LogicBio Therapeutics to Present Data on GeneRide™ Platform Technology at 2018 American Society of Gene & Cell Therapy Conference

May 14, 2018

Efficacy and durability of genome editing platform to be highlighted in presentations by NIH and others

Cambridge, Mass., May 14, 2018 – LogicBio Therapeutics, Inc., a genetic medicine company founded to develop safe medicines with lasting therapeutic benefit for children with life-threatening diseases, today announced it will be presenting data on the company's technology and robust preclinical pipeline at the annual meeting of the American Society of Gene & Cell Therapy (ASGCT) at the Hilton Chicago, from May 16-19, 2018.

The LogicBio team will present abstracts demonstrating the preclinical effectiveness of the company's platform technology, GeneRide™, in hemophilia B and methylmalonic acidemia (MMA)—a rare, life-threatening metabolic disease. GeneRide™ enables site-specific integration and lifelong expression of therapeutic transgenes, without the use of exogenous promoters or nucleases. LogicBio recently announced a development program targeting MMA.

In addition, LogicBio collaborators from the National Human Genome Research Institute (NHGRI) at the National Institutes of Health (NIH), Oregon Health and Science University (OHSU) and Stanford University will also highlight the preclinical effectiveness of the GeneRide™ platform in presentations during Saturday's oral abstract session. NIH scientist Randy Chandler will present data demonstrating the preclinical efficacy of GeneRide™ in mouse models of MMA; OHSU researcher Sean Nygaard will describe a novel approach for selecting gene edited hepatocytes; and Stanford researcher Gustavo De Alencastro will present data on a strategy to increase the efficiency of GeneRide™.

Details of LogicBio presentations:

Promoterless Targeting without Nucleases of Hyperactive Factor IX Corrects the Bleeding Diathesis in Hemophilia B Mice

Session: Gene Targeting & Gene Correction I
Abstract Number: 188
Time: Wednesday, May 16, 5:30 pm – 7:30 pm
Place: Stevens Salon C & D

Generide™, a Novel AAV Strategy to Treat Pediatric Patients with Methylmalonic Acidemia

Session: Gene Targeting & Gene Correction III
Abstract Number: 797
Time: Friday, May 18, 5:45 pm – 7:45 pm
Place: Stevens Salon C & D

Details of collaborator presentations:

Targeted Integration of MUT into the Albumin Locus Using a Promoterless AAV Vector (Generide™) Confers a Hepatocellular Growth Advantage in Mice with Methylmalonic Acidemia

Session: Oral Abstract Session 414: Liver Monogenic Diseases: Genome Editing, AAV Vectors, and Cell Therapy
Abstract Number: 980
Time: Saturday, May 19, 2018 10:15am
Place: Salon A-2

Efficient In Vivo Selection of Gene-Targeted Hepatocytes Using Acetaminophen-Induced Liver Toxicity

Session: Oral Abstract Session 410: Gene Targeting and Gene Correction
Abstract Number: 948
Time: Saturday, May 19, 2018 10:15am
Place: International Ballroom North

Improved Genome Editing through Inhibition of the FANCM Pathway

Session: Oral Abstract Session 410: Gene Targeting and Gene Correction
Abstract Number: 953
Time: Saturday, May 19, 2018 11:30am
Place: International Ballroom North

Self-Cleaving guideRNAs for Selective Expansion of Precisely Gene Edited Hepatocytes In Vivo

Session: Hematologic & Immunologic Diseases II
Abstract Number: 487
Time: Thursday, May 17, 2018 5:15 pm – 7:15 pm

Place: Stevens Salon C & D

All abstracts for the ASGCT meeting are available online at **2018 ASGCT Annual Meeting Abstracts**.

About LogicBio Therapeutics

LogicBio Therapeutics is a genetic medicine company, founded to deliver the benefits of genome editing to the fight against early onset childhood diseases. Launched in 2016 by pioneers in gene therapy from Stanford University and funded by leading life science investors, LogicBio is developing a pipeline of safe and durable treatments for pediatric indications. Our core technology, GeneRide™, enables the site-specific integration of a therapeutic transgene, in a nuclease-free and promotor-less approach by relying on the native process of homologous recombination to drive lifelong expression. With a world-class global team 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.

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