A Historic Possibility—The First Cure for a Neurological Disorder

ROADMAP TO A CURE

rett syndrome research trust

REVERSERETT.ORG/CURE

Rett Syndrome is a severe neurological disorder that afflicts 350,000 children and adults. A diagnosis of Rett is devastating to a family. Those afflicted develop normally through early toddlerhood. Then they regress, losing the ability to walk and talk. A host of extremely painful and debilitating symptoms ensue. It's difficult to imagine the symptoms of autism, Parkinson's, cerebral palsy, and epilepsy all in one child. But this is what those who have Rett Syndrome live with.

The **Rett Syndrome Research Trust (RSRT)** is the world leader in spurring and supporting scientific research on Rett. **RSRT has a singular goal of developing a cure,** and there are very good reasons to be confident that this is achievable. Because the cause of Rett is known—random mutations on a gene called *MECP2*—scientists have a target to attack. Remarkably, Rett symptoms have been reversed in mouse models, providing proof that it can be cured. Because Rett is not degenerative, afflicted children and adults are poised for the research to profoundly change their lives.

Since its founding in 2008 RSRT has invested $47 million, all of it contributed by affected families and their networks, to advance the research to its most promising stage ever. Now is the time for the most important push forward in Rett research. All resources and energy need to be put into translating lab science to make Rett the first neurological disorder that can be cured. This is why RSRT is implementing a bold strategic research plan called ***Roadmap to a Cure***. The plan, which has total costs of **$33 million over three years**, includes four components:

1. **CURE:** Four priority approaches designed to fix the underlying cause of Rett are being pursued: 1) Gene therapy, 2) *MECP2* Reactivation, 3) Protein Replacement, and 4) RNA Modifications. Informed by critical data generated by RSRT-supported researchers, the biotech company AveXis is now advancing the lead *MECP2* gene therapy candidate toward clinical trials. This will be the first clinical trial of a potentially curative approach for Rett. This is a key step towards RSRT delivering on its goal.
Funding needed: $13 million.

2. **TREAT:** Until a cure is achieved, RSRT will pursue treatment strategies with the greatest potential to improve quality of life for those suffering with Rett. Therapeutic interventions may improve multiple symptoms.
Funding needed: $3.3 million.

3. **ENABLE:** There are currently no clear outcome measures for clinical trials in Rett. Working with the Rett community, scientists, and clinicians, RSRT is establishing outcome measures that can be used by the FDA and other regulatory agencies. This will shorten the timeline for the testing and approval of potential cures and enable pharmaceutical and biotech industry investment. RSRT is also putting significant resources into providing clinics with the resources and personnel they will need to implement the most effective and efficient clinical trials.
Funding needed: $9.2 million.

4. **LEARN:** RSRT must continue investing in basic science to expand our understanding of the mutated gene that causes Rett, its protein product, and its function. This is vital to informing all approaches.
Funding needed : $4.5 million.

RSRT has exceptionally low overhead expenses. Operational costs for ***Roadmap to a Cure*** are $3 million, less than 10% of the total budget.

Every dollar matters. To contribute, please donate online at ***www.ReverseRett.org/Donate***
Or send check made out to "RSRT" to: ***RSRT 67 Under Cliff Road Trumbull CT 06611***

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