AveXis Reports Third Quarter 2016 Financial and Operating Results

November 10, 2016

– AVXS-101 demonstrated continued motor function improvement in ongoing study; majority of patients on proposed therapeutic dose achieved key developmental milestones –

– Confirmed single-arm design for pivotal study of SMA Type 1 in U.S. –

– Conference call of November 1, 2016, was in lieu of Q3 earnings conference call –

CHICAGO, Nov. 10, 2016 (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 reported financial results for the third quarter ended September 30, 2016, recent corporate highlights and upcoming milestones.

"This has been another eventful quarter for AveXis, with continued positive data from the ongoing Phase 1 study of AVXS-101 in SMA Type 1, including, for the first time, reporting of motor milestone achievements," said Sean Nolan, President and Chief Executive Officer of AveXis. "We look forward to progressing AVXS-101 into pivotal studies in the U.S. and EU in the first half of next year, and are eager to initiate a Type 2 study as we continue to explore gene therapy for all patients suffering from SMA."

Recent Highlights

- **Interim Phase 1 Data**: On October 10, 2016, AveXis reported interim data as of September 15, 2016 from the ongoing Phase 1 trial of AVXS-101 in SMA Type 1, as 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.
- AVXS-101 appears to have a favorable safety profile and to be generally well tolerated in patients studied as of September 15, 2016. There had been a cumulative total of 118 adverse events (AEs) reported, 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 2 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) were 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 was 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) had a pulmonary event in the third quarter. 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.
- 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 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 were 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.

Pivotal Trial Design

- On November 1, 2016, AveXis announced the pivotal study of AVXS-101 in SMA Type 1 will reflect a single-arm design, using natural history of the disease as a comparator, and will enroll approximately 20 patients. This update was based on the receipt of the minutes following a Type B meeting with the U.S. Food and Drug Administration (FDA).
- In addition to evaluating safety, the planned program is expected to evaluate 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.
- The FDA also indicated its preference for a design with co-primary endpoints consisting of a measure of developmental milestone achievement (such as sitting unassisted) along with a clinically meaningful measure of survival (such as time to an "event" as described above). Based on FDA's suggestions as well as other expert input, AveXis continues to evaluate a number of the details of the trial design. More specific information will be made available at the time the study is initiated, which is expected in the first half of 2017.
- The Company's strategy with the SMA Type 1 program is to complete the ongoing Phase 1 trial and, in parallel, execute on the single-arm pivotal trial, while continuing collaborative discussions with the FDA regarding the most expeditious pathways for FDA approval of AVXS-101.

Common Stock Offering

- On September 13, 2016, the Company completed an underwritten public offering of 4,887,500 shares of its common stock, 4,597,645 shares of which were issued and sold by the Company, including the exercise in full by the underwriters of their option to purchase 637,500 shares from the Company, and 289,855 shares of which were sold by PBM Capital Investments, LLC (PBM), an existing stockholder of the Company, each at a public offering price of $34.50 per share. After deducting the underwriting discounts and commissions and other offering expenses payable by the Company, the net proceeds to the Company were approximately $149.1 million. The Company did not receive proceeds from the sale of the common stock by PBM.

Clinical Development Milestones

- Provide quarterly updates on the ongoing Phase 1 trial of AVXS-101 in SMA Type 1.
- Initiate a Phase 1 safety and dosing study of AVXS-101 via intrathecal (IT) delivery in patients with SMA Type 2 in the second half of 2016.
- Report 13.6 months of data for all patients in the ongoing SMA Type 1 Phase 1 trial in the first quarter of 2017.
- Initiate pivotal trials of AVXS-101 in patients with SMA Type 1 in the United States and Europe in the first half of 2017.

Third Quarter 2016 Financial Results

- **Cash Position:** As of September 30, 2016, AveXis had $263.6 million in cash and cash equivalents.
- **R&D Expenses:** Research and development expenses were $14.1 million for the third quarter of 2016 (which included $2.7 million of non-cash stock-based compensation expense), compared to $6.3 million for the same period in 2015 (which included $5.3 million of non-cash stock-based compensation expense), an increase of $7.8 million. The increase in research and development expenses was primarily attributable to an increase in expenses necessary to support the advancement of the Company's manufacturing product development efforts, clinical and pre-clinical programs, primarily the ongoing trial of AVXS-101 in SMA Type 1, and increases in employee compensation. Partially offsetting the increase in R&D spending was lower stock-based compensation expense of $2.6 million.
- **G&A Expenses:** General and administrative expenses were $7.1 million for the third quarter of 2016 (which included $2.7 million of non-cash stock-based compensation expense), compared to $3.7 million for the same period in 2015 (which included $1.5 million of stock-based compensation expense), an increase of $3.4 million. The increase in general and administrative expenses was primarily attributable to an increase in employee compensation, legal and professional fees and other infrastructure costs to support the company's overall growth, and higher stock-based compensation expense.
- **Net Loss:** Net loss was $21.1 million, or $0.87 per share, for the third quarter of 2016, compared to a net loss of $9.9 million, or $1.32 per share, for the third quarter of 2015.

Selected Financial Information

Operating Results:

	Three Months Ended September 30,	Nine Months Ended September 30,

	2016	2015	2016	2015
Revenue	$ -	$ -	$ -	$ -
Total revenue				
Operating Expenses:				
General and administrative	7,082,683	3,740,077	17,324,863	6,651,233
Research and development	14,097,713	6,289,350	40,542,323	18,756,214
Total Operating Expenses	21,180,396	10,029,427	57,867,186	25,407,447
Interest income	98,588	88,769	230,313	94,410
Net loss and comprehensive loss	$ (21,081,808)	$ (9,940,658)	$ (57,636,873)	$ (25,313,037)
Weighted-average basic and diluted common shares outstanding	24,166,113	7,504,148	20,958,421	7,042,977
Basic and diluted net loss per common share	$ (0.87)	$ (1.32)	$ (2.75)	$ (3.59)

Balance Sheet Information:

	September 30, 2016	December 31, 2015
Cash and cash equivalents	$ 263,642,069	$ 62,251,860
Total assets	284,803,706	65,084,291
Total liabilities	17,581,342	6,877,304
Accumulated deficit	$ (116,187,393)	$ (58,550,520)

Conference Call Information

The AveXis' conference call and webcast of November 1, 2016, was conducted in lieu of the previously announced conference call scheduled for Thursday, November 10, 2016 at 4:30 p.m. EST. AveXis will not host a conference call and webcast related to third quarter 2016 financial and operating results. The November 1 webcast is archived through November 30, 2016, on the Events and Presentations page of the company's website, and is available for telephonic replay through November 14, 2016, by dialing (855) 859-2056 (Domestic) or (404) 537-3406 (International), conference ID 12184733.

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 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 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, expectations regarding design and timing of the pivotal trial of AVXS-101 as well as the overall clinical development of AVXS-101. 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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