rett syndrome research trust

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Key Facts

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WHO WE ARE

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.

RSRT is led by:

- *Eight-member Board of Trustees (seven are parents of daughters with Rett; one is a scientist)*
- *Five full time staff (Executive Director and Program Director have daughters with Rett)*
- *Thirteen-member Scientific Advisory Board includes some of the most respected scientists in the world*
- *Co-founder and Executive Director, Monica Coenraads, has been the one constant for the Rett research community since 1999*

OUR HISTORY

Monica became involved in Rett research in 1998 the day her then two-year-old daughter was diagnosed. She established the Rett Syndrome Research Foundation (RSRF) a year later with five other parents and served as Scientific Director. In 2007 RSRF merged with the older, family support-focused International Rett Syndrome Association (IRSA), founded by Kathy Hunter in 1984. The combined entity was renamed the International Rett Syndrome Foundation (IRSF). Monica, eager to focus strictly on research, resigned and launched RSRT in 2008 with Ingrid Harding.

Key Facts

WHAT WE DO

RSRT operates at the nexus of 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.

We identify, solicit, evaluate, prioritize, fund and monitor ambitious research projects that have the greatest likelihood of impacting the lives of those afflicted with Rett Syndrome.

We have provided financial support to:

- *44 scientists in 36 academic institutions*
- *4 biotech companies*
- *5 clinical trials testing 4 compounds*
- *Conceptualized and launched 4 consortia (MECP2 Consortium, Gene Therapy Consortium, Activating the Silent MECP2, Outcome Measures and Biomarker Development))*

YEAR	$ AWARDED TO RESEARCH	% OF DONATIONS TO RESEARCH
2008	2.3	98%
2009	0.6	94%
2010	1.3	96%
2011	3.6	96%
2012	4.3	97%
2013	7.2	96%
2014	5.8	93%
2015	8.8	TBD
2016	1.3 (YTD)	TBD
TOTAL	$35 MILLION	97% (AVERAGE)

Key Facts

WHAT WE DO *(Continued)*

We refute the conventional practice of labs working in isolation and promote and fund collaborations and consortia in which scientists work across multiple disciplines. We are risk takers who do not shy away from bold projects that are unlikely to be funded by NIH or other more traditional funding agencies.

Through close monitoring of scientific literature we identify and recruit the interest of scientists doing work relevant to our mission. We organize scientific meetings throughout the year favoring small, invitation-only gatherings to promote in-depth discussion and ensure participants of confidentiality, allowing them to share data long before publication, a process that can take years.

We provide accurate, timely research information to the Rett family community without bias or spin. We operate with transparency and strict accountability to the public and to Rett families in particular. We are funded almost entirely by parents of children with Rett and their networks in the US and abroad.

OUR ACCOMPLISHMENTS

Our funding to date has led to the following potentially clinically relevant discoveries:

- *Significant reversal of symptoms in Rett mouse models using gene therapy techniques that can be applied to people.*
- *Statins (cholesterol-lowering drugs) suppress some of the key Rett symptoms in a mouse model of the disease. A clinical study is now being designed.*
- *Evidence proving that the Rett protein is required throughout life to maintain healthy brain function.*
- *Identification of a critical novel domain on the Rett protein*
- *The revelation that glia play a key role in preventing the progression of the most prominent Rett symptoms displayed by mouse models of the disease.*