REGENXBIO Provides Year-End 2015 Corporate Update

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- *Company begins 2016 in strong financial position, on track to advance multiple lead gene therapy product candidate programs into clinic*
- *RGX-501 IND for treatment of homozygous familial hypercholesterolemia is active*
- *Five new NAV Vector licenses granted in the fourth quarter of 2015*

ROCKVILLE, Md., Jan. 07, 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 a year-end 2015 corporate update.

"We enter 2016 with great momentum and a strong balance sheet that will enable us to continue to advance our clinical and corporate objectives," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "As of December 31, 2015, our lead product candidate programs remain on track and are expected to enter clinical phase, and we've expanded the breadth and value of our NAV Technology Platform with the sublicensing of new programs. We've also grown our team in support of REGENXBIO's mission of developing and commercializing gene therapy products that transform the lives of patients suffering from severe diseases."

Year-End 2015 Highlights

Recent highlights related to REGENXBIO's internal lead product candidate programs and the sublicensing of its NAV Technology Platform include:

- An Investigational New Drug (IND) application to support a Phase I/II clinical trial to evaluate the effect of RGX-501 in the treatment of homozygous familial hypercholesterolemia (HoFH) is active. The IND was filed with the U.S. Food and Drug Administration (FDA) by the University of Pennsylvania, REGENXBIO's development partner.
- Orphan drug designation and rare pediatric disease designation were granted by the FDA to RGX-111 for the treatment of mucopolysaccharidosis Type I (MPS I).
- REGENXBIO granted five new commercial sublicenses, outside of REGENXBIO's internal core disease and therapeutic areas, for the development of gene therapy products based on its NAV Technology Platform in the quarter ended December 31, 2015.
    - Sublicense agreements with Audentes Therapeutics in two disease indications: Crigler-Najjar and Catecholaminergic polymorphic ventricular tachycardia (CPVT).
    - A sublicense agreement with Annapurna Therapeutics in two disease indications: Alpha-1 anti-trypsin (A1AT) deficiency and Allergy.
    - A sublicense with an existing NAV Technology Licensee for an additional disease indication.
- As of December 31, 2015, REGENXBIO's NAV Technology Platform was being applied in 23 partnered product candidates developed by third-party licensees.

Anticipated 2016 Milestones

Anticipated milestones related to the clinical development of REGENXBIO's internal lead product candidate programs include:

- A Phase I/II clinical trial evaluating RGX-501 for the treatment of HoFH is expected to be initiated during the first half of 2016.
- An IND application for RGX-111 for the treatment of MPS I is expected to be filed with the FDA

in the first half of 2016. The initiation of a Phase I/II clinical trial is planned for the second half of 2016.
- An IND application for RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) is expected to be filed with the FDA during the second half of 2016.

2016 Financial Guidance

As of December 31, 2015, REGENXBIO had more than $210 million in cash, cash equivalents and marketable securities. In 2016, REGENXBIO currently expects its cash burn to be between $60 million and $70 million, which will support the development of its lead product candidate programs and the anticipated growth of its internal pipeline.

Management Team Appointments

As of December 31, 2015, REGENXBIO had 37 full-time employees, including three recent management team appointments dedicated to the execution of REGENXBIO's lead programs: Mari Maurer, Vice President of Clinical Operations; Lynne Fahey McGrath, M.P.H., Ph.D., Vice President of Regulatory Affairs; and Jay Vora, Ph.D., Vice President of Program Management.

- Mari Maurer, Vice President of Clinical Operations
  - More than 28 years of biopharmaceutical experience, with 23 years in clinical operations and 10 years in rare and ultra-rare genetic disorders.
  - Previous positions include Senior Director, Clinical Operations at Ultragenyx Pharmaceutical and Director, Clinical Operations at BioMarin Pharmaceutical.
- Lynne Fahey McGrath, M.P.H., Ph.D., Vice President of Regulatory Affairs
  - More than 30 years of experience in regulatory affairs and pharmaceutical product development across a range of therapeutic categories; has directed worldwide approvals of more than 50 new drugs and indications.
  - Previous positions include Vice President, Global Head of Regulatory Affairs at Novartis Consumer Health and U.S. Head of Regulatory Affairs at Novartis Oncology.
- Jay Vora, Ph.D., Vice President of Program Management
  - More than 18 years of experience in the development and implementation of complex business strategies within the life sciences industry.
  - Previous positions include Executive Director, Product Development at BioMarin Pharmaceutical and Principal at PRTM Management Consultants.

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 expected cash burn for 2016, REGENXBIO's research, development and regulatory plans for RGX-111, RGX-314, RGX-501 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 prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission (SEC) on September 17, 2015 (the Prospectus) and Quarterly Report on Form 10-Q for the quarter ended September 30, 2015, each available on the SEC's website at www.sec.gov. Additional factors may be described in those sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2015, to be filed with the SEC in the first quarter of 2016. In addition to the risks described above and in the Prospectus, 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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