REGENX BIOSCIENCES AND AVEXIS ENTER INTO EXCLUSIVE LICENSE AGREEMENT FOR THE DEVELOPMENT OF TREATMENTS FOR SPINAL MUSCULAR ATROPHY USING NAV® rAAV9 VECTORS

Washington, DC and Dallas, TX - REGENX Biosciences, LLC (REGENX) and AveXis, Inc. (AveXis) announce that they have entered into an exclusive agreement for the development and commercialization of products to treat Spinal Muscular Atrophy (SMA) using **NAV** rAAV9 vectors.

Under the terms of the agreement, REGENX granted AveXis an exclusive, worldwide license, with rights to sublicense, to REGENX's **NAV** rAAV9 vector for treatment of SMA disease in humans. In return for these rights, REGENX receives an up-front payment, certain milestone fees and royalties on net sales of products incorporating **NAV** rAAV9.

"We believe this exclusive license agreement is important to the successful development of **NAV**-based gene delivery treatments for patients with SMA," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to be formally collaborating with AveXis which has assembled a world class team of scientific and clinical experts in SMA, led by Brian Kaspar, Ph.D. and his colleagues at Nationwide Children's Hospital and The Ohio State University, who have demonstrated tremendous dedication to the development of innovative gene therapy treatments for patients with SMA."

"AveXis is committed to the development of new treatments for patients with SMA using **NAV**-vector technology. We feel rAAV9 is the most promising vector to achieve this goal. We call it our 'special snowflake', because, AAV9 has unique properties that allow us to develop novel targeted treatments for infants with SMA. We've named the product chariSMA® from Greek origin meaning 'a gift of grace'," said John A. Carbona, CEO of AveXis. "Everyone associated with our SMA program is very pleased to establish this agreement with REGENX, which expands our leadership position in SMA gene therapy and supports a strong foundation for our team to continue to develop novel therapies for patients with all types of SMA."

About Spinal Muscular Atrophy

Spinal muscular atrophy (SMA) is an autosomal-recessive genetic disorder characterized by progressive weakness of the lower motor neurons. SMA is caused by a genetic defect in the *SMN1* gene, which codes SMN, a protein necessary for the survival of motor neurons. SMA kills more infants than any other genetic disease in the world today.

About REGENX Biosciences

REGENX Biosciences (www.regenxbio.com) is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary **NAV** vector technology platform, for a range of severe diseases with serious unmet needs. **NAV** vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX's leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, Audentes Therapeutics, Lysogene, and Esteve. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases. For more information regarding REGENX, please visit www.regenxbio.com.

About AveXis

Based in Dallas, Texas, AveXis is a private clinic-ready synthetic biology platform company establishing unique industry alliances to create innovative treatments for people with unmet medical needs. Spinal muscular atrophy is the company's first focus.

For more information regarding AveXis, please visit www.avexisinc.com.

Contacts:

REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio.com

AveXis
Corporate Contact:
John A. Carbona, Chief Executive Officer
972-725-7797 or jc@avexisinc.com
Media Contact:
Jillian Bowman, Administrative Specialist
972-725-7797 or Jillianb@avexisinc.com