LogicBio Announces Extension Of Collaboration With Children's Medical Research Institute

January 7, 2021

Collaboration seeks to develop next-generation viral vectors for gene therapy and gene editing applications in liver and two other tissues.

Program brings new levels of momentum to LogicBio as company plans for initiation of enrollment of SUNRISE Phase I/II clinical trial in treatment of methylmalonic acidemia (MMA) and other milestones.

LEXINGTON, Mass., Jan. 7, 2021 /PRNewswire/ -- LogicBio Therapeutics, Inc. (LogicBio - Nasdaq: LOGC), a clinical stage genetic medicines company, today announced the expansion of the Company's research partnership with Children's Medical Research Institute (CMRI) to develop next-generation adeno-associated virus (AAV) vectors for a range of gene therapy and gene editing applications in the treatment of serious diseases of the liver and two other tissues.

"I am very pleased to announce the expansion of our collaboration with CMRI, which over the past two years has already led to many important advances in our liver-targeting AAV vectors research," said Frederic Chereau, LogicBio President and CEO. "We now look forward to advancing the initial discovery efforts and to the expansion of our research into AAV capsids, the cell-targeting protein shell of viral vectors, for two additional tissues beyond the liver. We believe these broadening efforts will allow us to further expand the LogicBio pipeline and position us to explore a greater range of partnership opportunities."

New data highlighting the advantages of next-generation AAV capsids developed through the collaboration have been presented at multiple leading scientific conferences including the European Society of Gene and Cell Therapy and the American Society of Gene and Cell Therapy. To design next-generation AAV capsids, the CMRI team applies innovative genetic and molecular biology techniques including bioinformatics, machine learning and other computational methods. CMRI is also developing unique clinically predictive models to select and assess novel capsids. The Company anticipates that expansion of this collaboration will position LogicBio to advance research focusing on areas of unmet need in liver health and in two additional tissues to be announced in the near future.

The Company believes that these novel AAV capsids have broad potential applications in gene therapy and for gene editing platforms such as GeneRide™, LogicBio's proprietary gene editing technology that harnesses a cell's natural DNA repair process, known as homologous recombination, to insert a corrective copy of a gene at a precise spot in a patient's genome.

"The expansion of our research collaboration with CMRI comes at a very exciting time for LogicBio as we are poised to enroll the first patients in our SUNRISE Phase I/II clinical trial for LB-001 in the treatment of methylmalonic acidemia," Mr. Chereau added. "In addition, we are also advancing research with our investigational therapy LB-301 for the treatment of Crigler-Najjar along with our partners from Takeda. We have learned a great deal about the potential applications of GeneRide in these past four years and look forward to publishing additional proof of concept data and announcing our new target indications in 2021."

In August 2020, LogicBio announced that the FDA authorized the company to initiate the SUNRISE clinical trial and that the study would be open to patients with methylmalonic acidemia (MMA) as young as three years old, with the opportunity to treat patients as young as six months old as the trial progresses. Early treatment in MMA patients can allow for intervention prior to development or advance of nonreversible symptoms.

"The approval of our IND by the FDA for the SUNRISE trial to treat very young patients is a reflection of the quality of our pre-clinical data and the design of our clinical trial," said Dr. Daniel Gruskin, LogicBio SVP of Clinical Development.

LB-001 was granted Fast Track designation by the FDA in November 2020 and Rare Pediatric Disease designation in July 2019.

Upcoming LogicBio investor conference presentations:

- The 39th annual J.P. Morgan Global Virtual Healthcare Conference on Thursday, January 14, 2021 at 4:30 PM ET.
- The 2021 HC Wainwright BioConnect 2021 Conference, which will take place from January 10-14, 2021. Mr. Chereau's presentation will be available on demand to conference attendees for the duration of the event.

About LogicBio Therapeutics

LogicBio Therapeutics is dedicated to extending the reach of genetic medicine with pioneering 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 in the phase I/II SUNRISE clinical trial 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 have 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.

Forward-Looking Statements

This press release contains "forward-looking" statements within the meaning of the federal securities laws, including with respect to the Company's upcoming development milestones and opportunities, the collaboration's research efforts and the timing of patient enrollment in the phase I/II SUNRISE clinical trial. 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 March 16, 2020, the Company's Quarterly Report on Form 10-Q filed on May 11, 2020, and the Company's subsequent 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.

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