



REGENXBIO Appoints Dr. Steve Pakola as Chief Medical Officer

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ROCKVILLE, Md., April 17, 2019 /PRNewswire/ -- 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 the appointment of Steve Pakola, M.D., as its Senior Vice President and Chief Medical Officer. Dr. Pakola will report to Kenneth T. Mills, REGENXBIO's President and Chief Executive Officer.

Dr. Pakola joins REGENXBIO from Aerpio Pharmaceuticals, Inc., where he was Chief Medical Officer. He has held key leadership roles in preclinical and clinical development, regulatory affairs and medical affairs. While Dr. Pakola's therapeutic area experience encompasses multiple indications, his predominant focus has been the development of treatments for retinal disorders, including clinical development experience in diabetic retinopathy, diabetic macular edema and age-related macular degeneration (AMD). He was the lead inventor and program lead for the Jetrea[®] (ocriplasmin) program, from inception in 2002 through the therapy's United States and European regulatory submissions in 2012.

"In this role, Steve will leverage his rich industry experience and expertise in the discovery and development of novel treatments for retinal disease," said Mr. Mills. "Steve's background as an innovator in drug development is particularly relevant as we leverage our breakthrough science to advance five current proprietary therapeutic programs that focus on retinal, neurodegenerative and metabolic diseases. We are excited to welcome Steve to the team."

"I am thrilled to join REGENXBIO at this time of exciting momentum for the company as it advances the development of gene therapies," said Dr. Pakola. "REGENXBIO's NAV Technology Platform has the potential to transform the course of treatment for patients with rare diseases as well as in broader applications such as wet AMD. I look forward to working with my colleagues, investigators and the regulatory authorities to bring new and innovative treatments to patients."

Prior to Aerpio, Dr. Pakola served as Chief Medical Officer at Amakem NV and as Senior Vice President and Chief Medical Officer at ThromboGenics NV. Earlier in his career, Dr. Pakola served as Associate Director of Cardiovascular Clinical Research at Boehringer Ingelheim, where he was Global Medical Lead on the Lipid-Lowering Development Programme and the USA Medical Lead for the Direct Thrombin Inhibitor Development Programme. Prior to that, Dr. Pakola served in senior-level clinical development positions at Quintiles Cardiovascular Therapeutics and at Organon.

Dr. Pakola earned a B.A., *summa cum laude*, and an M.D. from the University of Pennsylvania, where he was elected to the Alpha Omega Alpha Honor Medical Society.

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 candidates in multiple therapeutic areas.

Forward-Looking Statements

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations and clinical trials. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to 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, 2018 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at www.sec.gov. All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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