

# IMPACT REPORT

2016



## OUR MISSION

- ➔ Accelerate full spectrum research for treatments and a cure for Rett syndrome
- ➔ Empower families with information, education and connectivity

## LOOKING AHEAD

2017

- ➔ Launch of new Phase 2 clinical trial with Anavex 2-73
- ➔ Data analysis of the completed Phase 2 clinical trials with trofinetide and IGF-1
- ➔ Enrollments to the NHS at 14 Rett syndrome Clinical Research Centers of Excellence throughout the U.S.

## DONATE

[www.rettsyndrome.org/donate](http://www.rettsyndrome.org/donate)

## PARTICIPATE

[www.rettsyndrome.org/get-involved](http://www.rettsyndrome.org/get-involved)

## SIGN UP

[www.rettsyndrome.org/join](http://www.rettsyndrome.org/join)



Rettsyndrome.org is a 501c(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.

☎ (800) 818-7388  
4600 Devitt Drive  
Cincinnati, OH  
45246-1104

🌐 <http://www.rettsyndrome.org> 📘 [/rettsyndrome](https://www.facebook.com/rettsyndrome) 📺 [/rettsyndrome](https://www.youtube.com/rettsyndrome)

©2017 Rettsyndrome.org



# IMPACT REPORT

2016

**\$40M**   
COMMITTED TO RESEARCH THROUGH 2016



**We are leading the revolution in Rett syndrome research**

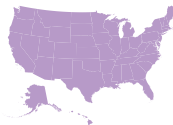
**\$5.0M+**   
RAISED

## 2016 HIGHLIGHTS



We invested \$2.1 million to support new research grants, to continue current research programs.

**\$2.7M**   
COMMITTED TO RESEARCH PROGRAM SERVICES IN 2016



Rettsyndrome.org designated 14 clinics as Research Centers of Excellence.

**40**   
ACTIVE RESEARCH & GRANT PROJECTS



To date, we have awarded over \$40 million cumulative research dollars in a quest to accelerate treatments and find a cure.

**11.7K**   
DONORS

## DoD FUNDING



Rettsyndrome.org, along with board members and families, undertook an ambitious advocacy campaign to enable Rett syndrome research to be funded by the Department of Defense. The campaign was a success and Rett syndrome is one of only 40 areas of study eligible for up to \$278 million of DoD research funds in 2016.

**46**   
MAJOR FUNDRAISERS

**89**   
FAMILY FUNDRAISERS

## CLINICAL TRIALS

**212**   
NEW PARENTS JOINED RETTSYNDROME.ORG

### Trofinetide (NNZ-2566)

→ Phase 2 trial in children ages (5-15) started enrollment in March 2016 and closed enrollment in November 2016.

→ A total of 82 subjects were enrolled to the study.

→ Concurrently, Neuren Pharmaceuticals is working with the FDA to design a Phase 3 pivotal study with trofinetide as a treatment for Rett syndrome.

**186K**   
UNIQUE USERS TO WWW.RETTSYNDROME.ORG

**3**  **STAR CHARITY**

WE'VE EARNED ONE OF CHARITY NAVIGATOR'S HIGHEST RATING



## IGF-1

→ Completed enrollment and trial study visits of 30 subjects.

→ Final data analysis is ongoing.