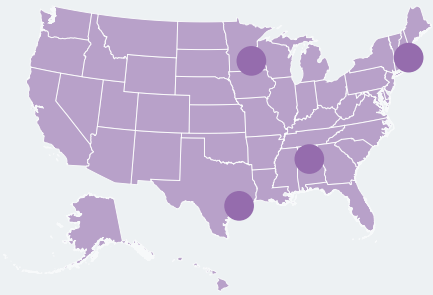
IMPACT REPORT
2014

Rettsyndrome.org
ACCELERATING RESEARCH. EMPOWERING FAMILIES.

RESEARCH TO MEDICINES
OUR STRATEGY

Rettsyndrome.org continues to cover the full spectrum of Rett syndrome research to accelerate research from basic discovery, where ideas are made, to translating them into medicines for future clinical testing and treatments.

80 GIRLS
CURRENTLY IN CLINICAL TRIALS



CLINICAL TRIAL
Trofinetide (NNZ-2566)

- Trofinetide granted fast-track approval
- Phase IIa effective and safe in Rett patients
  - A huge step toward a safe, effective treatment for Rett syndrome
- The data is promising
  - Trofinetide well tolerated at the dose levels tested after 28 days of treatment
  - No significant safety concerns identified
  - Dose-response and improvement over time indicated in the data
  - High dose used showed benefit vs placebo in group-level analysis & individual subject analysis

CLINICAL TRIAL
IGF-1

- Phase II
  - IGF-1 treatment was found to be safe and tolerable in Rett patients

HIGHLIGHTS

- The Rett syndrome, MECP2 Duplication, and Rett related disorders Natural History Study was approved for federal funding.
  - Rettsyndrome.org will take a central role in this endeavor as a member of the Coalition of Patient Advocacy Groups (CPAGs) within the Rare Diseases Clinical Research Network (RDCRN), and as administrative partner of the 5-year NIH U54 cooperative agreement awarded to the University of Alabama at Birmingham.
- Over $35M cumulative research dollars have been awarded in our quest to accelerate treatments through 2014
- Our investment supported new research grants and established a new neuro-habilitation program for those affected by Rett syndrome

$2.9M
SPENT ON RESEARCH

39
ACTIVE RESEARCH & GRANT PROJECTS

$5.5M
RAISED

16.5K
DONORS

50
MAJOR FUNDRAISERS

75
FAMILY FUNDRAISERS

223
NEW PARENTS JOINED RETTSYNDROME.ORG

250+
FAMILY CONFERENCE ATTENDEES

- Over 250 family members and caregivers attended the 30th annual family educational conference in Chantilly, VA.

We are leading the revolution in Rett syndrome research

125
REGISTERED RESEARCHERS & CLINICIANS

- We hosted the largest global gathering of Rett researchers and clinicians to establish research direction for the future

CHARITY NAVIGATOR

P.O. Box 706143 Cincinnati, OH 45270-6143 (800) 818-7388 http://www.rettsyndrome.org/donate /rettsyndrome /rettsyndrome

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Thank you!

We have amazing partners, families and donors. We would like to recognize and thank all of you for your special contributions in 2014.

OUR MISSION

Accelerate full spectrum research to cure Rett syndrome and empower families with knowledge and connectivity

2015

LOOKING AHEAD

- Launch of a new phase of the Natural History Study and growth of Rett clinics in the U.S.
- Expansion of the IGF-1 human clinical trial at Boston Children's Hospital and Greenwood Genetic Center
- Full release of the Trofinetide Phase II results that assisted in the acceptance of Neuren's applications for Orphan Drug status

RESEARCH TO REALITY FUND & CAMPAIGN LAUNCH

- Dedicated to identifying treatments and a cure for Rett syndrome
- 100% of Research to Reality donations fund research
- $1.5 million Board of Directors' commitment to the Research to Reality Campaign

> **We are in the time of Rett** when finding an effective treatment is truly possible.
>
> - Walter E. Kaufmann, MD

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