AveXis Presents Results from Phase 1 Trial of AVXS-101 in SMA Type 1 at the Annual Meeting of the American Academy of Neurology

April 25, 2017

– 10 of 12 patients on proposed therapeutic dose sat unassisted; patient videos demonstrate motor milestone achievement not seen in natural history of disease –

– 86% of patients without feeding support and 70% of patients without respiratory support prior to dosing continue without any support –

– 92% of patients on proposed therapeutic dose fed orally –

– Conference call and webcast April 25 at 4:30 p.m. EDT –

BOSTON, April 25, 2017 (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 presented results from the closeout of the Phase 1 trial of AVXS-101 in spinal muscular atrophy (SMA) Type 1 at the 2017 Annual Meeting of the American Academy of Neurology (AAN) in Boston. These data were presented during the Clinical Trial Plenary Session by Jerry Mendell, MD, principal investigator in the trial and Curran-Peters Chair of Pediatric Research, Professor of Pediatrics and Neurology at the Research Institute at Nationwide Children’s Hospital and The Ohio State University, Columbus, Ohio.

“Topline data from the Phase 1 study underscore the broad and consistent clinically transformative effect AVXS-101 appears to have on central nervous system and systemic features of SMA Type 1,” said Sean Nolan, President and Chief Executive Officer of AveXis. “These include event-free survival, motor function, pulmonary and nutritional support and achievement of developmental milestones – all of which are never seen in the well-characterized natural history of the disease.”

Results from the Phase 1 Trial of AVXS-101 in SMA Type 1 as Presented at AAN

The Phase 1, open-label, dose-escalating study was designed to evaluate the safety and tolerability of AVXS-101 in patients with SMA Type 1. The key measures of efficacy were the time from birth to an “event,” which was defined as either death or at least 16 hours per day of required ventilation support for breathing for 14 consecutive days in the absence of acute reversible illness or perioperatively, and video confirmed achievement of ability to sit unassisted. Additionally, several exploratory objective measures were assessed, including a standard motor milestone development survey and Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND).

Event-free Survival and Safety

- Data as of January 20, 2017, showed no new events, and 15 of 15 (100%) patients were event-free at 13.6 months. The expected event-free survival rate at 13.6 months based on the natural history of the disease is 25%. The median age at last follow-up was 20.2 months and 30.8 months for patients in the proposed therapeutic-dose cohort (Cohort 2) and low-dose (Cohort 1) respectively.

- AVXS-101 appeared to have a favorable safety profile and to be generally well tolerated, with no new treatment-related safety or tolerability concerns identified.

Nutritional and Respiratory Support

- According to natural history, nearly all Type 1 patients require nutritional and respiratory support by 12 months of age, and most SMA Type 1 babies are not able to swallow or speak effectively.

- As of January 20, 2017, six of seven (86%) patients in Cohort 2 that did not require feeding support before treatment continued without feeding support after treatment; seven of 10 (70%) patients that did not require bi-level positive airway pressure (BiPAP) support before treatment continued without any BiPAP after treatment.

- Further, as of January 20, 2017, 11 of 12 (92%) patients in Cohort 2 were fed orally, and six of 12 (50%) patients were exclusively fed orally; and eight of 12 (67%) patients were able to speak.

Motor Milestone Achievement

- As of January 20, 2017, 11 of 12 patients (92%) in Cohort 2 achieved head control, nine of 12 patients (75%) could roll a minimum of 180 degrees from back to both left and right, and 11 of 12 patients (92%) could sit with assistance.

- For the end-of-study assessment, AveXis evaluated three validated and well-established measures of sitting unassisted for periods of increasing duration. As of January 20, 2017, nine of 12 patients (75%) in Cohort 2 could sit unassisted for at least five seconds, seven of 12 patients (58%) could sit unassisted for at least 10 seconds and five of 12 patients (42%) could sit unassisted for 30 seconds or more.
- Dr. Mendell today reported three patients in Cohort 2 have achieved additional sitting unassisted milestones since the January 20, 2017 evaluation date. Ten of 12 patients (83%) in Cohort 2 could sit unassisted for at least five seconds, nine of 12 (75%) patients could sit unassisted for at least 10 seconds and eight of 12 patients (67%) could sit unassisted for 30 seconds or more in the post-January 20 analysis.

- As of January 20, 2017, two patients in Cohort 2 could crawl, pull to a stand, and stand and walk independently. For the end-of-study assessment, all motor milestone achievements were assessed and adjudicated by an independent third-party reviewer using video evidence. The post-January 20 developmental milestones were confirmed internally by video. Detailed Cohort 2 motor milestone data is included in the chart below.

### Motor Milestone Achievement as of January 20, 2017*

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<th>Cohort 2</th>
<th>Age at Gene Transfer (mos)</th>
<th>Brings Hand to Mouth</th>
<th>Head Control</th>
<th>Partial Roll*a</th>
<th>Rollb</th>
<th>Sitting with Assistance</th>
<th>Sitting Unassisted</th>
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- Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back in only one direction.
- Bayley Scales of Infant and Toddler Development, item #20, rolls a minimum 180° from back to both left and right.
- Sitting unassisted for ≥5 seconds is in accordance with the criteria of item 22 in the Bayley Scales of Infant and Toddler Development – gross motor subtest and surpasses the three-second count used as a basis for sitting (test item 1) in the Hammersmith Functional Motor Scale – Expanded for SMA (HFMSE).
- Sitting unassisted for ≥10 seconds is in accordance with the criteria in the World Health Organization – MultiCentre Growth Reference Study.
- Sitting unassisted for ≥30 seconds defines functional independent sitting and is in accordance with the criteria of item 26 in the Bayley Scales of Infant and Toddler Development – gross motor subtest.

*Milestone achievements as of January 20, 2017, except those indicated by an O, which were achieved after the January 20, 2017 cutoff.

“Babies born with SMA Type 1 that are untreated will never reach or maintain developmental milestones, which is why the videos presented today at AAN showing the achievement of motor milestones after a one-time infusion of AVXS-101 – including the majority of patients in Cohort 2, the proposed therapeutic-dose cohort, who were able to roll and sit unassisted – is incredibly remarkable,” said Dr. Mendell. “Importantly, AVXS-101 appears to demonstrate continued improvements past 13.6 months of age, with three new patients reaching sitting thresholds since the January 20, 2017 evaluation date.”

### Research Presented at AAN Further Scientific Understanding of AVXS-101

Brian Kaspar, PhD, Senior Vice President and Chief Scientific Officer of AveXis, will present his poster today, “CSF Delivery of AAV9-mediated Gene Therapy for SMA, a Lethal Neuromuscular Disease in Children: A Dose-response Study in Mice and Nonhuman Primates – data regarding a dose-response study for the CSF delivery of AVXS-101,” data from which support the use of intrathecal delivery of gene therapy for neurological diseases, such as SMA Types 2 and 3. The study offers insight into vector distribution and its correlation with transgene expression and provides guidance for future AAV9-based clinical trials in SMA, as well as other neurodegenerative disorders.

Douglas Sproule, MD, Vice President of Clinical Development, and Medical Affairs of AveXis, presented, “AVXS-101 Phase 1 Gene Therapy Clinical Trial in SMA Type 1: Experience with Preexisting Anti-AAV9 Antibody in the SMA1 Population,” results of which suggest pre-existing antibodies to AAV9 are quite uncommon in the pediatric population and will not impact use of gene therapy for the vast majority of SMA Type 1 patients. Two patients in the Phase 1 study who had modestly elevated anti-AAV9 titers, presumably resulting from maternal antibody transfer through breastfeeding or placental-transfer, showed resolution at retesting that enabled dosing.

Linda Lowes, PT, PhD, Director of Clinical Therapies Research and a member of the Center for Gene Therapy at the Research Institute of Nationwide Children’s Hospital, presented, “AVXS-101 Phase 1 Gene Therapy Clinical Trial in SMA Type 1: Correlation between CHOP-INTEND and Motor Milestone Achievements,” providing further evidence of the correlation between motor function and motor milestone achievement in patients with SMA Type 1. Specifically, this data demonstrates that the degree of treatment outcome appears to be influenced by age at dosing and baseline motor function.
AveXis will host a conference call and webcast at 4:30 p.m. EDT today, April 25, to discuss these data. 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 10345273. 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 until its next quarterly earnings call 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 10345273.

About the Phase 1 Trial Design
The Phase 1 open-label, dose-escalation clinical trial of AVXS-101 in patients with SMA Type 1 initiated in April 2014. Enrollement of 15 patients across two dosing cohorts was completed in December 2015. Patients received a one-time intravenous infusion of AVXS-101 over a one-hour period in a peripheral limb vein.

Patients in Cohort 1, the low-dose cohort (n=3), received 6.7E13 vg/kg. Patients in Cohort 2, the proposed therapeutic dose cohort (n=12), received 2.0E14 vg/kg. Key inclusion criteria included SMA Type 1 patients with clinical symptoms before six months of age, bi-allelic SMN1 gene deletions or point mutations and with two copies of the SMN2 backup gene, as determined by genetic testing. Of note, all patients in the trial had bi-allelic Exon 7 deletions in SMN1. A key exclusion criteria was the presence of c.859G>C point mutation in SMN2 (Exon 7 modifier). Additionally, patients must have been no older than nine months of age (for the first nine patients) and six months of age (for the last six patients) at the time of vector infusion.

The primary outcome measure of the Phase 1 trial was safety and tolerability. The key measures of efficacy were time from birth to an “event,” which was defined as either death or at least 16 hours per day of required ventilation support for breathing for 14 consecutive days in the absence of acute reversible illness or perioperatively, and video confirmed achievement of ability to sit unassisted. Additionally, several exploratory objective measures were assessed, including a standard motor milestone development survey and Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND).

The primary analysis for efficacy was assessed in January 2017 when all patients reached 13.6 months of age. A follow-up safety analysis will be completed when the last patient reaches 24 months post-dose.

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.

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, recently completed a 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 and statements about the effects of SMA Type 1 on developmental milestones. 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” section of AveXis’ Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on March 16, 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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