Press Release:
Rett Syndrome Research Trust Names Randall L. Carpenter as Chief Scientific Officer

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The Rett Syndrome Research Trust (RSRT) is delighted to announce that Randall L. Carpenter is joining the organization in the role of Chief Scientific Officer. In this newly created position, Dr. Carpenter, who will start on March 1, will be responsible for accelerating the translation of basic science discoveries into medications that will profoundly improve the lives of individuals with Rett Syndrome and MECP2 disorders. *"Every decision we make at RSRT,"* states its co-founder and Executive Director Monica Coenraads, *"is made with one goal in mind – healing our children as fast as we can.* ***Having Randy on board will dramatically expedite this goal.****"*

Dr. Carpenter begins his work at RSRT at a very exciting time for Rett Syndrome research. The field has seen explosive growth in the last fifteen years. Tenacious work has uncovered a rich array of potential drug targets including strategies that target the root cause of the disorder. These successes have shone a spotlight on Rett and have incited considerable attention on the disorder from both pharmaceutical and biotech companies. With the foundation laid, much of it with RSRT funding, it's time to translate the discoveries from lab to clinic.

Neither the FDA nor its European equivalent, the EMA, have ever approved a drug for Rett Syndrome. Successful drug approval will require defining a regulatory pathway that includes the identification and adoption of clear outcome measures for clinical trials. Importantly, the pathway will also elucidate clinical trial designs that take into account the rare nature of the disorder and the challenge of broad symptom variability. Educating the FDA about the severity of the disorder and its debilitating symptoms is also crucial. Defining such a regulatory pathway will require an intense and thoughtful initiative that will engage Rett clinicians and scientists, industry, investors, NIH, FDA and affected families.

With the ideal mix of drug development skills and translational medicine experience, Dr. Carpenter is well positioned to lead such an initiative. His over 30 years of experience in the pharmaceutical and biotechnology industries include roles as Co-Founder, President and CEO of Seaside Therapeutics; President and CEO of Sention; VP of Clinical Research & Development and Regulatory Affairs at Adolor Corporation and a member of Astra Pain Control's Global Therapeutic Area Team.

While in industry Dr. Carpenter led translational medicine teams responsible for eight successful IND (investigational new drug) submissions to the FDA and dozens of clinical trials. Dr. Carpenter's experience with the EMA and undertaking clinical trials in Europe will be very useful.

Beyond defining the regulatory pathway Dr. Carpenter will manage RSRT's growing research portfolio, prioritize existing and new opportunities in academia and industry, and develop strategies to address research knowledge gaps.

Dr. Carpenter currently serves on the External Advisory Board for the Translational Neuroscience Center at Boston Children's Hospital, the Scientific Advisory Board of EU-AIMS, and the Board of Directors of Forum Pharmaceuticals. He recently completed a 4-year term as a member of the National Advisory Mental Health Council.

You can view his ***profile page*** on our website.

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"I've known Randy for about a decade and have routinely gone to him for advice and brainstorming. I could not be happier that he will now be focused exclusively on Rett and MECP2 disorders. Having spent eight years as CEO of a company developing treatments for Fragile X, Randy understands the challenges of working in the neurodevelopmental space and has the skillset and urgency to lead the Rett community through the important next phase. I look forward to working closely with him on behalf of every child and adult suffering with Rett and MECP2 disorders."

MONICA COENRAADS
Executive Director, RSRT

"Decades of progress in genetics and fundamental neurobiology have produced technologies enabling research that, for the first time, provide insights for developing therapeutics that specifically target the molecular mechanisms underlying Rett Syndrome. I am thrilled to join RSRT during this auspicious moment in time, and look forward to helping Monica leverage the strong relationships already established with numerous stakeholders, establish new innovative collaborations and rapidly facilitate progress in translational medicine."

DR. RANDALL CARPENTER
Chief Scientific Officer, RSRT

"Late last year Monica came to the board with a recommendation and plan for hiring a CSO. We recognized that with increased industry interest and a growing number of drug targets the stakes were getting higher and the time was right. RSRT undertook an extensive search and we are delighted that it ended with Randy joining our team. The combination of Monica's passion and knowledge of the Rett field and Randy's experience will make for a formidable partnership."

TONY SCHOENER
Chairman of the Board, RSRT

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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 girls of speech, hand use, normal movement often including the ability to walk. As the girls 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.

About the MECP2 Disorders

Beyond Rett Syndrome MECP2 disorders can range from mild learning disabilities to neonatal encephalopathy and syndromic or non syndromic intellectual disability. The MECP2 Duplication Syndrome is caused by having an area of the X chromosome (Xq28), which includes the MECP2 gene, erroneously duplicated. The core phenotypes include infantile hypotonia, developmental delay, absent to minimal speech, recurrent infections, progressive spasticity especially of the lower limbs, ataxia, autistic features, and seizures.

About the Rett Syndrome Research Trust

The Rett Syndrome Research Trust (RSRT) is a non-profit organization with a highly personal and urgent mission: a cure for Rett Syndrome and related MECP2 disorders. In search of a cure and effective treatment options, RSRT operates at the nexus of global scientific activity. We enable advances in knowledge and drive innovative research through constant engagement with academic scientists, clinicians, industry, investors and affected families. These relationships catalyze the development and execution of a research agenda that neither academia nor industry could achieve alone. RSRT refutes the conventional practice of labs working in isolation, instead seeking out, promoting and funding collaborations and consortia in which scientists work across multiple disciplines. Since 2008, RSRT has awarded $34 million to research projects. To learn more about the Trust, please visit **www.ReverseRett.org**.