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AveXis to Present AVXS-101 Data at the Annual Meeting of the American Academy of Neurology

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Includes Initial Data from SMA Type 1 Pivotal Trial (STR1VE) and 24-Month Follow-Up Data from Phase 1 Trial

CHICAGO, April 19, 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 initial results from the pivotal study (STR1VE) of AveXis' proprietary gene therapy, AVXS-101, for the treatment of spinal muscular atrophy (SMA) Type 1, and 24-month follow-up data from the Phase 1 trial, will be presented at the 2018 Annual Meeting of the American Academy of Neurology (AAN) taking place April 21-27 in Los Angeles.

"AVXS-101 Gene Replacement Therapy Pivotal Trial in SMA Type 1: Pivotal Study (STR1VE) Update," will be presented during the Emerging Science Poster Session IV on April 25 at 11:30 a.m. - 7:00 p.m. PDT. John W. Day, MD, PhD, Professor, Neurology & Neurological Sciences Director, Stanford Neuromuscular Disorders Program, Stanford University Medical Center, will be available starting at 5:30 p.m. PDT.

Four platform presentations will be delivered consecutively during S29: Child Neurology and Developmental Neurology I on April 25 from 1:00 - 1:48 p.m. PDT :

- "AVXS-101 Phase 1 Gene Replacement Therapy Clinical Trial in SMA Type 1: Event Free Survival and Achievement of Developmental Milestones after 24-Months Post-Dosing," by Samiah Al-Zaidy, MD, Co-Investigator for the study.
- "AVXS-101 Phase 1 Gene Replacement Therapy Clinical Trial in SMA Type 1: Patients Treated Early with the Proposed Therapeutic Dose Were Able to Sit Unassisted at a Younger Age (24-Month Follow-Up Data Set)," by Linda Lowes, PhD, Director of Clinical Therapies Research and a member of the Center for Gene Therapy at the Research Institute of Nationwide Children's Hospital.
- "AVXS-101 Phase 1 Gene Replacement Therapy Clinical Trial in SMA Type 1: Continued Independence from Nutritional and Ventilatory Support in Patients Dosed Early in Disease Progression After 24-Months Post-Dosing," by Richard Shell, MD, member of the Section of Pulmonary Medicine at Nationwide Children's Hospital.
- "AVXS-101 Trial Experience: CHOP-INTEND Effectively Demonstrates Marked, Early, Rapid, and Sustained Improvements Following Treatment and Precedes Major Motor Milestone Achievement (24-Month Follow-Up Data Set)," by Dr. Linda Lowes.

Abstracts can be accessed through the AAN website at www.aan.com.

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
AveXis' initial product candidate, AVXS-101, is its proprietary gene therapy currently in development for the one-time treatment of 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 effective targeting of both central and systemic features.

About AveXis, Inc.
AveXis, Inc. is a clinical-stage gene therapy company, dedicated to developing and commercializing novel treatments for patients suffering from rare and life-threatening neurological genetic diseases. Our initial product candidate, AVXS-101, is our proprietary gene therapy currently in development for the treatment of spinal muscular atrophy, or SMA, Type 1, the leading genetic cause of infant mortality, and SMA Type 2. The U.S. Food and Drug Administration, or FDA, has granted AVXS-101 Orphan Drug Designation for the treatment of all types of SMA and Breakthrough Therapy Designation, as well as Fast Track Designation for the treatment of SMA Type 1. In addition to developing AVXS-101 to treat SMA Type 1 and Type 2, we also plan to develop other novel treatments for rare neurological diseases, including Rett syndrome and a genetic form of amyotrophic lateral sclerosis caused by mutations in the superoxide dismutase 1 (*SOD1)* gene.

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

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