REGENXBIO Honors Rare Disease Day® 2017

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ROCKVILLE, Md., Feb. 28, 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, joins the global rare disease community today in honoring Rare Disease Day® 2017, dedicated to raising awareness of rare diseases and their impact on the lives of patients and their families. This year's theme, "Research," recognizes the importance that scientific research plays in identifying and increasing understanding of unknown diseases, enabling doctors to make correct diagnoses and generating the development of treatments and potentially of cures.

"REGENXBIO is proud to continue our support of Rare Disease Day to raise awareness of the importance of research and therapeutic development in rare diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "REGENXBIO is dedicated to advancing a broad pipeline of NAV-based gene therapies to address a wide range of rare diseases with high unmet need. There are currently over 20 development programs based on NAV Technology including our internal development efforts and the work of our NAV Technology Licensees."

In recognition of Rare Disease Day, REGENXBIO will participate in activities in Washington, D.C., and will host a company-wide discussion at its Rockville, Maryland, headquarters with people affected by Mucopolysaccharidosis Type I (MPS I) and Mucopolysaccharidosis Type II (MPS II), two rare diseases for which NAV-based gene therapies are in development.

"Partnering with the communities that we serve is crucial to the development of innovative gene therapies that address the needs of individuals and their families," said Vivian Fernandez, Director of Patient Advocacy at REGENXBIO. "We are actively engaged with a number of patient advocacy organizations including the FH Foundation and the National MPS Society, and we look forward to continuing to work closely with them as our lead gene therapy programs advance toward the clinic."

REGENXBIO has received orphan drug designations from the United States Food and Drug Administration (FDA) for:

- RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH);
- RGX-111 for the treatment of MPS I; and
- RGX-121 for the treatment of MPS II.

The FDA has also granted rare pediatric disease designation to the RGX-111 and RGX-121 programs.

For more information on REGENXBIO's rare disease programs, please visit REGENXBIO's website at www.regenxbio.com. To sign up for updates, please visit the Patients & Families section of REGENXBIO's website.

About Rare Disease Day

Rare Disease Day takes place every year on the last day of February to raise awareness among the general public and decision-makers about rare diseases and their impact on patients' lives. Each year, organizations around the world hold a variety of activities that highlight rare and genetic diseases. Rare Disease Day was established in Europe in 2008 by the European Organization for Rare Diseases (EURORDIS), the organization representing the rare disease community in Europe, and is now observed in more than 80 nations. Rare Disease Day is sponsored in the United States by the National Organization for Rare Disorders (NORD), a leading independent, non-profit organization committed to the identification, treatment and cure of rare diseases.

For additional information on Rare Disease Day in the United States, please visit: www.rarediseaseday.us. For global activities, please visit: www.rarediseaseday.org.

About REGENXBIO Inc.

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 research, development and regulatory plans for RGX-111, RGX-121, 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 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 to conduct clinical trials and to commercialize REGENXBIO's product candidates, the labeling for any approved products; the scope, progress, expansion, and costs of developing and commercializing REGENXBIO's product candidates; competitive products; 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; 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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