



## **Voyager Therapeutics and REGENXBIO Announce Exercise of Options for Rights to NAV Vectors**

November 29, 2016

CAMBRIDGE, Mass., and ROCKVILLE, Md., Nov. 29, 2016 (GLOBE NEWSWIRE) -- Voyager Therapeutics, Inc. (NASDAQ:VYGR), a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the central nervous system (CNS), and 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 (NAV), today announced that Voyager has exercised commercial options for the use of REGENXBIO's NAV vectors for the development and commercialization of gene therapies for specific neurological diseases.

Upon exercise of the options, REGENXBIO has granted Voyager a non-exclusive worldwide commercial license, with rights to sublicense, to three specific NAV vector sequences covered by REGENXBIO's NAV Technology Platform, each for the treatment of a specific neurological disease. In return for these rights, REGENXBIO will receive undisclosed upfront payments, ongoing fees, milestone payments and royalties on net sales of products incorporating the licensed intellectual property.

"Today's announcement aligns with our stated goal of advancing our multiple preclinical programs during this year and into 2017 towards selection of lead clinical candidates," said Steven Paul, M.D., President and Chief Executive Officer of Voyager Therapeutics. "Exercising the commercial options with REGENXBIO represents an important step for our preclinical pipeline programs, and we are excited with the progress of our continued collaboration with REGENXBIO."

"These option exercises serve as further validation of our proprietary NAV Technology Platform, the breadth of our intellectual property portfolio, and the unique properties of NAV vectors for targeting the central nervous system and a number of other organ systems," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "As a leader in next-generation AAV gene therapy, REGENXBIO is pleased to continue our collaboration with Voyager and its pursuit of developing and delivering life-changing treatments for severe diseases of the CNS."

### **About Voyager Therapeutics**

Voyager Therapeutics is a clinical-stage gene therapy company developing life-changing treatments for severe diseases of the CNS. Voyager is committed to advancing the field of AAV (adeno-associated virus) gene therapy through innovation and investment in vector engineering and optimization, manufacturing and dosing and delivery techniques. The Company's pipeline is focused on severe CNS diseases in need of effective new therapies, including advanced Parkinson's disease, a monogenic form of ALS, Friedreich's ataxia, Huntington's disease, frontotemporal dementia, Alzheimer's disease and severe, chronic pain. Voyager has broad strategic collaborations with Sanofi Genzyme, the specialty care global business unit of Sanofi, and the University of Massachusetts Medical School. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics is headquartered in Cambridge, Massachusetts. For more information, please visit [www.voyagertherapeutics.com](http://www.voyagertherapeutics.com). Follow Voyager on [LinkedIn](#).

### **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 September 30, 2016, REGENXBIO's NAV Technology Platform was being applied in the development of 29 product candidates for a variety of diseases, including five internally developed candidates and 24 partnered candidates developed by REGENXBIO's licensees.

### **Voyager Forward-Looking Statements**

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities law. The use of words such as "may," "might," "will," "should," "expect," "plan," "anticipate," "believe," "estimate," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify forward-looking statements. For example, all statements Voyager makes regarding the initiation, timing, progress, enrollment and reporting of top-line and other results of its preclinical programs and clinical trials and its research and development programs, its ability to advance its AAV-based gene therapies into, and successfully complete, clinical trials, its ability to continue to develop its product engine, its ability to add new programs to its pipeline, its expected cash, cash equivalents and marketable securities at the end of a fiscal year and anticipation for how long expected cash, cash equivalents and marketable securities will last, and the timing or likelihood of its regulatory filings and approvals, are forward looking. All forward-looking statements are based on estimates and assumptions by Voyager's management that, although Voyager believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Voyager expected. These statements are also subject to a number of material risks and uncertainties that are described in Voyager's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission, as updated by its future filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Voyager undertakes no obligation to publicly update or revise any forward-looking

statement, whether as a result of new information, future events or otherwise, except as required by law.

### **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 NAV Technology Platform, REGENXBIO’s collaborations with its NAV Technology Licensees and the research, development and regulatory plans of REGENXBIO’s NAV Technology Licensees. 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, 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 REGENXBIO’s product candidates and technology; 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, including those with NAV Technology Licensees; REGENXBIO’s expenses and revenue, the sufficiency of REGENXBIO’s cash resources and needs for additional financing, 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 September 30, 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 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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