



## ABOUT 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 by Rett are girls and women. The symptoms have been described as having autism, cerebral palsy, Parkinson's, epilepsy, anxiety disorder, and scoliosis, all in one person. Most victims of Rett are unable to walk, speak, or use their hands.

There are many reasons to be optimistic about a cure for Rett syndrome:



The cause of Rett is known: a random mutation on a gene called MECP2. This means that scientists have a target to attack the disorder.



Rett is not neurodegenerative, meaning brain cells don't die, they just don't function properly.



Rett symptoms have been reversed in mice.

## THE RETT SYNDROME RESEARCH TRUST (RSRT)

The goal of the Rett Syndrome Research Trust is to accelerate a genetic-based cure for the disorder. Since its founding in 2008, RSRT has awarded more than \$64 million to leading scientists to advance us toward a cure.

Every dollar of that \$64 million has been contributed by individuals and families who want to make a difference to the lives of those with Rett.

RSRT has prioritized six approaches that aim to fix the underlying cause of Rett: gene replacement, gene editing, MECP2 reactivation, RNA editing, RNA trans-splicing, and protein replacement.

RSRT partners with biopharmaceutical companies to translate lab research to clinical trials and a treatment. To date, eight companies have Rett programs, every one of them facilitated by RSRT. Two of these companies are developing the first clinical trials in gene replacement for Rett.

RSRT urgently needs funding to continue its momentum toward a cure. As an exceptionally lean and efficient non-profit, RSRT spends an average of 94% of its funding on its research program.

This is a disease that causes great suffering and disability. We have a chance now as never before to change lives and cure Rett syndrome.

To support, please go to [reverserett.org/donate](http://reverserett.org/donate) or send a check made out to "RSRT" to the address below.

*RSRT tax identification number is 26-0687439.*

### RETT SYNDROME RESEARCH TRUST

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### QUESTIONS?

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or call 609.815.5102

