Rettsyndrome.org

ACCELERATING RESEARCH. EMPOWERING FAMILIES.

RETTSYNDROME.ORG
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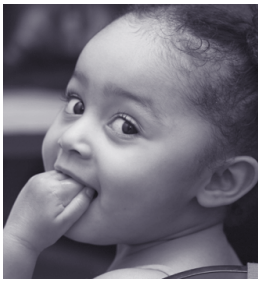

About Rettsyndrome.org

Rettsyndrome.org is accelerating research for treatments and a cure for Rett syndrome. As the world's leading private funder of Rett research, we have funded more than $38 million in peer-reviewed research grants and programs to date. We empower families to make a difference. We are a 501(c)3 organization, earning Charity Navigator's most prestigious 4-star rating. Visit www.rettsyndrome.org to learn more, or call (800) 818-7388.

About Rett syndrome

Rett syndrome is a rare non-inherited genetic postnatal neurological disorder that occurs almost exclusively in girls and leads to severe impairments, including seizures, scoliosis, digestion, and affects nearly every aspect of the child's life: their ability to speak, walk, eat, and even breathe easily. The hallmark of Rett syndrome is near constant repetitive hand movements while awake. Cognitive assessment in children with Rett syndrome is complicated, but we know that they understand far more than they can communicate to us, evidenced by their bright and attentive eyes, and their ability to express a wide spectrum of moods and emotions.

Why help now?

With the association of the MECP2 gene with Rett syndrome (1999), research in the lab that proves the theory of reversibility of the disease (2007) and the launch of multiple disease-modifying human clinical trials (present), we now reach an unprecedented and historic moment in time where we must do everything possible to fund the truly life-changing research in front of us. We hope you will choose to help us help all those who are patiently waiting.

Rettsyndrome.org is the leading Rett syndrome research organization in the world.

Here are some of the highlights in 2015:

- Following a successful Phase 2 trial with trofinetide in adult women with Rett syndrome, and anencouraging meeting with the FDA, Neuren is moving forward with a Phase 2 trial for tolerability inchildren with Rett syndrome. Concurrently, Neuren is working with the FDA to design a Phase 3 pivotalstudy with trofinetide as a treatment for Rett.
- An investment of $3.3 million was made to support new research grants, current research programs, and establish a new Read-Through Program.
    - To date, $38 million cumulative research dollars have been awarded in a quest to acceleratetreatments.

- The Rett Syndrome, MECP2 Duplication Disorder, and Rett related disorders Natural History Study was approved for federal funding in 2014 , and has been working to get all sites activated for enrollment.
    - In 2015, Rettsyndrome.org was able to sponsor three additional sites to the Rett Consortium,which will involve a total of 15 participating sites. This will expand clinical infrastructure and expertise across the country for our rare disorders.
- Rett syndrome became one of only 40 areas of study eligible for up to $278 million of Department of Defense research funds after a successful advocacy campaign by Rettsyndrome.org.

Here are some of the highlights to look forward to in 2016:

- Launch of new Phase 2 clinical trial with trofinetide in a pediatric population with Rett syndrome.
- Enrollments to the NHS at 15 sites throughout the U.S.
- 2016 Rett Syndrome Research Symposium and first ever Related Disorders Family Conference.

CHARITY NAVIGATOR
★★★★
Four Star Charity