

Study Using NAV Vector Highlights Potential for Gene Therapy To Treat Diseases That Affect The CNS

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- Results support REGENX's plans to advance gene therapies for MPS I and MPS II into human clinical trials

WASHINGTON, DC August 27, 2014 – REGENX Biosciences, LLC today announced that gene transfer mediated by REGENX's NAV® AAV9 vector can be accomplished in vivo and globally correct central nervous system (CNS) manifestations of MPS I, a lysosomal storage disease caused by the body's inability to produce the α -L-iduronidase (IDUA) enzyme. Data from a study performed by researchers at the Perelman School of Medicine at the University of Pennsylvania ("Penn") show the production of stable levels of IDUA that are clinically meaningful after a single injection of AAV9 expressing the IDUA gene, restoring to normal the histological and biochemical features of MPS I in the CNS. The study, which was funded in part by a grant from REGENX, has been published online in *Molecular Therapy*.

"We are encouraged by the results of this study and have been using the approach in REGENX's active development programs investigating gene therapies for the treatment of MPS I and MPS II, also known as Hurler syndrome and Hunter syndrome, respectively," said Ken Mills, President and CEO of REGENX. "We anticipate presenting the safety and efficacy that have been demonstrated in this study when we meet with the U.S. Food and Drug Administration later this year to discuss the progression of our gene therapy treatment candidates into human clinical trials. We believe the approach used in this study might also serve as a broad platform for the treatment of other lysosomal storage diseases that affect the CNS."

James M. Wilson, MD, PhD, professor and director of the Gene Therapy Program in the Department of Pathology and Laboratory Medicine at Penn, added, "This first demonstration of the efficacy of intrathecal gene therapy in a large animal model of a lysosomal storage disease will help pave the way for gene therapies, such as the NAV-based gene delivery treatments REGENX is developing."

The study, titled "Intrathecal Gene Therapy Corrects CNS Pathology in a Feline Model of Mucopolysaccharidosis I," is available online at: <http://www.nature.com/mt/index.html>.

Editor's Note

J.M. Wilson is an advisor to REGENX Biosciences and Dimension Therapeutics, and is a founder of, holds equity in, and receives grants from REGENX Biosciences and Dimension Therapeutics; in addition, he is a founder, advisor and consultant to several other biopharmaceutical companies and is an inventor on patents licensed to various biopharmaceutical companies.

About REGENX Biosciences

REGENX Biosciences is the leading next-generation AAV gene therapy company, developing a new class of personalized therapies based on its proprietary NAV® vector technology platform for a range of severe diseases with serious unmet needs. NAV vector technology includes novel AAV vectors rAAV7, rAAV8, rAAV9, and rAAVrh10. The company is developing gene therapy treatments to address lysosomal storage disorders and ocular diseases. REGENX 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, REGENX formed Dimension Therapeutics, a company focused on the development and commercialization of NAV-based gene therapies for rare diseases.

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

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Contact:

REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio.com

REGENX Biosciences (Media)
Annie Starr, 973-415-8838
astarr@6degreespr.com