REGENXBIO Provides Update on Lead Gene Therapy Development Programs

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- *Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia is actively recruiting and screening patients; expect to enroll first patient in second half of 2016*
- *IND submission for RGX-314 for the treatment of wet age-related macular degeneration planned for first quarter of 2017*
- *IND submissions for RGX-111 for the treatment of Mucopolysaccharidosis Type I and RGX-121 for the treatment of Mucopolysaccharidosis Type II planned for first half of 2017*

ROCKVILLE, Md., July 05, 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 an update on its four lead gene therapy development programs.

"The development and clinical operations teams at REGENXBIO and the University of Pennsylvania are working to ensure that our first-in-human gene therapy clinical trials are well-designed and enroll the patients most likely to benefit from treatment, which supports our long-term clinical development plans and our mission of improving the lives of patients suffering from severe diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We look forward to dosing the first patients in the RGX-501 clinical trial in the coming months and expect to have active INDs for our four lead programs in 2017."

RGX-501 Phase I/II Clinical Trial Update

REGENXBIO and trial sponsor, the University of Pennsylvania (Penn), are actively recruiting and screening participants in the Phase I/II clinical trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), and expect to enroll the first patients in the second half of 2016. RGX-501 uses the AAV8 vector to deliver the human low-density lipoprotein receptor (LDLR) gene to liver cells.

Due to the novel mechanism of RGX-501, REGENXBIO and Penn have implemented a screening and clinical protocol that is innovative for HoFH clinical trials, including requiring the discontinuation of most current therapies for up to six months before treatment administration. However, REGENXBIO and Penn recently submitted revisions to the clinical protocol that would allow for the inclusion of patients who receive treatment with PCSK9 inhibitors without requiring a discontinuation period.

The Phase I/II clinical trial is an open-label, single-center study evaluating the safety and efficacy of RGX-501 in up to 12 patients with HoFH. The primary endpoint is to assess the safety of a single intravenous administration of RGX-501. The secondary endpoint is to determine the percent of change from baseline of LDL cholesterol at 12 weeks. This is a dose-escalation study with patients receiving a single dose of 2.5×10^{12} (GC/kg) or 7.5×10^{12} (GC/kg).

RGX-314 Update

REGENXBIO and WuXi AppTec, Inc. plan to begin production of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) in the third quarter of 2016, to support REGENXBIO's planned Phase I clinical trial. REGENXBIO plans to deliver RGX-314 subretinally using an AAV8 vector, which encodes a gene for a monoclonal antibody fragment that binds to anti-vascular endothelial growth factor (VEGF) and neutralizes VEGF activity. REGENXBIO is in the process of completing preclinical studies that include animal evaluations recommended by the U.S. Food and Drug Administration's Center for Drug Evaluation and Research during a pre-Investigational New Drug meeting in April of 2016. REGENXBIO expects to submit an Investigational New Drug application (IND) for RGX-314 in the first quarter of 2017.

RGX-111 Update

REGENXBIO is conducting additional preclinical studies and expects to submit an IND for a Phase I/II clinical trial of RGX-111 in the U.S. and Canada in the first half of 2017. RGX-111 is a product candidate for the treatment of Mucopolysaccharidosis Type I (MPS I), which uses the AAV9 vector to deliver the human α-l-iduronidase (IDUA) gene to the central nervous system (CNS). The Phase I/II clinical trial design for RGX-111 is a first-in-human study that involves a novel, intracisternal route of administration that has demonstrated a range of partial to complete effect in diseased animals. As REGENXBIO advances RGX-111 in preclinical development, the Company continues to learn more about the opportunity to optimize RGX-111's clinical effect. The Company has begun additional dose-ranging studies with RGX-111 in animals that REGENXBIO believes will enable it to more fully evaluate an optimal initial dosing plan for this study.

RGX-121 Update

REGENXBIO plans to initiate preclinical studies for RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II) in the third quarter of 2016 and begin production of RGX-121 in the fourth quarter of 2016 to support its planned Phase I/II clinical trial. RGX-121 uses the AAV9 vector to deliver the human iduronate-2-sulfatase (IDS) gene to the CNS. The Company reiterates its prior guidance regarding the planned submission of an IND in the first half of 2017.

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 March 31, 2016, REGENXBIO's NAV Technology Platform was 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, anticipated timing of dosing of participants in REGENXBIO's RGX-501 clinical trial and updates regarding REGENXBIO's RGX-501, RGX-111, RGX-314 and RGX-121 development programs. 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. 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 its product candidates and technology; REGENXBIO's ability to establish and maintain development partnerships, including those with NAV Technology Licensees; REGENXBIO's expectations regarding REGENXBIO's expenses and revenue, the sufficiency of REGENXBIO's cash resources and needs for additional financing, 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 and Quarterly Report on Form 10-Q for the quarter ended March 31, 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 Form 10-Q for the quarter ended June 30, 2016, to be filed with the SEC in the third quarter of 2016. In addition to the risks described above and in 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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