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AveXis Announces Expanded Clinical Development Program for AVXS-101 in Spinal Muscular Atrophy " >

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– Company to expand study of AVXS-101 into additional SMA populations including pre-symptomatic, older pediatric Type 2 and Type 3 SMA patients –

– First patient dosed in Phase 1 trial of AVXS-101 in SMA Type 2 –

CHICAGO, Jan. 16, 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 overview of the expanded clinical development program for the company's initial gene therapy candidate, AVXS-101, for the treatment of spinal muscular atrophy (SMA). In addition to the ongoing pivotal trial in SMA Type 1 (STR1VE) and the ongoing Phase 1 trial in SMA Type 2 (STRONG), the company plans to initiate three studies to further evaluate AVXS-101, including in new SMA patient populations. Additionally, the company announced the first patient has been dosed in the Phase 1 trial of AVXS-101 in SMA Type 2.

"Our focus has always been to serve the SMA community, and our expanded clinical development program is designed to evaluate the impact of AVXS-101 in a broader set of SMA patients," said Dr. Sukumar Nagendran, Chief Medical Officer of AveXis. "We believe the year ahead has the potential to be one of significant clinical progress as we continue toward our ultimate goal of bringing AVXS-101 to the patients and families devastated by SMA."

Clinical Development Program Overview of AVXS-101 for the Treatment of SMA

Ongoing Clinical Trials

- **Pivotal Trial of AVXS-101 in SMA Type 1 (STR1VE):** The ongoing, open-label, single-arm, single-dose, multi-center trial is designed to evaluate the efficacy and safety of a one-time IV infusion of AVXS-101 in patients with SMA Type 1. The trial is expected to 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. Three patients have been dosed to date.

- **Phase 1 Trial of AVXS-101 in SMA Type 2 (STRONG):** The on-going, open-label, dose-comparison, multi-center Phase 1 trial is designed to evaluate the safety, optimal dosing, and proof of concept for efficacy of AVXS-101 in two distinct age groups of patients with SMA Type 2, utilizing a one-time intrathecal (IT) route of administration. The trial is expected to enroll 27 infants and children who are symptomatic with a genetic diagnosis consistent with SMA, including the bi-allelic deletion of *SMN1* and three copies of *SMN2* without the *SMN2* genetic modifier, who are able to sit but have no historical or current ability to stand or walk. One patient has been dosed to date.

Planned Trials in SMA

- **Pivotal Trial of AVXS-101 in SMA Type 1 in Europe (STR1VE EU):** The planned trial is expected to reflect a single-arm design, using natural history of the disease as a comparator, and is expected to enroll

approximately 30 patients with SMA Type 1 who are less than six months of age at the time of gene therapy. The trial is designed to evaluate safety and efficacy of a one-time IV dose of AVXS-101, including achievement of motor milestones, specifically patients' ability to sit unassisted, as well as an efficacy measure defined by the time from birth to an "event," defined as death or requiring at least 16 hours per day of ventilation support for breathing for greater than two weeks in the absence of an acute reversible illness, or perioperatively. AveXis incorporated scientific advice from the European Medicines Agency into the protocol design, and expects to initiate the trial in the first half of 2018.

- **Pre-Symptomatic SMA Types 1, 2, 3 (SPRINT):** The planned multi-national trial is expected to enroll approximately 44 patients with two, three and four copies of *SMN2* who are less than six weeks of age and pre-symptomatic at the time of gene therapy. The trial is designed to evaluate appropriate clinical endpoints, including developmental milestones, survival, bulbar function and safety, of a one-time IV infusion of AVXS-101. AveXis expects to initiate the trial in the first half of 2018, and will provide more design details at the time of initiation.

- **Pediatric "All Comers" with SMA Types 1, 2, 3 (REACH)** : The planned multi-national trial is expected to enroll approximately 50 patients between approximately six months and 18 years of age who do not qualify for other AVXS-101 trials at the time of gene therapy. The trial is designed to evaluate a one-time IT dose of AVXS-101. AveXis expects to initiate the trial in late Q4 2018 or early 2019, and will provide more trial design details at the time of initiation.

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' research, development and regulatory plans for AVXS-101, including expected trial design, planned enrollment and timing of anticipated clinical trials in additional SMA patient populations, 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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