REGENXBIO and AveXis Announce Expansion of Relationship through Amended License Agreement for the Development and Commercialization of Treatments for Spinal Muscular Atrophy

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- *AveXis acquires exclusive rights to entire NAV Technology Platform for the development of treatments for SMA*
- *Amended agreement permits assignment by AveXis upon a change of control without REGENXBIO's consent*
- *REGENXBIO could receive up to $260 million, including $140 million in guaranteed upfront and annual payments*

ROCKVILLE, Md. and CHICAGO, Jan. 08, 2018 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (Nasdaq:RGNX) and AveXis, Inc. (Nasdaq:AVXS) today announced that they have entered into an amendment which expands upon the exclusive, worldwide license agreement they entered into in March 2014 (2014 License Agreement) for the development and commercialization of products to treat spinal muscular atrophy (SMA). AveXis' initial proprietary gene therapy candidate, AVXS-101, is in a pivotal trial for the treatment of SMA Type 1, and a Phase 1 trial for SMA Type 2. AVXS-101 uses REGENXBIO's NAV AAV9 vector.

"An expanded relationship further aligns REGENXBIO with AveXis in their commitment to develop and commercialize novel gene therapy treatments for SMA, and serves as additional validation of our NAV Technology Platform and its potential utility in this therapeutic area," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are encouraged by the innovation and progress of AVXS-101 and are pleased to broaden our relationship with AveXis by providing deeper technology access to AveXis as it advances its mission towards commercialization."

Under the terms of the amendment, REGENXBIO granted AveXis exclusive, worldwide rights to all vectors in REGENXBIO's NAV Technology Platform for the treatment of SMA in addition to adding and amending certain terms of the 2014 License Agreement, including the modification of the assignment provision to now permit assignment in the event of a change of control by AveXis without REGENXBIO's consent. REGENXBIO will receive, in addition to the payments and royalties owed under the original 2014 License Agreement, an upfront payment of $80 million, an additional payment of $30 million after one year and an additional payment of $30 million after two years, and REGENXBIO is eligible to receive potential commercial milestone payments of up to $120 million. For any product developed for the treatment of SMA using the NAV AAV9 vector, REGENXBIO will continue to receive mid-single to low double-digit royalties on net sales as defined in the original 2014 License Agreement, and for any product developed for the treatment of SMA using a NAV vector, other than NAV AAV9, REGENXBIO will receive a low double-digit royalty on net sales.

"We have been very encouraged by the clinical data demonstrated to date by AVXS-101 utilizing the NAV Technology Platform from REGENXBIO in infants with SMA Type 1 and are optimistic that we may also see robust results in our recently initiated Phase 1 trial in SMA Type 2," said Sean Nolan, President and Chief Executive Officer of AveXis. "We believe this expanded partnership with REGENXBIO, which provides exclusive rights to the entire REGENXBIO NAV Technology Platform, will further strengthen our leadership position in gene therapy treatments for this devastating disease."

About SMA

SMA is a severe neuromuscular disease characterized by the loss of motor neurons leading to progressive muscle weakness and paralysis. SMA is caused by a genetic defect in the SMN1 gene that codes SMN, a protein necessary for survival of motor neurons. The incidence of SMA is approximately one in 10,000 live births.

The most severe form of SMA is Type 1, a lethal genetic disorder characterized by motor neuron loss and associated muscle deterioration, which results in mortality or the need for permanent ventilation support before the age of two for greater than 90 percent of patients. SMA Type 1 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.

About AveXis, Inc.

AveXis is a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases. The company's initial proprietary gene therapy candidate, AVXS-101, is in the pivotal phase of study for the treatment of SMA Type 1, and a Phase 1 trial for SMA Type 2. The company also intends to expand the study of gene therapy into two additional rare neurological monogenic disorders: Rett syndrome (RTT) and a genetic form of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (SOD1)

gene.

REGENXBIO 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 collaboration with AveXis to develop and commercialize products to treat SMA, and future potential milestone and royalty payments by AveXis to REGENXBIO. 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 of REGENXBIO’s clinical trials; the timing and success of preclinical studies and clinical trials 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, 2016 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.

AveXis 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, AveXis’ continued collaboration with REGENXBIO to develop and commercialize products to treat SMA, future potential milestone and royalty payments by AveXis to REGENXBIO, AveXis’ research, development and regulatory plans for AVXS-101, including and the potential of AVXS-101 to positively impact quality of life and alter the course of disease in patients with SMA Type 1. 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 scope, progress, expansion, and costs of developing and commercializing AveXis’ product candidates; regulatory developments in the U.S. and EU, as well as other factors discussed in the “Risk Factors” and the “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections of AveXis’ Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 16, 2017, and AveXis’ Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, filed with the SEC on November 9, 2017. In addition to the risks described above and in the 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 AveXis’ results. There can be no assurance that the actual results or developments anticipated by AveXis will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, AveXis. 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. AveXis cautions investors not to rely too heavily on the forward-looking statements AveXis 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). AveXis 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, except as required by law.

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