LogicBio Therapeutics Extends Sponsored Research Agreement with Oregon Health & Science University to Explore Translation of Pharmaceutically-Driven Selective Advantage for Future GeneRide™ Candidates

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CAMBRIDGE, Mass., Dec. 17, 2019 (GLOBE NEWSWIRE) -- LogicBio Therapeutics, Inc. (Nasdaq:LOGC), a genome editing company focused on developing medicines to durably treat rare diseases in pediatric patients, today announced it has entered into an exclusive license with Oregon Health & Science University ("OHSU") to intellectual property rights owned by OHSU while also extending a sponsored research agreement ("SRA") to explore methods for enhancing selective advantage of edited hepatocytes using pharmacological agents with the laboratory of Markus Grompe, M.D., professor at OHSU. 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 will focus on translating the enhancement strategy to non-human primates, a critical step before clinical translation to future GeneRide candidates and other technologies. GeneRide™ is LogicBio's proprietary promoterless, nuclease-free genome editing technology, which is designed to provide a stable therapeutic effect by harnessing homologous recombination to precisely integrate corrective genes into a patient's genome and leveraging endogenous promoters to drive gene expression.

LogicBio is currently working primarily in disorders where patients can benefit substantially even when only a modest percentage of their cells are modified and begin expressing the corrective transgene introduced by GeneRide. The Company has found, however, that in some genetic contexts, integrating the transgene gives hepatocytes a naturally-occurring selective advantage over cells that have not been modified. Over time, the percentage of modified cells expressing that transgene rises, potentially leading to more robust patient benefits. This was observed in an experiment in which a murine GeneRide construct was introduced into mice with and without a functioning copy of the *Mut* gene (deficient in the pediatric disease methylmalonic acidemia) in the liver. The initial GeneRide integration frequency was less than 1% in both sets of mice. Over time, this percentage remained stable in heterozygous mice that naturally express *Mut* in the liver (Mut+/- in liver). However, the share of cells expressing *Mut* increased to approximately 25% over more than a year in the mice genetically deficient in liver *Mut* (Mut-/- in liver). This selective advantage could be attributed to improvements in mitochondrial function as a result of *Mut* expression and restoration of the deficient essential metabolic pathway. These data were presented at the 2019 American Society of Gene & Cell Therapy Annual Meeting and can be found on the LogicBio website at the following link: https://investor.logicbio.com/events-and-presentations/presentations.

The goal of the expanded SRA with OHSU is to refine the pharmacological approach to providing a selective advantage to gene modified cells even when the transgene does not naturally confer a selection advantage at the cellular level. One such method involves adding an element to a GeneRide construct that gives cells incorporating that element a selective advantage when patients are treated with an external approved pharmacological agent. This research could enable expansion of the GeneRide platform to address genetic disorders in which clinical benefit emerges only after a higher percentage of cells are modified and begin expressing the corrective transgene.

"We are excited to explore novel methods for enriching the number of cells expressing the therapeutic gene," said Dr. Grompe. "Such methods could improve the likelihood that patients derive long-term therapeutic benefit from a single treatment. They could also expand the range of serious genetic disorders we can address with GeneRide."

Dr. Grompe's lab studies monogenic disorders, particularly metabolic liver diseases affecting children. He has focused extensively on the use of *in vivo* selection to enhance cell and gene therapies. Dr. Grompe received the E. Mead Johnson Award for research excellence from the Society for Pediatric Research in 2002. He retains an active clinical practice, focused on metabolic disease.

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.

Forward Looking Statements
This press release contains "forward-looking" statements within the meaning of the federal securities laws. 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.

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