

Rett Syndrome Research Trust



67 Under Cliff Road
Trumbull, CT 06611
(203) 445-0041
www.rsrt.org



Mission

The Rett Syndrome Research Trust (RSRT) was established to accelerate and intensify scientific exploration of Rett Syndrome, which is the most physically disabling of the autism spectrum disorders. RSRT is a nonprofit devoted exclusively to science. It emphasizes transparency, accountability, and dedication in pursuit of its mission to develop effective treatments or a cure for Rett Syndrome.

Strategy/Approach

RSRT pursues two primary approaches to reversing Rett Syndrome. The first is to understand the function of the MeCP2 protein and to design rational drugs to compensate for its deficit. The second approach is to identify the various outcomes of having an MeCP2 deficiency and screen for anything that ameliorates that outcome. RSRT funds and supports research relevant to both approaches.

- The **MeCP2 Gene Therapy Consortium** comes after the success of the MeCP2 Consortium, which led to initial gene therapy findings. With a budget of \$1.6 million, RSRT is mobilizing the international gene community to get to clinical trials as quickly as possible.
- Two **gene therapy projects** supported by RSRT pair experienced basic scientists with deep knowledge of the Rett animal models with researchers in the world of gene therapy.

The trust also closely monitors not only the efforts of scientists working on MeCP2 but also the research community at large so that it can quickly identify and recruit the interest of scientists doing synergistic work. Beyond financial support to those scientists, it provides access to intellectual capital and facilitates the sharing of research tools and data.

Additionally, RSRT raises general awareness of Rett through its events, public campaigns, documentaries, and its website, Twitter and Facebook accounts. RSRT also builds knowledge and awareness about Rett within the scientific community via conferences convened around specific sets of questions.

Research Portfolio

RSRT is not a traditional grant-making organization. Rather, it focuses on identifying the research agenda and assisting in its execution. Most of the projects it funds were originated by RSRT and its advisors. The trust identifies promising research concepts, seeks out individual projects, and offers to fund the research.

RSRT supports research in three categories:

- **Treatment**—targeting downstream effects of MECP2 mutations via drugs and/or procedures. Largely a symptom-by-symptom approach to improving quality of life
- **Basic Science**—expanding the knowledge base of the neurobiology of Rett
- **Reversal**—approaches that target the underlying cause of Rett, aberrations in MECP2. Includes gene therapy, activation of silent MECP2 genes, and identification and deployment of modifier genes.

Partnership Practices

RSRT stimulates development of treatments and a cure via ongoing engagement with individual labs, biotech companies, and in-house pharmaceutical research. RSRT also fosters alliances with national and international Rett Syndrome organizations.

Financials

RSRT is a 501(c)(3) charitable organization. The most recent financial information available is from 2013.¹

Year ending 12/31/2013

- Revenue: \$5,671,351
- Total Assets: \$5,774,064
- Gifts received: \$5,655,936
- Expenditures: \$7,713,085
- Grants: \$7,152,602

Key Accomplishments

- To date, the trust has committed \$20 million to research.
- In 2013 alone, it awarded \$7.2 million to new research
- Ninety-six percent of every dollar donated is channeled directly to research.
- A paper published in 2013 by one of the MECP2 Consortium members showed for the first time reversal of symptoms in mice using gene therapy techniques that have the potential to be used in humans. This led to the creation of the MeCP2 Gene Therapy Consortium.
- An RSRT-funded investigator found concrete evidence proving that MeCP2, the protein that, when mutated, causes Rett Syndrome, is required throughout life to maintain healthy brain function.

¹ Information obtained from Foundation Center Directory, <http://fconline.foundationcenter.org>, May 2012.

- An RSRT public service announcement was featured on the Port Authority billboard in Times Square at the close of 2011 and beginning of 2012.
- RSRT and its executive director were featured in a 2010 documentary called RETT: There is Hope. It was recognized at the Canadian International Film festival and was chosen to be screened at two other festivals.

Leadership

RSRT is governed by **Trustees, Scientific Advisory Board, and Professional Advisory Council.**

- **Executive Director:** Monica Coenraads, monica@rsrt.org
- **Co-Founder:** Ingrid Love Harding, ingrid@rsrt.org
- **Chair:** Tony Schoener, tschoener@gmail.com
- **Vice-Chairman:** Heidi Epstein, heidi@rsrt.org
- **Program Director:** Tim Freeman, tim@rsrt.org