REGENXBIO Publishes Data from Ongoing Preclinical Studies of NAV® Gene Therapy in Neurodegenerative Diseases

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- *Preclinical study of RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I) demonstrates disease correction from a single administration of NAV AAV9 in a canine model; expected to help establish minimum effective dose in humans*
- *Preclinical study of RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) demonstrates disease correction from a single administration of NAV AAV9 in a mouse model*
- *Data support REGENXBIO's efforts to advance a broad platform of NAV gene therapies to address neurodegenerative diseases*

ROCKVILLE, Md., Sept. 13, 2016 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (Nasdaq:RGNX), a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy based on its proprietary NAV® Technology Platform, today provided an update on the recent publication of data from ongoing preclinical studies of NAV Gene Therapy for the treatment of Mucopolysaccharidosis Type I (MPS I) and Mucopolysaccharidosis Type II (MPS II).

"We are pleased to share additional positive preclinical results, which were generated by our development partners at the University of Pennsylvania, on the potential of our NAV Technology Platform in MPS I and MPS II," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "These results support our long-term clinical development plans and our mission of utilizing NAV gene therapies to improve the lives of patients suffering from severe neurodegenerative diseases."

RGX-111 for the treatment of MPS I

Data from a preclinical study of RGX-111 for the treatment of MPS I in a canine model were published online in July 2016 in the journal *Molecular Genetics and Metabolism.* RGX-111 uses the NAV AAV9 vector to deliver the human α-l-iduronidase (IDUA) gene to the central nervous system (CNS).

The results demonstrate dose-dependent expression of IDUA and correction of disease pathology in the brain, as well as reduction in spinal cord compression after a single administration of NAV AAV9 vectors expressing IDUA. These data are expected to help establish the minimum effective dose for REGENXBIO's planned first-in-human studies.

The study, titled "Neonatal tolerance induction enables accurate evaluation of gene therapy for MPS I in a canine model," is available online at: http://www.mgmjournal.com/article/S1096-7192(16)30105-6/abstract.

REGENXBIO expects to submit an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) and a Clinical Trial Application (CTA) with Health Canada for a Phase I/II clinical trial of RGX-111 in the first half of 2017. RGX-111 has been granted both orphan drug designation and rare pediatric disease designation by the FDA.

RGX-121 for the treatment of MPS II

Data from a preclinical study of RGX-121 for the treatment of MPS II in a mouse model were published online in August 2016 in the journal *Human Gene Therapy*. RGX-121 uses the NAV AAV9 vector to deliver the human iduronate-2-sulfatase (IDS) gene to the CNS.

The results demonstrate dose-dependent expression of IDS and correction of disease pathology in the brain after a single administration of NAV AAV9 vectors expressing IDS. Levels of IDS in the brain tissue, cerebrospinal fluid (CSF), and serum all approached or exceeded normal levels. The treated mice also demonstrated improvement in long-term memory in a novel object recognition test, as well as evidence of correction of disease in the liver and heart.

The study, titled "Delivery of an adeno-associated virus vector into CSF attenuates central nervous system disease in mucopolysaccharidosis type II mice," is available online at: http://online.liebertpub.com/doi/abs/10.1089/hum.2016.101.

REGENXBIO expects to submit an IND to the FDA for a Phase I/II clinical trial of RGX-121 in the first half of 2017. RGX-121 has been granted both orphan drug designation and rare pediatric disease designation by the FDA.

About REGENXBIO

REGENXBIO is a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. REGENXBIO's NAV® Technology Platform, a proprietary AAV gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO's mission is to transform the lives of patients suffering from severe diseases with significant unmet medical need by developing and commercializing *in vivo* gene therapy products based on REGENXBIO's NAV Technology Platform. REGENXBIO seeks to accomplish this mission through a combination of internal development efforts and third-party NAV Technology Platform licensees. As of June 30, 2016, REGENXBIO's NAV Technology Platform was being applied in the development of 29 product candidates for a variety of diseases, including five internally developed candidates and 24 partnered candidates developed by REGENXBIO's

licensees.

Forward Looking Statements

This press release contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995, regarding, among other things, REGENXBIO's research, development and regulatory plans for its gene therapy treatments, including RGX-111 and RGX-121. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could cause actual results to differ materially from those projected by such forward-looking statements. All of REGENXBIO's development timelines could be subject to adjustment depending on recruitment rate, regulatory agency review and other factors that could delay the initiation and completion of clinical trials. Meaningful factors which could cause actual results to differ include, but are not limited to, the timing of enrollment, commencement and completion of REGENXBIO's clinical trials; the timing and success of preclinical studies and clinical trials conducted by REGENXBIO, its development partners and its NAV® Technology Licensees; the ability to obtain and maintain regulatory approval to conduct clinical trials and to commercialize REGENXBIO's product candidates, and the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing REGENXBIO's product candidates; REGENXBIO's ability to obtain and maintain intellectual property protection for our product candidates and technology; trends and challenges in REGENXBIO's business and the markets in which REGENXBIO operates; REGENXBIO's ability to attract or retain key personnel; the size and growth of the potential markets for REGENXBIO's product candidates and the ability to serve those markets; the rate and degree of market acceptance of any of REGENXBIO's product candidates; REGENXBIO's ability to establish and maintain development partnerships, including those with NAV Technology Licensees; REGENXBIO's expenses and revenue, the sufficiency of REGENXBIO's cash resources and needs for additional financing, regulatory developments in the United States and foreign countries, as well as other factors discussed in the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2015 and Quarterly Report on Form 10-Q for the quarter ended June 30, 2016, which are available on the SEC's website at www.sec.gov. In addition to the risks described above and in REGENXBIO's filings with the SEC, other unknown or unpredictable factors also could affect REGENXBIO's results. There can be no assurance that the actual results or developments anticipated by REGENXBIO will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, REGENXBIO. Therefore, no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

All forward-looking statements contained in this press release are expressly qualified by the cautionary statements contained or referred to herein. REGENXBIO cautions investors not to rely too heavily on the forward-looking statements REGENXBIO makes or that are made on its behalf. These forward-looking statements speak only as of the date of this press release (unless another date is indicated). REGENXBIO undertakes no obligation, and specifically declines any obligation, to publicly update or revise any such forward-looking statements, whether as a result of new information, future events or otherwise.

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