

JUST THE FACTS



ABOUT RETT SYNDROME

Rett syndrome is a rare, non-inherited genetic neurological disorder that occurs almost exclusively in girls and leads to severe impairments, including seizures, scoliosis, and digestive difficulties. It 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. Children with Rett syndrome 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?

Our understanding of what it will take to conquer Rett syndrome has never been greater and we are committed to funding the life-changing research in front of us. We have four compounds in clinical trials and are testing hundreds more for potential use in Rett syndrome. We continue to support research in gene therapy and it shows great promise for the future. We are developing devices to improve neurological function and enhance educational assessments for our children. Our research has led to earlier diagnosis, higher standards of clinical care and is producing results that are bringing hope to families all over the world.

During this time of such tremendous progress in research, it is essential that we continue to provide services that support the families affected by Rett syndrome. We never forget that Rett syndrome affects the entire family and our dedication to them is unwavering.

Accelerating research and empowering families – not just a tagline, but the core of everything we do. We hope you will partner with us as we continue to transform lives



ABOUT RETTSYNDROME.ORG

Rettsyndrome.org's purpose is to transform the lives of all those affected by Rett syndrome. Through the relentless pursuit of advanced and comprehensive research, we work to deliver treatments and ultimately a cure for this disorder. We never forget that Rett syndrome impacts the entire family and we empower our families with the tools they need on their journey. As the world's leading private funder of Rett research, we have funded more than \$44 million in peer-reviewed research grants and programs to date. We have also invested over \$10 million in family programs and services to date.



HIGHLIGHTS FROM 2017

• **\$2.4 million** awarded for 2018 research grants

- **19 clinics** in the US now provide specialized clinical care for Rett syndrome as part of our clinical network. **14** have earned our designation of Clinical Research Center of Excellence.
- **2500+ children and adults** have received care from one of the clinics in our network
- **50 post-doctoral researchers** training through our Mentored Fellowship Training program
- **232 newly diagnosed families** received a comprehensive care packet
- **120 families** held fundraising events
- Rett syndrome experts are acknowledging the role of BOTH neuro-habilitation therapies and treatment medications as essential components of a complete cure for Rett syndrome.
- New methods to evaluate cognitive ability have been developed by our researchers and have shown that children with Rett syndrome have far greater intelligence levels that many have previously thought
- Dr. Jenny Downs' Neuro-Habilitation research confirmed the importance of environmental enrichment to stimulate development in children with Rett syndrome, providing a tool for parents to share with a child's intervention, therapy and IEP teams.



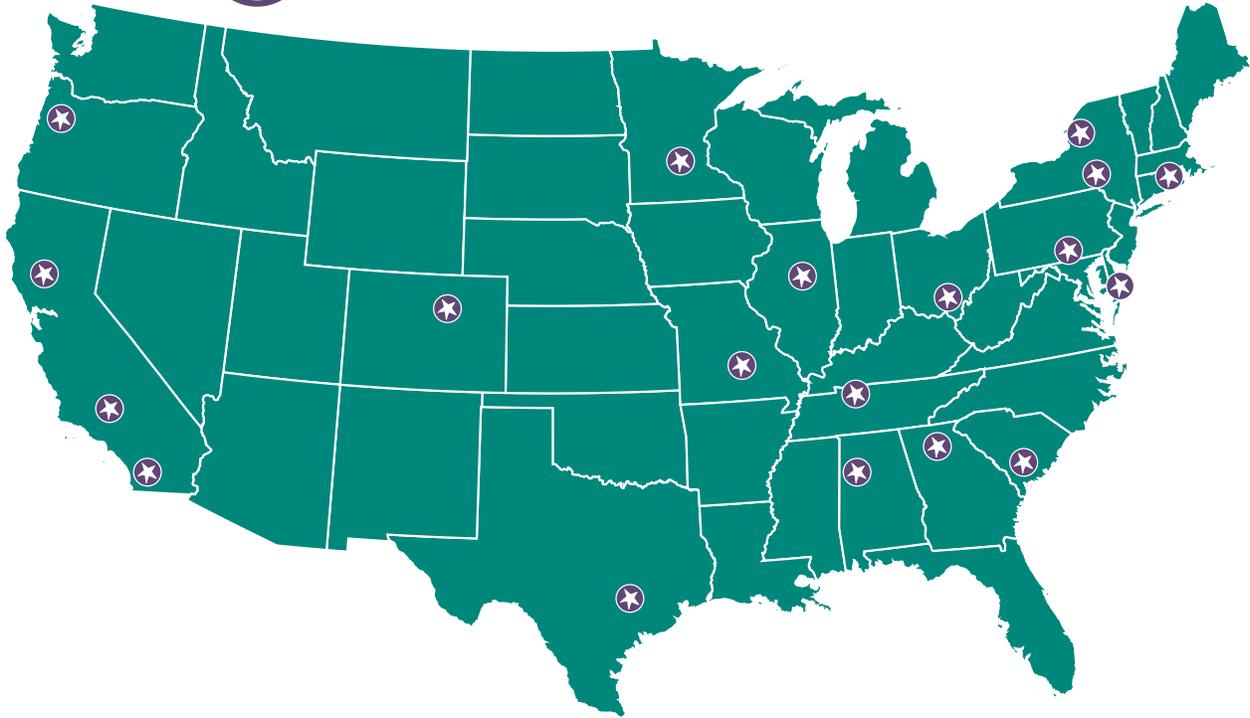
LOOKING FORWARD TO 2018

• **Phase 3 Clinical trial of trofinetide to begin** after planning and study design are complete.

- **Phase 2 clinical trial of Anavex 2-73 to begin.**
- The **Natural History study continues in 15 US sites**, making it one of the largest studies of its kind for a developmental disability.
- **Phase 2/3 clinical trial of Sarizotan is slated to complete enrollment in 2018**, with trial results ready in 2019.
- **Phase 1 clinical for AMO-04 being planned**
- **Continued expansion of the Scout Program** which has moved compounds with potential for use in Rett syndrome from preclinical studies to clinical trials.



RETT SYNDROME CLINICS



ALABAMA – BIRMINGHAM*
Rett Clinic and Research Center
 UAB - Civitan International
 Research Center
 1.800.822.2472, opt. 7

CALIFORNIA – OAKLAND*
**Katie's Clinic for Rett Syndrome
 and Related Disorders**
 UCSF Benioff Children's
 Hospital Oakland
 1.925.979.4055

CALIFORNIA – LOS ANGELES
USC UCEDD Rett Syndrome Clinic
 Children's Hospital Los Angeles
 1.323.361.3849

CALIFORNIA – SAN DIEGO*
Rett Syndrome Clinic
 Rady Children's Hospital – San Diego
 1.858.966.6458

COLORADO – AURORA*
Rett Syndrome Clinic
 Children's Hospital Colorado
 1.720.777.4363

GEORGIA – NORCROSS
**Rett syndrome and MECP2
 Related Disorders Clinic**
 Center for Rare Neurological Diseases
 1.617.984.9091

ILLINOIS – CHICAGO*
Rett Syndrome Clinic
 Rush University Medical Center
 1.312.942.4036

MARYLAND – BALTIMORE
Department of Neurogenetics
 Kennedy Krieger Institute, John's Hopkins
 School of Medicine
 1.800.873.3377, Ext. 2778

MASSACHUSETTS – BOSTON*
Rett Syndrome Program
 Boston Children's Hospital/Harvard
 Medical School
 1.617.355.8994

MINNESOTA – ST. PAUL*
Integrated Care Program
 Gillette Children's Specialty Healthcare
 1.651.229.3897

MISSOURI – ST. LOUIS*
**Rett Syndrome and Related Spectrum
 Disorders Clinic**
 Saint Louis Children's Hospital
 1.314.454.6120

NEW YORK – BRONX
Tri-State Rett Syndrome Center
 Children's Hospital at Montefiore
 1.347.640.2671 or 1.718.920.4378

NEW YORK – ROCHESTER*
Paciorkowski Lab research program
 University of Rochester Medical Center
 1.585.275.2808

OHIO – CINCINNATI*
Rett Syndrome Clinic
 Cincinnati Children's Hospital
 Medical Center
 1.513.636.4222

OREGON – PORTLAND
Rett Syndrome Clinic
 Oregon Health and Sciences University
 1.800.452.3563

PENNSYLVANIA – PHILADELPHIA*
Division of Neurology, Rett syndrome
 Children's Hospital of Philadelphia
 1.215.590.1719

SOUTH CAROLINA – GREENWOOD*
**Rett Syndrome and Related
 Disorders Program**
 Greenwood Genetic Center
 1.888.442.43630

TENNESSEE – NASHVILLE*
Rett Syndrome Clinic
 Vanderbilt University School of Medicine
 1.615.875.9311

TEXAS – HOUSTON*
The Blue Bird Circle Rett Center
 Texas Children's Hospital
 1.888.430.7388 (RETT)

**Denotes the clinic is a Natural History Study site*

Rettsyndrome.org attempts to verify the accuracy of information regarding Rett syndrome clinics and health care providers prior to publishing. Rettsyndrome.org does not guarantee the accuracy of information or the quality of medical care received at any institution, clinic, or by a medical provider listed. All material contained or referenced in this document is for informational purposes only.