



## **REGENXBIO and Biogen Enter Exclusive License Agreement for the Development of Gene Therapy Treatments for Rare Genetic Vision Disorders**

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ROCKVILLE, Md., May 16, 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 announced an exclusive worldwide license agreement with Biogen for the development of gene therapy product candidates based on the NAV Technology Platform for the treatment of two rare genetic vision disorders. The NAV Technology Platform is an AAV gene delivery platform consisting of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10.

Under the terms of the agreement, REGENXBIO has granted Biogen an exclusive worldwide research license, with rights to sublicense, to REGENXBIO's NAV AAV8 and AAV9 vectors for the development of gene therapy product candidates for the treatment of two rare genetic vision disorders in humans. Upon selection of a single vector for each indication, the research license will convert to a commercial license. In return for these rights, REGENXBIO will receive an undisclosed upfront payment, ongoing fees, milestone payments and royalties on net sales of products incorporating the licensed intellectual property.

"This license agreement provides new validation of the potential of our NAV Technology Platform in ocular indications and is an important step in advancing NAV-based gene therapies to people suffering from rare genetic vision disorders," said Kenneth T. Mills, President and CEO of REGENXBIO. "We are pleased that Biogen, a respected biotechnology leader, has selected our NAV Technology Platform for the development of innovative gene therapies to improve treatment options in areas of significant unmet need."

"We're continually looking for opportunities to advance gene therapies to people lacking adequate treatments, through improved delivery vectors, like REGENXBIO's NAV Technology Platform," said Olivier Danos, Ph.D., Senior Vice President, Cell & Gene Therapy at Biogen. "This collaboration will enable us to expand our pipeline of treatments with the potential to improve health outcomes in diseases of the eye, an ideal setting for the delivery of targeted gene therapies."

### **About Biogen**

Through cutting-edge science and medicine, Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological, autoimmune, and rare diseases. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For more information, please visit [www.biogen.com](http://www.biogen.com).

### **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, REGENXBIO's licensing of NAV AAV8 and AAV9 vectors for the development of gene therapy product candidates for the treatment of certain eye diseases by Biogen. 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. 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](http://www.sec.gov). 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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REGENXBIO Inc.