REGENXBIO Announces New License Agreement with Pfizer for the Treatment of Friedreich's Ataxia Using NAV® AAV9 Vector

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ROCKVILLE, Md., July 31, 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 it entered into a license agreement with Pfizer Inc.

Under the terms of the agreement, REGENXBIO has granted Pfizer a non-exclusive worldwide license, with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapies for the treatment of Friedreich's ataxia, the most common hereditary ataxia. In return for these rights, REGENXBIO will receive an upfront payment, and has the potential to receive ongoing fees, development and commercial milestone payments, and royalties on net sales of products incorporating the licensed intellectual property.

"This license agreement further validates the strength of our intellectual property portfolio and the potential of NAV AAV9 for the treatment of systemic and CNS manifestations of movement disorders," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to establish our relationship with Pfizer as they advance this program to develop a potential gene therapy treatment for Friedreich's ataxia."

"We are excited to partner with REGENXBIO on the use of the NAV AAV9 vector for the treatment of Friedreich's ataxia, a condition with significant unmet medical need," said Seng Cheng, Senior Vice President and Chief Scientific Officer of Pfizer's Rare Disease Research Unit. "We believe the AAV9 vector has the potential to have a profound impact on patients with severe and debilitating diseases where treatment options are limited today."

"Friedreich's ataxia is a debilitating, life-shortening, degenerative neuro-muscular disorder that affects about one in 50,000 people in the United States, and there are currently no treatments available," said Ronald J. Bartek, President, Director and Co-Founder of the Friedreich's Ataxia Research Alliance. "We applaud these efforts to develop new potential NAV Technology-based gene therapies for our under-served patient community."

About Friedreich's Ataxia

Friedreich's ataxia (FA) is the most common hereditary ataxia. FA patients have a genetic mutation in the FXN gene, which limits the production of the protein frataxin, causing a variety of debilitating symptoms and complications including loss of coordination and balance, muscle weakness, impaired vision, hearing and speech, scoliosis, diabetes, and cardiomyopathy.

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 cash flow. 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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