REGENXBIO Announces Initiation of Phase I/II Clinical Trial of RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia

March 07, 2017 05:05 PM EST

- *First AAV gene therapy treatment for HoFH; interim trial update expected late 2017*

ROCKVILLE, Md., March 07, 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 the first patient in a Phase I/II clinical trial of REGENXBIO's investigational gene therapy RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH) has been dosed. RGX-501 uses the NAV AAV8 vector to deliver a functional copy of the human low-density lipoprotein receptor (LDLR) gene to liver cells, which may enable liver cells to make the LDLR protein they need to process LDL cholesterol.

"REGENXBIO is pleased to announce that we have advanced into the clinic with the dosing of the first patient in one of our lead development programs. This significant milestone is a tribute to the hard work of teams at Penn and REGENXBIO to bring this potentially life-changing therapy to patients with HoFH," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We look forward to continuing enrollment, and expect to report interim trial results by the end of 2017."

"Homozygous familial hypercholesterolemia is an inherited disorder that leads to aggressive premature cardiovascular disease. In those born with HoFH, heart attack or sudden death can occur as early as the teenage years," said Katherine Wilemon, Founder and Chief Executive Officer of the FH Foundation. "The treatment of a patient in REGENXBIO's clinical trial represents an important step forward in advancing research to discover a potential new approach to treating this life-threatening disease."

For further details on the trial, enrollment criteria and eligibility, please contact patientadvocacy@regenxbio.com or visit http://www.clinicaltrials.gov/ct2/show/NCT02651675.

About the Phase I/II Clinical Trial of RGX-501

The Phase I/II first-in-human clinical trial is an open-label, single-center study evaluating the safety and efficacy of RGX-501 in up to 12 patients with HoFH in the U.S. and Canada. The primary objective is to assess the safety of a single intravenous administration of RGX-501. The secondary endpoints are the percent of change from baseline of LDL cholesterol at 12 weeks and other lipid outcome measures. 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).

About RGX-501

RGX-501 is being developed as a novel, one-time intravenous treatment for HoFH. RGX-501 is designed to use the NAV AAV8 vector to deliver a functional copy of the LDLR gene to liver cells. This may enable liver cells to make the LDLR protein they need to process elevated levels of LDL cholesterol (LDL-C). The liver is a preferred target for HoFH gene therapy as it is the most important organ for expressing LDLRs, and contributes to greater than 90 percent of the capture and breakdown of LDL-C. RGX-501 has received orphan drug product designation from the U.S. Food and Drug Administration.

About Homozygous Familial Hypercholesterolemia

HoFH occurs when people inherit an abnormal copy of the LDLR gene from each of their parents. Individuals with HoFH have very low levels of – or are completely missing – LDLR, resulting in high levels of LDL-C in the blood. High levels of LDL-C (or "bad" cholesterol) are associated with premature and aggressive buildup of plaque in arteries, life-threatening coronary artery disease and an increase in the risk of a heart attack or stroke. If untreated, individuals with HoFH can suffer serious cardiac events before the age of 30. Current treatments do not provide a cure and may not lower cholesterol to optimal levels.

About REGENXBIO Inc.

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 Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of product candidates in multiple therapeutic areas.

Note Regarding Penn

The University of Pennsylvania (Penn) has licensed certain Penn-owned AAV technologies to REGENXBIO, including rights related to RGX-501.

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-501. 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, 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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