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AveXis Announces Alignment with FDA on Next Steps Toward a BLA Submission for AVXS-101 in SMA Type 1

Jan 04, 2018

– Company to submit information requested by FDA to the IND on an on-going basis –

– AveXis plans to request a pre-BLA meeting in Q2 2018 –

– Conference call and webcast today at 4:30 pm EST –

CHICAGO, Jan. 04, 2018 (GLOBE NEWSWIRE) -- AveXis, Inc. (NASDAQ:AVXS), a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases, today provided an update following the receipt of minutes from the end-of-Phase 1 meeting with the U.S. Food and Drug Administration (FDA) conducted on December 5, 2017, regarding the company's primary gene therapy candidate, AVXS-101, for the treatment of spinal muscular atrophy (SMA) Type 1.

The goal of the end-of-Phase 1 meeting was to review the non-clinical, clinical and Chemistry, Manufacturing and Controls (CMC) data that has been generated by AveXis to date, and to align with the FDA on next steps leading to a Biologics License Application (BLA) submission. The FDA provided detailed information requests in each of the areas discussed, which the company plans to address by submitting the requested information to the investigational new drug (IND) application on an on-going basis. AveXis has been working on many of these areas of focus in anticipation of the requests at some point during the review process. AveXis also plans to provide available data from its on-going pivotal trial of AVXS-101 in SMA Type 1 (STR1VE) prior to the pre-BLA meeting.

"We are very pleased that the constructive and collaborative discussion during the end-of-Phase 1 meeting resulted in the identification of the specific next steps we must take on our path to a BLA submission for AVXS-101 in SMA Type 1," said Sean Nolan, President and Chief Executive Officer of AveXis. "We greatly appreciate the level of clarity we received from the FDA and will provide our responses on an on-going basis through a series of submissions to the IND, with the expectation that we will request a pre-BLA meeting in the second quarter of 2018."

The general purpose of the pre-BLA meeting is to outline what information is to be submitted in the BLA and how that information will be submitted. AveXis intends to make the requested data submissions to the IND in advance of the pre-BLA meeting, which may allow the meeting itself to focus on how the BLA and supportive information will be submitted.

Today's Conference Call Information
AveXis will host a conference call and webcast at 4:30 pm EST today, January 4, 2018. Analysts and investors can participate in the conference call by dialing (844) 889-6863 for domestic callers and (661) 378-9762 for international callers, using the conference ID 8188476. The webcast can be accessed live on the Events and Presentations page in the Investors and Media section of the AveXis website, www.AveXis.com. The webcast will be archived on the company's website for 90 days and will be available for telephonic replay for 14 days

following the call by dialing (855) 859-2056 (Domestic) or (404) 537-3406 (International), conference ID 8188476.

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 and is the leading genetic cause of infant mortality.

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 2 typically presents between six and 18 months of age, and those affected will never walk without support and most will never stand without support. SMA Type 2 results in mortality in more than 30 percent of patients by the age of 25.

About AVXS-101

AVXS-101 is a proprietary gene therapy candidate of a one-time treatment for SMA Types 1 and 2, designed to address the monogenic root cause of SMA and prevent further muscle degeneration by addressing the defective and/or loss of the primary SMN gene. AVXS-101 also targets motor neurons, providing rapid onset of effect and crossing the blood brain barrier to allow targeting of both central and systemic features.

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.

For additional information, please visit www.avexis.com.

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 AVXS-101, including the expected timing of future interactions with the FDA, including AveXis' expected timing to request a pre-BLA meeting with the FDA and the submission of requested information to the FDA 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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