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AveXis Enters into Licensing Agreement with Genethon

Includes exclusive worldwide rights to AAV9-SMN product and route of administration

Chicago, Ill. and Evry, France (March 13, 2018) – AveXis, Inc. (NASDAQ:AVXS) and Genethon today announced they have entered into an exclusive, worldwide license agreement for *in vivo* gene therapy delivery of AAV9 vector into the central nervous system (CNS) for the treatment of spinal muscular atrophy (SMA).

"Adding to our robust intellectual property estate, this agreement further strengthens our position by providing freedom to operate when using intravenous or intrathecal routes of administration to deliver the AAV9 vector into the CNS for the treatment of SMA," said Sean Nolan, President and Chief Executive Officer of AveXis. "With our proprietary gene therapy, AVXS-101, currently being evaluated in patients with SMA in ongoing clinical trials in the U.S., and soon in Europe, we are pleased to have this exclusive worldwide agreement in place."

Under the terms of the agreement, Genethon granted AveXis a license to patents in the U.S., Europe and Japan, for the AAV9 SMN product and *in vivo* gene therapy delivery of AAV9 vector into the CNS using intrathecal or intravenous routes of administration for the treatment of SMA.

"Genethon is pleased to enter into this agreement with AveXis and to contribute to the efforts for the development of treatments for SMA patients who have urgent medical needs," said Frédéric Revah, Chief Executive Officer of Genethon. "It demonstrates Genethon's capability to develop effective first-in-class technologies and the excellence of our translational research driven by the commitment to treat patients living with rare diseases."

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 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.

About Genethon
Created by the AFM-Telethon, the French Muscular Dystrophy Association (AFM), Genethon, located in Evry, France, is a non-profit R&D organization dedicated to the development of biotherapies for orphan genetic diseases, from the research to clinical validation. Genethon is specialized in the discovery and development of gene therapy drugs and has multiple ongoing programs at clinical, preclinical and research stage for neuromuscular, blood, immune system, and liver diseases.

AveXis 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' freedom to operate afforded by the license agreement with Genethon and 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; 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, 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.

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