REGENXBIO Provides Year-End 2016 Corporate Update

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- *Filed IND for RGX-314 Phase I clinical trial for wet AMD*
- *Announced initiation of protocol changes for RGX-501 Phase I/II clinical trial for HoFH; patient enrollment is now projected to begin first half 2017*
- *Anticipate enrolling patients in clinical trials for three lead programs and filing an additional IND in 2017*
- *Enhanced advanced manufacturing and analytics infrastructure and capabilities*
- *Ended 2016 with greater than $155 million in cash, cash equivalents and marketable securities*

ROCKVILLE, Md., Jan. 06, 2017 (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 2016 corporate update.

"In 2016, REGENXBIO saw substantial progress in meeting the development goals for our lead product candidate programs and building our core organization; we expect this to continue in 2017 as we work toward generating a robust clinical pipeline of product candidates that hold the potential to improve treatment in many diseases. This year we anticipate clinical data reporting from programs using our NAV Technology, including our trials of RGX-314 for the treatment of wet age-related macular degeneration, RGX-501 for the treatment of homozygous familial hypercholesterolemia, and trials from our licensees AveXis, Dimension and Audentes," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "In addition, we expect several new INDs for NAV Technology programs to be filed in 2017, further expanding the clinical pipeline of NAV-based gene therapies that have the potential to benefit patients in need."

Year-End 2016 Highlights

Recent highlights related to REGENXBIO's internal lead product candidate programs include:

- REGENXBIO filed an Investigational New Drug (IND) application with the U.S. Food and Drug Administration (FDA) during the first week of 2017 to support the Phase I clinical trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD). IND-enabling studies for RGX-314 and the manufacture of material to support initiation of the Phase I clinical trial were completed in 2016.
- REGENXBIO and trial sponsor the University of Pennsylvania had commenced screening for patients with homozygous familial hypercholesterolemia (HoFH) for inclusion in a Phase I/II clinical trial of RGX-501. REGENXBIO today announced that the sponsor has initiated changes to the protocol intended to broaden the eligible and available patient population. As a result of these changes, which are expected to facilitate enrollment in this trial as well as future trials for RGX-501, the Phase I/II clinical trial is now projected to begin enrolling in the first half of 2017. REGENXBIO plans to provide further updates during the first quarter of 2017.
- IND-enabling studies for RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I) are nearing completion, and the manufacture of material to support initiation of a Phase I/II clinical trial has been completed.
- IND-enabling studies for RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) are expected to be completed in the second quarter of 2017. REGENXBIO also initiated the manufacture of material to support the planned Phase I/II clinical trial.
- REGENXBIO invested in internal capabilities, expanded its contract manufacturing network and opened an advanced manufacturing and analytics lab.

Anticipated 2017 Milestones

REGENXBIO expects to meet the following anticipated milestones related to the clinical development of internal lead product candidate programs:

- RGX-314 for the treatment of wet AMD
  - Begin enrollment in the Phase I clinical trial in mid-2017;
  - Present interim update from the Phase I clinical trial in late 2017.
- RGX-501 for the treatment of HoFH
  - Begin enrollment in the Phase I/II clinical trial in the first half of 2017;
  - Present interim data from the Phase I/II clinical trial in late 2017.
- RGX-111 for the treatment of MPS I
  - File an IND application with the FDA in the first half of 2017;
  - Begin enrollment in a Phase I/II clinical trial in the second half of 2017.
- RGX-121 for the treatment of MPS II
  - File an IND application with the FDA in mid-2017;
  - Complete the manufacture of material by the end of 2017 to support initiation of a Phase I/II clinical trial.

NAV Technology Licensee Program Highlights

As of December 31, 2016, REGENXBIO's NAV Technology Platform was being applied in more than 20 partnered product candidates developed by nine NAV Technology Platform Licensees (NAV Licensees). There were a number of NAV Licensee advancements announced in 2016, which included:

Clinical Development of NAV Technology Platform

- AVXS-101 (AveXis, Inc.), which uses the NAV AAV9 vector for the treatment of spinal muscular atrophy Type I:
  - Positive interim data from the Phase I clinical trial in 2016, and the initiation of the pivotal study expected in the first half of 2017.
- DTX101 (Dimension Therapeutics, Inc.), which uses the NAV AAVrh10 vector for the treatment of hemophilia B:
  - Initiation of the Phase I/II clinical trial in 2016, and interim data expected in January 2017.
- AT132 (Audentes Therapeutics, Inc.), which uses the NAV AAV8 vector for the treatment of X-Linked Myotubular Myopathy:
  - Initiation of a Phase I/II clinical trial in 2017, and preliminary data expected by the end of 2017.

Expansion of NAV Technology Platform

- Biogen Inc. became REGENXBIO's ninth NAV Licensee and is expected to use NAV vectors to develop gene therapy product candidates in the treatment of achromatopsia and choroideremia, two rare genetic vision disorders.
- Voyager Therapeutics, Inc. exercised commercial options for the use of NAV vectors for the development and commercialization of gene therapies for specific neurological diseases.

2017 Financial Guidance

As of December 31, 2016, REGENXBIO had more than $155 million in cash, cash equivalents and marketable securities, completing 2016 within the previously updated cash burn guidance range of between $55 million and $60 million. REGENXBIO expects full-year 2017 cash burn to be between $75 million and $85 million, which will support the continued development of its lead product candidate programs.

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. REGENXBIO and its licensees are applying the NAV Technology Platform in the development of a broad pipeline of 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 NAV Technology Platform, REGENXBIO’s collaborations with its NAV Technology Licensees, REGENXBIO’s financial guidance, REGENXBIO’s research, development and regulatory plans for RGX-111, RGX-121, RGX-314, RGX-501 and other gene therapies and the research, development and regulatory plans of REGENXBIO’s NAV Technology Licensees. 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 REGENXBIO’s 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 September 30, 2016, which are available on the SEC’s website at www.sec.gov. Additional factors may be set forth in those sections of REGENXBIO’s Annual Report on Form 10-K for the year ended December 31, 2016, to be filed in the first quarter of 2017. 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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