Preclinical Data from REGENXBIO's RGX-314 and RGX-121 Gene Therapy Programs to be Presented at the American Society of Gene & Cell Therapy 19th Annual Meeting

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- *High expression of anti-VEGF antibody achieved in rhesus macaques with administration of RGX-314 for treatment of wet age-related macular degeneration*
- *Gene transfer mediated by RGX-121 shown to globally correct central nervous system manifestations of mucopolysaccharidosis Type II in animal models*
- *Data support FDA discussions on advancement of RGX-314 and RGX-121 into human clinical trials*

ROCKVILLE, Md., April 19, 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 announced that preclinical data from studies supported by REGENXBIO at the Perelman School of Medicine at the University of Pennsylvania will be presented in two poster sessions at the American Society of Gene & Cell Therapy (ASGCT) 19th Annual Meeting, to be held May 4 to May 7 at the Marriott Wardman Park in Washington, D.C. These data highlight the use of REGENXBIO's investigational gene therapies RGX-314, for the treatment of wet age-related macular degeneration (wet AMD), and RGX-121, for the treatment of mucopolysaccharidosis Type II (MPS II), in preclinical animal models.

"RGX-314 has the potential to be a one-time treatment for people with wet AMD by delivering the highest expression of anti-VEGF antibodies through the use of our NAV AAV8 vector. The concentrations achieved in these studies demonstrate that RGX-314 could supplant the need for repeated intraocular injections associated with the existing standard of care," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "In addition, a single administration of RGX-121 provided broadly distributed and sustained enzyme delivery to the central nervous system in animals with MPS II. The NAV AAV9 vector and route of administration used in RGX-121 is identical to our RGX-111 program for the treatment of MPS I, and indicates the potential of our broad platform for the treatment of other diseases that affect the central nervous system. These data also support our discussions with the FDA and our plans to file IND applications for RGX-314 in the second half of 2016 and for RGX-121 in the first half of 2017."

Details of the posters to be presented are as follows:

Title: Therapeutic Gene Transfer as a Treatment Option for Age-Related Macular Degeneration
Session date / time: Wednesday, May 4, 5:30 – 7:30 p.m. EDT
Session title: Gene Therapy for Neurosensory Diseases
Room: Exhibit Hall C & B South
Abstract number: 189
Authors: Erik Wielechowski[1], Angelica Medina-Jaszek[1], Omua Ahonkhai[1], Muhammad Arif[1], Jean Bennett[2], Albert Maguire[2], Anna Tretiakova[1], James M. Wilson[1] [1]Gene Therapy Program, Department of Medicine, University of Pennsylvania, Philadelphia, PA, [2]Department of Ophthalmology, University of Pennsylvania, Philadelphia, PA

Title: AAV9 Delivery into Cerebrospinal Fluid Corrects CNS Disease in a Murine Model of Mucopolysaccharidosis Type II
Session date / time: Thursday, May 5, 6 – 8 p.m. EDT
Session title: Diabetes, Metabolic and Genetic Diseases II
Room: Exhibit Hall C & B South
Abstract number: 346
Authors: Christian Hinderer[1], Nathan Katz[1], Jean-Pierre Louboutin[2], Peter Bell[1], Hongwei Yu[1], Mohamad Naval[1], Karen Kozarsky[3], Timothy O'Brien [4], Tamara Goode[1], James M. Wilson[1] [1]Gene Therapy Program, Department of Medicine, University of Pennsylvania, Philadelphia, PA, [2]Section of Anatomy, Department of Basic Medical Sciences, University of West Indies, Kingston, Jamaica, [3]REGENXBIO, Rockville, MD, [4]Department of Neuroscience, University of Pennsylvania, Philadelphia, PA

Additional information on the meeting can be found on the ASGCT website: http://www.asgct.org.

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 December 31, 2015, REGENXBIO's NAV Technology Platform is being applied in the development of 28 product candidates for a variety of diseases, including five internally developed candidates and 23 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 RGX-121, RGX-314, RGX-111 and other gene therapies. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual results to differ materially from those projected in its forward-looking statements. Meaningful factors which could cause actual results to differ include, but are not limited to, the ability to obtain and maintain regulatory approval of 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; REGENXBIO's ability to establish and maintain development partnerships; REGENXBIO's expectations regarding federal, state and foreign regulatory requirements; 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, which is available on the SEC's website at www.sec.gov. In addition to the risks described above and in the Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other 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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