

PRESS RELEASE:

AveXis to Advance Gene Therapy Program Initiated by the Rett Syndrome Research Trust

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TRUMBULL, CT – The Rett Syndrome Research Trust (RSRT) is excited to announce that AveXis, Inc., has committed to advance a gene therapy candidate for Rett Syndrome into human clinical trials. AveXis, a pioneering clinical-stage gene therapy company, has already advanced a transformative gene therapy treatment for spinal muscular atrophy Type 1, a devastating disease that strikes infants and kills in toddlerhood. Remarkably, in a Phase 1 clinical trial, infants treated with the gene therapy achieved developmental milestones, including the ability to sit unassisted, talk and walk in some cases, – achievements never seen in untreated babies with the disease. It is our shared belief that gene therapy, the introduction of healthy *MECP2* genes to compensate for the mutated ones, may also produce benefits for individuals with Rett Syndrome.

This milestone represents the culmination of research originating in 2010 when RSRT funded the collaboration between Dr. Gail Mandel and Dr. Brian Kaspar, the now scientific founder and Chief Scientific Officer of AveXis, to explore the potential of gene therapy for the treatment of Rett Syndrome. Encouraged by the results of that collaboration, in 2014 RSRT conceived of, recruited the scientists for and funded the *Gene Therapy Consortium* to definitively evaluate the feasibility of gene therapy as a treatment for Rett Syndrome.

The *Consortium* scientists, Stuart Cobb, Steve Gray, Brian Kaspar and Gail Mandel, exceeded our expectations by developing a gene therapy product candidate with impressive efficacy, safety and delivery characteristics. Importantly, therapeutic benefits in the mouse models are much greater than that of any drug ever tested for Rett Syndrome.

Adrian Bird's 2007 reversal study showing that symptoms in mice can be dramatically reversed suggests that a diagnosis of Rett need not translate to lifelong disability. While gene therapy seems an obvious approach for any of the numerous single gene disorders, the quality of the data generated by the *Consortium* was an essential factor in AveXis' decision to prioritize development of a gene therapy for Rett Syndrome.

"RSRT recognized early on that the gene therapy approach would move forward only if we aggressively championed it. We brought together scientists with strong track records of success and provided them with the financial support and infrastructure to work effectively and efficiently. Our strategy worked and I am thrilled that AveXis, a leader in gene therapy, is now taking this work forward at an industry scale. I am beyond grateful to the many Rett families who believe in our vision and to the generosity of their networks that make our vital work possible," said Monica Coenraads, Executive Director of RSRT and mother to a young woman with Rett Syndrome.

"As was made clear in RSRT's recently launched strategic research plan, *Roadmap to a Cure*, our mission is a cure for Rett Syndrome. Today's announcement is a huge step forward towards our goal. By addressing the core cause of Rett, gene therapy has the potential to be a life-changer. We have complete confidence in the expertise and leadership of the AveXis team and will continue to provide our full support as this program moves forward," said Randall Carpenter, Chief Scientific Officer, RSRT.

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“Without RSRT championing gene therapy for the treatment of Rett Syndrome, we would not be where we are preclinically in our understanding of the potential for gene therapy to treat this devastating disease,” said Brian Kaspar, Chief Scientific Officer of AveXis. “Bringing our expertise and focus on rare monogenic diseases, we are excited by the possibility that gene therapy may address the needs of individuals with Rett Syndrome.”

About the Rett Syndrome Research Trust

The Rett Syndrome Research Trust (RSRT) is a nonprofit organization with a highly personal and urgent mission: a cure for Rett Syndrome and related *MECP2* disorders. In March of 2017 RSRT announced *Roadmap to a Cure*, a three-year, \$33 million strategic research plan. The plan prioritizes four curative approaches with gene therapy as our lead program. RSRT operates at the nexus of global scientific activity enabling advances in knowledge and driving innovative research through constant engagement with academic scientists, clinicians, industry, investors and affected families. Since 2008, RSRT has awarded \$42 million to research. To learn more, please visit www.reverserett.org

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, recently completed a Phase 1 clinical trial for the treatment of SMA Type 1. For additional information, please visit www.avexis.com

About Rett Syndrome

Rett Syndrome is a genetic neurological disorder that almost exclusively affects girls. It strikes randomly, typically at the age of 12 to 18 months, and is caused by random mutations of the *MECP2* gene on the X chromosome. Rett Syndrome is devastating as it deprives young children of speech, hand use, normal movement often including the ability to walk. As the children enter childhood the disorder brings anxiety, seizures, tremors, breathing difficulties, severe gastrointestinal issues. While their bodies suffer, it is believed that their cognitive abilities remain largely intact. Although most children survive to adulthood, they require total round-the-clock care.