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# AveXis to Initiate Screening for Remaining Patients in Pivotal Trial of AVXS-101 for SMA Type 1 Following Review of Preliminary Data from First Three Patients

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CHICAGO, Jan. 30, 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 announced that, following review of safety data and early signals of efficacy from the first three patients dosed in its pivotal trial of AVXS-101 for spinal muscular atrophy (SMA) Type 1, the company, with agreement from the U.S. Food and Drug Administration, will initiate screening for the remaining patients in the trial as per the protocol.

The open-label, single-arm, multi-center trial (STR1VE) is designed to evaluate the efficacy and safety of a one-time intravenous (IV) infusion of AVXS-101. There was at least a four-week interval scheduled between dosing the first three patients to allow review of the safety analysis as well as early signals of efficacy.

"We are pleased to share that we will be initiating screening for the remaining patients to be enrolled in STR1VE as planned, following review of safety data and early signals of efficacy from the first three patients who received a one-time IV infusion of AVXS-101," said Dr. Sukumar Nagendran, Chief Medical Officer of AveXis. "We know there is an urgent need for new treatment approaches, and we are eager to rapidly enroll the remaining patients in the pivotal trial as we further our clinical understanding of AVXS-101."

The trial will enroll a minimum of 15 patients with SMA Type 1 who are less than six months of age at the time of gene therapy, and who have one or two copies of the *SMN2* backup gene as determined by genetic testing and bi-allelic *SMN1* gene deletion or point mutations. The intent-to-treat population is defined as patients who are less than six months of age and symptomatic at the time of gene therapy, with two copies of the SMN2 gene as determined by genetic testing, bi-allelic SMN1 gene deletion and no c.859G>C mutation in SMN2.

**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 is also designed to target 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 its development 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' continued enrollment of patients in the STR1VE trial, its research, development and regulatory plans for AVXS-101, and the planned expansion of the company's development of gene therapy into additional disorders. 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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