


\$38M 
COMMITTED TO RESEARCH THROUGH 2015



We are leading the revolution in Rett syndrome research

\$5.1M 
RAISED

\$3.3M 
COMMITTED TO RESEARCH IN 2015

41 
ACTIVE RESEARCH & GRANT PROJECTS

16.5K 
DONORS

56 
MAJOR FUNDRAISERS

89 
FAMILY FUNDRAISERS

224 
NEW PARENTS JOINED RETTSYNDROME.ORG

195K 
UNIQUE USERS TO WWW.RETTSYNDROME.ORG

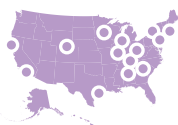
4 ★★★★★
STAR CHARITY
WE'VE EARNED CHARITY
NAVIGATOR'S HIGHEST
RATING



2015 HIGHLIGHTS



We invested \$3.3 million to support new research grants, to continue current research programs, and to establish a new Read-Through Program.



Rettsyndrome.org sponsored 3 additional sites to the NIH Natural History Study, which will now involve a total of 14 participating sites. This expands clinical infrastructure and expertise across the country for our rare disorders.



To date, we have awarded over \$38 million cumulative research dollars in a quest to accelerate treatments and find a cure.

DoD FUNDING



Rettsyndrome.org along with board members and families, undertook an ambitious advocacy campaign in January 2015 to enable Rett syndrome research to be funded by the Department of Defense. The campaign was a success and now Rett syndrome is one of only 40 areas of study eligible for up to \$278 million of DoD research funds.

CLINICAL TRIALS

Trofinetide (NNZ-2566)

- Phase 2 trial in adult women closed in September 2014.
- Moving forward with a Phase 2 trial for safety and tolerability in children with Rett syndrome.
- Concurrently, Neuren Pharmaceuticals is working with the FDA to design a Phase 3 pivotal study with trofinetide as a treatment of Rett syndrome.

IGF-1

- After meeting with the FDA they recommended the expansion of the IGF-1 human clinical trial to include 17 additional patients.
- Added Chicago, IL and Greenwood, SC as sites.



OUR MISSION



Accelerate full spectrum research for treatments and a cure for Rett syndrome



Empower families with knowledge and connectivity

LOOKING AHEAD

2016

- Launch of new Phase 2 clinical trial with trofinetide in a pediatric population with Rett syndrome
- Expansion of the IGF-1 human clinical trial in Boston, MA, Chicago, IL and Greenwood, SC
- Enrollments to the NHS at 14 sites throughout the U.S.
- 2016 Rett syndrome Research Symposium and first ever Related Disorders Family Conference



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PARTICIPATE

www.rettsyndrome.org/get-involved



SIGN UP

www.rettsyndrome.org/join



Rettsyndrome.org

Rettsyndrome.org is a 501(c)(3) non-profit corporation registered as The International Rett Syndrome Foundation and established in 2007 through the strategic merger of the Rett Syndrome Research Foundation and the International Rett Syndrome Association.

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