AveXis Gene Therapy AVXS-101 Granted Access into EMA PRIME Program for Spinal Muscular Atrophy Type 1

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PRIME intended to optimize development and speed evaluation of AVXS-101 through enhanced interactions and early dialogue with EMA

CHICAGO, Jan. 31, 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 announced that the European Medicines Agency (EMA) has granted access into its PRIority MEdicines (PRIME) program for the company's proprietary gene therapy, AVXS-101, for the treatment of spinal muscular atrophy (SMA) Type 1. The PRIME application was based on data from both preclinical evaluations and the ongoing Phase 1 clinical trial of AVXS-101 as of September 15, 2016.

PRIME is intended to enhance support for the development of medicines – specifically those that may offer a major therapeutic advantage over existing treatments or benefit patients without treatment options – through early and proactive support by EMA to optimize the generation of robust data and development plans, and potentially expedite the assessment of the Marketing Authorization Application (MAA) so these medicines may reach patients sooner.

"The acceptance of AVXS-101 into the PRIME program reflects the urgent need for innovative treatment options for the patients diagnosed with SMA in the European Union," said James L'Italien, Ph.D., Senior Vice President, Chief Regulatory and Quality Officer of AveXis. "We are eager for this enhanced collaboration with the EMA to ensure we are taking the most appropriate and expeditious path toward the development of a robust Marketing Authorization Application submission, and to potentially streamlining the time needed to bring AVXS-101 to patients in the EU suffering from this devastating disease."

In notifying the company of the acceptance, EMA noted: "The preliminary clinical observations following AVXS-101 administration include positive impact on survival, pulmonary function, nutritional support, preservation of motor function and the attainment of development milestones, all of which are unexpected within the framework of the natural history and disease progression for SMA Type 1. These clinically meaningful responses in the patients treated with AVXS-101 are sufficient preliminary clinical evidence of treatment effect that has the potential to address an unmet need in this devastating pediatric disease."

In July 2016, the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation, a comparable program to PRIME used by the FDA, for AVXS-101 for the treatment of patients with SMA Type 1.

AveXis intends to initiate a pivotal trial of AVXS-101 in patients with SMA Type 1 in the European Union before the end of 2017.

About PRIME

The EMA launched the PRIME initiative in March 2016 to foster research and development of medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. PRIME aims to strengthen clinical trial designs to facilitate the generation of high quality data for the evaluation of an application for marketing authorization. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on preclinical and/or early clinical data. These medicines are considered priority medicines within the European Union.

After an investigational candidate has been selected for PRIME, developers are assigned a rapporteur from the Committee for Medicinal Products for Human Use (CHMP) to provide continuous support and help to build knowledge ahead of a Marketing Authorization Application (MAA). A multidisciplinary group of experts will provide broader guidance on the overall development plan and regulatory strategy of the product. Companies are also eligible for accelerated assessment at the time of their regulatory application.

For more information, please visit the research and development section of www.ema.europa.eu.

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. Approximately 30 percent of cases are expected to be SMA Type 2. SMA Type 2 typically presents between six and 18 months of age. Affected patients will never walk without support, and SMA Type 2 results in mortality for more than 30 percent of patients by age 25.

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, 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 benefit patients with SMA Type 1, the potential expediting and streamlining of the European regulatory approval process for AVXS-101 resulting from the acceptance of AVXS-101 into the EMA's PRIME program, expectations regarding timing of the planned European Union pivotal trial of AVXS-101 in patients with SMA Type 1 and 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 European Union, 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' Quarterly Report on Form 10-Q for the quarter ended September 30, 2016, filed with the SEC on November 10, 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.

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

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

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