REGENXBIO and AveXis Announce New Exclusive Worldwide Licenses for the Treatment of Two Rare Neurological Monogenic Disorders Using NAV AAV9 Vector

June 07, 2017 04:02 PM EDT

REGENXBIO grants AveXis new licenses to NAV AAV9 vector for the development and commercialization of treatments for Rett syndrome (RTT) and amyotrophic lateral sclerosis (ALS)

ROCKVILLE, Md. and CHICAGO, Ill., June 07, 2017 (GLOBE NEWSWIRE) -- REGENXBIO Inc. (NASDAQ:RGNX) and AveXis, Inc. (NASDAQ:AVXS) today announced an exclusive worldwide license agreement for AveXis to develop and commercialize gene therapy treatments using REGENXBIO's NAV AAV9 vector to treat two 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.

Under the terms of the license agreement, REGENXBIO will receive an upfront payment upon execution, ongoing fees, milestone payments and royalties on net sales of products incorporating the NAV AAV9 vector.

"This license agreement for our NAV AAV9 vector highlights the strength of our relationship with our existing NAV Technology Licensee, AveXis, and our commitment to bringing important new NAV-based gene therapies to patients with severe diseases with significant unmet medical need," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "As a leader in AAV-based gene therapy, REGENXBIO continues to selectively and strategically license our NAV Technology Platform for specific vector and indication combinations in a way that allows us to maintain our focus on internal product development while at the same time advancing the overall field by expanding the pipeline of NAV-based gene therapies."

"Building on our experience and the success we have seen to date with the use of REGENXBIO's NAV AAV9 vector in our spinal muscular atrophy clinical trials, this new license agreement reflects progress on executing our corporate strategy and our vision of becoming the leader in the treatment of rare and life-threatening neurological genetic diseases," said Sean Nolan, President and Chief Executive Officer of AveXis. "While we remain intensely focused on the development and commercialization of AVXS-101 for the treatment of spinal muscular atrophy, we are excited by the potential for gene therapy to address the needs of patients with RTT and ALS – two devastating diseases for which there are no cures and insufficient existing treatments."

Preclinical data demonstrating promising efficacy and safety of gene therapy treatments for RTT and ALS using NAV AAV9, generated by AveXis' Chief Scientific Officer Dr. Brian Kaspar at Nationwide Children's Hospital, has been licensed by AveXis. AveXis intends to move forward with initiating IND-enabling studies in both RTT and ALS and plans to provide more details on these programs in the second half of 2017.

About Rett Syndrome

Rett syndrome (RTT) is a devastating, rare neurological disorder characterized by slowed growth, loss of normal movement and coordination and loss of communication skills. RTT is caused by an X-linked dominant mutation in the methyl CpG binding protein 2 (MECP2) gene, which results in problems with the protein production critical for brain development. Rett Syndrome occurs in approximately one of every 10,000 female births and usually begins to show signs and symptoms in infants between six and 18 months of age. Current treatments only offer symptomatic relief and do not target the genetic cause of the disease, leaving a significant unmet need.

About Genetic Amyotrophic Lateral Sclerosis

Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease that affects nerve cells in the brain and the spinal cord. Familial or inherited forms of ALS reflect five to 10 percent of ALS cases, or approximately one to two thousand people in the U.S., and can be caused by mutations in several genes known to be associated with ALS. Approximately 20 percent of these cases are caused by mutations in the gene that produces the copper zinc superoxide dismutase 1 (SOD1) enzyme, which leads to a progressive degeneration of motor neurons affecting movement and muscle control. ALS usually occurs in people between the ages of 40 and 70. Current treatments only offer modest benefits and do not target the genetic cause of the disease, leaving a significant unmet need

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, recently completed a Phase 1 clinical trial for the treatment of SMA Type 1. For additional information, please visit www.avexis.com.

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 research, development and regulatory plans in connection with its NAV Technology Platform and gene therapy treatments. 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 and its development partners; the ability to obtain and maintain regulatory approval of 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 establish and maintain development partnerships; REGENXBIO's expenses and revenue; regulatory developments in the United States and foreign countries; the sufficiency of REGENXBIO's cash resources and needs for additional financing; and 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, 2016 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2017, which are on file with the Securities and Exchange Commission (SEC) and available at 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.

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' research, development and regulatory plans for its programs for treatment of RTT and ALS, its expectations regarding initiation of IND-enabling studies for these programs and timing of providing an update on these programs. 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" section of AveXis' Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 16, 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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