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rett gazette



Introducing Rettsyndrome.org

It's the beginning of a new era for the Rett syndrome community. The International Rett Syndrome Foundation isn't going away; we're evolving into a more focused comprehensive research and family empowerment organization doing business as Rettsyndrome.org.

We understand the urgency that exists with the speed of progress of research and the overall quality of care available to those dealing with the challenges of Rett syndrome.

We've advanced our comprehensive strategy to accelerate discoveries in fundamental research toward clinical to improve the lives and treatment of those living with Rett syndrome. Our emphasis on translational and clinical research will ensure that new ideas are constantly flowing into the drug pipeline and neuro-habilitation platforms aimed at accelerating the treatments for Rett syndrome.

We've committed to the growth of quality of life research woven together with reliable information, tools, resources, and communication forums to promote healthy and empowered families, educators, medical professionals, and the caring community. We have strong beliefs about what it's going to take to achieve this goal:

We believe the fastest way to achieve our research goal is by working together as a community. We're calling on all individuals, Rett syndrome organizations and communities around the world to unite and pool our resources to focus on only the most promising avenues (scientifically reviewed and vetted by a panel of unbiased and qualified evaluators) to help those dealing with the symptoms and daily realities of Rett syndrome.

We believe the best way to support someone dealing with Rett syndrome on a day-to-day basis is by sharing resources as a community. We are calling on all individuals, Rett syndrome organizations and communities around the world to unify and pool our information, intelligence, tools, and best practices to create a single, user-friendly, crowd-sourced family resource (vetted by a panel of experienced and qualified experts).

We believe the only way research can be adequately funded is by reaching out beyond the community of those directly impacted by Rett syndrome. We are calling on all individuals, Rett syndrome organizations and communities around the world to leverage the latest social and traditional media tools and resources to send creative personal communications and take attention-grabbing actions and to help raise awareness, recruit participation, develop fruitful relationships, and solicitations from individuals and organizations at a level never before achieved. In today's online era, we can crowd-source the cure.

The goal of Rettsyndrome.org is to make this vision a reality. We want to eliminate the threat of Rett syndrome as soon as possible. We want to help those living with Rett syndrome right now. We must accelerate the funding of research to levels never dreamed of before.

Our new logo brings this goal to life by including a hand reaching for a star. The star not only represents a cure, but also our ambitions to find answers that will shine light on and help those we love with Rett syndrome. We know most of our loved ones don't have full use or control of their hands, but this image signifies our goal of them one day reaching for and achieving anything they have their beautiful minds set on. We are reaching for the stars and if we all come together we will achieve this!

We can only do this with your help. Together we can empower change.

Rettsyndrome.org is YOUR source for achieving this shared goal. Join us.



We extend our deepest thanks to our friends at Attention Span Media for their help overhauling our new website, Wynne Creative Group for the creation of our beautiful new logo and all of our wonderful friends and volunteers like Barry Rinehart, Duncan Millar, Gus Rodriguez and so many more who helped us develop our new Rettsyndrome.org brand ALL PRO-BONO! We are forever grateful for the support each of you has given.

The Rett Gazette is published periodically by Rettsyndrome.org, a non-profit 501(c)(3) organization. The core mission of the IRSF is to fund research for treatments and a cure for Rett syndrome while enhancing the overall quality of life for those living with Rett syndrome by providing information, programs, and services.

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ABOUT RETT SYNDROME

Rett syndrome (RTT) is a devastating postnatal neurological disorder that occurs almost exclusively in females. It is usually caused by a mutation of the MECP2 gene on the X chromosome. Rett syndrome is found in all racial and ethnic groups throughout the world, and in every socio-economic class. RTT affects 1 in 10,000 live female births. Early developmental milestones appear normal, but between 6-18 months of age, there is a delay or regression in development, particularly affecting speech, hand skills, and coordination. A hallmark of Rett syndrome is repetitive hand movements that may become almost constant while awake. Other features may include seizures, irregular breathing, swallowing difficulties, deceleration of head circumference with age, and curvature of the spine. Many individuals with Rett syndrome live well into adulthood. There is currently no cure.

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Call 1-800-818-RETT (7388).

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POLICY STATEMENT

In an effort to improve the quality of life for those with Rett syndrome we welcome ideas from our readers on various techniques of management and care, general and educational suggestions that individuals have investigated and/or found beneficial. Parents and others have the opportunity to decide if they want to follow through with the suggestions.

As a foundation, we do not support or endorse any particular treatment, therapy, or medication. We encourage parents to support one another with suggestions and to contact their child's physician for final approval.

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WELCOME

Shannon Starkey-Taylor, Chief Operating Officer

Please welcome Shannon Starkey-Taylor as the new Chief Operating Officer of

Rettsyndrome.org. Shannon, who has specialized in the fields of strategic development, early childhood education and social work for over 20 years, will oversee the day to day operation of the foundation. Shannon was previously the Vice President of Strategic Development for The Children's Home of Cincinnati where she provided leadership to staff in child welfare, early care and education, alternative special education and family support services. Shannon has extensive experience in designing and implementing new programs, non-profit leadership, and establishing community collaborations.

"Shannon is a mission-focused and seasoned leader who will help the foundation deliver measurable, cost-effective results that make the vision a reality," says Rajat Shah, chair of the Rettsyndrome.org board. "Shannon's established success and strategic background will propel the pursuit of the Rettsyndrome.org strategic plan to expand our position as the leading private funder of Rett syndrome research and to vigorously pursue new endeavors."

Shannon joins at a critical time for the foundation with a heavy focus of raising crucial dollars for Rett syndrome research. Rettsyndrome.org anticipates that research momentum will accelerate towards additional clinical trials in the near future. Whether support for expansion of the current trials to Phase 3 or for implementation of Phase 1 trials for new drug candidates, Rettsyndrome.org is at the forefront of clinical research for Rett syndrome.

"Words cannot adequately express the gratitude we feel for our Rettsyndrome.org Board of Trustees. Thank you to Michael Joyce, Dr. Richard Altschuler and Barry Rinehart who have just termed off the board this summer. Your generosity and your investment of time has been a true gift. We are fortunate to have your continued leadership for key initiatives of the foundation. Thank you."

WELCOME Back to a Leading Board Member

Rettsyndrome.org is proud to welcome long time Board of Directors member, Gordon (Gordy) Rich, back to the Rettsyndrome.org Board.

Gordy, with the support of his wife Anne, created the Kelly Rich Rett Research Foundation in 1997 for their daughter Kelly, who had just been diagnosed with Rett syndrome, as well as funded the formation of the Kelly O'Leary Center for Pervasive Developmental Disorders at Cincinnati Children's Hospital. Kelly was diagnosed with Rett syndrome at Johns Hopkins Kennedy Krieger in 1997 at the age of 20 months.

In November of 1999, The Rett Syndrome Research Foundation (RSRF) was formed by a passionate group of parents, led by Gordy, who wanted to more aggressively pursue biomedical research to accelerate the discovery of treatments and a cure. In its first year the foundation funded over \$1M in research grants. Their achievements in funding new research and expanding the scope of knowledge was unsurpassed (including the discovery that most symptoms of Rett syndrome may one day be reversed).

In 2007 RSRF joined forces with The International Rett Syndrome Association (IRSA) to form the International Rett Syndrome Foundation (IRSF), which today is known as Rettsyndrome.org.

Even after his term ended, Gordy continued to play a key role in the foundation's fundraising efforts. With the Rettsyndrome.org headquarters located at Reading Rock, a family run company where Gordy is President, the employees of Reading Rock have played an important role in supporting the foundation through various fundraisers; one huge undertaking being the Tri-State Strollathon. The Strollathon program, Rettsyndrome.org's signature fundraising event, raised over \$8 million in the first ten years. The Tri-State Strollathon has totaled over \$1,798,646 making it the foundation's most successful strollathon since inception.

After four years, Gordy has decided to add Rettsyndrome.org board membership back to his mission. "Before Kelly was even diagnosed with Rett syndrome, I knew I needed to be a part of the cure for whatever my daughter had. Once we had the diagnosis, I knew that I needed to step up to raise the funds needed for treatments and a cure. I gave 100% before my leave and plan to give 110% now that I'm back."



Gordy, Kelly and Anne



Message from the
Chief Science Officer of Rettsyndrome.org

Steve Kaminsky, PhD

The timing of the 13th Rett Syndrome Symposium in June brought great excitement with the spectrum of ongoing research from basic discovery to clinical research. The timing of the

symposium brought great excitement with the spectrum of ongoing research from basic discovery to clinical research. As you know, Rettsyndrome.org is currently sponsoring two clinical trials that are moving forward each day, with data collection ending in the fall for the NNZ-2566 trial and the closing of enrollment for the IGF-1 trial this summer. Our greatest hope is that when the data is decoded, we find that one or both of the drugs is found to be an effective treatment for Rett syndrome so we can move to a phase 3 trial. Like us, the Rett community is very anxious to see the results; we all know how much work goes into moving basic and translational research to clinical trials today. Rettsyndrome.org and our Rett research community will continue to make new discoveries, and build upon these findings to bring even more treatments into the pipeline for testing in future clinical trials.

With these potential treatments on the horizon, Rettsyndrome.org has coined the phrase that our pharmacologic treatments are designed to "correct Rett biology". However, as we proceed down this road, we have to identify new technologies and therapies that "reset the neurology" in Rett syndrome. To this end, we have initiated a new program this year and it is called our Neuro-Habilitation Program.

We dedicated one of the sessions at this year's science symposium solely to this topic. The goal of the program is to identify the best cognitive therapies, physical therapies, occupational therapies and speech therapies that will aid in establishing the networks critical for skill development in Rett syndrome. We believe that the time is right to begin looking at this area of research so that the fruits of this labor mature at the same time as our programs that identify pharmacologic treatments. By conducting research on these two fronts, our hope is that the work will converge on each other, and we can match the best practices in drug therapy to the best practices in neuro-habilitation therapy. Together, we are hopeful that these two areas of research will be the fastest way to improve the quality of life for those suffering with Rett syndrome. Our keynote speaker for this year's symposium, Dr. Michael Merzenich, has stated:

"It is an important time for scientists to begin to more aggressively integrate a developmental/brain plasticity perspective, for both understanding the origins of the expressions of Rett, and for incorporating brain plasticity training both to increase resilience, and potentially drive correction."

Thank you to our community of families and researchers who are so committed to bringing research to reality. We at Rettsyndrome.org are grateful to make an impact in the lives of all families touched by Rett syndrome.

Sincerely,

Steve Kaminsky
Steve Kaminsky, PhD
Chief Science Officer



Opening doors to discoveries

The 13th Rett Syndrome Symposium has come and gone so quickly this year. Over 130 researchers, clinicians, therapists, allied health professionals and research advocates had joined this special science symposium. There were 31 speakers giving talks in four sessions (Basic Research, Translational Research, Neuro-Habilitation Research and Clinical Research), and 44 posters were presented at the Poster Session.

As Dr. Kaminsky highlighted in this accompanying article, the Neuro-Habilitation track is new to our symposium. Therefore, we invited Dr. Michael Merzenich, our special keynote speaker to address the importance of Brain Plasticity-based therapeutics and their relevance for Rett syndrome.

We would like to thank our session chairs for putting together this illuminating meeting and to all our attendees for participating and promoting Rett syndrome research. Communication and collaboration will bring us closer to treatments for our children. Thank you for your commitment to Rettsyndrome.org and our community.



Dr. Sacha Nelson, session chair of the Basic Discovery session, opens up the symposium.

Clinical Trial Resources

Two Rettsyndrome.org publications were written to help parents and legal guardians of children with Rett syndrome understand the importance of clinical trials, and what participation means, what to expect, what your rights are as well as the rights of your participating child. As part of our mission to empower families, Rettsyndrome.org has created these resources in response to parent questions about the two incredibly important clinical trials supported by Rettsyndrome.org today, the IGF-1 and NNZ-2566 clinical trials, and future trials that we will fund through the continued generous support of our donor community. Be informed!

A Participant's Guide to Drug Research in Rett Syndrome

Download: <http://www.rettsyndrome.org/document.doc?id=156>

Placebo Controls in Clinical Trials

Download: <http://www.rettsyndrome.org/document.doc?id=139>

In case you missed it....

Here are some 2013-14 Research Highlights.

June 2014 – A new review article gives perspective on targeting Rett syndrome with 5-HT1A agonists by Dr. Ana Paula Abdala and Dr. Julian Paton of Bristol University, Dr. John Bissonnette of Oregon Health and Science University, and Dr. Adrian Newman-Tancredi of Neurolix. Here they examine the therapeutic potential of drugs that activate serotonin 5-HT1A receptors. They are currently funded to study a 5-HT1A agonist called NLX-101, which could potentially ameliorate symptoms including autonomic dysfunction (e.g. breathing difficulties), mood deficits (e.g. anxiety) and motor dysfunction (e.g. dystonia). Scientists at the University of Bristol have received an ANGEL award from Rettsyndrome.org in partnership with Cure Rett to test this powerful new drug NLX-101, which may have the potential to alleviate at least one of the most distressful symptoms – the breathing abnormality.



Dr. Walter Kaufmann and Jennifer Endres talk about the IGF-1 clinical trial Phase I results!

March 2014 IGF-1 Phase 1 clinical trial data from Boston Children's Hospital was published. IGF-1 was deemed safe for treatment in Rett syndrome. Breathing abnormalities and anxiety in the small number of patients tested also improved. With your support, Rettsyndrome.org is now funding the larger IGF-1 Phase 2 trial in Boston. The purpose of Phase 2 is to now prove efficacy of IGF-1.

October 2013 – The Rett Syndrome gene MECP2 –Function Redefined 14 years after its Discovery. A new paper from Dr. Rudolf Jaenisch's laboratory of the Whitehead Institute was published in the journal *Cell Stem Cell* that will change how we think about MECP2. Using cutting edge technology, the researchers created new tools to model Rett syndrome at the cellular level. This research means that (1) We have to start looking at MECP2 and the genes it 'turns on' differently; (2) We must continue our search for relevant pathways to target therapeutically; (3) We have new cellular tools that could be used for in vitro testing of compound; and (4) IGF-1 therapy is one that has great potential and illustrates the wisdom behind our support of the current Phase 2 IGF-1 clinical trial at Boston Children's Hospital and the Phase 2 NNZ-2566 clinical trial at Baylor College of Medicine. This work was co-funded in part by Rettsyndrome.org, Rett Syndrome Association of Massachusetts, Simons Center for the Social Brain, Brain and Behavior Research Foundation, Croucher Foundation, Swedish Research Council, National Institutes of Health, Koch Institute, Curt Marble Cancer Research Fund, Simons Foundation Autism Research Initiative, and Ethel Louise Armstrong Foundation.

Visit <http://www.rettsyndrome.org/research-irflash/orsam-2013> to read the full commentary.

July 2013 - Dr. Monica Justice and her colleagues at Baylor College of Medicine in Houston, TX, published a new study in the journal *Nature Genetics* about a novel basic discovery and its translation to an early preclinical study. The results: a new therapeutic avenue for RTT to pursue. They showed that the cholesterol metabolic pathway is a possible target for treatment, and that treatment with statin drugs can improve the symptoms in RTT mice. This exciting work was funded by Rettsyndrome.org, RSRT, ASF, and the NIH.

Check out a blog we wrote along with our partner CureRett. Visit: <http://www.rettsyndrome.org/our-role-and-impact/news/blog>

January and July 2013 – Two papers on gene therapy in the mouse model of Rett syndrome were published. In January 2013, the first paper on gene therapy discussed the possibility of using a virus, called AAV9, to do gene replacement in Rett mice. Essentially, they used AAV9, an altered version of the common cold virus, to encapsulate healthy MECP2 and sneak it into the brain cells. In this study scientists Stuart Cobb, Steven Gray and their teams demonstrated reversibility of RTT-like phenotypes in mice. This suggests that MECP2 gene replacement is a potential therapeutic option in patients and that these results support the concept of MECP2 gene therapy for RTT.

The study reported improvements in motor function, tremors, seizures and compulsive movements. One symptom which didn't respond to the therapy was abnormal breathing.

They concluded the paper by saying that this work shows, at "proof-of-concept level", that MECP2 can be delivered at a tolerable level via AAV2/9 vectors to the brain of Mecp2-null mice. Longer-term studies in female mice are the next steps before any human application can be considered. The first study into gene therapy for Rett syndrome concluded that, yes, it is possible that a virus could deliver healthy MECP2. But it ended with the next step being the need to now test in female mice.

A second paper published in the *Journal of Neuroscience* from Dr. Gail Mandel's lab at Oregon Health and Sciences University, used a similar virus as the first study. The same results were seen as in the first study, including non-reversal of breathing problems. What was different about this particular study (other than the fact that it was done using female mice) is that a 10-15% uptake of MECP2 in the brain was achieved to get a reversal of symptoms as opposed to only 5% uptake in the first study.

These gene therapy studies are examples of discovery based research and there is still a lot of work before they can be transferred to the clinical environment.

Check out the full commentary on a blog we wrote along with our partner CureRett. Visit: <http://www.rettsyndrome.org/our-role-and-impact/news/blog>

February 2013 - A basic research study from Dr. Huda Zoghbi's group was published in the journal *Cell*, and reported a highly stimulating discovery of an important protein characteristic and function of MeCP2 on DNA structure. On the basis of clinical observations of male patients who harbored different mutations in the MECP2 gene and exhibited very different symptoms, the Zoghbi group set out to generate two new mouse models, bearing two different truncation mutations (G273X and R270X -both cause shortened MeCP2 protein). The results suggest that MeCP2 with the G273X mutation retains the ability to compact chromatin and influence ATRX-DNA binding, while MeCP2 R270X protein is lacking a key protein structure called the AT-Hook along with this structure's essential functions.

Read the full commentary at <http://www.rettsyndrome.org/irflash/march-2013>

2013-2014 Research Funding News

During 2013 and the spring of 2014, Rettsyndrome.org funded

- ✓ 20 new research grants at leading global research institutions;
- ✓ implemented a new Translational Research Program;
- ✓ renewed 2 Rett Database contracts;
- ✓ started a working group of scientists to study Rett syndrome in a rat model;
- ✓ approved additional funding for current human clinical trials.

The funding awarded in 2013 covers the spectrum of Rett syndrome research, from basic discovery starting at the genetic level all the way to treating the girls diagnosed with Rett syndrome. Awards are not restricted by geographic boundaries, and several of the new Rettsyndrome.org grants are invested in researchers from Canada, Italy, and Australia. Rettsyndrome.org remains steadfast in recruiting talented young scientists to the research bench for Rett syndrome, and welcomes three new Rettsyndrome.org fellows into the Rettsyndrome.org training fellowship program.

New 2014 Translational Research Awards

ANGEL Award

- Julian Paton, PhD, University of Bristol
NLX-101 - a novel, highly selective and potent serotonergic 5-HT_{1A} receptor agonist for the treatment of respiratory arrhythmias in Rett Syndrome: a pre-clinical proof-of-principle study in murine models

HeART Awards

- Michela Fagiolini, PhD, Boston Children's Hospital
Assessing NMDA_R modulators to ameliorate cortical regression in Rett Syndrome

HeART Awards – Neuro-Habilitation Program

- David Koppenhaver, PhD, Appalachian State University

Investigating Visual Attention to Print in Children with Rett Syndrome

- Charles Nelson, III, PhD, Boston Children's Hospital

New Methods to Assess Cognition and Affect in Girls with Rett Syndrome

- Susan Bruce, PhD, Boston College
Sensory Integration to Increase Functional Reaching in Children with RTT/Rett-Related Disorders

- Sarika Peters, PhD, Vanderbilt University Medical Center

Auditory processing, language, and learning in Rett and Rett-related disorders

- Pamela Diener, PhD MS OTL, Marymount University and Jack Engsberg, PhD, Washington University in St. Louis

Improving Upper Extremity Motor Skills in Children with RTT Using Selected Movements to Control Internet Virtual Reality Computer Games

New 2013 Translational Research Awards

- Theresa E. Bartolotta, PhD, Seton Hall University and Patricia Remshifski, PhD, Monmouth University

Eyetracking in Rett syndrome: A preliminary investigation of receptive and expressive vocabulary

- John Christodoulou, AM, Sydney Children's Hospital Network, Australia
Preclinical Evaluation of Tubastatin A, a Novel Therapy for Rett Syndrome

- Steven Gray, PhD, University of North Carolina at Chapel Hill
BDNF gene transfer for the treatment of RTT

- Jeannie Lee, MD, Ph.D, Massachusetts General Hospital
Treating Rett syndrome via selective reactivation of the silenced MECP2 allele

New Basic Research Awards-Regular Research Grants

- Kerry Delaney, PhD, University of Victoria, Canada

In vivo imaging of spine turnover rates in the heterozygous female Rett mouse brain

- Xinyu Zhao, PhD, University of Wisconsin-Madison

Restoring network integration of MeCP2-deficient neurons

- Serena M. Dudek, PhD, National Institute of Environmental Health Sciences
Role of IGF1R in hippocampal CA2 plasticity and function: interaction with MeCP2

New Mentored Training Fellowships

- Farshad Moradpour, PhD, University of Victoria, Canada

Neurophysiology of cortical neurons in heterozygous female Rett mouse brain

- Deivid Rodrigues, PhD, The Hospital For Sick Children, Canada

Post-transcriptional control of MECP2 expression during human development and disease

- Rocco George Gogliotti, PhD, Vanderbilt University Medical Center

Temporal divergence of hypoconnectivity and excitotoxicity in Rett syndrome

In the Fall 2013 newsletter, we announced the Scout Program.

Progress has been made, and the program is now implemented and the Contract Research Organization is geared up to test compounds now. As a refresher, the Scout Program is a Drug Discovery Screen in a mouse model of Rett syndrome that will aggressively accelerate the testing of compounds, whether new or repurposed, through standardized preclinical studies. The Scout Program was created to replicate and confirm encouraging results stemming from studies performed in academic labs as well as testing of compounds no labs are pursuing. PsychoGenics, Inc. is the testing bed for this program and will independently test selected drugs using the set standards in preclinical studies for Rett syndrome. Outcomes from this program will provide the complementary high quality data required to move promising compounds through federal review and along the drug development pipeline toward the clinic.

New Rettsyndrome.org Scout Program

2013 Contract Research Organization

- Daniela Brunner, PhD, PsychoGenics, Inc.
A Drug Discovery Screen in a Mouse Model of Rett Syndrome

2014 HeART Awards – Scout Program

- David Katz, PhD, Case Western Reserve University

Preclinical evaluation of LM22A-4

- Bianca De Filippis, PhD, Istituto Superiore di Sanità

Preclinical evaluation of the bacterial protein CNF1 as a novel therapeutic approach for Rett syndrome

- Maurizio Giustetto, PhD, Università di Torino
Restoring the AKT/mTOR pathway to treat Rett syndrome

2014 Database Contracts

- John Christodoulou, AM, Sydney Children's Hospital Network, Australia *RettBASE*
- Helen Leonard, MBChB MPH, Telethon Institute for Child Health Research, Australia *InterRett*

Supplemental Funding for Current ANGEL Awards

- Walter Kaufmann, MD, Boston Children's Hospital

A Phase 2b placebo-controlled cross-over study of rh-IGF1 (mecasermin [DNA] injection) for treatment of Rett syndrome and development of Rett-specific novel biomarkers of cortical and autonomic function

- Daniel Glaze, MD, Baylor College of Medicine
A randomized, double-blind placebo controlled trial of NNZ-2566 (IGF-1{1-3}, glycyl-L-2-methylprolyl-L-glutamine acid) with open label extension in adults with Rett syndrome

Check out the Rettsyndrome.org blog!

It's a new online resource for everything our community is interested in from research topics to family empowerment. Website: <http://www.rettsyndrome.org/our-role-and-impact/news>

A Human Clinical Trial Study on NNZ-2566

4/17/2014

Contributors: Elizabeth Halford, Wordsmith, Cure Rett; Nancy E. Jones, PhD, Senior Director, Clinical Development and Medical Affairs, Neuren Pharmaceuticals; Steve Kaminsky, PhD, Chief Science Officer, Rettsyndrome.org; Janice Ascano, PhD, Manager of Grants & Research, Rettsyndrome.org

If you follow Rett research closely (or maybe even loosely), NNZ-2566 will be a drug name you're familiar with. But if you're looking for information about this potentially groundbreaking new medication and the implications it may have for Rett syndrome, please read this Research Digest we developed with Cure Rett and Neuren to answer questions.

NNZ-2566 is a medication being developed by the small pharma company Neuren. Neuren is a New Zealand company which has offices in Australia and the United States. They have special interests in areas of neuroscience where there are gaps in treatment development. Neuren currently has a growing portfolio on traumatic brain injury, neurodegenerative disorders and neurodevelopment disorders. Learn more about Neuren at <http://www.neurenpharma.com/>

There are very few human clinical trials actually going on for Rett syndrome and NNZ-2566 is one of them. The trials are happening in America as we speak.

WHAT

So the first question we aim to answer for you is "what is it?" We have a protein prevalent in our bodies called IGF-1. Its function is to help cells grow. It is an important component in the actions of the growth hormone in all of our bodies. In the brain, it helps cells to divide and make synaptic connections from neuron to neuron. However, people with Rett syndrome have issues with their IGF-1 because MeCP2 affects its expression. Now, where NNZ-2566 comes into this mix is this: by looking at the IGF-1 protein and which bits of the whole package could be beneficial in treating Rett, scientists have found that the first 3 amino acids of IGF-1 are what's needed.

IGF-1 does not readily pass from our circulation into the brain. However, a small piece of IGF-1, called a tripeptide, is able to pass from the blood and into the brain. NNZ-2566 is a synthetic version of the tripeptide that has been modified so that it can be absorbed orally, passing from the digestive tract into the circulation and then into the brain. Evidence from the laboratory suggests that the tripeptide is the part of IGF-1 that seems to be effective in treating Rett Syndrome. A trial of NNZ-2566 is underway in adolescents and adults with

Rett Syndrome at Baylor College of Medicine, Gillette Children's Specialty Healthcare and the University of Alabama - Birmingham. A trial of IGF-1 in young girls is underway at Boston Children's Hospital.

HOW

NNZ-2566 is delivered as an oral, strawberry flavored liquid whereas IGF-1 has to be injected.

CONTRAINDICATIONS

Just like any drug, IGF-1 isn't suitable for everyone. When growth hormones are given after puberty, they can cause continued bone growth so there's a window of time when this could be administered as a treatment. However, NNZ-2566, which is a peptide and not the whole protein, does not appear to have those same issues. The clinical trial with NNZ-2566 is designed to show that it is suitable for teenagers and adults with Rett. This means NNZ-2566 could have the potential to be suitable for anyone with Rett at any period of their life, something that no other suggested treatment we've ever seen can claim.

THE BENEFITS

You may remember a while back, we posted about gene therapy and said that, while treatment with vectors is an interesting idea, there are great challenges. That these methods were like using a shotgun when what we need is a sniper rifle. Well, IGF-1 is like a sniper rifle. Through the current trials, scientists are hoping to deliver more growth enhancers into the central nervous system which gets right to the central problem immediately, bypassing the need to deliver a new gene with a vector and hope it is expressed to correct the defective MeCP2.

But of course, we've already mentioned that IGF-1 needs to be injected while NNZ-2566 is an oral medication to hopefully deliver the exact same results and be potentially suitable for any age.

THE CHALLENGES

Like every current suggested method for the treatment of Rett, there are a few challenges to face. The fundamental challenge with NNZ-2566 is that it is a new drug, and new drugs have to go through a lot of rigorous testing before they can be used on all age groups. Since NNZ-2566 was developed by Neuren for traumatic brain injury, it has had a sizable amount of safety testing already completed and has been shown to be generally safe and well tolerated by adult human subjects. However, this fact does not remove the need for safety evaluation in Rett patients.

CURRENT STUDIES

NNZ-2566 is currently being studied in human clinical trials for both Rett syndrome and traumatic brain injury. Both conditions have something in common in that there are

weak synapses in the brain. A study in Fragile X syndrome has started this year. Phase 1 (the safety tests) have been completed. We know that it's a well-tolerated drug.

Phase 2A began in March 2013 and hopes to end in September this year. These are currently being undertaken at Baylor College of Medicine with Drs. Dan Glaze and Jeff Neul at the University of Alabama - Birmingham with Dr. Alan Percy, and at Gillette Children's Specialty Healthcare with Drs. Tim Feyma and Arthur Beisang. This trial consists of 60 participants between the ages of 16-45.

The Phase 2 study examines the safety profile, looking for unexpected adverse changes, deterioration or anything that wouldn't be expected in the natural progression of Rett syndrome. This is where the ongoing Natural History Study adds value. Unless we study Rett in the long run and learn what happens and the effects we can expect, it's difficult to measure the effectiveness of drugs we want to trial for Rett. The data extracted during the Natural History Study gives us baselines of what to expect so that during these types of trials, we can know what would normally have been expected and gives scientists a greater base of knowledge for use when monitoring such trials.

This phase is also measuring efficacy (effectiveness).

Based on the tests with mouse models, we're hoping to see improvements in breathing and cardiac function, and decreases in seizures. The patients are also being observed for changes in many other behaviors.

THE NEXT STEP

The next step for NNZ-2566 will be proof of concept studies in the pediatric population. Remember, the youngest person in the current study is 16 years old because the FDA guidelines are such that a drug usually cannot be tested on juveniles until the trial has first been successful with adults (when adult patients are available).

RECENT UPDATE:

Enrollment for the NNZ-2566 clinical trial was completed in June! Read Neuren's press release at <http://www.proactiveinvestors.com.au/companies/news/55870/neuren-pharmaceuticals-completes-enrolment-for-rett-syndrome-clinical-trial-55870.html>.

FUNDING

This is where partnerships are incredibly important. Rettsyndrome.org started the Baylor study with a \$600,000 ANGEL grant to get it going, but this doesn't even come close to covering the cost. Partnership with Neuren is extremely important to bring more resources to the table for these trials.

CLINIC SPOTLIGHT

Kennedy Krieger Institute
707 North Broadway
Baltimore, MD 21205
For appointment, call
KKI's Care Management office: 1.888.554.2080
Visit: www.kennedykrieger.org/patient-care/diagnoses-disorders/rett-syndrome



The Rett syndrome outpatient clinic at the Kennedy Krieger Institute (KKI) has been in effect since the 80's. More recently it is being made into a comprehensive multidisciplinary outpatient clinic to maximize medical input during the same visit where patients can obtain services from multiple specialists. This multidisciplinary clinic is being established to avoid the parents having separate evaluations with various subspecialists for ongoing care, and to optimize the interaction among the specialists. This multidisciplinary clinic dedicated to children with Rett syndrome proposes to improve total care for the child, where the subspecialists communicate with each other during the child's visit.

These specialists at KKI and Johns Hopkins have vast experience with multiple aspects of Rett syndrome because of the long history of clinical and research activities they have conducted over the past 25 years. These specialists will include neurologists, physiatrists (OT, PT), communication specialists, endocrinologists specializing in bone health and a gastroenterologist with a nutritionist. While utilizing all the services may not be essential for some cases, the visits to the Rett clinic will be tailored to the need of each child. For example, should a patient have severe rigidity or dystonia, a physiatrist and neurologist along with a specialist in baclofen pumps would provide input towards the patient's care. Should behavioral issues be a concern, an experienced psychiatrist will be requested to evaluate the child. If bone injury is suspected as a cause of pain or behavior change, X-ray facilities are available for immediate confirmation and an orthopedist can attend on an as needed basis.

The Rett multidisciplinary outpatient clinic will be headed by Drs. Constance Smith-Hicks and SakkuBai Naidu.

Families may call Kennedy Krieger's Care Management office, Toll free: 888-554-2080, for pre-admission intake to the Clinic. We anticipate the Clinic being open shortly.



Dr. SakkuBai Naidu, Rettsyndrome.org's 2014 Art of Caring award recipient, pictured in 1985 with Dr. Andreas Rett and others, landmark meeting in Baltimore, 1985



BACK TO SCHOOL IS NEARLY UPON US

School is very exciting. It can also be a time of additional stress and worries for parents and caregivers of children with Rett syndrome. We want to help make this school year as successful as possible. With a little summer-time planning and preparedness, the school year will start off just right.

Visit <http://www.rettsyndrome.org/for-families/school-and-day-programs> for ideas. Don't wait to have all adapted equipment sized, checked, and prepped. It may take awhile to get the appointment, and you may never get back around to it. Her safety and comfort are essential to a great school year.

"Childhood is not a race to see how quickly a child can read, write and count."

"Childhood is a small window of time to learn and develop at the pace that is right for each individual child."

OCTOBER RETT SYNDROME AWARENESS MONTH IS ALMOST HERE!



Let's shine a spotlight on Rett syndrome throughout October. Our children deserve to be counted. Visit www.rettsyndrome.org/get-involved/october-awareness-month to kickstart your planning ideas and find support materials tailored just for you.

Check www.facebook.com/RettSyndrome daily throughout October to see who's doing what, and pickup Facts to Share.

**Together we are making a difference.
We can't wait to see and hear about your
October Rett Syndrome Awareness activities!**

30TH ANNUAL FAMILY CONFERENCE

The 2014 family conference provided attendees from around the world with hope, inspiration, and the power of progress towards our goal of real treatments and a cure for Rett syndrome. Information about how to keep our children healthy and engaged; and how all of us can stay empowered and hopeful along the way was shared in many ways, and from many sources. Beyond the learning of tools and information, valuable contacts and relationships were made that will carry attendees until we can all meet again. We especially learned so much from our "First Families", those who attended the very first conferences in the 1980's and came to be with us again.

FEATURED THIS YEAR

Between the Science Symposium, Family Conference, and Tribute Dinner, over 600 people passed through our halls, were touched by our mission, and learned from the experts and each other at the beautiful Westfields Marriott Washington Dulles. This was our first ever collaborative conference with our closely related disorder and research partner: the International Foundation for CDKL5 Research. Harnessing the power of collaboration underscored how much we have in common and how much more quickly we will realize research progress and provide care for families when we work together.

Sessions kicked off with an outstanding keynote motivational talk from Emily Perl Kingsley, author of the legendary poem "Welcome to Holland", and Sesame Street writer who broke barriers for inclusion of children of all abilities on television.



2014 AWARD RECIPIENTS

Each year we recognize individuals who have made significant contributions towards RettSyndrome.org's mission to accelerate research and empower families. The following awards were presented:

- **Sakku Bai Naidu MD**, Director of Neurogenetics Dept and Rett Syndrome research at the Kennedy Krieger Institute - Art of Caring Award for 30 years of devotion to Rett syndrome.
- **Meir Lotan, Professor, PhD, M.Sc.P.T.**, Physiotherapy Department Ariel University, Israel - Circle of Angels Award, for his enduring commitment to Rett syndrome clinical research.
- **Bill Babiarz**, father to Cammy - Giving Hope a Hand Award, for his inspirational acts of advocacy and awareness, truly an award to the entire Babiarz family.
- **Joyce Opinsky**, mother to Lilly - Volunteer of the Year Award, for her outstanding dedication to RettSyndrome.org's mission.

All of us congratulate and thank our award recipients for going above and beyond for our children. You are an inspiration to us all!

Please visit www.rettSyndrome.org for a detailed account of this amazing event, for session handouts and recordings and more.

REGIONAL REPRESENTATIVE PROGRAM

Here are messages from just a few of our dedicated Regional Reps. Contact yours today! Please visit <http://www.rettSyndrome.org/for-families/regional-representatives>

A MESSAGE FOR CONNECTICUT FAMILIES FROM YOUR REGIONAL REP

Join our annual Lemonade STAND for Rett on August 16th in Fairfield, CT. Also, please make sure your calendar is marked for our CT Strollathon on September 20th at Camp Harkness in Waterford, CT. Please contact Andrea Zak for more information (ehaze35@cox.net). Finally, join us on our "Connecticut RettSyndrome.org Families" page on Facebook!

--Ariane McMahan (arianem@optonline.net)

A MESSAGE FOR PENNSYLVANIA FAMILIES FROM ONE OF YOUR REGIONAL REPS

On April 26th, six families joined together in DuBois, PA for a demo by special guest, My Tobii Representative, Mary DeCoen. We talked a lot about the Tobii, communication in general, and what our girls need to succeed in communicating. It was a wonderful time and it was great meeting families that share this journey with us! Please contact me anytime; I would love to hear from you.

--Julie Shaffer (shafferjm@yahoo.com)

A MESSAGE FOR TEXAS FAMILIES FROM ONE OF YOUR REGIONAL REPS

Several Texas families gathered for Camp Kaity, outside of Houston, TX at the Gordan Ranch, for a weekend of fun and community. It was a great event hosted by Jana & David Tovey with coordination from Susan & José Rangel, sponsored by the Blue Bird Circle at Texas Children's Hospital. We were privileged to have several of the physicians from the Blue Bird Circle Rett Center join us to share information and updates. We're already looking forward to next March when the 2015 Camp Kaity takes place. Please contact me for more information.

--Amber Weigl (amberweigl@gmail.com)

A MESSAGE FOR ILLINOIS FAMILIES FROM YOUR REGIONAL REP

On May 18th after our Chicagoland Strollathon, 11 girls with Rett syndrome, and their families, were able to attend a meet and greet with Clint Black. He could not have been more gracious to have us and was so very sweet. While talking at a whisper to save his vocal chords for the show, he took the time to talk and take photos with each girl; it was a special experience for all.

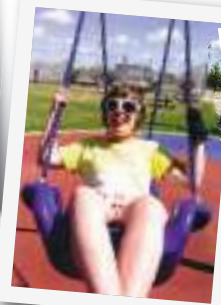
--Shelby Swann (Shelby.swann@yahoo.com)

POSSIBILITIES

Just because our children have the diagnosis of Rett syndrome, it does not mean they cannot do everything their siblings and peers do. With a little effort and planning ahead as well as some patience and flexibility, your child can take part in a wide array of activities while remaining safe and sound. Participating feels good for the whole family but most importantly, it builds self-esteem while promoting important neuronal plasticity in your special and unique child.



Paula sky diving



Skylar enjoys the swing



Jaidyn Skiing



Olivia getting a pedicure



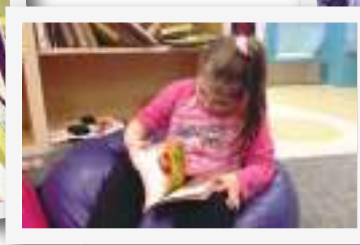
Lara takes part in a pageant



Melia Cross Country Skiing



Jillian rides in a go-cart



MJ reads a book

GRANDPARENTS



Grandma with Chelsie



Pappaw with Karlie



Larry and Charlotte



Pappy with Keira

Be sure you have registered for the Grandparents Network at [Rettsyndrome.org](https://www.rettsyndrome.org). Join hundreds of others as you unite to support each other, support your grandchild and become a part of the Rett syndrome community.

Here are just a few of our special grandparents with their granddaughters. Visit the website to register and to also read about our featured Grandparents of the Month.

<https://www.rettsyndrome.org/families/grandparents-network>

"There is nothing like a grandchild in your arms to put a smile on your face, a lump in your throat and a warm feeling in your heart."

SIBLINGS

Having a sibling with Rett syndrome can pose its challenges at times but the love, compassion and strength that becomes apparent overtime is incredibly touching and quite remarkable. Search for "Rettsyndrome.org Siblings Network" on Facebook and meet other siblings! Rettsyndrome.org applauds all siblings everywhere!

WITHOUT WORDS

By Kacie Hymers for Lyndsay Hymers

Kacie age 15

Lyndsay age 21

It's amazing how one person
can touch so many hearts

Without Words

Somehow they work their way
into your heart, grasp it
and never let go

Without Words

They have a way of leaving
an eternal impression in the
minds of everyone they meet

Without Words

On a bad day, they seem to
always find a way to make it brighter

Without Words

When someone makes them feel
like they "aren't normal" or "different"
and whisper "what's wrong with her"
it takes all I have not to react.

Without Words

I want to say "what's wrong with you?
You wish you were different.
That you could have such an
impact on the world."

Without Words

But they know who they are
and they don't let words hurt them.
They know how strong and ambitious
they are.

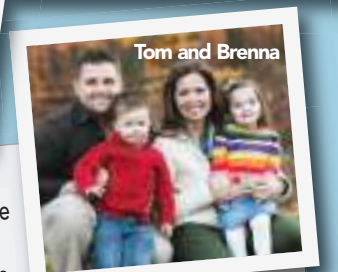
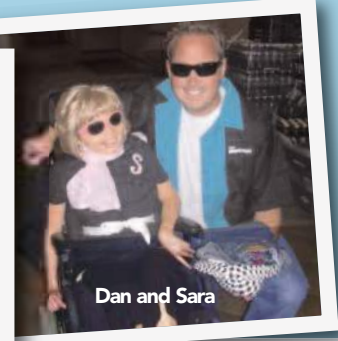
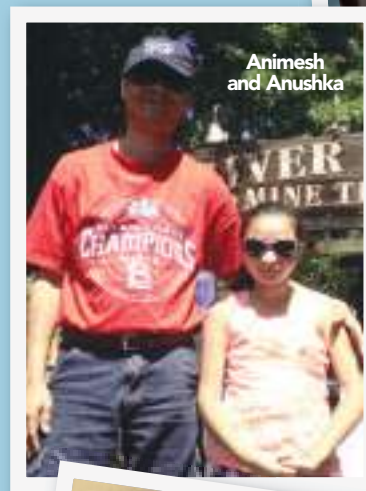
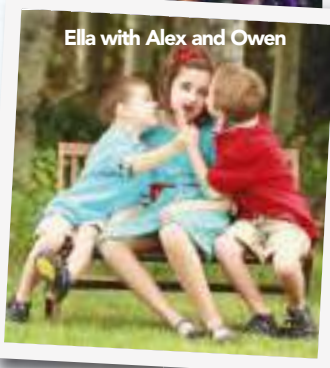
Without Words

For 14 years, 2 months and 15 days
they have created an unbreakable
love within me

Without Words

Everyday our love grows stronger.
Our hope also grows, that one day
we will be able to speak together.

WITH WORDS



Hello Rett Dads,
My name is Tom Stearns and I am one
of the Dad Representatives for
Rettsyndrome.org. I currently handle
the "Rettsyndrome.org Dads Network"
page on Facebook as well as a monthly conference call. This is a
"closed" group for males only. We encourage grandfathers,
brothers or any other leading men in a "Silent Angel's" life to join
our page and attend the calls. We have had great appreciation so
far and our group continues to grow. Please take the time to join
myself and other Dads to network together, share ideas, address
current issues and help one another be the best Rett father and
family man possible! To join the Facebook page please friend me
and I will add you - I'm Thomas Stearns. If you're not on
Facebook please feel free to email me at rettdadrep@gmail.com
and I will forward the conference call information for our next call.
Sincerely,
Tom

DADS

There is something very special about the bond
between a father and daughter. The fathers in the
Rett community are some of the most caring and loving
men in this world. Rettsyndrome.org honors all the special
dads out there and welcome them to join other dads who
are traveling the same journey.

LIFE AFTER RETT SUPPORT GROUP

"Life After Rett, a Facebook group for family and friends who have lost a loved one to Rett syndrome, is the group no one wants to belong to, but its members offer each other support and remember important events in their lost ones' lives with photos and stories. It currently is made up of 80 members who also discuss issues on monthly phone calls, talking about loss and also how to better support all those who have lost a child to Rett."

– Jane George, mother of Aniko, 9/22/75 – 8/7/05

"This group, of all the parent or grandparent groups that I belong to, has helped me the most. Although our lives and journeys are different, they are the most alike because we all speak the same language of Rett syndrome. I have received as much advice as I have given out and I feel so much more connected to all who have suffered a loss to this, as yet, incurable syndrome. I am heartbroken to "meet" people in this way but also comforted."

– Elaine Dolecheck, mother of Danielle, 7/4/94 – 11/8/12

For more information please contact Jane at jgeorge@globetrotter.net and Elaine at cowgirlndanidee@comcast.net.

FROM KNOWLEDGE WILL COME A CURE

Organ and tissue donation is one critical 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 if she is having a routine blood draw or undergoing a medical or surgical procedure (e.g., skin or muscle biopsy, endoscopy, scoliosis surgery). A known MECP2 mutation and completion of a clinical questionnaire is required for this research.

A difficult thing to think about in times of good health is organ donation, but it is an important gift of hope towards the discovery of treatments and a cure for those living with RTT today, and for future generations to come. 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 and contact us for more information

today. It is also helpful to know that the donation process must begin within hours of passing, and before any preparations. We partner with the Harvard Brain Tissue Resource Center, and 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 pnues@rettsyndrome.org; 510.499.3858 or call the office at 1-800-818-7388.



MEMORIALS

The loss of every child is a loss for our entire community. Please join us in a moment of reflection for all of our beautiful children, and especially for those families who need our support during their difficult time.

We promise to learn from each and every one of them, remember them always, and allow their lessons to guide our efforts forward. In an effort to let the community extend their support, Memorials will now be posted to www.rettsyndrome.org.



GET CONNECTED

Connect with over 15,000 families and friends on our Rettsyndrome.org Facebook page. Find a closed group experience by finding your "(State) Rettsyndrome.org Families" state family page. Get Rett syndrome trending by including "#Rettsyndromeorg" & "#rettsyndrome" in all of your posts and tweets. Not on Facebook? Visit our website to subscribe to our email or print newsletter(s). Contact admin@rettsyndrome.org to ensure we have your current contact information.



Family Empowerment Board

This is such an exciting and transformative time at Rettsyndrome.org. From a family support standpoint we are very active as well. The Family Empowerment Board consists of 12 passionate family members that drive leadership in family needs, regularly connect and act as a sounding board for the Rettsyndrome.org Board, work with newly diagnosed families, and act as the voice for the end consumer. We are fortunate to be a part of an organization that does not lose track of family needs with balancing the need for research and awareness.

I wanted to take the opportunity to provide you an update on some of the activities this inspiring group of folks have been engaged in over the past few months. We have "FEB" members holding positions in Fundraising, Conference, Survey, and Branding Committees to communicate our ideas as we shape this organization. This year we changed the name of the board from "Family Support Board" to "Family Empowerment Board." We did so to align ourselves with the organization's goal of truly empowering families via information, support, and advise to become their own advocates, fundraisers, and awareness builders. We also launched a family survey at the end of last year with over 480 responses. This survey helps receive feedback on direction, gaps, and needs from the families and those affected by Rett syndrome so we be in tune with your needs. We are filtering through the meaningful results as we speak and sharing insights with the Board and to communicate to you all as well. If you were one that participated, thank you.

Unfortunately we lost a valuable FEB member and Rett syndrome advocate this year in Maria McTernan. Each year Rettsyndrome.org provides the ability for some to attend the national conference who may not otherwise afford to do so through scholarships. In recognition of Maria's dedication to families and inspiration within the Rett Community, we elected to name this the "Maria McTernan Conference Scholarship." In addition, the FEB members funded, on their own, an attendee to this year's conference in June as a tribute to Maria.

Families please visit the family empowerment section on the new website, located at www.rettsyndrome.org/for-families, and reach out to us directly or inquire how you can be a part of the Family Empowerment Board in the future www.rettsyndrome.org/our-role-and-impact/our-leadership. All the best to you and your families.

Sincerely,
 Danny Warner
 Dad to Jaidyn, age 12 (R270x)
 Chairman, Family
 Empowerment Board



Danny and Jaidyn



Maria McTernan (front row, far left)
 with the Boston Marathon team at her home

health matters

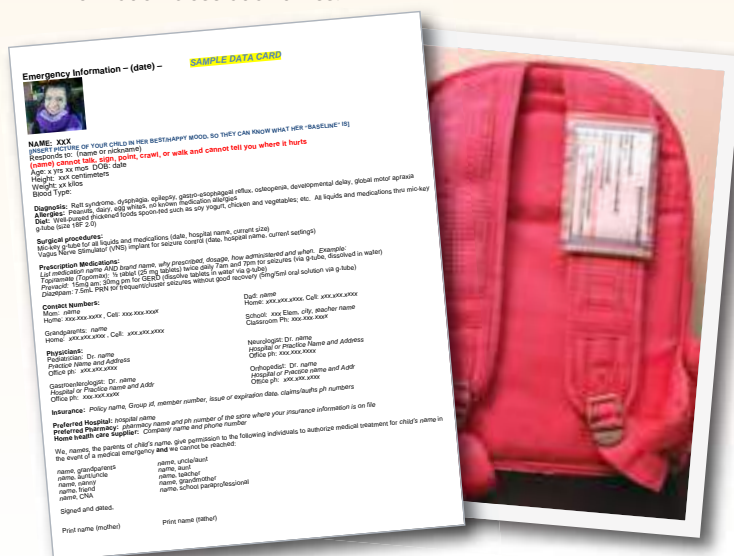
Be Prepared In Case of Emergency

We hope this is never needed, but always best to be prepared. Visit <http://www.rettsyndrome.org/for-families/resources> to download a sample Emergency Information Form, and read about a wonderful parent developed product to fashionably keep this information close at all times.

We all try to be optimistic about our health and safety, but it is crucial to be prepared for an emergency before it occurs. Having an emergency contact sheet prepared will help the medical team take appropriate steps and care. Anyone who cares for your child in your absence should also have an emergency sheet.

The thought of being in an accident where I am unable to speak on either of my daughters' behalf is a frightening thought. Having this form in a visible spot provides me peace of mind. It could save the medical team vital time if they knew my daughter had Rett syndrome. It would help them to know that she is unable to speak, walk, point or indicate where it hurts, her blood type, emergency contacts, doctor contact information, allergies, medications, surgeries and a copy of my insurance card. We have copies of our emergency form in several places for all situations, including her backpack, wheelchair, car seatbelt and posted in our home. They are tucked into a clear pocket on Snap wraps, which Velcro around straps so they are visible.

An emergency contact sheet is important for anyone, not just someone with special needs. It is important to update any information. Snap wraps to hold emergency sheets are available at www.kissaroo.com. Jackie Corrado Babiarz, mom of "Cammy Can", and the creator of Snap Wraps.



Fall is on the way and so are the United Way and Combined Federal Campaign drives.

In 2013, friends and family of girls with Rett Syndrome pledged almost \$33,000 to the foundation through local Federal Campaigns. It is great to know that so many federal employees have "united" with us in our campaign to find the cure for Rett syndrome and we hope that they will continue to do so. If you have a friend or relative that works for the federal government or is in the armed services here or abroad, please let them know that Rettsyndrome.org's CFC Code is 11046 so they can designate their donation to the foundation.

And speaking of being "united" we have almost 80 different United Way organizations in our database. It's easy to designate your United Way donation to Rettsyndrome.org - just write our (F)EIN - 31-1682518 on the designation line and if there is room, we also recommend writing in our name and address - Rettsyndrome.org, 4600 Devitt Drive, 45246. Thanks so much for spreading the word.

Shop Amazon for Rett Syndrome

Do you like to shop online? When purchasing on Amazon, use www.smile.amazon.com instead of your usual 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. They also run special promotions to make a \$5 donation on top of the 0.5% during certain periods, so check back often and follow updates on our Facebook page at www.facebook.com/RettSyndrome.

Since the launch of the AmazonSmile program in October 2013, we have received more than \$1,200 through your support while shopping on www.smile.amazon.com. Please continue to use the program and spread the word among your friends and family. Thank you!

Shop at AmazonSmile

and Amazon will make a donation to:



Get started

amazon smile

Rettsyndrome.org Partners Make Our Work Possible

We believe it takes an army of multi-talented people using every ounce of creativity, strength, know-how and energy to win the battle against Rett syndrome. We must be united in this effort. Over the years, we have developed strong partnerships with many organizations and through our collaboration, we have been able to move closer to our common goal of ending Rett syndrome. Their support allowed us to invest in projects that led to critical discoveries, just recently proving that IGF-1 is safe and well-tolerated in patients with Rett syndrome; Sarizotan reduces the incidence of apnea in Rett mouse models; AT-Hook domain in MeCP2 determines the clinical course of Rett syndrome; function of MEP2 was redefined using cutting edge genome editing technology called TALEN; and gene therapy showed some promise in Rett mice as a potential therapeutic option.

We would like to thank the following organizations that have supported us in 2014 for their exceptional financial and in-kind support. Let's celebrate our collaboration and make an even greater impact.

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
- Ella Foard Foundation for Rett Syndrome
- Hershey Entertainment & Resorts Co.
- ICAP Services North America, LLC
- JKK Charity Fund
- IMC Chicago Charitable Foundation
- Main Street Bank
- Manhattan Beer Distributors, LLC
- Pinnacle Health System
- PotashCorp
- Rochester Area Community Foundation
- Southern Ionics, Inc.
- T.S. & Juanita Ballance Foundation
- Tobii Assistive Technology, Inc.
- Transwestern RBJ LLC
- United Way Of Buffalo & Erie County

Natural History Study Partner

- National Institutes of Health (NIH)

In-kind Contributors

- Attention Span Media
- University of Alabama Birmingham
- Baylor University
- Kennedy Krieger Institute/Johns Hopkins Medical School
- University of California San Diego
- Boston Children's Hospital
- Wiley Ryan
- Reading Rock
- Wynne Creative Group

Please visit <http://www.rettsyndrome.org/our-role-and-impact/our-partners> to read more about our partners. If you're interested in partnering with Rettsyndrome.org, please contact Eva Dillon at edillon@rettsyndrome.org or (301) 961-1549. Help End Rett!

ANGEL GALLERY...

Let the picture tell her story

Inspire the community with your angel's picture. Send us your pictures of your angels, family gatherings and fundraisers to update print materials and create our Angel Gallery.

Contact lgeglein@rettsyndrome.org to submit your photo.



FEATURED ANGEL - Liliana "Lily" Salerno O'Mahoney

Be a part of Rettsyndrome.org's monthly Featured Angel. Rettsyndrome.org spotlights one of our many inspiring Rett angels in the monthly Rettsyndrome.org e-newsletter. The Featured Angel's intent is to give our Rett community an introduction to a few of the Rett angels for whom we are fighting to turn Research into Reality.

Read more about Lily and all of the Rettsyndrome.org Featured Angels at rettsyndrome.org - News & Media - Rettsyndrome.org Features. Contact lgeglein@rettsyndrome.org to add your angel's story.



Lily

Hi, my name is Liliana but I go by 'Lily'. I just turned 3 years old on February 8th. Both my mommy and daddy tell me all that time that I'm the sweetest, smartest, prettiest, hardest working, bravest and most amazing little girl that they know. I think it's because I give them tons of hugs and kisses and smiles.

I've been getting almost 30 hours a week of therapy, through Early Intervention, for over a year which has helped me in so many ways.

Lucky for the therapists that I'm motivated by berries and music (my two favorite things in the whole world, second to my parents and third to my therapists and nanny). My boyfriend Johane (ok, really he's my physical therapist) gets me

moving up and down stairs to keep my body strong. I give him plenty of cuddles and kisses along the way. Lynda, my occupational therapist, comes over almost every day and we do so many things like crawl through tunnels, pick up heavy objects and jump on the trampoline (though I'm still working on my jumping skills).

FUN WITH FUNDRAISING

"There is nothing more gratifying than being able to raise funds to advance the goals of the Foundation. It doesn't matter if it is a bake sale, a Strollathon or a Benefit, just do what you feel comfortable doing and it will be a success."

- Gordy Rich, OH

Together, we can change lives! Events like Jackie and Bill Babiarz's Cammy Can's Cinderella Story, Tom Guidi's Bruins Hockey event, Casino Night for Rett hosted by Melanie Gregory, the Herold family's Live Rett Free Walk, the Color Run (PA) chaired by Nicole Karnash, Diane and Mick Ross' Chicago Run for Rett have raised \$210,851. Galas such as the Reach for the Stars Gala chaired by the Lebson, Diamond and Schurr families, and the Picture a Cure Gala hosted by

the Seiler family have raised \$321,369; Golf tournaments like Mike Joyce's Boston Golf Tourney, John Corpus' Samantha Corpus Golf event, the New Jersey Golf tourney hosted by Leslie and Mike Greenfield, Jim Bathie's Cure for Keira Golf, and new golf events such as Rip it for Rett Golf hosted by Lynn Peel, Josh Faulkner's BNSF Rail Golf and the McCool family's Golf Fore Ellie have raised \$461,096.





The Lemonade STAND for Rett fundraiser is perfect for:

- Anyone new to fundraising
- Adults wanting to introduce the spirit of giving to children, younger family members, schoolmates and friends
- Service organizations wanting to support Rettsyndrome.org
- Anyone short on time or resources to plan a larger event like a Strollathon, gala, or tournament
- Anyone looking for a fun new add-on to a current annual event like a Strollathon, run, or race day

Our glass is always half full. You'll know what we mean once you experience the turnout at

your own Lemonade Stand. It's so easy to make this traditional end-of-the-driveway warm weather childhood activity into something meaningful for our children with Rett syndrome. 38 Lemonade STANDS have raised over \$15,000 so far this year!



Visit www.rettsyndrome.org/get-involved/lemonade-STAND-for-rett to start your stand, order a Starter Kit and get started.

Going the Distance

The Rett Racer program continues to grow. In 2014, races in Louisiana, Massachusetts, Illinois, New York, Michigan, Virginia, Minnesota, Florida, South Dakota, California, Pennsylvania, and Nebraska have already raised \$171,368. If you are signed up for a race or plan to sign up, we can help you fundraise as you train. Online fundraising pages and resources will help you achieve success, and special incentive recognition is available beginning at \$500 raised. Visit www.retracers.org to sign up or for more information on our Rett Racer program.



Strolling Into 2014

As the weather warms up, so do our volunteer efforts in planning local Strollathon events. Strollathons will be coming to at least 30 locations in 2014 and this is a great time to get involved. Taking the first step, whether registering a team for the first time or reaching out with interest in joining a committee or hosting an event in your

community, can be nerve-racking. It is THAT first step that opens you to great support, either from the local Strollathon chair and committee or from Rettsyndrome.org staff – and in many cases, both – and the opportunity to meet new families living with Rett syndrome in your local community! Strollathons are fundraisers, friend-raisers, and family support rolled into one. The St. Louis Strollathon chaired by Joyce Opinsky, the Chicagoland Strollathon, chaired by Shelby Swann and the Farmington Michigan

Strollathon chaired by Lisa Fenberg have contributed over \$150,000 to the combined total of \$214,674 raised by all strollathons for Rett syndrome just this year! A list of fall strollathons is on the back page of this newsletter, please visit our website for additional information and register for the event closest to you, the location where your loved one lives, or where you have family or friends who might also want to get involved to support your efforts. We look forward to seeing you in 2014!

Family Fundraisers!

In addition to the signature-type events, 37 unique and sometimes very innovative events have been held in many states by families on an individual basis. The following events collectively have raised almost \$92,000:

- 2nd Annual Lil' Bit of Fun Golf Tournament, IHO Liliana Mueller
- 7-iron Challenge, IHO Liliana Mueller
- Bake Sale, IHO Abrianna Madsen
- Benefit Concert, IHO Grace Ann Patton
- Cased Meats for a Cure, IHO Emelia Foster
- Cler Family Valentine, IHO Colleen Cler
- Community Christmas, Ladies Philoptochos Society Westfield, NJ
- Davis Open House, IHO Jaidyn Warner
- Dog Biscuit Sales, Girl Scouts of N. California
- Dress Down Friday, IHO Hanna Blankenship
- Dress in Denim Day, IHO Michelle Albanese
- East Hanover Council Fundraiser, IHO Jennifer DaSilva
- Erika's Walk, IHO Erika Miller
- First Friday Casual, East Coast Chair Co.
- Galata School Staff
- Glow Run, IHO Jaelyn Seiber
- Go Purple Southmoore
- Harlem vs Rett Warriors, IHO Chelsie Brackett
- Heroes of the Silent Angels, IHO Dierdre Galyon
- Knight Golf Scramble, IHO Jacalyn Knight
- Maddie Schurr Mitzva, IHO Jessie Lebson
- Magnolia's Hope, IHO Magnolia Tesler
- Meals for Maddie, IHO Madlyn McKnight
- MFP Walk for Rett, IHO Olivia Lang
- One House Release, IHO Ariel Kean
- New Jersey Golf IHO Heather Greenfield
- Paddle for Sorel, IHO Sorel Kolendrianos
- Quaker Steak & Lube, IHO Kate Allen
- Race for Grace, IHO Grace Reddington
- Revelry to End Rett, IHO Violet Berlin
- RMRA Walk n' Roll (MO)
- Runnin' & Gunnin' for Rett, IHO Jillian Mortensen
- Sam's 21st
- Spike it for Rett, IHO Jessica Brunner/Laurel
- Cooper/Anna Guess
- Spirit Night
- St. Margaret's Choir, IHO Chelsea Anderson
- Sugar on Snow, IHO Payton Boutin
- Tri4Cure, IHO Tia Kanemura
- Walk for a Cure, IHO Chloe Bell
- Watseka Park District, IHO Tayla Maple

*IHO – In Honor Of

Thank you all!

THANKS TO EVERYONE who has hosted an event – the foundation truly appreciates all of your hard work and dedication to help us find treatments and a cure for Rett syndrome.

Could there be an event on this list that you would like to duplicate? Please contact the office at 1-800-818-7388 for help with getting your event started.

2014 FALL EVENTS

8/21/2014	Casting 4 A Cure, Victor, ID
8/30/2014	Ohio Walk for Rett, Massillon, OH
8/30/2014	Forever Emma Lou's Ride for a Cure, Cherryville, NC
9/6/2014	Pennsylvania Strollathon, PA
9/6/2014	Roanoke Strollathon, Roanoke, VA
9/7/2014	Sixth Annual Charlotte Troy Golf, MA
9/13/2014	Tristate Strollathon, Cincinnati, OH
9/20/2014	Maine Strollathon, Augusta, ME
9/20/2014	Connecticut Strollathon, CT
9/20/2014	Nebraska Strollathon, Grand Island, NE
9/21/2014	Grand Rapids Strollathon, MI
9/21/2014	Birdies for Bennett, Elk River MN
9/27/2014	San Antonio Strollathon, TX
9/27/2014	Kansas Strollathon, Olathe, KS
9/27/2014	Raleigh Strollathon, NC
9/27/2014	Cape Cod Strollathon, MA
9/27/2014	Portland Strollathon, OR
TBD	Louisiana Strollathon, LA
10/3/14	Connecticut Rett Angels Golf, Portland, CT
10/4/2014	Reno Strollathon, NV

10/4/2014	Central Florida Strollathon, Lakeland, FL
10/4/2014	Georgia Strollathon, Macon, GA
10/4/2014	Walk n' Roll, Lebanon, MO
10/4/2014	Bay Area Water Utilities Softball Tournament, Pleasanton, CA
10/5/2014	New Jersey Strollathon, Hoboken, NJ
10/5/2014	Belton TX Strollathon, TX
10/10/2014	San Diego Golf Tournament, San Diego, CA
10/12/2014	NorCal Strollathon, Moraga, CA
10/12/2014	Minnesota Strollathon, Savage, MN
10/12/2014	National Capital Strollathon, Silver Spring, MD
10/25/2014	SoCal Strollathon, Whittier, CA
10/25/2014	Hannah's Hope 5K, Mason, TX
10/26/2014	Charlotte Strollathon, NC
10/27/2014	Grapevine Strollathon, TX
11/1/2014	Las Vegas Strollathon, Sparks, NV
11/1/2014	The Woodlands Strollathon, TX
11/15/2014	Color Run, Pittsburgh, PA
11/1/2014	Coconut Creek Strollathon, FL

