YOUR HELP IS OUR HOPE!

ABOUT RETTSYNDROME.ORG

Rettsyndrome.org, a 501(c)3 organization, is the most comprehensive nonprofit dedicated to accelerating research of treatments and a cure for Rett syndrome and related disorders while providing information and family empowerment. Rettsyndrome.org funds high-quality, peer-reviewed research grants and programs. The organization hosts the largest global gathering of Rett researchers and clinicians to establish research direction for the future. Rettsyndrome.org, a 501(c)3 organization, has earned Charity Navigator’s most prestigious 4 star rating.

The core mission of Rettsyndrome.org is to accelerate full spectrum research to cure Rett syndrome and empower families with knowledge and connectivity.

JOIN US IN OUR MISSION

• Make a donation or volunteer your time
• Coordinate or participate in a fundraising event.
  – Strollathon
  – Social event or gala
  – Golf tournament

Direct donations are appreciated online or mailed to:
Rettsyndrome.org
P.O. Box 706143
Cincinnati, OH 45270-6143
RESEARCH STRATEGY

Our strategy is to accelerate research and aggressively move more potential drug treatments to clinical trials by expanding basic discovery. We will translate foundational research into promising treatments.

RESEARCH PROGRESS

1999
Causative gene discovered
Mouse models developed

2004
Natural history study of Rett syndrome initiated

2007
First publication on the genetic reversibility of Rett syndrome in mice (Adrian Bird Lab)

2009
IGF-1 treatment reverses symptoms of Rett syndrome in mice (Mriganka Sur lab)

2010
First gene therapy publication (labs of Stuart Cobb, and Steven Gray)

2013
Phase I & IIa clinical study of NNZ-2566 (trofinetide) started

2014
Treatment of IGF-1 is found to be safe and tolerable in Rett Patients
First multisite Phase II clinical study of trofinetide completed and found to be efficacious
NIH awards an agreement for Natural History Study Launch of Neuro-Habilitation program

2015
Launch of Read Through Program Expansion of Phase II IGF-1 trial to two additional sites
Three sites added to NHS for a total of 14

2016
Phase II trofinetide trial in a pediatric population will begin enrollment
The 50th anniversary of Dr. Andreas Rett first publishing information about Rett syndrome

RESEARCH TO REALITY

Since discovering the association of the MECP2 gene with Rett syndrome in 1999, our pace of research is accelerating, with reversibility in mice established in 2007, and two clinical trials for treatments progressing through Phase II.

Rett syndrome is caused by a mutation in MECP2, a crucial gene for brain development and synaptic function.

Research aimed at developing therapies specific to the MECP2 gene will have an impact reaching far beyond Rett syndrome. Research toward understanding MECP2 will greatly advance our understanding of other synaptic disorders like these.

“[Rettsyndrome.org] has been an amazing force in advancing our understanding of Rett syndrome research over the last 25 years. One day, we will have a great treatment for these girls. The ‘Research to Reality’ is a really great way to capture our state of affairs with Rett syndrome. Research gave us the reality about how important this protein is for brain function”

RETTSYNDROME.ORG ACCELERATES RESEARCH

- We have invested $35 million over the past 10 years.
- The National Institute of Health consistently supports Rettsyndrome.org-funded research.
- Rettsyndrome.org funded projects have consistently produced data recognized by the National Institute of Health to warrant a five times leverage of our research dollars through federal funding.
- We have sponsored 2 out of the top 10 drug trials in autism and related neurodevelopmental disorders cited by the scientific journal Nature Biotechnology, more than any other nonprofit.

A girl with Rett syndrome is born every 2 hours

Rett syndrome...

- is a rare non-inherited genetic postnatal neurological disorder affecting mostly females.
- affects seemingly normal girls usually starting when they are 6-18 months old.
- is caused by a mutation in MECP2, master gene for normal neurologic development.
- affects 1 in 10,000 live female births worldwide, it is more common than Huntington’s disease and Muscular Dystrophy.
- has a wide range of symptoms, with many Autism characteristics, often including the loss of speech, the ability to walk and purposeful use of hands. Other symptoms include seizures, breathing irregularities and extreme anxiety.

The hallmark sign of Rett syndrome is near constant repetitive hand movements. Cognitive assessment in children with Rett syndrome is complicated, but they understand far more than they can communicate to us, evidenced by their bright and attentive eyes, and ability to express moods and emotions.

Dr. Huda Zoghbi