Preclinical Data from REGENXBIO RGX-314 Gene Therapy Program for Wet Age-Related Macular Degeneration to be Presented at Upcoming Conferences

May 4, 2017 8:07 PM EDT

- *Preclinical data for RGX-314 program for the treatment of wet AMD to be presented at the Retinal Cell and Gene Therapy Innovation Summit, the Association for Research in Vision and Ophthalmology, and the American Society for Gene and Cell Therapy*
- *Data support the active IND for RGX-314 Phase I clinical trial*

ROCKVILLE, Md., May 04, 2017 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (Nasdaq:RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced that preclinical data from studies supported by REGENXBIO at the University of Pennsylvania's Gene Therapy Program and Center for Advanced Retinal and Ocular Therapeutics and at the Johns Hopkins Wilmer Eye Institute will be shared in one presentation and four posters at upcoming conferences including the Retinal Cell and Gene Therapy Innovation Summit, the Association for Research in Vision and Ophthalmology (ARVO), and the American Society of Gene and Cell Therapy (ASGCT). These data support further clinical research regarding the use of REGENXBIO's investigational gene therapy RGX-314 for the treatment of wet age-related macular degeneration (wet AMD).

"RGX-314 has the potential to be a one-time treatment for people with wet AMD by delivering high expression of anti-VEGF antibodies through the use of our NAV AAV8 vector. We are pleased to share additional positive preclinical results, which were generated by our development partners at the University of Pennsylvania and Johns Hopkins, which support our active IND," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "REGENXBIO is on track to begin enrollment in the RGX-314 Phase I clinical trial by mid-2017 and to provide an interim trial update by the end of 2017."

Details of the upcoming presentation and posters are as follows:

Presentation at Retinal Cell and Gene Therapy Innovation Summit

Title: Preclinical gene therapy studies to select RGX-314 doses to treat wet age-related macular degeneration
Presenter: Jean Bennett, PhD, Department of Ophthalmology, University of Pennsylvania, Philadelphia, PA
Session date/time: Friday, May 5, 9:20 a.m. – 9:30 a.m. EDT
Session title: Gene Therapy, Outcome Measures, and Novel Therapies, Session 1: Preclinical Aspects — Vector Design/Animal Models
Room: Holiday 1-3, Hilton Baltimore, Baltimore, MD

Posters at Association for Research in Vision and Ophthalmology

Title: RGX-314, an AAV8 expressing an anti-VEGF protein, strongly suppresses subretinal neovascularization and vascular leakage in mouse models
Authors: Ji-kui Shen[1], Yuanyuan Liu[1], Seth D. Fortmann[1], Stephen Yoo[3], Karen Kozarsky[2], Jiangxia Wang[1], Peter A. Campochiaro[1]. [1]Ophthalmology, Johns Hopkins Wilmer Eye Inst, Baltimore, Maryland, United States; [3]REGENXBIO Inc, Rockville, Maryland, United States
Session date/time: Sunday, May 7, 8:30 a.m. – 10:15 a.m. EDT
Session title: Cytokines; Growth factors; Antiangiogenic drugs
Room: Exhibit/Poster Hall, Baltimore Convention Center, Baltimore, MD
Abstract number: B0230

Title: Subretinal delivery of RGX-314 AAV8-anti-VEGF Fab gene therapy in NHP
Authors: Anna Tretiakova[1], Tomas S. Aleman[3], Arkady Lyubarsky[3], Elaine J. Zhou[4], Erik Wielechowski[1], Gui-Shuang Ying[2], Erin Bote[1], Leah Makaron[1], Stephen Yoo[5], Jean Bennett[3,6], Albert M. Maguire[3,6], James Wilson[1]. [1]Gene Therapy Program, Department of Medicine, University of Pennsylvania, Philadelphia, Pennsylvania, United States; [2]Center for Preventative Ophthalmology and Biostatistics, University of Pennsylvania, Philadelphia, Pennsylvania, United States; [3]Center for Advanced Retinal and Ocular Therapeutics, Scheie Eye Institute, University of Pennsylvania, Philadelphia, Pennsylvania, United States; [4]Perelman School of Medicine, University of Pennsylvania, Philadelphia, Pennsylvania, United States; [5]REGENXBIO, Rockville, Maryland, United States; [6]Center for Cellular and Molecular Therapeutics, The Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States
Session date/times: Wednesday, May 10, 11:00 a.m. – 12:45 p.m. EDT
Session title: Gene editing and gene therapies
Room: Exhibit/Poster Hall, Baltimore Convention Center, Baltimore, MD
Abstract number: B0164

Title: Normal parameters of the full field ERG recorded with bipolar electrodes in Cynomolgus Macaque (*Macaque fascicularis*)
Authors: Arkady Lyubarsky[1,2], Erik Wielechowski[3], Tomas S. Aleman[4], Albert M. Maguire[1,4], Gui-Shuang Ying[4], Erin Bote[3], Leah Makaron[3], James Wilson[3], Jean Bennett[1,4], Anna P. Tretiakova[3]. [1]Center for Advanced Retinal and Ophthalmic Therapeutics, SOM Univ. of Pennsylvania, Philadelphia, Pennsylvania, United States; [2]Vision Research Center, University of Pennsylvania, Philadelphia, Pennsylvania, United States; [3]Gene Therapy Program, University of Pennsylvania SOM, Philadelphia, Pennsylvania, United States; [4]Scheie Eye Institute, University of Pennsylvania SOM

Ophthalmology, Philadelphia, Pennsylvania, United States
Session date/times: Thursday, May 11, 8:30 a.m. – 10:15 a.m. EDT
Session title: Retinal Function – ERG studies
Room: Exhibit/Poster Hall, Baltimore Convention Center, Baltimore, MD
Abstract number: B0441

Additional information on the meeting can be found on the ARVO website: http://www.arvo.org

Poster at American Society of Gene and Cell Therapy

Title: Safety of RGX-314 AAV8-anti-VEGF Fab Gene Therapy in NHP Following Subretinal Delivery
Authors: Tomas S. Aleman[1], Anna P. Tretiakova[2], Arkady L. Lyubarsky[1], Jessica I. W. Morgan[2], Elaine J. Zhou[3], Erik Wielechowski[2], Gui-Shuang Ying[4], Erin Bote[2], Leah Makaron[2], Stephen Yoo[5], Jean Bennett[1], Albert M. Maguire[1], James M. Wilson[2]. [1]Center for Advanced Retinal and Ocular Therapeutics, Scheie Eye Institute, University of Pennsylvania, Philadelphia, PA,[2]Gene Therapy Program, Department of Medicine, University of Pennsylvania, Philadelphia, PA,[3]Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA,[4]Center for Preventative Ophthalmology and Biostatistics, University of Pennsylvania, Philadelphia, PA,[5]REGENXBIO, Rockville, MD.
Session date/times: Thursday, May 11, 5:15 p.m. – 7:15 p.m. EDT
Session title: Neurologic Diseases (including Ophthalmic and Auditory Diseases) II
Room: Exhibit Hall A & B South, Marriot Wardham Park Hotel, Washington, DC
Abstract number: 427

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

Note Regarding Penn

Penn has licensed certain Penn-owned AAV intellectual property to REGENXBIO, including rights related to RGX-314. Dr. Wilson is an advisor to REGENXBIO and is a founder of, holds equity in, and receives sponsored research support from REGENXBIO.

About REGENXBIO

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV ® Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Licensees are applying the NAV Technology Platform in the development of a broad pipeline of product candidates in multiple therapeutic areas.

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 in connection with its NAV Technology Platform and gene therapy treatments. 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 and its development partners; 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 REGENXBIO's product candidates and technology; REGENXBIO's growth strategies; REGENXBIO's competition; 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; REGENXBIO's expenses and revenue; regulatory developments in the United States and foreign countries; the sufficiency of REGENXBIO's cash resources and needs for additional financing; and 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, 2016. In addition to the risks described above and in REGENXBIO's filings with the Securities and Exchange Commission, 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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