

The Rett Syndrome Research Trust



WHAT IS RETT SYNDROME?

- Rett Syndrome is a severe neurological disorder that afflicts 350,000 children and adults around the world. The vast majority of those affected are girls and women.
- With the exception of women's cancers, Rett is the **leading genetic cause of severe impairment in females**.
- Symptoms have been described as like having anxiety disorder, autism, cerebral palsy, Parkinson's, epilepsy, and scoliosis **all in one person**.
- Research on Rett is extremely promising because we know the cause: a random mutation of a gene called MECP2. This fact gives scientists a target.
- **Rett has been proven to be reversible in mouse models.**

WHO IS RSRT?

- Since its founding in 2008, **RSRT has strategically awarded over \$85 million** to leading scientists, more than any other Rett organization worldwide.
- Every dollar of that has been donated from private sources, **mostly individuals and families**.
- RSRT prioritizes genetic-based approaches that aim for a cure, including gene therapy, gene editing, MECP2 reactivation, RNA editing, and RNA trans-splicing.
- RSRT has recruited some of the **world's top scientists** and has incentivized them to work in consortia to **maximize acceleration toward a cure for Rett**.
- RSRT facilitates biopharmaceutical companies to take on Rett research, a critical step to developing clinical trials. To date, eight companies have launched Rett programs.
- RSRT is exceptionally efficient, with an **average of 94% of funding spent on its research program**.
- The gene therapy trials for Rett that are currently proceeding were initiated and incubated with RSRT funding.
- In 2024, RSRT launched a bold \$40 million initiative called Roadmap to Cures, with a goal to bring three genetic medicines, beyond the current gene therapies, to clinical trials by 2028.

HOW CAN I HELP?

- RSRT urgently needs funding to build on its momentum and aggressively advance its programs.
- **We have a chance now, as never before, to dramatically change lives and**

GET IN TOUCH

Send checks made to "RSRT" to:
RSRT / 67 Under Cliff Road
Trumbull, CT 06611

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