



LogicBio Therapeutics Reports Second Quarter 2020 Financial Results and Provides Business Updates

August 10, 2020

FDA Clears IND Application for LB-001 for the Treatment of Methylmalonic Acidemia in Pediatric Patients

LEXINGTON, Mass., Aug. 10, 2020 (GLOBE NEWSWIRE) -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC) (LogicBio or the Company), a company dedicated to extending the reach of genetic medicine with pioneering targeted delivery platforms, today reported financial results for the quarter ended June 30, 2020, provided a business update and announced the U.S. Food and Drug Administration (FDA) has cleared the Company's Investigational New Drug (IND) application for LB-001 for the treatment of methylmalonic acidemia in pediatric patients. LogicBio released a separate press release this morning providing further details on the planned Phase 1/2 clinical design for LB-001.

"We are thrilled to have received clearance to move forward with this first-in-human clinical trial with our lead product candidate, LB-001, for the treatment of methylmalonic acidemia, a life-threatening congenital genetic disease with no current therapeutic treatment options. This represents a significant milestone in our goal of bringing a treatment to MMA patients as well as for our GeneRide platform. We have maintained continuous dialogue with the centers of excellence that are planned to participate in the Phase 1/2 clinical trial, and we look forward to activating these sites as quickly as possible," said Fred Chereau, CEO of LogicBio. "We have instituted systems attempting to mitigate COVID-19 dynamics on our study start-up process and, based on our best estimates, we plan to enroll our first patient in early 2021."

Commenting on the Next Generation Capsid Program, Mr. Chereau said, "We are very excited about the recent advances in our novel capsid program, which has generated liver-tropic capsids intended for use in gene editing technologies such as GeneRide and other gene therapy approaches. We are focused on executing across all of our programs and look forward to sharing further details on our novel capsids in early 2021."

Appointment of Daniel Gruskin, M.D. to SVP, Head of Clinical Development

Daniel Gruskin, M.D. was appointed as SVP, head of clinical development in August 2020. Dr. Gruskin has served as interim head of clinical development of LogicBio since June 2020. In April 2020, Dr. Gruskin started consulting with the Company as a special advisor. Previously, Dr. Gruskin served in roles of increasing responsibility at Sanofi Genzyme, most recently as vice president, head of global medical affairs, rare disease, in which capacity he oversaw medical affairs, life cycle management, scientific affairs and other medical and development activities related to metabolic, rare and/or genetic diseases. Prior to his role at Sanofi Genzyme, Dr. Gruskin served as assistant professor, human genetics and pediatrics at Emory University School of Medicine, where he was also the chief of the genetics section at Children's Healthcare of Atlanta.

"Daniel has been instrumental in leading LB-001 clinical development efforts including getting the IND cleared. His deep experience in genetic medicines and metabolic diseases will serve LogicBio well as we look to execute on our goals for both the GeneRide and Next Generation Capsid platforms in search of transformative medicines," said Mr. Chereau.

Anticipated Milestones for 2020 and 2021:

- **GeneRide Platform**
 - **LB-001 for MMA**
 - **Early 2021:** First patient enrollment in Phase 1/2 trial
 - **Platform**
 - **Early 2021:** Translational model data
- **Next Gen Capsid Platform**
 - **Early 2021:** Translational model data

Second Quarter 2020 Financial Results

Three Months Ended June 30, 2020 and 2019

- **R&D Expenses:** Research and development expenses for the three months ended June 30, 2020 were \$5.9 million, compared to \$7.9 million for three months ended June 30, 2019. The decrease was primarily due to decreases of approximately \$2.3 million in external development and manufacturing expenses for the Company's lead product candidate, LB-001, and a decrease of \$0.1 million in personnel-related costs as increased headcount was offset by salary cuts related to the COVID-19 pandemic. These decreases were partially offset by an increase of \$0.3 million in other research and development expenses as the Company increased its overall research and development activities related to general platform development. While there may be fluctuations on a quarterly basis, the Company expects that its research and development costs will decrease during 2020 as compared to 2019 as it has already incurred a significant proportion of the LB-001 external development and manufacturing costs needed to bring LB-001 into clinical development.
- **G&A Expenses:** General and administrative expenses were \$3.0 million for the three months ended June 30, 2020, compared to \$2.5 million for the three months ended June 30, 2019. The increase was primarily due to increases in

corporate legal expense, D&O insurance premiums and non-capitalizable facilities expenses. The Company expects that general and administrative expenses will remain relatively consistent during 2020 as compared to 2019 although there may be fluctuations on a quarterly basis.

- **Net Loss:** Net loss was \$8.2 million, or \$0.35 per share, for the three months ended June 30, 2020, compared to a net loss of \$10.0 million, or \$0.45 per share, for the three months ended June 30, 2019.
- **Cash Position and Financial Guidance:** Cash and cash equivalents were \$36.7 million as of June 30, 2020. The Company believes that its cash and cash equivalents as of June 30, 2020 will enable the Company to fund operating expenses and capital expenditure requirements into the third quarter of 2021.

About LogicBio Therapeutics

LogicBio Therapeutics is dedicated to extending the reach of genetic medicine with pioneering targeted delivery platforms.

LogicBio's proprietary genome editing technology platform, GeneRide, enables the site-specific integration of a therapeutic transgene without nucleases or exogenous promoters by harnessing the native process of homologous recombination. LogicBio has received FDA clearance for the first-in-human clinical trial of LB-001, a wholly owned genome editing program leveraging GeneRide for the treatment of methylmalonic acidemia. Patient enrollment is expected to begin in early 2021. In addition, LogicBio has a collaboration with Takeda to research and develop LB-301, an investigational therapy leveraging GeneRide for the treatment of the rare pediatric disease Crigler-Najjar syndrome.

LogicBio is also developing a Next Generation Capsid platform for use in gene editing and gene therapies. Data presented have shown that the capsids deliver highly efficient functional transduction of human hepatocytes with improved manufacturability with low levels of pre-existing neutralizing antibodies in human samples. Top-tier capsid candidates from this effort demonstrated significant improvements over benchmark AAVs currently in clinical development. LogicBio is developing these highly potent vectors for internal development candidates and potentially for business development collaborations.

LogicBio is headquartered in Lexington, Mass. For more information, please visit www.logicbio.com.

Forward Looking Statements

This press release contains "forward-looking" statements within the meaning of the federal securities laws, including those related to the Company's plans to initiate, advance and complete its planned SUNRISE Phase 1/2 clinical trial of LB-001 in MMA; the timing, progress and results of the Company's research and development activities, including those related to the GeneRide technology platform and Next Generation Capsid Program; its plans for LB-301 in Crigler-Najjar; and the sufficiency of its cash and cash equivalents to fund operating expenses and capital expenditure requirements. 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 potential future clinical trials. In particular, the impact of the COVID-19 pandemic on the Company's ability to progress with its research, development, manufacturing and regulatory efforts, including the Company's plans to initiate, advance and complete its Phase 1/2 clinical trial for LB-001 in MMA, and the value of and market for the Company's common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the United States and in other countries, and the effectiveness of actions taken globally to contain and treat the disease. 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 16, 2020 with the SEC, the Company's Quarterly Report on Form 10-Q filed on May 11, 2020, and the Company's subsequent Quarterly Reports on Form 10-Q and other 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.

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,	
	2020	2019	2020	2019
REVENUE				
Service revenue	\$ 965	\$ —	\$ 1,986	\$ —
Total revenue	965	—	1,986	—
OPERATING EXPENSES				
Research and development	5,895	7,934	13,068	\$ 13,420
General and administrative	3,029	2,524	6,221	5,156
Total operating expenses	8,924	10,458	19,289	18,576
LOSS FROM OPERATIONS	(7,959)	(10,458)	(17,303)	(18,576)
OTHER INCOME (EXPENSE), NET:				
Interest income	10	411	177	854
Interest expense	(273)	—	(545)	—
Other expense, net	(5)	(1)	(11)	(1)
Total other (expense) income, net	(268)	410	(379)	853

Loss before income taxes	(8,227)	(10,048)	(17,682)	(17,723)
Income tax provision	—		—		—		(22)
Net loss	\$ (8,227)	\$ (10,048)	\$ (17,682)	\$ (17,745)
Net loss per share—basic and diluted	\$ (0.35)	\$ (0.45)	\$ (0.76)	\$ (0.79)
Weighted-average common stock outstanding—basic and diluted	23,326,018		22,479,511		23,250,910		22,396,780	

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

	As of	
	June 30, 2020	December 31, 2019
Cash, cash equivalents and investments	\$ 36,697	\$ 50,647
Other assets	10,646	5,013
TOTAL ASSETS	\$ 47,343	\$ 55,660
Accounts payable, accrued expenses and other liabilities	\$ 19,187	\$ 13,373
Stockholders' equity	28,156	42,287
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	\$ 47,343	\$ 55,660

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