

# rett gazette

## Neuren's NNZ-2566 Successful in Phase 2 Clinical Trial for Rett Syndrome

We've known that effective treatments and a cure for Rett syndrome will come in small and incremental steps. But we've just learned that a clinical trial we supported has achieved promising results.

Neuren Pharmaceuticals' human clinical trial using NNZ-2566 as a potential treatment for Rett syndrome was shown to be well tolerated at the dose levels tested after 28 days of treatment. The study found no significant safety concerns, and the data indicated dose-response and improvement over time. In fact, the high dose used in this study showed benefit versus the placebo – both in the group-level analysis and in the individual subject analysis.

The study was conducted at Baylor College of Medicine (Drs. Daniel Glaze and Jeffery Neul); University of Alabama at Birmingham (Dr. Alan Percy); and the Gillette Specialty Healthcare (Drs. Tim Feyma and Art Beisang).

The study of NNZ-2566 dosing for safety, tolerability, and efficacy is the first step needed to develop a drug for treating Rett syndrome. Neuren's next steps are to meet with the FDA and to apply for Orphan Drug Status and Breakthrough Therapy Status. This is exciting for all of us involved with Rett syndrome. Rettsyndrome.org will continue to support Neuren's development of NNZ-2566.



*"These are exciting times for Rett syndrome. The results will enable engagement with the FDA on the further development of NNZ-2566.*

*To date Rettsyndrome.org has invested over \$1 million dollars to conduct this trial and the Natural History Data it was built on."*

– Steven Kaminsky, Ph.D.  
Chief Science Officer,  
Rettsyndrome.org



## Meet Ellie & Mickie

Parent Mickie is grateful that 16-year-old Ellie participated in the recent Neuren Pharmaceutical trial of NNZ-2566. After so many years of dreaming that one day drug trials would begin, Mickie now believes that Ellie's historical "fingerprints" are helping us understand more about Rett syndrome and possible drug therapies.

When so much research is beginning to translate from dreams to reality, your financial gift is especially important. You too have your fingerprints on our research – fingerprints that continue to expand our understanding of Rett syndrome and will one day identify a cure.

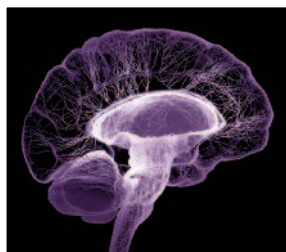
**Support our annual appeal to continue progress of our funded research. Visit [Rettsyndrome.org/donate](http://Rettsyndrome.org/donate) to help today!**



# The NIH Renews Funding for the Natural History Study

In October, the National Institute of Health (NIH) renewed funding for continuation of the Natural History Study. The study (which will be led by Dr. Alan Percy, University of Alabama at Birmingham) will include our 11 specialty Rett centers.

The study will investigate the longitudinal natural history of Rett syndrome, MECP2 Duplication disorder, CDKL5 disorder, and FOXP1 syndrome. Other areas of investigation include: neuro-behavioral assessments; biomarkers; outcome measures; neurophysiological assessments; and neuroimaging – in relation to disease severity.



As Dr. Percy explains, “the potential for developing effective clinical trials is now tangible with other disease modifying agents rapidly coming to the clinics.”

Over the next five years, the study has three primary goals:

1. Identify and understand the clinical features of each disorder
2. Identify factors that can modify the severity of the disorders
3. Understand the relationship between patients’ symptoms, their brain imaging, and electroencephalography alterations.

*“Through this Natural History Study we will transform Rett syndrome and the Rett-related disorders care initiatives by stimulating more clinical research that will help draft guidance on how to standardize and optimize the best care for the children and adults with these four disorders as soon as possible through building a collaborative clinical network.”*

*Steven Kaminsky, Ph.D. - Chief Science Officer, Rettsyndrome.org*

Rettsyndrome.org will again play a central role as an administrative partner of the 5-year cooperative agreement and as a member of the Coalition of Patient Advocacy Groups. Enrollment information for this version of the Natural History Study will be available in the coming months. The last study allowed our researchers to follow nearly 1,200 girls and women with typical and atypical Rett syndrome.

We are very hopeful that the Rett community will again be enthusiastic about participating in the study. We are also grateful for the commitment of our physician research community and the NIH.

*Below is a list of the 11 specialty Rett centers.*

*Please contact Paige Nues at [pnues@rettsyndrome.org](mailto:pnues@rettsyndrome.org) for enrollment questions.*

## Participating Sites

1. University of Alabama at Birmingham – Alan K. Percy, M.D.
2. Baylor College of Medicine, Texas Children’s Hospital – Daniel G. Glaze, M.D.
3. Harvard Medical School, Boston Children’s Hospital – Walter E. Kaufmann, M.D.
4. Greenwood Genetics Center, Greenwood, SC – Steven A. Skinner, M.D.
5. Children’s Hospital of Philadelphia – Eric D. Marsh, M.D., Ph.D.
6. University of Rochester Medical Center – Alex R. Paciorkowski, M.D. FACMG
7. University of Colorado Medical School, Denver – Timothy A. Benke, M.D. Ph.D.
8. University of California, San Diego, Rady Children’s Hospital – Jeffrey L. Neul, M.D., Ph.D.
9. Vanderbilt University Medical Center – Sarika U. Peters, Ph.D.
10. Rush University Medical Center (Chicago) – Peter T. Heydemann, M.D.
11. University of California, San Francisco, Benioff Children’s Hospital – Mary Jones, M.D.

## Current Clinical Trials in Rett Syndrome



See our recent blogs where Steve Kaminsky Ph.D. of Rettsyndrome.org discusses the drug interventions covered by the ClinicalTrials.gov web site. All of these trials are important.

Steve discusses all of them, not just those that Rettsyndrome.org is involved with. Don't miss this expanded blog packed with information and updates. [rettsyndrome.org/our-role-and-impact/blog](http://rettsyndrome.org/our-role-and-impact/blog)

## Drugs in active trial:

IGF-1  
NNZ-2566  
Desipramine  
Fingolimod  
Dextromethorphan  
Copaxone  
EPI-743



# Thank You for Raising Over \$2.7 Million through Events

Many thanks to all of you who helped to raise over \$2.7 million this year. Whether you chaired a Strollathon, ran as a Rett Racer, or hosted another event, your help is our hope! This \$2.7 million brings us one step closer to discovering treatments and a cure for Rett syndrome.

**Stroll & Roll for Rett Syndrome:** This year's strollathons raised the largest portion of this \$2.7 million. Held in more than 25 cities, these events raised over \$980,000. Some cities marked their 10th anniversary or even longer. For example, in Roanoke Virginia, Strollathon Chair, Petrina Pate had amazing success in her 10th year – raising almost \$44,000.

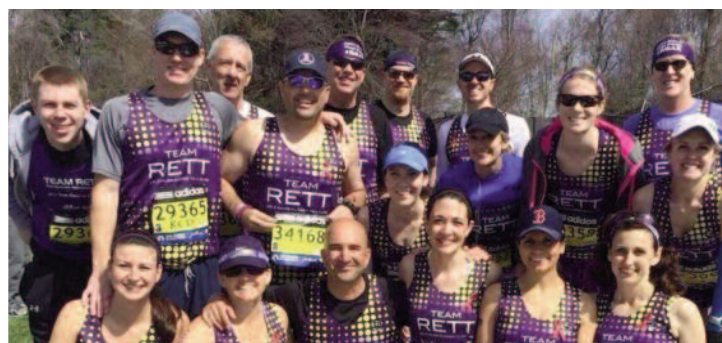
In its 11th year, the Cincinnati Tristate Strollathon was the most successful – raising nearly \$158,000. Linda Klare contributed significantly by donating \$18,260 that she raised from the sale of a cookbook that she wrote.

Florida and Texas Strollathons also enjoyed tremendous success. The Allocco family of Coconut Creek, Florida was again the largest contributor for their local strollathon – which has been chaired by Henry

Perez for five years. And in Texas, there were strollathons in San Antonio, Grapevine, and The Woodlands hosted by Rosie Ortega, Vanessa Peace, and Kenna Seiler, respectively.

We would also like to recognize five new Strollathon chairs: Gwen Noecker (of Raleigh North Carolina) raised over \$20,000, Erica Foreman (of Charlotte, North Carolina) raised over \$14,000, Andrea Zak (of Waterford, CT) raised over \$8,500, Julie Peden and Julie Langzettell (Millvale, Pennsylvania) raised nearly \$14,000, and Brenda Turley (of Reno, Nevada) raised over \$12,000.

These are just a few of the cities and people who contributed to this year's success to whom we are extremely grateful.



**Going the Distance:** Our Rett Racers have also been busy putting in the miles this year. In over 15 cities, they have collectively covered more than 450 miles and raised nearly \$212,000. This year we were a registered charity in two marathons – the Louisiana Marathon, which raised over \$141,000, and the Medtronic Twin Cities Marathon, which raised over \$22,000. Thank you Kathryn Kissam and Erin and Mitch Bleske for bringing these events to the Foundation!

Other notable racers were those that raced in the Boston Marathon. After 2013's tragic event, these steadfast Boston runners returned to finish the race. They ran in honor of those that lost their lives, those that were injured, and they ran in honor of our girls and women with Rett syndrome. Thank you!



**Golf Tournaments:** We've heard about the strollers and the runners, but those who drive around in golf carts also deserve a grateful Thank YOU for raising over \$640,600 for Rett syndrome. The Samantha Corpus golf event raised over \$250,000 for the second year in a row while the San Diego golf tournament (hosted by Dierdre Gonzales and her army of volunteers) had another great year. The McCool Family of St. Louis hosted their first tournament, raising over \$40,000, and they intend to make this an annual event. Some other golf events were held by Mike Joyce of Boston; Lynn Peel of Maine; Leslie Greenfield of New Jersey; Jim Bathie of Wisconsin; and Rick Troy of Massachusetts. The Fay family of Connecticut held their 18th annual tourney, while BNSF Railroad hosted their first time golf event in California.





## Raising Over \$2.7 Million through Events *continued*

**Family Fundraisers, Lemonade Stands & Special Events:** Many families raise money by hosting unique family fundraisers. This year, more than 50 families held online fundraisers, raising over \$245,000; 15+ Lemonade Stands contributed \$63,500; and a number of special events raised nearly \$598,000.

Seventy-two year old grandfather, Edward Curtis is one example of the creative ways that families raise money. Edward decided that he wanted to do something for his granddaughter, Alia, so he did what he knows best – cycling. He created the Rett Riders and managed his own team, Team Alia, which raised over \$23,000 and rode 100 miles! In Ed's words:

*"On September 21, 2014, Team Alia competed in the "Ride the Rivers Century" bike race from the Mississippi to the Missouri river. The Rett Riders rode 100 miles. The wind was tough, and I spent more than seven hours in the saddle. But I kept thinking of Alia. My body was toast, but I still felt happy."*



## Thank YOU for Making National Awareness Month a Huge Success

October is our special month for raising awareness of Rett syndrome and its immeasurable impact on our children, our families, and our community. Every two hours a girl is born who will develop Rett syndrome. Despite its prevalence, very few people know about Rett syndrome. We greatly appreciate all of your fundraising and awareness events during October. And thank you for participating in the What Would You Give campaign!

## Cheer on our Rowdy Rett Racers in Baton Rouge

We are thrilled to be an official charity partner of the 2015 Louisiana Marathon which will be held January 16th-18th in Baton Rouge, LA. Join the fun as a runner, a fundraiser or cheerleader for our Rowdy Rett Racers. \$50,000 in sponsorship has already been donated by the Albemarle Foundation.

Kathryn Kissam, Rettsyndrome.org board member, will host our race weekend activities including a dinner on Saturday as well as a gathering area to celebrate after the run.



Nike will reward our Rett Racers who reach various fundraising levels. Contact Melissa Florio, Development Coordinator, at [mflorio@rettsyndrome.org](mailto:mflorio@rettsyndrome.org) for details.



# Research to Reality Fund

## Our single, urgent goal: Treat and Cure Rett Syndrome in our Lifetime

Rettsyndrome.org has geared up for a multi-year fundraising initiative – the Research to Reality Fund. Our scientific strategy is to accelerate research and aggressively move more potential drug treatments to clinical trials. The Fund is our most important fundraising need. Dedicated to identifying treatments and a cure for Rett syndrome, the Fund builds upon our successful history of high-quality and peer-reviewed research.

By donating to the Research to Reality Fund, you will be helping improve the lives of thousands with Rett syndrome and tens of thousands more people who also have an MECP2 related disorder. The time is now. Please donate today. Together we can accelerate research to reality.

For more information, please contact Robert Copeland, Chief Development Officer, at 513-454-1301 or [rcopeland@rettsyndrome.org](mailto:rcopeland@rettsyndrome.org).



*"[Rettsyndrome.org] has been an amazing force in advancing our understanding of Rett syndrome research over the last 25 years. One day, we will have a great treatment for these girls. "*

*– Dr. Huda Zoghbi*

## Meet our New Team Members

The key to success starts with the right people. Rettsyndrome.org is pleased to welcome Robert S. Copeland as Chief Development Officer and two new board members – Peter White and Scott Parven. Robert will be responsible for developing a strategic fundraising plan and overseeing our development team. Additionally, Peter and Scott bring important marketing and government relations skills to our team.

**Robert Copeland** has nearly 25 years of fundraising experience at several universities. Since 1998, he has held a number of positions at Wright State University. Most recently, he served as Assistant Vice President for Advancement in the Boonshoft School of Medicine. In his previous role as Wright State's Associate Vice President of Advancement, Robert was the chief architect for the university's first-ever comprehensive campaign, raising \$123 million two years ahead of schedule and more than tripling the \$40 million goal. Prior to his Wright State positions, Robert worked in the fundraising offices at Wilberforce University and the University of Dayton.

Similar to the expertise that Robert brings to our team, our two new board members also have professional skills that are important to our work. **Peter White**, for example, is the Chairman and Cofounder of Attention Span Media and Chairman and Cofounder of Global Oversight. We are already grateful for his commitment to Rettsyndrome.org, because he and members of his Attention Span Media team helped to design our new website and the new marketing materials for our name change to Rettsyndrome.org.

New board member, **Scott Parven** also brings specialized professional skills to our team. Currently a partner at the law firm Akin Gump, Scott specializes in government relations and public policy. His political acumen and policy advice have earned him accolades from numerous publications, including "Best In The Business" by *The Hill* newspaper and *National Journal* magazine.



*"I am absolutely thrilled about the opportunity to build and sustain a culture of philanthropy that will dramatically expand our research and the families that we are all working vigorously to support."*

*– Robert Copeland,  
Chief Development  
Officer*



## Meet an Angel, Francesca Scarano

We like to regularly spotlight one of our many inspiring Rett angels, so please meet Featured Angel, Francesca Scarano.

Hi! My name is Francesca. I am the apple of my dad's eye; he works endlessly to make sure I have everything I need. I have a brother, Domenico, who is 16 years old. Domenico is my favorite.

When I'm scared or in pain, I always look for him to comfort me. I also have the special privilege of living with my Grandmother, who has dedicated every free minute she has to help my family with me. My family thinks I'm quite a character. Despite this horrible diagnosis, they see how I have managed to allow my wonderful personality to shine through.

Visit [rettsyndrome.org](http://rettsyndrome.org) to meet all of our featured angels.



### Angel Awareness Cards

Tell the world about your angel with Rett syndrome with these [Rettsyndrome.org](http://Rettsyndrome.org) personalized Angel Awareness Cards. Request your [Rettsyndrome.org](http://Rettsyndrome.org) Angel Awareness cards - then print and share them with your community.

## GIVE THE GIFT OF AWARENESS FOR THE HOLIDAYS

Check out [Rettsyndrome.org](http://Rettsyndrome.org)'s NEW merchandise and spread awareness with every gift you give for the Holidays! From bracelets to t-shirts to hats and more, [Rettsyndrome.org](http://Rettsyndrome.org) has just what you need to help spread awareness.



## Let's Vacation at Disneyworld in 2015



[Rettsyndrome.org](http://Rettsyndrome.org) will host a virtual conference in 2015. Presentations will be streamed online with Q & A opportunities for the audience. All presentations will be recorded and available for future viewing. We will meet together again for a traditional conference and science symposium in June of 2016 in the Chicago area.

We realize that 2016 is too long to go without connecting in person! So we encourage families to meet with us at a destination resort in Orlando in October, 2015.

Contact Paige Nues at [pnues@rettsyndrome.org](mailto:pnues@rettsyndrome.org) for details.

## Join a Network!

One of the ways Rettsyndrome.org provides connectivity to families is by hosting virtual communities through Facebook and e-mail. These networks cater to specific groups – such as educators, dads, single parents, grandparents, etc. We also host a bereavement group – the Life After Rett Network.

In October, Debra and Clarence Stage were featured as “Grandparents of the Month.” The Grandparents Network is primarily an email community that helps grandparents understand Rett syndrome and connect with others who are experiencing the emotional turmoil of supporting both their adult child and grandchild who is struggling with Rett syndrome.

Visit [rettsyndrome.org/for-families/get-support](http://rettsyndrome.org/for-families/get-support) to read more about our networks, meet our featured network members, and register for the one that best supports you.



## From Knowledge Will Come A Cure

Organ and tissue donations are one way that families can help researchers unravel the mysteries of Rett syndrome. You can help with this research by donating a sample of your child's blood or tissue when she has a routine blood draw or undergoes a medical or surgical procedure (e.g., skin or muscle biopsy, endoscopy, scoliosis surgery). A difficult thing to think about in times of good health is organ donation. It is an important gift of hope towards the discovery of treatments and a cure. The best time to consider this option is before you have to. We urge U.S. and Canadian families to please consider this selfless and courageous gift. It is also helpful to know that the donation process must begin within hours of passing before any preparations. We partner with the Harvard Brain Tissue Resource Center. Their kind and respectful staff will handle all arrangements directly with the hospital, hospice/palliative care nurse, coroner, or pathologist. For more information, contact Paige Nues at 510.499.3858 or [pnues@rettsyndrome.org](mailto:pnues@rettsyndrome.org).

## Life After Rett

We encourage all families who have lost a family member with Rett syndrome to request to join the "Life After Rett" Facebook group. This is the place to find support and sympathy from those who understand. Members support each other and offer encouraging ways to commemorate their loved ones through continued involvement in the Rett syndrome community. This support network is sponsored by Rettsyndrome.org and moderated by parents who have been there, Jane [jgeorge@globetrotter.net](mailto:jgeorge@globetrotter.net) and Elaine [cowgirdanidee@comcast.net](mailto:cowgirdanidee@comcast.net).

## Memorials

*The loss of every child is a loss for our entire community. Please join us in a moment of reflection for all the beautiful children who have gone before us, and especially for these families who have asked for our support during their loss this year. We promise to learn from each and every one of them, remember them always, and allow their lessons to guide our efforts forward. In remembrance of:*

Anne Stuart-Grantham – age 26  
Brianna Violet Frost – age 12  
Brooke Pinsonat – age 23  
Casey Rose Calista – age 13  
Clancy Stuart – age 32  
Gabriella Cellini – age 32  
Jasmine Lara – age 13  
Jenna Brobst – age 30  
Karissa Fabiano – age 14  
Katelyn True – age 20  
Robert Mohr – age 19  
Rylie Francis – age 11  
Sherita Smith – age 26  
Susannah Hall – age 49

*To allow our community to extend their support, full Memorials will now be posted to [www.rettsyndrome.org](http://www.rettsyndrome.org)*



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## THANK YOU TO OUR PARTNERS FOR MAKING OUR WORK POSSIBLE

### HER Heroes (\$50,000+)



### HER Knights (\$25,000 - \$49,999)



### HER Angels (\$10,000 - \$24,999)

- Allen, Matkins, Leck, Gamble, Mallory & Natsis, LLP
- The Anastasi Fund
- Brookfield Financial Properties, LP
- Casting 4 A Cure
- The Cornelius F. Doolan Memorial Fund
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- Hershey Entertainment & Resorts Company
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## Thank You for Your Support through the United Way & the Combined Federal Campaign

A special thanks to those of you who donated through your local United Way campaign or Combined Federal Campaign. Your donations help us continue research of Rett syndrome and its eventual cure. We greatly appreciate your generosity! If you have a friend or relative who works for the federal government or is in the armed services, please consider letting them know that Rettsyndrome.org's CFC Code is 11046 so they too can designate their donation for our important work.

## Shop Amazon for Rett Syndrome During the Holidays

Shop at AmazonSmile  
and Amazon will make  
a donation to:



Get started

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Raise money for Rett syndrome while working your way down you holiday list! When purchasing on Amazon, use [www.smile.amazon.com](http://www.smile.amazon.com) instead of your usual [www.amazon.com](http://www.amazon.com) and select Rettsyndrome.org (International Rett Syndrome Foundation) as your charity of choice. Amazon will donate 0.5% of your purchase to Rettsyndrome.org. Thank you!

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