# REGENXBIO Enhances and Expands Scientific Leadership

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## Appoints Gene Therapy Industry Leader Olivier Danos, Ph.D., as Chief Scientific Officer

ROCKVILLE, Md., March 28, 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 Olivier Danos, Ph.D., is joining REGENXBIO in the newly created position of Chief Scientific Officer. Dr. Danos will report to Kenneth T. Mills, REGENXBIO's President and Chief Executive Officer.

Dr. Danos joins REGENXBIO from Biogen Inc., where he was Senior Vice President, Cell and Gene Therapy. At Biogen, Dr. Danos led company efforts dedicated to identifying and developing new technologies for gene transfer and genome engineering. Dr. Danos also co-founded and is an executive member of the board of directors of Lysogene, a NAV Technology Licensee focused on the development of gene therapy product candidates for the treatment of Mucopolysaccharidosis Type IIIA.

"Olivier brings established industry leadership and scientific expertise in the development of gene therapies and the field of genome engineering to REGENXBIO," said Dr. James M. Wilson, M.D., Ph.D. REGENXBIO's scientific founder and director of the University of Pennsylvania (Penn) Gene Therapy Program. "My relationship with Olivier can be traced back to the early stages of our careers, when he and I shared a bench at the Whitehead laboratories. He is an innovative scientist who has been at the forefront of the industry, and I look forward to collaborating with him on the advancement of scientific research at REGENXBIO."

"We are excited to enhance the breadth and depth of our scientific research team. Olivier and Jim are two renowned gene therapy pioneers who have been key players in the success of the field," said Mr. Mills. "We look forward to leveraging Olivier's rich industry experience and remarkable understanding of the space, including his work with the NAV Technology Platform, as we build a robust clinical pipeline of gene therapy product candidates with the goal of improving treatment options in many diseases."

"The NAV Technology Platform has the potential to significantly alter the course of disease and deliver enhanced health outcomes to patients in need," said Dr. Danos. "I am thrilled to have the opportunity to lead scientific research at REGENXBIO as we continue to advance this groundbreaking technology in a broad range of diseases that are not effectively addressed by existing drug classes."

Prior to Biogen, Dr. Danos served as Senior Vice President, Molecular Medicine, Synthetic Biology and Gene Regulation at Kadmon Pharmaceuticals. Earlier in his career, Dr. Danos was Director of the Gene Therapy Consortium of the University College of London, Scientific Director at Genethon and Senior Director of Research at Somatix Therapy Corporation. Dr. Danos has directed research focused on gene therapy at the Necker - Enfants Malades Hospital in Paris, the French National Centre for Scientific Research and the Pasteur Institute in Paris.

Dr. Danos received a Ph.D. in Biology at the University of Paris Diderot and the Pasteur Institute, and a Master in Science in Genetics and Mathematics from the University of Paris Orsay. Dr. Danos is a founding member of the European Society of Gene and Cell Therapy.

**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**

Penn has licensed certain Penn-owned AAV technologies to REGENXBIO.

**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 in connection with its NAV Technology Platform and gene therapy treatments. 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. 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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