PRESS RELEASE:

RSRT Names Timothy A. Riley Chief Scientific Officer and Chief Business Officer; Randall Carpenter Transitions to Chief Medical Officer

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TRUMBULL, CT — The Rett Syndrome Research Trust (RSRT) is delighted to announce that Timothy A. Riley, Ph.D. has joined the organization in the role of Chief Scientific Officer and Chief Business Officer. Randall Carpenter, M.D. who previously held the position of Chief Scientific Officer is transitioning to Chief Medical Officer.

The decision to expand the research team is in response to numerous recent scientific breakthroughs that enable potentially transformative therapeutics for individuals with Rett Syndrome. RSRT has a growing portfolio of therapeutic development programs that require expert oversight and concurrently a number of other therapeutics are advancing toward clinical trials. Dr. Riley will focus his deep expertise in translating scientific discoveries into therapeutics and his business development experience toward advancing and successfully partnering these programs with biopharmaceutical companies. Dr. Carpenter will now focus his attention on the programs that are advancing toward clinical trials.

Dr. Riley, a seasoned professional with a thirty-year track record in the pharmaceutical industry brings expertise in rare diseases, polymer technologies, large molecules, peptides and proteins, and small molecules. He has led groups in drug discovery, pre-clinical development, non-clinical pharmacokinetic/pharmacodynamic modeling, early formulations, pre-clinical toxicology and quality. His business development experience as an Entrepreneur-in-residence at Yale University and as Associate Vice Chancellor for Innovation & Business Development at the University of Massachusetts Medical School will be highly relevant as RSRT facilitates relationships among researchers, investors, and industry to bring discoveries out of the lab and into the clinic. Dr. Riley, who holds a BS in chemistry and a PhD in organic chemistry from Brigham Young University, will manage the research portfolio, prioritize existing and new opportunities in academia and industry, and develop strategies to address research knowledge gaps.

"The time is right to cure Rett. I am absolutely thrilled to join this great team at RSRT and appreciate their confidence in me. Dr. Carpenter, Dr. von Hehn and Monica Coenraads have made so many advances in a very short period of time with their strategically chosen industry and academic partners. I look forward to working with them and the rest of the RSRT team, board, companies, and academic investigators to move multiple programs into the clinic and find a cure for Rett Syndrome," shared Dr. Riley.

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With the ideal mix of drug development skills and translational medicine experience gained during his tenure as Chief Medical Officer and Chief Executive Officer of several biopharmaceutical companies, Dr. Carpenter is well positioned to assume the new role of Chief Medical Officer. He will oversee a number of clinical efforts including the identification and adoption of clear outcome measures for trials, the search for biomarkers, and our Clinical Trial Consortium. Dr. Carpenter received his medical degree from the University of Michigan Medical School.

"Recent scientific advances make it possible for the first time to pursue therapeutic development strategies that have a realistic potential to cure Rett Syndrome. The addition of Dr. Riley to our team greatly increases the likelihood that we will successfully translate these discoveries into novel therapeutics," said Dr. Carpenter.

Rounding out the research team is Dr. Jana von Hehn, Director of Research, whose experience in clinical development planning, protocol development, novel endpoint design, clinical trial execution, data analysis and reporting is invaluable. Dr. von Hehn earned her Ph.D. in Genetics and Molecular Biology from Emory University.

Collectively the RSRT research team of Drs. Carpenter, Riley and von Hehn has:

- Over 60 years of drug development experience
- Over 40 years of clinical trial experience
- Successfully opened 17 INDs (investigational new drug application)
- Moved 9 programs from basic research to clinical stage which led to over $6 billion in market capitalization
- Responsible for 94 trials performed in compliance with FDA standards for Good Clinical Practice and over 25 additional academic clinical trials

"As RSRT celebrates its 10th anniversary this week it is my distinct pleasure to welcome Tim Riley to our research team. Together our team has the experience, passion and urgency to drive our mission forward to a cure. We have and will continue to take full advantage of exciting novel research technologies to ensure success," stated Monica Coenraads, Executive Director of RSRT and mother of a young woman with Rett Syndrome.

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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. RSRT operates at the nexus of global scientific activity enabling advances in knowledge and driving innovative research. In March of 2017 RSRT announced *Roadmap to a Cure*, a three-year, $33 million strategic research plan. The plan, for which nearly $18 million has been pledged or contributed by generous donors, prioritizes four curative approaches with gene therapy as our lead program. In June of 2017 the biotechnology company, AveXis, announced its intent to advance RSRT's gene therapy program to clinical trials. Since 2008, RSRT has awarded $47 million to research. To learn more, please visit ***www.reverserett.org***

About Rett Syndrome

Rett Syndrome is a genetic childhood neurological disorder caused by random mutations of the *MECP2* gene on the X chromosome that affects predominately girls but can rarely also affect boys. Its symptoms typically become apparent between the ages of 12 to 18 months. Rett Syndrome is devastating as it deprives young children of speech, hand use, and normal movement often including the ability to walk. As the children enter childhood the disorder brings anxiety, seizures, tremors, breathing difficulties, and 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.