U NOVARTIS

AveXis to Report Fourth Quarter and Full Year 2017 Financial and Operating Results

Feb 20, 2018

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

CHICAGO, Feb. 20, 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, will report financial and operating results for the fourth quarter and full year ended December 31, 2017, as well as recent corporate highlights, on Tuesday, February 27, 2018, after the close of U.S.-based financial markets. AveXis management will host a conference call and live webcast at 4:30 p.m. Eastern Standard Time.

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, <u>www.AveXis.com</u>. 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. 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. 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 is a clinical-stage gene therapy company developing treatments for patients suffering from rare and life-threatening neurological genetic diseases. The company's initial product candidate, AVXS-101, is its proprietary gene therapy currently in the pivotal phase of study for the treatment of SMA Type 1, and in a Phase 1 trial for SMA Type 2. The company also intends to expand the study of gene therapy into two additional rare neurological monogenic disorders: Rett syndrome (RTT) and a genetic form of amyotrophic

lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (SOD1) gene.

For additional information, please visit <u>www.avexis.com</u>.

Media Inquiries: Lauren Barbiero W2O Group 646-564-2156 Ibarbiero@w2ogroup.com

Investor Inquiries: Jim Goff AveXis, Inc. 650-862-4134 jgoff@avexis.com

AveXis, Inc.

Source URL: https://prod1.novartis.com/news/media-releases/avexis-report-fourth-quarter-and-full-year-2017-financial-and-operating-results

List of links present in page

- 1. https://prod1.novartis.com/news/media-releases/avexis-report-fourth-quarter-and-full-year-2017-financialand-operating-results
- 2. http://www.AveXis.com
- 3. https://www.avexis.com
- 4. mailto:lbarbiero@w2ogroup.com
- 5. mailto:jgoff@avexis.com