# AveXis to Use Intended Commercial GMP Product in SMA Type 2 Study

December 21, 2016

*– SMA Type 2 study to initiate in Q2 2017 –*
*– Streamlines development of Type 2 clinical data set –*
*– Conference call and webcast December 21, at 4:30 p.m. EST –*

CHICAGO, Dec. 21, 2016 (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 reported that, based on emerging data from its intended commercial Good Manufacturing Practice (GMP) development work and ongoing discussions with the U.S. Food and Drug Administration (FDA), the company has made the strategic decision to use the intended commercial GMP derived product in all future studies of the company's proprietary gene therapy candidate, AVXS-101, including the planned spinal muscular atrophy (SMA) Type 2 trial.

The company had originally planned to use existing inventory for its initial trial in SMA Type 2, and to then use its intended commercial GMP derived product in later Type 2 trials. By using the intended commercial GMP derived product for the duration of its study of AVXS-101 in Type 2 patients, the need for a comparability analysis later in program development is eliminated, potentially accelerating the timeframe for gathering clinical data that may be used as part of a data set to support a future SMA Type 2 indication.

"We believe this strategic decision has the potential to streamline the development of the clinical data set for AVXS-101 in SMA Type 2," said Sean Nolan, President and Chief Executive Officer of AveXis. "We are pleased with the advances we have seen from our process development efforts and believe the optimal approach is to use the scalable process in our clinical evaluation of AVXS-101 in SMA Type 2 from the outset."

The company now expects to initiate the planned study of AVXS-101 in SMA Type 2 patients in Q2 2017, assuming a positive outcome from its Type B manufacturing meeting with the FDA, currently anticipated to occur in Q1 2017.

This approach is intended to potentially expedite the SMA Type 2 program and does not impact the development program for AVXS-101 in SMA Type 1. AveXis expects to initiate a U.S. pivotal trial of AVXS-101 in patients with SMA Type 1 in the first half of 2017, and plans to use the same intended commercial GMP derived product for that study.

**Conference Call Information**
AveXis will host a conference call and webcast at 4:30 p.m. EST today, December 21, 2016, to discuss this clinical development update for AVXS-101.

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 43591277. 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 30 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 43591277.

**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. SMA 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. Approximately 30 percent of cases are expected to be SMA Type 2. SMA Type 2 typically presents between six and 18 months of age. Affected patients will never walk without support, and SMA Type 2 results in mortality for more than 30 percent of patients by age 25.

**About AVXS-101**
AVXS-101 is a proprietary gene therapy candidate of a one-time treatment for SMA Type 1 and is designed to address the monogenic root cause of SMA and prevent further muscle degeneration by addressing the defective and/or loss of the primary SMN1 gene. AVXS-101 also targets motor neurons providing rapid onset of effect, and crosses the blood brain barrier allowing an IV dosing route and effective 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 an ongoing Phase 1 clinical trial for the treatment of SMA Type 1. 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 potential of AVXS-101 to positively impact quality of life and alter the course of disease in children with SMA Type 1, expectations regarding design and timing of the SMA Type 2 trial of AVXS-101 and the overall clinical development of AVXS-101 and AveXis' manufacturing processes. 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 United States and foreign countries, as well as other factors discussed in the "Risk Factors" included as Exhibit 99.1 to the Company's Current Report on Form 8-K filed with the Securities and Exchange Commission on September 7, 2016 and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of AveXis' Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, filed with the SEC on November 10, 2016. 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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