REGENXBIO'S NAV® Technology Demonstrates Correction of Cardiovascular Symptoms of MPS I

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- Results highlight potential for development of novel AAV-mediated gene therapy treatments for lysosomal storage diseases

WASHINGTON, DC September 29, 2014 – REGENXBIO Inc. today announced that gene transfer mediated by REGENXBIO's NAV® AAV8 vectors resulted in sustained serum α-L-iduronidase (IDUA) expression, as well as correction of systemic features of MPS I, or Hurler syndrome, a lysosomal storage disease (LSD) caused by the body's inability to produce the IDUA enzyme. Data from a study performed by researchers at the Perelman School of Medicine at the University of Pennsylvania ("Penn") show animals treated with a single intravenous injection of NAV AAV8 vectors expressing the IDUA gene not only demonstrated meaningful improvements in the biochemical features of MPS I in most tissues, but the majority also exhibited complete resolution of aortic valve lesions. This effect is significant since it has not been previously observed in MPS I patients treated with current therapies or animal models. The study, which was funded in part by a grant from REGENXBIO, has been published online in *Proceedings of the National Academy of Sciences of the United States of America* (PNAS).

"This study by our collaborators at Penn demonstrates the potential of NAV Technology-based treatments to address cardiovascular symptoms of LSDs that are not well-managed with existing standards of care, which consists of hematopoietic stem cell transplantation or weekly enzymereplacement therapy," said Ken Mills, President and CEO of REGENXBIO. "While REGENXBIO's current development programs focus on a profile for treating the central nervous system manifestations of Hurler syndrome (MPS I) and Hunter syndrome (MPS II), our research interests are rooted in a broad commitment to improve patient outcomes for all features of these and other LSDs."

James M. Wilson, MD, PhD, professor and director of the Gene Therapy Program in the Department of Pathology and Laboratory Medicine at Penn, added, "Our research underscores the prospect for AAV mediated gene therapy to be further developed as a potentially safe and effective treatment for MPSI, as well as other LSDs that require lifelong systemic enzyme replacement."

The study, titled "Liver directed gene therapy corrects cardiovascular lesions in felinemucopolysaccharidosis type I," is available online at: http://www.pnas.org/content/early/2014/09/24/1413645111.abstract

Editor's Note

J.M. Wilson is an advisor to REGENXBIO, and is a founder of, holds equity in, and receives grants from REGENXBIO; REGENXBIO holds license and option rights to technologies developed by Dr. Wilson at the University of Pennsylvania; in addition, Dr. Wilson is a founder, advisor and consultant to several other biopharmaceutical companies and is an inventor on patents licensed to various biopharmaceutical companies, including REGENXBIO.

About REGENXBIO

REGENXBIO Inc. is the leading next-generation AAV gene therapy company, developing a new class of personalized therapies based on its proprietary NAV® Technology platform for a range of severe diseases with serious unmet needs. NAV Technology includes novel AAV vectors AAV7, AAV8, AAV9, and AAVrh10. The company is developing gene therapy treatments to address lysosomal storage disorders and ocular diseases. REGENXBIO has enabled leading global partners including Baxter Healthcare, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, AveXis, AAVLife and Voyager Therapeutics to use its NAV Technology. In addition, together with Fidelity Biosciences, REGENXBIO formed Dimension Therapeutics, a company focused on the development and commercialization of NAV-based gene therapies for rare diseases.

For more information about REGENXBIO, please visit www.regenxbio.com.

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