AveXis Gene Therapy Awarded SAKIGAKE Designation for Spinal Muscular Atrophy Type 1

Mar 27, 2018

CHICAGO, March 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 announced that Japan's Ministry of Health, Labour and Welfare (MHLW) awarded the company's initial product candidate, AVXS-101, SAKIGAKE Designation (SAKIGAKE) for the treatment of spinal muscular atrophy (SMA) Type 1. The designation was based on data from the Phase 1 clinical trial of the company's proprietary gene therapy.

SAKIGAKE is intended to promote research and development in Japan for innovative new medical products that satisfy certain criteria, such as the severity of the intended indication, by providing prioritized consultation review during the early stages of development and by shortening the target review period for license applications from 12 months to as few as six months. The benefits of SAKIGAKE Designation are similar to the Breakthrough Therapy Designation in the United States and access into the PRIority MEdicines (PRIME) scheme in the EU, both of which have already been granted to AVXS-101.

"This designation by the MHLW underscores the agreement by the Japanese government that there is an urgent need for a new therapeutic approach to treat Japanese patients diagnosed with SMA Type 1, and allows for enhanced discussions between AveXis and the agency to potentially expedite the timeline for approval of AVXS-101," said Sean Nolan, President and Chief Executive Officer of AveXis.

James L'Italien, Chief Regulatory and Quality Officer of AveXis, stated, "We look forward to continuing to work closely with the Japanese authority as well as regulatory authorities around the globe in our effort to bring this gene therapy to SMA patients and their families as safely and efficiently as possible."

About SAKIGAKE Designation
The MHLW in Japan formed the "Strategy of SAKIGAKE" in June 2014 in an effort to facilitate the review and approval of innovative medical products. The Strategy of SAKIGAKE consists of two measurements – SAKIGAKE Designation and the Scheme for Rapid Authorization of Unapproved Drugs – covering basic research to clinical research and trials, approval reviews, safety measures, insurance coverage, improvement of infrastructure and the environment for corporate activities, and global expansion. The SAKIGAKE Designation System intends to promote research and development in Japan aiming at shortening premarket review period for innovative new medical products that satisfy certain criteria, such as severity of intended indication, by designating such products during the early stages of development, and providing prioritized consultation services and premarket pharmaceutical affairs review.

For more information, please visit Strategy of SAKIGAKE section of http://www.mhlw.go.jp.

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 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 SMA Type 1, the leading genetic cause of infant mortality, and SMA Type 2. The U.S. Food and Drug Administration 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' research, development and regulatory plans for 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 and regulatory developments in the U.S., EU and Japan, 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, 2017, filed with the SEC on February 28, 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.

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

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

Source: AveXis, Inc.

Source URL: *https://prod1.novartis.com/news/media-releases/avexis-gene-therapy-awarded-sakigake-designation-spinal-muscular-atrophy-type-1*

List of links present in page

1. https://prod1.novartis.com/news/media-releases/avexis-gene-therapy-awarded-sakigake-designation-spinal-muscular-atrophy-type-1
2. http://www.mhlw.go.jp
3. https://www.avexis.com
4. mailto:lbarbiero@w2ogroup.com
5. mailto:jgoff@avexis.com