.96)	\$ (1.10)
Weighted-average common stock outstanding—basic and diluted	32,443,960	23,599,052	32,181,912	23,367,804

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

	As of	
	September 30, 2021	December 31, 2020
Cash and cash equivalents	\$ 59,581	\$ 70,075
Other assets	8,605	10,565
<b>TOTAL ASSETS</b>	<b>\$ 68,186</b>	<b>\$ 80,640</b>
Accounts payable, accrued expenses and other liabilities	\$ 29,422	\$ 19,213
Stockholders' equity	38,764	61,427
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<b>\$ 68,186</b>	<b>\$ 80,640</b>

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