

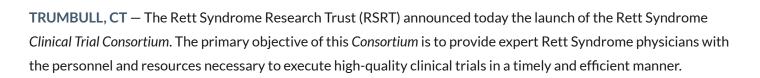
PRESS RELEASE:

RSRT Launches Rett Syndrome Clinical Trial Consortium with \$1.8 Million Award

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During the last decade, the Rett Syndrome basic science community, fueled by millions of dollars in research investments, has made phenomenal progress in understanding the disease. Coupled with technological advances in a number of fields, including gene therapy, we are now on the brink of advancing potentially life-changing therapeutics into human clinical trials.

These human clinical trials will generally be undertaken at Rett Syndrome Clinics, many of which are facing a number of key challenges due to lack of funding.

Firstly, there are a limited number of Rett physicians who have sufficient expertise and resources to participate in clinical research that meets rigorous FDA standards. Secondly, most Rett Clinics are resource constrained and constantly challenged to support and retain the highly trained personnel necessary to conduct rigorous clinical research. Lastly, lack of funding and trained personnel impacts the Rett clinician's ability to analyze and publish data in a timely fashion, potentially delaying the sharing of important information.

For all these reasons, the time is right to invest in a *Clinical Trial Consortium* to support rigorous testing of the therapeutics we have worked so hard to develop.

The *Clinical Trial Consortium* will launch with an initial investment of \$1.8 million to support four Rett Clinics. As our funding allows, we hope to grow the number of Clinics in the *Consortium*. This initial grant of \$1.8 million will support full-time research support staff (research coordinators, nurses and/or physicians in training) for three years at the following Clinics:

- 1. Center for Rare Neurological Diseases (CRND, Atlanta); Dr. Daniel Tarquinio
- 2. Montefiore Medical Center (MMC, Bronx NY); Dr. Sasha Djukic
- 3. Children's Hospital of Philadelphia (CHOP); Dr. Eric Marsh
- 4. Boston Children's Hospital (BCH); Drs. David Lieberman & Mustafa Sahin

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While this funding will allow the v to focus their efforts on clinical trials that provide the most benefit to the Rett community, a complementary outcome will be to leverage the resources and infrastructure developed in our *Outcome Measures and Biomarker Development Consortium* (OMBD) to improve clinical care for individuals with Rett Syndrome. Specifically, the outcome measures developed in the OMBD will be used to track and evaluate whether changes in clinical care (e.g., drugs or biologicals, diet, therapy) result in improved health for individual patients.

"As the principal investigator or sponsor of over 75 clinical trials during my academic and biotechnology career, I intimately understand the challenge of performing high quality clinical trials in a busy medical practice. I look forward to working closely with these investigators, openly sharing our insights and results with the many other expert investigators that we were regrettably unable to fund in this initial effort, and hopefully, ultimately recruiting these investigators into the *Clinical Trial Consortium*," said Randall Carpenter, MD, Chief Scientific Officer of RSRT.

"It's a very exciting time for Rett research as we've reached the point where clinical trials of potentially curative approaches are imminent. I could never thank our generous donors and the families that fundraise for RSRT enough. We're at this stage because of you," said Monica Coenraads, Executive Director of RSRT and mother to a young woman 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. 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 prioritizes four curative approaches with gene therapy as our lead program. This June the biotechnology company, AveXis, announced its intent to advance RSRT's gene therapy program to clinical trials. Since 2008, RSRT has awarded \$44 million to research. To learn more, please visit <u>www.reverserett.org</u>

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, 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.