



## LogicBio Reports Full Year 2019 Financial Results and Provides Business Updates

March 16, 2020

CAMBRIDGE, Mass., March 16, 2020 (GLOBE NEWSWIRE) -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC) (LogicBio or the Company), a genome editing company focused on developing medicines to durably treat rare diseases in pediatric patients, today reported financial results and provided a corporate update for the year ended December 31, 2019.

"2019 was a productive year for LogicBio marked by multi-faceted progress, including our new collaboration with Takeda to advance GeneRide™ in Crigler-Najjar, our newly announced second indication, and continued work to move our lead candidate, LB-001 towards the clinic," said Fred Chereau, CEO of LogicBio. "We are focused on resolving the FDA's questions on the LB-001 IND application as quickly as possible and remain firmly committed to bringing LB-001 to pediatric patients living with MMA as part of our broader mission to bring the promise of genomic medicines to children with rare diseases."

In January 2020, we announced the submission of an IND to support the initiation of a Phase 1/2 clinical trial in pediatric patients with MMA, which the FDA placed on clinical hold. Subsequently, we received a letter from the FDA specifying its questions related to the clinical hold. The clinical hold was based on questions that were clinical and nonclinical in nature, including questions related to the studies conducted for our IND filing, but did not relate to chemistry, manufacturing, and controls. We expect to have interactions with the FDA regarding their questions through mid-2020, after which we plan to provide guidance on the anticipated timing for the initiation of the Phase 1/2 clinical trial for LB-001.

### Business Highlights Include:

- **Established collaboration with Takeda to leverage the GeneRide platform in a second indication, Crigler-Najjar Syndrome.** LogicBio and Takeda agreed to further research and develop LB-301, an investigational pediatric genome editing therapy based on LogicBio's GeneRide technology for the treatment of CN. The LB-301 construct, utilizing the modularity of GeneRide, is expected to share several components with LB-001 to facilitate development. Those components include: LK-03 as the capsid; the albumin gene as the target genetic locus for integration; and a 2A peptide sequence to facilitate polycistronic expression and serve as a circulating biomarker.
- **Extended Sponsored Research Agreement with Oregon Health & Science University to Explore Translation of Pharmaceutically-Driven Selective Advantage of Future GeneRide™ Candidates.** The initial phase of the research program provided proof-of-principle of enhanced selective advantage for cells edited by GeneRide in pilot murine experiments. This extension phase focuses on translating the enhancement strategy beyond murine models with the goal of enhancing the clinical translation of future GeneRide candidates and other approaches to genetic medicines.
- **Initiated Retrospective Natural History Study in MMA.** This study is designed to evaluate disease progression in pediatric patients (born since 2010) with severe MMA, with the aim of informing LogicBio's future development in MMA and its discussions with regulatory agencies.
- **Reported positive data on Next Generation Capsid Development program at European Society of Gene and Cell Therapy Annual Meeting.** Data were presented from a set of novel, synthetic adeno-associated virus (AAV) capsid candidates tested against references AAV2, AAV8, and LK-03. All capsids showed selective tropism and more potent transduction and gene expression than the reference capsids in human hepatocytes of a chimeric FRG mouse model. LogicBio, working in partnership with the Children's Medical Research Institute, intends to advance this research and present additional findings at a scientific conference in 2020.

### Full Year 2019 Financial Results

*Twelve Months Ended December 31, 2019 and 2018*

- **R&D Expenses:** Research and development expenses for the year ended December 31, 2019 were \$30.7 million, compared to \$11.1 million for year ended December 31, 2018. The increase was primarily due to an increase of approximately \$14.1 million related to external development and manufacturing expenses for our lead product candidate LB-001. Personnel-related costs for the year ended December 31, 2019 included stock-based compensation expense of \$0.8 million compared to \$0.3 million for the year ended December 31, 2018.
- **G&A Expenses:** General and administrative expenses were \$10.4 million for the year ended December 31, 2019, compared to \$6.9 million for the year ended December 31, 2018. The increase was primarily due to increases in legal and professional fees and personnel-related costs primarily due to an increase in headcount. Stock-based compensation expense included in general and administrative expenses was \$1.0 million and \$0.8 million for the years ended December 31, 2019 and 2018, respectively.
- **Net Loss:** Net loss was \$40.1 million, or \$1.78 per share, for the year ended December 31, 2019, compared to a net loss

of \$17.6 million, or \$3.04 per share, for the year ended December 31, 2018.

- **Cash Position and Financial Guidance:** Cash, cash equivalents and investments were \$50.6 million as of December 31, 2019. Based upon our current operating plan, which takes into account expenditures that are contingent based on corporate developments, we believe that our cash and cash equivalents and short-term investments as of December 31, 2019 will enable us to fund our operating expenses and capital expenditure requirements into the first quarter of 2021.

#### About LogicBio Therapeutics

LogicBio Therapeutics is a genome editing company focused on developing medicines to durably treat rare diseases in pediatric patients with significant unmet medical needs using GeneRide™, its proprietary technology platform. GeneRide enables the site-specific integration of a therapeutic transgene in a nuclease-free and promoterless approach by relying on the native process of homologous recombination to drive potential lifelong expression. Headquartered in Cambridge, Mass., LogicBio is committed to developing medicines that will transform the lives of pediatric patients and their families.

For more information, please visit [www.logicbio.com](http://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 resolve the FDA’s clinical hold on the IND for LB-001; the timing, progress and results of the Company’s research and development activities, including those related to the GeneRide technology platform; and the anticipated construct for LB-301 in Crigler-Najjar. 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. 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 April 1, 2019 with the SEC, 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.

##### Consolidated Statements of Operations

(In thousands, except share and per share data)

	Year Ended December 31,	
	2019	2018
OPERATING EXPENSES:		
Research and development	\$ 30,656	\$ 11,079
General and administrative	10,385	6,864
Total operating expenses	41,041	17,943
LOSS FROM OPERATIONS	(41,041	) (17,943
OTHER INCOME (EXPENSE), NET:		
Interest income	1,500	569
Interest expense	(546	) (2
Other expense, net	(19	) (159
Total other income, net	935	408
Loss before income taxes	(40,106	) (17,535
Income tax provision	(22	) (86
Net loss	\$ (40,128	) \$ (17,621
Net loss per share—basic and diluted	\$ (1.78	) \$ (3.04
Weighted-average common stock outstanding—basic and diluted	22,602,954	5,801,533

#### LogicBio Therapeutics, Inc.

##### Condensed Consolidated Balance Sheets

(In thousands)

	As of December 31, 2019	December 31, 2018
Cash, cash equivalents and investments	\$ 50,647	\$ 80,906
Other assets	5,013	2,004
TOTAL ASSETS	\$ 55,660	\$ 82,910
Accounts payable, accrued expenses and other liabilities	\$ 13,373	\$ 2,685
Stockholders’ equity	42,287	80,225
TOTAL LIABILITIES AND STOCKHOLDERS’ EQUITY	\$ 55,660	\$ 82,910

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Source: LogicBio Therapeutics