REGENXBIO Receives $100 Million Accelerated License Payment Due to Acquisition of AveXis by Novartis

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- $100 million license payment consists of annual fees and a commercial milestone fee accelerated upon the change of control of AveXis

- Novartis, through its acquisition of AveXis, holds exclusive rights to the REGENXBIO NAV Technology Platform for the development of treatments for SMA, which is the leading genetic cause of infant mortality

- In April 2018, AveXis reported that the six SMA patients who were at least one-month post gene transfer in the pivotal trial for AVXS-101, which uses the NAV AAV9 vector, were exhibiting motor function improvements

ROCKVILLE, Md., June 11, 2018 /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 that it has received an accelerated license payment of $100 million under its license agreement (the License Agreement) with AveXis, Inc. (AveXis) for the development and commercialization of products to treat spinal muscular atrophy (SMA), due to the acquisition of AveXis by Novartis AG (Novartis).

The accelerated license payment consisted of $60 million in annual fees and a commercial milestone fee of $40 million. Under the License Agreement, in addition to the accelerated license payment, REGENXBIO remains eligible to receive a potential commercial milestone fee of $80 million and certain royalties on net sales for any product developed for the treatment of SMA using REGENXBIO's NAV technology.

Novartis now holds exclusive rights to the NAV Technology Platform for the development of treatments for SMA, including AVXS-101, which uses REGENXBIO's NAV AAV9 vector. In April 2018, AveXis reported that the six SMA patients who were at least one-month post gene transfer in the pivotal trial for AVXS-101 were exhibiting motor function improvements. SMA is the leading genetic cause of infant mortality.

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, 2017 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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