# AveXis Reports Interim Data from Ongoing Phase 1 Clinical Trial of AVXS-101 in Spinal Muscular Atrophy Type 1 as Presented at the International Annual Congress of the World Muscle Society

October 8, 2016

*-- Majority of patients on the proposed therapeutic dose achieved key developmental milestones including sitting unassisted; two patients walking independently --*

*-- Conference call and webcast October 10 at 8:30 a.m. EDT --*

CHICAGO--(BUSINESS WIRE)--Oct. 8, 2016-- 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 update on interim data from the ongoing Phase 1 trial of AVXS-101 in spinal muscular atrophy (SMA) Type 1 as of September 15, 2016. The data were presented by Jerry Mendell, M.D., director of the Center for Gene Therapy at The Research Institute at Nationwide Children's Hospital, at the 21st International Annual Congress of the World Muscle Society in Granada, Spain.

For the first time, interim data from the trial were presented that highlighted patient achievement of key motor development milestones as of September 15, 2016. Two-thirds of patients in Cohort 2 (the proposed therapeutic dose) had achieved the ability to sit unassisted, including one patient whose achievement of this milestone was confirmed after September 15. In Cohort 2, 11 of 12 patients achieved head control, 7 of 12 patients could roll over completely and 11 of 12 patients could sit with support. Two patients are now walking independently, including one whose achievement of this milestone was confirmed after September 15. These two patients each achieved earlier and important developmental milestones such as crawling, standing with support, standing alone and walking with support.

"To date, the majority of patients who received the proposed therapeutic dose of AVXS-101 have achieved key milestones and two-thirds of these patients can sit independently – a fact completely inconsistent with the known disease course, as children with untreated SMA Type 1 will never sit unassisted," said Sean Nolan, President and Chief Executive Officer, AveXis. "We are encouraged by these interim data, and continue to work diligently to bring this gene therapy to the children suffering from this devastating condition."

**Interim Phase 1 Data as of September 15, 2016**

- Data as of September 15, 2016 showed AVXS-101 continued to demonstrate a favorable safety profile and was generally well tolerated, with no new treatment-related safety or tolerability concerns identified.
    - There has been a cumulative total of 118 adverse events (AEs) reported as of September 15, 2016, 34 of which were determined to be serious adverse events (SAEs) and 84 were determined to be non-serious AEs. As previously reported, a total of 5 AEs in 4 patients were treatment-related. Two were deemed treatment-related SAEs (experienced by 2 patients) and three were deemed non-serious AEs (experienced by 3 patients). All consisted of clinically asymptomatic liver enzyme elevations.
    - All of the elevated liver enzyme AEs and SAEs were clinically asymptomatic and resolved with prednisolone treatment. There were no clinically significant elevations of gamma-glutamyl transferase (GGT), alkaline phosphatase or bilirubin, and as such Hy's Law was not met.
    - Other non-treatment-related AEs were expected and were associated with SMA.
- All patients in Cohort 2 (proposed therapeutic dose) are event-free, 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. The median age at last follow-up for Cohort 2 is 17.3 months, with the oldest patient at 27.4 months of age.
    - As previously reported, one patient in Cohort 1 (the low-dose cohort) did have a pulmonary event after July 1, 2016. The patient had increased use of bi-level positive airway pressure (BiPAP) in advance of surgery related to hypersalivation, a condition experienced by some SMA patients; the event was determined by independent review to represent progression of disease and not to be related to the use of AVXS-101.
- Mean increases in CHOP-INTEND scores of 9.0 points in Cohort 1 and 24.8 points in Cohort 2 were observed, reflecting improvement in motor function. The Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) is a test developed to measure motor skills of patients with SMA Type 1.
    - 11 out of 12 patients in Cohort 2 achieved CHOP-INTEND scores of at least 40 points.
    - 9 out of 12 patients in Cohort 2 achieved CHOP-INTEND scores of at least 50 points.
    - 3 out of 12 patients in Cohort 2 achieved CHOP-INTEND scores of at least 60, which is in a range considered to be normal.
- Patients on the proposed therapeutic dose of AVXS-101 consistently achieved and maintained key developmental motor milestones.

- As of September 15, 2016, 11 out of 12 patients achieved head control; 7 out of 12 patients could roll over (completely); 11 out of 12 patients could sit with support; and 8 out of 12 patients could sit unassisted, including one patient whose achievement of this milestone was confirmed after September 15.
- In addition, 7 patients are able to feed themselves, including one patient whose achievement of this milestone was confirmed after September 15, and 5 patients are speaking (1 bilingual).
- 4 patients are now standing with support, including two whose achievements of this milestone were confirmed after September 15.
- 2 patients are now walking independently, including one whose achievement of this milestone was confirmed after September 15. These two patients each achieved earlier and important developmental milestones such as crawling, standing with support, standing alone and walking with support.

Detailed Cohort 2 motor milestone data is included in the chart below:

**Motor Milestone Achievement as of September 15, 2016**

| **Cohort 2** | | | | | | | |
|---|---|---|---|---|---|---|---|
| **2.0e14 vg/kg** | **Age at Gene Transfer (mos)** | **Brings Hand to Mouth** | **Head Control** | **Rolls Over (partial)** | **Rolls Over (complete)** | **Sitting with Assistance** | **Sitting Unassisted** |
| **E.04** | 6 | X | X | X | | X | X |
| **E.05** | 4 | X | X | X | X | X | X |
| **E.06** | 2 | X | X | X | X | X | X |
| **E.07** | 4 | X | X | X | | X | X |
| **E.08** | 8 | X | | | | | |
| **E.09** | 5 | X | X | X | X | X | X |
| **E.10** | 1 | X | X | X | X | X | X |
| **E.11** | 2 | X | X | X | | X | X* |
| **E.12** | 3 | X | X | X | X | X | X |
| **E.13** | 1 | X | X | X | X | X | |
| **E.14** | 4 | X | X | X | X | X | |
| **E.15** | 2 | X | X | | | X | |

* Achievement confirmed after Sept 15, 2016

“The preliminary clinical observations of extended event-free survival, sustained increases in motor function and achievement of developmental milestones in patients receiving a one-time infusion of AVXS-101 have far exceeded what has been observed with natural history,” said Suku Nagendran, MD, Senior Vice President and Chief Medical Officer, AveXis. “These preliminary results demonstrate the potential of AVXS-101 to positively impact quality of life and as such alter the course of disease in these children with SMA Type 1.”

**Conference Call Information**

AveXis will host a conference call and webcast at 8:30 a.m. EDT Monday, October 10, 2016, to discuss the AVXS-101 clinical update.

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 93427499. 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 5 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 93427499.

**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.

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 1 is the leading genetic cause of infant mortality.

**About AVXS-101**

AVXS-101 is a proprietary gene therapy candidate of a one-time treatment for SMA Type 1 and is the only clinical-stage gene therapy in development for SMA. AVXS-101 is designed to address the monogenetic 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 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. 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' Annual Report on Form 10-Q for the quarter ended June 30, 2016, filed with the SEC on August 12, 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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Source: AveXis, Inc.

Media Inquiries:
W2O Group
Lauren Barbiero, 646-564-2156
lbarbiero@w2ogroup.com
or
Investor Inquiries:
AveXis, Inc.
Jim Goff, 650-862-4134
jgoff@avexis.com