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AveXis Reports Fourth Quarter and Full Year 2017 Financial and Operating Results

Feb 27, 2018

– On track to request pre-BLA meeting with FDA in Q2 2018 –

– Intends to initiate pivotal trial in SMA Type 1 in Europe and multi-national pre-symptomatic SMA study in the first half of 2018 –

– Anticipates IND submissions for Rett syndrome and genetic ALS in late 2018/early 2019 –

– Conference call and webcast February 27 at 4:30 p.m. EST –

CHICAGO, Feb. 27, 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 reported financial results for the fourth quarter and full year ended December 31, 2017, recent corporate highlights and upcoming milestones.

"2017 was marked by significant progress for AveXis across multiple fronts, including alignment with the U.S. Food and Drug Administration (FDA) on our commercial manufacturing process for AVXS-101, publication of Phase 1 results for spinal muscular atrophy (SMA) Type 1 in the *New England Journal of Medicine* , and the initiation of pivotal SMA Type 1 and Phase 1 SMA Type 2 trials in the United States," said Sean Nolan, President and Chief Executive Officer of AveXis. "As we continue this momentum into 2018, we look forward to the pre-Biologics License Application (BLA) meeting with the FDA for AVXS-101, as well as to completing enrollment in our ongoing SMA Types 1 and 2 studies, and to expanding our clinical development program into other SMA sub-populations, and programs beyond SMA."

Recent Corporate Highlights

- **Expanded Product Development Pipeline:** On June 7, 2017, AveXis obtained exclusive worldwide rights to AAV9 from REGENXBIO for Rett syndrome (*MECP2* gene) and a genetic form of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (*SOD1*) gene. AveXis had previously licensed the preclinical data developed by Dr. Brian Kaspar from Nationwide Children's Hospital. These are rare, life-threatening, neurological monogenic diseases that have significant unmet need with either minimally effective or no available treatment options. AveXis expects to leverage its Good Manufacturing Practice manufacturing platform for these programs, which AveXis believes will reduce overall development timelines.
- **Announced Alignment with the FDA on Next Steps in Regulatory Pathway for AVXS-101 in SMA Type 1:** On January 4, 2018, following the receipt of minutes from the end-of-Phase 1 meeting with the FDA, AveXis announced plans to address detailed information requests from the agency by submitting the requested information to the investigational new drug (IND) application on an ongoing basis, and plans to submit the request for a pre-BLA meeting in the second quarter of 2018.
- **Acquired Exclusive Worldwide Rights from REGENXBIO to Entire NAV Technology Platform to**

Develop Treatments for SMA : On January 8, 2018, AveXis announced an amended licensing agreement, which expands the exclusive, worldwide license agreement to REGENXBIO's entire NAV platform for SMA, and permits license assignment by AveXis upon a change of control without consent from REGENXBIO.

- **Initiated Phase 1 Trial of AVXS-101 in SMA Type 2 (STRONG) Using Intrathecal Delivery and Product from the Company's Good Manufacturing Practice Commercial Process:** On January 16, 2018, AveXis announced the first patient was dosed in STRONG. Two patients have been dosed to date, with a four-week interval between the dosing of the first three patients for each dose cohort per the trial protocol.
- **Completed Public Offering with Net Proceeds of $431.9 Million :** On January 22, 2018, AveXis closed an underwritten public offering with net proceeds of $431.9 million. AveXis intends to use the net proceeds to fund its research, manufacturing and clinical activities to support its programs in SMA, Rett syndrome and genetic ALS; to fund pre-commercial activities, including medical affairs, development of commercial initiatives for the potential launch of AVXS-101; licensing activities; and for general corporate purposes and working capital.
- **Expanded SMA Clinical Development Program:** On January 26, 2018, AveXis announced plans to initiate three studies to further evaluate AVXS-101, including in new SMA patient populations.
- **Initiated Screening for Remaining Patients in U.S. Pivotal Trial of AVXS-101 for SMA Type 1 (STR1VE):** On January 30, 2018, following review of safety data and early signals of efficacy from the first three patients dosed, and with agreement from the FDA, AveXis initiated screening for the remaining patients in the trial. To date, five patients have been dosed.

Upcoming Milestones

- **Pre-BLA Meeting Request:** AveXis expects to submit the request for a pre-BLA meeting related to AVXS-101 in SMA Type 1 in the second quarter of 2018.
- **Pivotal Trial of AVXS-101 in SMA Type 1 in Europe (STR1VE EU):** The single-arm trial is expected to initiate in the first half of 2018 and enroll approximately 30 patients who are less than six months of age and naÃ¨ve to SMA treatment. STR1VE EU is designed to evaluate a one-time intravenous (IV) infusion of AVXS-101, including safety, event-free survival and achievement of the developmental milestone of sitting without support for 10 seconds, and will study children up to 18 months of age.
- **Pre-Symptomatic SMA Types 1, 2, 3 (SPRINT):** The multi-cohort, multi-national trial is expected to initiate in the first half of 2018 and enroll approximately 44 patients with two, three and four copies of *SMN2* who are less than six weeks of age and pre-symptomatic. SPRINT is designed to evaluate appropriate clinical endpoints, including developmental milestones, survival, bulbar function, and safety of a one-time IV infusion of AVXS-101. The primary efficacy endpoint by cohort is as follows:
  - Cohort with two copies of *SMN2* : proportion of patients who achieve functional independent sitting for at least 30 seconds, up to 18 months of age;
  - Cohort with three copies of *SMN2* : proportion of patients who achieve the ability to stand without support for at least three seconds, up to 24 months of age;
  - Cohort with four copies of *SMN2* : proportion of patients who do not manifest symptoms consistent with SMA Type 3 based on a scaled score on Bayley V.3 Gross and Fine Motor Subtests within 1.5 standard deviations of chronological development reference standard, as assessed at 36 months of age.
- **Pediatric SMA Types 1, 2, 3 (REACH)** : Expected to initiate late in the fourth quarter of 2018 or early 2019.
- **Rett Syndrome and Genetic ALS:** Expected to submit IND applications for AVXS-201 for Rett syndrome (*MECP2*) and AVXS-301 for genetic ALS (*SOD1*) in late 2018/early 2019.

Fourth Quarter and Full Year 2017 Financial Results

- **Cash Position:** As of December 31, 2017, AveXis had $324.1 million in cash and cash equivalents. This amount does not include the net proceeds from the recently completed public offering described above.
- **R&D Expenses:** Research and development expenses were $51.4 million for the fourth quarter of 2017 (which included $4.9 million of non-cash stock-based compensation expense), compared to $18.3 million for the same period in 2016 (which included $1.6 million of non-cash stock-based compensation expense), an increase of $33.1 million. The increase in research and development expenses was primarily attributable to increases in third-party clinical and manufacturing research and development spending related to AveXis' clinical trials and clinical trial product manufacturing, salaries and personnel-related expenses driven by increased headcount across all research, development and manufacturing functions, license fees and other research and development expenses.
- **G&A Expenses:** General and administrative expenses were $31.1 million for the fourth quarter of 2017 (which included $3.0 million of non-cash stock-based compensation expense), compared to $7.2 million for the same period in 2016 (which included $2.4 million of stock-based compensation expense), an increase of $23.9 million. The increase in general and administrative expenses was primarily attributable to a loss incurred on a non-cash common stock settlement and increases in pre-commercial marketing expenses, salaries and personnel-related costs driven by increased headcount across all general and administrative functions to support our overall growth, and legal, professional and consulting fees and other administrative costs.
- **Net Loss:** Net loss was $81.7 million, or $2.55 per share, for the fourth quarter of 2017, compared to a net loss of $25.4 million, or $0.92 per share, for the fourth quarter of 2016.

Selected Financial Information

Operating Results (In thousands, except per share data):

	Three Months Ended December 31,		Year Ended December 31,	
	2017	2016	2017	2016
Revenue	$ -	$ -	$ -	$ -
Operating Expenses:				
General and administrative	31,078	7,198	69,976	24,523
Research and development	51,445	18,350	150,391	58,892
Total Operating Expenses	82,523	25,548	220,367	83,415
Loss from operations	(82,523)	(25,548)	(220,367)	(83,415)
Interest income	858	173	2,316	403
Net Loss	$ (81,665)	$ (25,375)	$ (218,051)	$ (83,012)

Weighted-average basic and diluted common shares outstanding	32,030	27,678	29,935	22,648
Basic and diluted net loss per common share	$ (2.55)	$ (0.92)	$ (7.28)	$ (3.67)

Balance Sheet Information (In thousands):

	December 31, 2017	December 31, 2016
Cash and cash equivalents	$ 324,117	$ 240,430
Total assets	391,578	270,575
Total liabilities	58,838	24,443
Accumulated deficit	$ (359,613)	$ (141,562)

Conference Call Information

AveXis will host a conference call and webcast at 4:30 p.m. EST today, February 27, to discuss the fourth quarter and full year 2017 financial and operating results, recent business highlights and upcoming development milestones.

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 1599399. 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 1599399.

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 Rett Syndrome

Rett syndrome is a devastating, rare neurological disorder characterized by slowed growth, loss of normal movement and coordination and loss of communication skills. Rett syndrome is caused by an X-linked dominant mutation in the methyl CpG binding protein 2 (*MECP2*) gene, which results in problems with *MECP2*

protein production critical for brain development. Rett syndrome occurs in approximately one of every 10,000 female births in the U.S., and affected infants usually begin to show signs and symptoms between six and 18 months of age. Current treatments only offer symptomatic relief and do not target the genetic cause of the disease, leaving a significant unmet need.

About Genetic Amyotrophic Lateral Sclerosis

Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease that affects motor neurons in the brain and the spinal cord. Inherited forms of ALS comprise five to 10 percent of ALS cases, or approximately 1,000 to 2,000 people in the U.S., and can be caused by mutations in several genes known to be associated with ALS. Approximately 15 to 20 percent of these cases are caused by mutations in the gene that produces the copper zinc superoxide dismutase 1 (*SOD1*) enzyme, which leads to a progressive degeneration of motor neurons affecting movement and muscle control. ALS usually occurs in people between the ages of 40 and 70. Current treatments only offer modest benefits and do not target the genetic cause of the disease, leaving a significant unmet need.

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.

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' clinical 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 Type 2, the expected timing of future meetings with the FDA, the continued enrollment of patients in the STR1VE and STRONG clinical trials, the initiation of AveXis' planned future clinical trials of AVXS-101, AveXis' clinical development and regulatory plans for AVXS-201 and AVXS-301, and the planned expansion of AveXis' development of gene therapy into additional disorders. 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 and the information included in AveXis' Current Report on Form 8-K filed with the SEC on January 16, 2018. 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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