



# Rett GAZETTE

Accelerating Research.  
Empowering Families.

## CELEBRATING PROGRESS & COMMUNITY



Melissa Kennedy

***"It takes each  
of us to make  
a difference  
for all of us"***

~ Jackie Mutcheson

I could not think of a quote that does a better job summarizing this edition of Rettsyndrome.org's Rett Gazette. In this issue, we celebrate the power of our community: our community of families, physicians, clinics, researchers, regional associations and donors.

Now more than ever, we are dependent upon one another. Families need to be connected to research opportunities and excellent clinical care. Clinicians need families to try emerging treatment modalities. Pharmaceutical companies are looking for experts to help build clinical protocols. And we all need resources!

Now more than ever, our community needs a single organization that supports research and also empowers families. We appreciate that the most impactful change will happen because of the synergy between families and researchers. Our two-pronged mission of accelerating research and empowering families gives us the unique ability to create connections and enhance collaboration.

Our organization exists to serve and support you. We hope that you find comfort knowing that you are an important part of this strong and resilient community of people who love someone with Rett syndrome. Thank you for the privilege of serving you.

Melissa

Melissa Kennedy  
Executive Director  
Rettsyndrome.org



Steve Kaminsky, PhD

In the six years I have been with the foundation, the focus of the research community has sharpened on getting basic discoveries from the bench to the bedside. As clinical trials move forward, we must continue to look at emerging technologies and pathways.

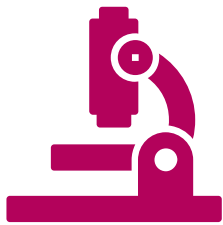
Each of these technologies will have to traverse the demanding regulatory hurdles that make these endeavors expensive and time consuming. Navigating these hurdles will stretch available resources. We must face the challenges head on and make the difficult choices to determine the technologies that hold the most promise.

Even as we push for drug intervention to move forward and for new technologies to emerge, we also have to recognize the immense value in continuing to develop therapies that can improve our children's lives most immediately. To truly help those with Rett syndrome, we must have a strategy to correct the underlying biology that is abnormal, and at the same time rebuild the neural networks that are essential to everyday skill development. Physical therapy, occupational therapy and cognitive therapy research are still in an early state in regard to Rett syndrome. If we want to change the lives of those suffering with Rett syndrome, then we must push these two frontiers together.

I invite you to join us as we push forward. Together we can make a difference and our mutual goal must be to make this happen as soon as possible. Many are waiting and their hope lies in all of us.

Steve Kaminsky, Ph.D.  
Chief Science Officer  
Rettsyndrome.org

# 2017 RESEARCH TO RESULTS



accelerating  
research.

**\$44M** COMMITTED  
TO RESEARCH  
THROUGH 2017



**37** ACTIVE RESEARCH &  
GRANT PROGRAMS

In 2017, our training program has reached over 50 post-doctoral researchers ensuring a promising future for Rett syndrome research.



Continued expansion of our proprietary Scout Program yielded two compounds that will begin Phase 2 clinical trials in 2018.



FDA approval to launch the first Phase 3 trial for Rett syndrome studying trofinetide. Trial slated to begin in 2018.



Rettsyndrome.org 2016 advocacy efforts led Congress to add Rett syndrome as a 2017 topic of interest for the US Department of Defense and Congressionally Directed Medical Research Program. Dr. Colleen Niswender received \$1.9 million dollars for drug discovery research.



empowering  
families.

**\$10M+** INVESTED IN FAMILY  
PROGRAMMING AND  
SERVICES THROUGH 2017



**10,202**  
DONORS IN 2017



- Expert clinical care available at 22 hospitals in North America reaching 2,500+ children and adults
- 14 Clinical Research Centers of Excellence
- Natural History Study continues in 2018 in 15 US sites, making it one of the largest studies of its kind for a developmental disability.



**120** FAMILY  
FUNDRAISING  
EVENTS



**232** NEWLY DIAGNOSED  
FAMILIES RECEIVED  
A COMPREHENSIVE  
CARE PACKET



“As parents of a newly diagnosed one year old, we were given renewed hope when we read about the promising research trials on drugs like trofinetide and IGF-1. RSO has been vital to our getting through these first few months...”

*Lindsey Benjamin, mom to Leah age 1*



# Collaboration and Progress in Research

On February 15, 2018 Rettsyndrome.org gathered with a group of renowned clinical and scientific experts in Atlanta. **Our ask: Help us make sure that our research strategy is meeting the needs of researchers, clinicians and families.** Together, these experts represent the research areas that define our comprehensive research strategy.

Dr. Steven Kaminsky, our Chief Science Officer, led an in-depth review of Rettsyndrome.org's 48 current research projects — those that are underway and those that will begin soon. The group discussed the direction of future research to ensure that we address areas of unmet need when awarding our next cycle of research grants. The varied backgrounds and perspectives represented within the group were valuable in prioritizing current and future research.

Because research and clinical care must be focused on families, Paige Nues, Director of Family Empowerment, gave the group a unique perspective of what the daily lives of families are like as they care for their child with Rett syndrome. As our research strategy progresses and more clinical trials begin, we must make sure to keep the needs of families at the center of our decisions.

Rettsyndrome.org came out of this meeting with confirmation that our research strategy is uniquely comprehensive, encompassing biologics, pharmaceuticals and neuro-habilitation. We are incredibly grateful to this group of experts who gave generously of their time and talent. No one succeeds in fighting Rett syndrome alone. It takes a united team of researchers, families, patients and care givers to win this battle.

## OUR CLINICAL AND SCIENTIFIC EXPERTS



**Theresa Bartolotta, PhD, CCC-SLP**  
Professor, Department of Speech-Language Pathology, Monmouth University



**Timothy Benke, MD, PhD**  
The Ponzio Family Chair in Pediatric Neurology Research, Director of Research-Neurosciences Institute, Children's Hospital Colorado



**Pamela S. Diener, PhD, MS, OT/L**  
Professor, Occupational Therapist  
Department of Neuroscience,  
Georgetown University Medical Center



**James H. Eubanks, PhD**  
Senior Scientist, Krembil Research Institute (Krembil), Research Division Head, Krembil Research Institute (Krembil)



**Larry Glass, BA**  
Executive Director and Chief Science Officer, Neuren Pharmaceuticals



**Daniel G. Glaze, MD**  
Medical Director, The Blue Bird Circle Rett Center Baylor College of Medicine and Medical Director The Sleep Center Texas Children's Hospital, Professor Departments of Pediatrics and Neurology Baylor College of Medicine



**Dena Howland, PhD**  
Associate Professor, Department of Neurological Surgery, Laboratory of Neural Repair, Plasticity and Functional Recovery, University of Louisville



**Walter Kaufmann, MD**  
Ravenel Boykin Curry Chair in Genetic Therapeutics, Professor of Neurology, Greenwood Genetic Center



**Jane Lane, RN, BSN**  
Research Nurse Manager and  
Rett Consortium Project Manager,  
The University of Alabama at  
Birmingham Research



**Eric Marsh, MD, PhD**  
Pediatric Neurologist, The Children's  
Hospital of Philadelphia



**John McCall, PhD**  
Consultant and Board Chair  
Reveragen BioPharma



**Alysson Muotri, PhD**  
Professor, University of California  
San Diego, UCSD Stem Cell Program  
School of Medicine, Dept. Pediatrics/  
Rady Children's Hospital Sanford  
Consortium



**Jeffrey L. Neul, MD, PhD**  
Annette Schaffer Eskind Chair and  
Director, Vanderbilt Kennedy Center;  
Professor of Pediatrics, Division  
of Neurology, and Pharmacology  
Investigator, Vanderbilt University  
Medical Center



**Alan Percy, MD**  
Medical Director, Rett Syndrome Clinic  
and Research Center, UAB - Civitan  
International Research Center, The  
University of Alabama at Birmingham



**Lucas Pozzo-Miller, PhD**  
Professor, Associate Director,  
Comprehensive Neuroscience Center,  
Interim Scientific Co-Director, Civitan  
International Research Center

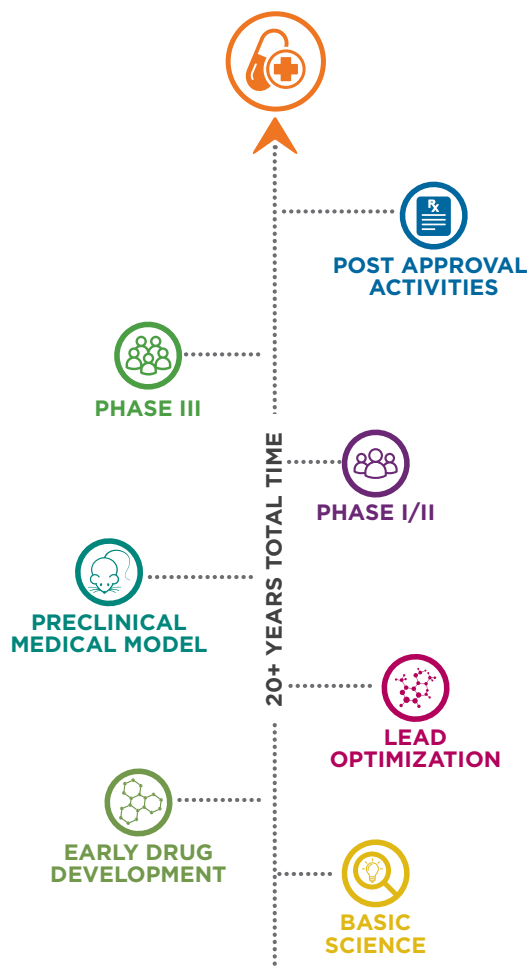


**Bernhard Suter, MD**  
Assistant Professor and Associate  
Director, MECP2 Duplication Clinic -  
Department of Pediatrics and Neurology,  
Baylor College of Medicine, The Blue  
Bird Circle Rett Center, Baylor College of  
Medicine and Texas Children's Hospital

# Trofinetide Phase 3 Update

"Neuren has not yet confirmed the timeline, but there is a huge amount of preparation underway by Neuren and others to get ready for the trial, which is larger, longer and more complex than the previous two studies. Things are moving along nicely, but many different aspects have to be perfect before the trial starts, including approval of the protocol, manufacturing, trial sites, and logistics. All of these aspects are being worked on. Neuren will provide further updates in due course." Jon Pilcher, CFO & Company Secretary, Neuren Pharmaceuticals

Rettsyndrome.org does not influence the timing of the Phase 3 launch. There are many behind the scenes activities taking place to ensure a successful trial. We are waiting in anticipation just as you are and will keep you updated with the latest timing information.



# Newly Funded Research

Rettsyndrome.org has, to date, invested over \$44 million dollars in research. In 2018 we are taking bold steps to expand our support of basic research that includes gene therapies, protein therapies, and reactivating the normal *MECP2* gene. We also continue to support neuro-habilitation research, which is bringing effective therapies to our children now. **We feel ethically compelled to ensure that our children with Rett have the best quality of life today, while we invest in a future cure.**



## Gene Therapy

Dr. Roux is researching gene therapy in mice using BDNF (Brain Derived Neurotrophic Factor) instead of *MECP2*.

**Jean-Christophe Roux, PhD**  
Aix Marseille



## Activating the Silent X

Dr. Lee is leading gene therapy research focused on reactivating the silent *Mecp2* gene (on the inactive X-chromosome) to treat Rett syndrome.

**Jeannie Lee, MD, PhD**  
Massachusetts General Hospital



## Gene Therapy & Environmental Enrichment

Dr. Sinnett is leading research to demonstrate how environmental enrichment (neuro-habilitation) can enhance the benefits of gene therapy.

**Sarah Sinnett, PhD**  
University of North Carolina



## Brain Changes After Ketamine Dosing

Dr. Fagiolini is using live brain imaging of the mouse model to evaluate changes in the brain directly after Ketamine treatment.

**Michela Fagiolini, PhD**  
Boston Children's Hospital



## Gene Therapy Dosage

Dr. Niswender is leading research to address one of the challenges of gene therapy — gene dosage issues.

**Colleen Niswender, PhD**  
Vanderbilt University



## Biologic Target Validation

Dr. Niswender is studying whether metabotropic glutamate receptor 3 (mGlu3), can be a potential therapeutic target for Rett syndrome and *MECP2* Duplication Syndrome.

**Colleen Niswender, PhD**  
Vanderbilt University



## Protein Therapy

Dr. Sheiner is evaluating a new way to deliver *MeCP2* protein into the brain by using a parasite. This is a cutting edge approach and is being conducted on animal models.

**Lilach Sheiner, PhD**  
University of Glasgow



## Understanding Molecular and Physiological Processes in RTT

Dr. Lau is studying the impact of *Mecp2* mutations on the adult brain's neural networks, gene expression and behavior.

**Billy Lau, PhD**  
University of Tennessee – Knoxville



## Activating the Silent X/CRISPR

Dr. Fink is working on new CRISPR/Cas9 technology to reactivate the healthy, silenced *Mecp2* gene.

**Kyle Fink, PhD**  
University of California – Davis



## Evaluating Potential New Drug Candidates

Dr. Chang will evaluate 13 potential drug candidates for Rett syndrome. He will do his research in cells and on mice.

**Qiang Chang, PhD**  
University of Wisconsin – Madison



### Evaluating Potential Compounds in the BDNF Pathway

Dr. Jones is investigating compounds that can stimulate the Brain Derived Neurotrophic Factor (BDNF), which enhances the function of the brain. He will test drugs already approved by the FDA for other uses, in order to find those that could be effective in treating Rett syndrome.

**Kevin Jones, PhD**  
University of Colorado



### New Method to Screen Potential Compounds

Dr. Katsanis is developing a new, faster drug candidate screening method using zebrafish that have a *Mecp2* mutation. This method will increase our Scout Program's drug screening capacity from 8-10 compounds per year to hundreds per year.

**Nicholas Katsanis, PhD**  
Duke University



### MECP2 Nonsense Mutations

Dr. Pozzo-Miller is working collaboratively with Southern Research (SR) to identify compounds that best suppress the effects of MECP2 nonsense mutations (which affect approximately 35% of Rett syndrome patients).

**Lucas Pozzo-Miller, PhD**  
University of Alabama – Birmingham



### Screening Compounds in Rettsyndrome.org's Scout Program

Dr. Hanania is doing research on potential drug candidate compounds. This is a unique program that increases the pace of testing, allows for potential treatments to move more quickly into clinical trials, and create partnerships with pharmaceutical and biotech companies.

**Taleen Hanania, PhD**  
Psychogenics, Inc



### Improving Motor Skills for Children with RTT

Dr. Diener is studying how virtual reality computer games can be used to improve upper body motor skills. This funding will expand the number of study sites for the project to 2 sites.

**Pamela Diener, PhD MS OTL**  
Georgetown University



### Learning with Eye-Gaze Technology

Dr. Rose is leading research to better understand and assess learning with eye-glaze technology. She intends to demonstrate that they can measure memory, anticipation, and attention with eye-gaze and use those as outcome measures for clinical trials.

**Susan Rose, PhD**  
Albert Einstein College of Medicine



### Clinical Outcome Measures

Dr. Buchanan will refine clinical outcome measures using anxiety biomarkers, such as cortisol level, heart rate variability and inflammatory markers. Dr. Buchanan's goal is to improve diagnosis and treatment of anxious behaviors in RTT.

**Carrie Buchanan, MD**  
Greenwood Genetic Center



### Seizure Biomarkers

Dr. Fu is investigating seizures in Rett syndrome in a new way. His group will use clinical data from the Natural History Study and use mouse models to determine characteristics that can predict seizures.

**Cary Fu, MD**  
Vanderbilt University



### Clinical Trial Biomarkers

Drs. Sur, Tropea and Kaufmann are conducting international research to identify biomarkers that can predict an individual's response to either IGF1 or trofinetide.

**Mriganka Sur, PhD**  
Massachusetts Institute of Technology



**Daniela Tropea, PhD**  
Trinity College Dublin



**Walter Kaufmann, MD**  
Greenwood Genetic Center



### Wearable Home Sensors

Dr. Brunner is developing wearable, home sensor technology to measure gait, movement, heart rate, and sleep in girls with Rett syndrome.

**Daniela Brunner, PhD**  
Early Signal Foundation



# IGF-1 Results... What We Learned

Results for the Phase 2 IGF-1 clinical trial have been published. Walter Kaufmann, MD, scientific advisor and a principal investigator, of the Phase 1 and Phase 2 clinical trials of IGF-1 in Rett syndrome has provided us with highlights of the Phase 2 study's results.

## Highlights of the Phase 2 IGF-1 trial

- Mecasermin (injectable human recombinant IGF-1) is well tolerated by girls with Rett syndrome, confirming the findings of the Phase 1 trial.
- The Phase 2 trial did not confirm the positive effects of IGF-1 on anxiety and breath holding observed in the Phase 1 trial. Therefore, for FDA purposes, the trial should be considered negative due to lack of effectiveness. We are still investigating several factors that may have contributed to these negative results.
- Some symptoms, such as hyperventilation, may have worsened. The severity of hyperventilation was increased in caregiver reports but not through the testing that monitored breathing patterns.
- There were positive findings from this study as well, including an indication that social communication improved substantially. A minor improvement in repetitive behavior was also noticed.
- The positive findings need to be confirmed by follow up analyses.
- Indirect benefits of the trial include the recruitment of patients to participate in the development of a new developmental cognitive test for Rett syndrome, which can be used for clinical and research assessments. This new test was published December 2017.

Rettsyndrome.org's Chief Science Officer Steve Kaminsky, PhD, comments "As this clinical trial demonstrates, Rett syndrome is a complex disorder and although we had hoped for more positive results, these findings help us as we look forward. The research team built new tools to measure outcomes in Rett syndrome clinical trials as a result of this trial. I am proud of the families and researchers who venture down this road; they have added to our knowledge and have focused our attention."

Mustafa Sahin, MD, PhD, Director of the Translational Neuroscience Center at Boston Children's and a principal investigator of the study adds "Only by testing novel therapies through rigorous placebo-controlled studies, we can improve our chances of finding a safe and effective treatment for Rett syndrome. We have learned a lot about biomarkers and outcome measure during these trials with IGF-1. These will help us design better trials in the future. We are grateful to the pioneering individuals and families with Rett syndrome who took part in this trial."

We know it can feel disheartening to see that a trial should be considered "negative" for FDA purposes. It is so important to remember that clinical trials are just that — they are trials. We learn from all trials. Both failures and successes become critical in setting the direction for future research.

# A Grandmother's Story

by Leslie Mayorga, grandmother to Maddie

Becoming a grandmother is the best thing that ever happened to me. I knew I'd love my grandchildren but I wasn't prepared for the depth of emotion and love that overcame me with the arrival of each of my precious grandbabies. They are my heart and soul.

I prayed for healthy grandbabies. Boy or girl, the sex didn't matter as long as they were healthy. Madelyne, my three-year-old granddaughter was a charmer from day one. Just one look at that angel's face and my heart melted. She reached her developmental milestones right on time and always with a sweet smile on her precious face.

Maddie's first birthday came and went and Maddie wasn't walking but she was crawling and climbing the stairs at breakneck speed. Her pediatrician wasn't concerned and assured us that Maddie was fine. She had a vocabulary of 25 or so words and "she was just a late walker."

Around the age of 18 months, Maddie started having one ear infection after another. She still wasn't walking and the words she used to say like "pretty, baby doll, baby help, momma, dada, pizza and brother" had disappeared. The last time I heard Maddie speak was the night before her tympanostomy tube surgery when she clamored for "pizza."

Knowing something wasn't right we changed pediatricians and embarked on a journey to get answers. After countless appointments, referrals, tests, therapy sessions and hospital visits my granddaughter was diagnosed with Rett syndrome on January 5, 2017. I had so many questions. Would Maddie ever talk? Would she go to college? Would she get married and have children? The answer to each question was a gentle but emphatic no. In an instant, all the hopes and dreams I had for my granddaughter were shattered. At the same time my heart broke for my daughter and son-in-law and the pain they were going through.

I came home from the hospital and cried for three days. Then I got busy. I Googled Rett syndrome and found Rettsyndrome.org. What a blessing to find such a wealth of information so quickly. Knowledge is power! I registered with Rettsyndrome.org and contacted the Family Empowerment Representative in my state. I found answers to so many of the questions that had been running wild in my head. And I found resources, many of them local. I had a copy of the Rett syndrome Handbook within a few days. Thus began my journey to spread awareness about Rett syndrome.

I've met a lot of supportive families through Rettsyndrome.org. Each and every one has embraced us and encouraged us. Our "Rett Family" is always there to answer a question, offer a suggestion and shower us with love and prayers. Knowing we are not alone is a huge comfort.

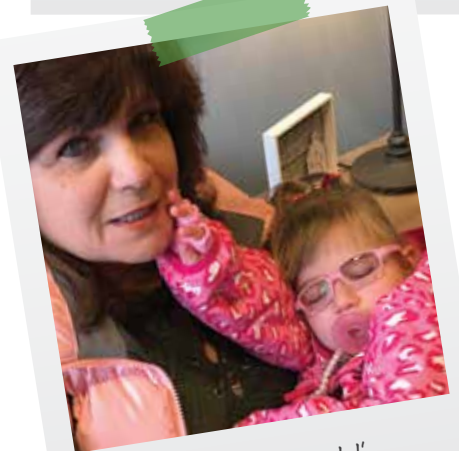
We celebrate all of Maddie's victories, no matter how small. I refuse to live in desperation and despair.



Grampa & Maddie



Maddie & Grampa

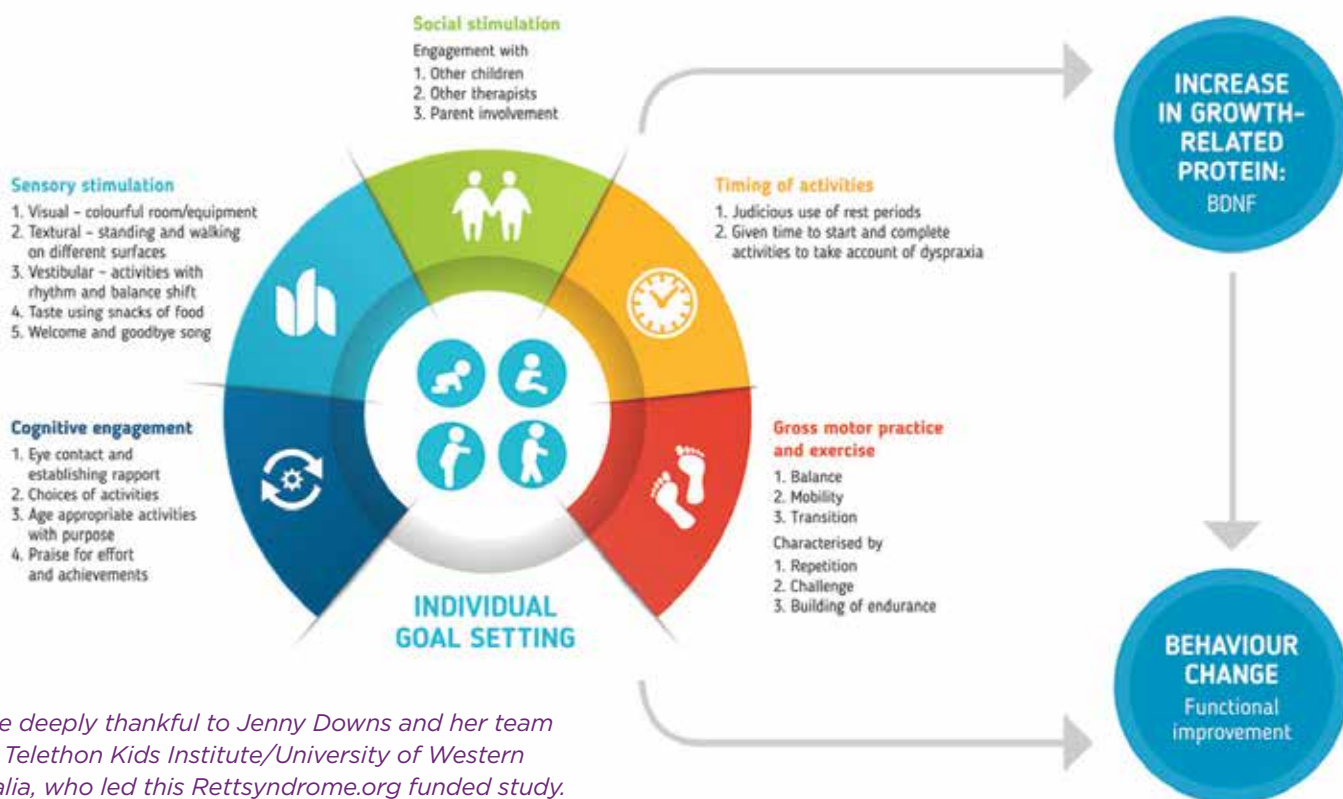


Grampa & Maddie

# Share This With Your School

A landmark study recently concluded that environmental enrichment — rich social, cognitive and sensory experiences — can enhance everyday life for a child with Rett syndrome. The effects are impressive, improving gross motor skills, BDNF levels, growth, sleep and mood.

## Some things you can begin doing today:



*We are deeply thankful to Jenny Downs and her team at the Telethon Kids Institute/University of Western Australia, who led this Rettsyndrome.org funded study. This truly represents the intersection of research and family empowerment!*

## Family Advisory Council

We are excited to announce that we will be establishing a Family Advisory Council. This council will advise Rettsyndrome.org leadership in program development and community engagement. Please contact Melissa Kennedy at [mkennedy@rettsyndrome.org](mailto:mkennedy@rettsyndrome.org) if you are interested in serving on the council.

### Are you the caregiver or parent to an adult with Rett syndrome?

Visit [www.rettsyndrome.org/for-families/adults-with-rett-syndrome](http://www.rettsyndrome.org/for-families/adults-with-rett-syndrome) to find resources and to connect and share with others.



# When Blessings Come in Pairs

by Kassie Morell

Is there anything more fascinating than twins? Twins interact with each other before they are born and form a bond. Forty percent of twins develop their own special “language” that they use to communicate to each other as babies. They can share features, feelings, moods and habits — or not. Twin births are rising and so is the number of twins with Rett syndrome. Kassie Morell is mom to twin daughters, Kenzie and Kaylie. This is her story.

When our daughters, Kenzie and Kaylie, were diagnosed 2 years ago, we were devastated as you can imagine. Raising twins is inherently challenging and having twins with Rett can feel overwhelming. Like many families, we started our search for information on Google even before the girls were officially diagnosed. We found [Rettsyndrome.org](http://Rettsyndrome.org) right away and it was our greatest source of information. After the diagnosis, I knew I had found a place to connect with others for support, advice, and encouragement. The Facebook Twins Network is a great place to begin!

[Rettsyndrome.org](http://Rettsyndrome.org) was the first organization to reach out to us and come along side of us in our journey. They connected us with a nearby family representative. Just talking with her gave us tremendous hope for the future. Connecting with other parents of twins helped

**Although identical twins have the same DNA, the progression of Rett in each will be unique. Twins with Rett syndrome are a special group within a rare disease and can give us insight into how environmental factors affect the progression of the disease.**

us face our fears about Rett and helped us learn how to find balance with our twins — we often feel like we are not doing enough for one of them — it can feel like an impossible balancing act.

We began the process of healing our hearts and spreading the message of hope for the future of those affected by Rett. Early on, we jumped right in to fundraising which made us feel more connected and gave us a sense of control. Joining the fight with other parents has been a great experience for us. We have learned much from them and are now able to share the lessons we have learned. And we have learned that we have much to be grateful for. Kenzie and Kaylie are amazing. One of the privileges of having twins is seeing their amazing bond! Even being nonverbal, they have their own language of communication through eye contact, sounds, and infectious giggles. They love being near each other, and doing activities together. It's so overwhelming having two children with special needs, but it's a blessing that they have each other.

We are also grateful for all of the dedicated people at [Rettsyndrome.org](http://Rettsyndrome.org). We are very involved and will continue to be because we are certain that in the near future our girls will benefit from the amazing research taking place to help end Rett syndrome.



**Rett Syndrome  
Education Day**  
APRIL 21, 2018



**THANK YOU FOR PARTNERING WITH US FOR YOUR EDUCATION DAY!**

# “The Road to 100: What I’ve Learned”

by Kelly Zwald featuring Libby Stromer and Megan Hill Mitchum



In spring 2017, Kelly & Libby, aunts to Alia decided to host the first ever Iowa Strollathon. Much to their surprise they were able to raise almost \$90,000 and this year have their sights set on \$100,000. We caught up with Co-Chair, Kelly Zwald, to learn more about their inspirational story.

## How the journey began...

When our niece, Alia, was diagnosed at the age of 2, we were devastated. As families do, we rallied around Alia and her family and did everything we could to support them, all the while falling more in love with Alia every day. This girl, who couldn't tell us how much she loves us, made it obvious through her smiling eyes when we would arrive and through her sad tears anytime we would leave. We wanted to do more to help. Alia's mom and our sister, Regan, pointed us to Rettsyndrome.org and the flagship Strollathon fundraiser. And the seed was planted.

And because every great story involves a glass of wine (or three), soon after that seed was planted, we were sitting with our friend Megan, enjoying wine and cheese on a sunny patio, and brought up the idea of hosting a Strollathon. Megan was all in, no convincing needed. She even added the idea of a silent auction and oh-so-generously offered her husband as the entertainment.

The next step was to meet our Iowa families. And, oh boy, once they were involved there was no looking back! While we aren't "Rett parents" ourselves they still welcomed us with open arms. It quickly became apparent that they were willing to do anything to get this event off the ground and make it a success.

**Although the original fundraising goal was \$10,000, the Strollathon raised almost \$90,000!**

Our success with the Stroll was due entirely to the commitment of the Iowa Rett families and their families and friends. These girls grab you by the heart, so once

we provided a platform through which to get involved, there was no stopping them! We made sure our event had a wide appeal by including fun activities, a walk for all the families, a silent auction and beer. Beer helps... are you sensing a theme?

Our success was also due to one person who doesn't get nearly enough credit — Jackie from Rettsyndrome.org. Not only was she a constant resource for us during the planning process (and I mean constant... like day or night...), she also attended our event in person and did it all with a huge smile and a contagious laugh.

**Kelly, Libby & Megan want to raise \$100,000 this year and have a plan to get there.**

This year will be more of the same, but better! Aiming for such a big number is a little intimidating, but we're confident that all of our families, friends, and supporters will step up.

## They have gained as much as they have given.

I've learned that, as Iowans, we are humble. We know that everyone has their struggles and, while some of us have been given more challenges than others, we know how lucky we really are. We also know that everyone needs help at times. I've learned that when you ask for help, share your story, your struggles and your pain, people step up. Iowans may be humble but, perhaps more so, we are generous.

Everyone has something unique to give. By tapping into the talents and resources of everyone whose heart has been touched by one of these girls, we've been able to spread our message to people and places that would otherwise not have been possible.

I think I knew this deep down somewhere, but this experience has confirmed that people are good and generous and selfless. **We couldn't agree more.**



## BOYS WITH RETT SYNDROME

On January 18, 2018, Rettsyndrome.org held an online seminar for parents of boys to meet some of our top clinic medical directors. Information was shared, families were heard, and we learned about the future of research for this small but mighty group. You can listen to the recording of the call and read the Q&A from the call at [www.rettsyndrome.org/about-rett-syndrome/boys-with-mecp2](http://www.rettsyndrome.org/about-rett-syndrome/boys-with-mecp2). We want everyone to open your hearts to these handsome guys and their determined parents.

## SIERA AND LISA RUSHIN



In February, Siera Rushin turned 30 in style. Mom, Lisa, threw Siera a magnificent 30th birthday party that was also a fundraiser for Rettsyndrome.org. The 200+ guests were treated to an evening that included dinner, an auction, live music and, of course, birthday cake! **Together, they raised an impressive \$10,000.** Though they have been active in fundraising for years, Lisa says that “until a cure is found and as long as I’m still around, we’ll be in the fight along with everyone else.” Lisa has been active for years as a parent representative, event host, and volunteer.

Thank you, Lisa and Siera for being an inspirational part of our Rett community.

WHO'S  
INSPIRING  
US!



## KENNA SEILER AND DEBORAH PATTERSON

Kenna and Deborah represented Rettsyndrome.org at a Rare Disease Day event in Houston, Texas. The event was attended by medical professionals in the rare disease community, local researchers and families affected by rare diseases.

We celebrate Kenna and Deborah’s unwavering commitment to advocating for and supporting those with Rett syndrome. Rare Disease Day takes place in late February each year and was created to make policy makers and the public aware of rare diseases and their impact on patients’ lives and to reinforce their importance as a public health priority.



## MORGAN'S WONDERLAND!

Last fall, Rettsyndrome.org hosted a much needed get away for our families at Morgan’s Wonderland in San Antonio. Friendships were made, first-time travel adventures were tried and enjoyed, supplies were collected and donated to RTT families affected by Hurricane Harvey, and Judy Barrish, RN joined us for research and care updates. Morgan’s Wonderland was truly a magical place to enjoy!

*“We had a blast! Thank you to all the families that made this weekend one to remember. Many more to come with new friends.”*

We hope to see you at our NEXT getaway!



## Meet Samantha

### Rettsyndrome.org's NEW Family & Community Engagement Manager

Hi all!!! My name is Samantha Brant and I am honored to be your new Family and Community Engagement Manager! My husband Scott and I have 2 girls, Cede (Sadie) is 18 and Macy is 3. Cede is a freshman in college studying Radiology Technology. Macy is our little one who has Rett syndrome. Macy has and will continue to be the backbone to our family that we never knew we needed. She truly was the pivotal change in my life that brought me to my passion... this position!

I was a childcare provider for about 15 years and spent several years working for a Fortune 500 company as an Executive Team Leader. I am a graduate of Mid America Nazarene University with a degree in Human Relations and Management. Since Macy's diagnosis I have dedicated my life to bringing awareness, showing and teaching inclusion and raising funds for research towards clinical trials.

I am not only over the moon to bring families together, but I am also excited to meet each of you at strolls, events, or even over a phone call. I hope you will reach out to me if you need an ear or guidance. I am dedicated to helping you. Thank you from the bottom of my heart for welcoming me!

Best,

Samantha Brant  
sbrant@rettsyndrome.org

***Rettsyndrome.org is thrilled to add Samantha to our team. Samantha said that she was honored for this opportunity and we feel the same! Please help us welcome her!***

Many cities feature organizations like ours in their monthly publications as a way to raise awareness and encourage donations. If you would like help creating an ad for your area, just let us know.

[admin@rettsyndrome.org](mailto:admin@rettsyndrome.org)



## 2018 SPRING/ SUMMER EVENTS

Below are some of our events coming up in the next few months. To see a complete list of all of our events, please visit [www.rettsyndrome.org/events-calendar](http://www.rettsyndrome.org/events-calendar).

### SPRING/SUMMER EVENTS

- St. Louis Strollathon - 5/5/18
- Iowa Strollathon - 5/5/18
- Live Rett Free, OH - 5/6/18
- Golf Fore a Cure, FL - 5/12/18
- Charamella Golf Tournament, PA - 5/14/18
- Samantha Corpus Golf Tournament, CA - 5/15/18
- Maryland Strollathon - 5/19/18
- Farmington MI Strollathon - 5/20/18
- Ella Foard Golf Tournament, NC - 5/21/18
- Flower Power Fun Run, CA - 5/26/18
- Rip it for Rett, ME - 6/29/18
- Rett Syndrome Charity Golf Classic, CA - 7/27/18
- Casting 4 a Cure, ID - 8/9/18 - 8/12/18



Gillette's Rett Syndrome Program began in 2005 at the request of the newly named Midwest Rett Syndrome Foundation (MRSF) parents. Today, the program specializes in treating children, adolescents and adults who have disabling medical conditions that begin during childhood, including Rett syndrome. They focus on providing specialty care to maximize both ability and quality of life.

Gillette serves patients in a five-state region and beyond, so excellent care coordination is essential, and it begins well before the first appointment. The core team works closely with the family and the many primary providers and specialists who might already be involved in a child's care. Each family and often the patient's primary care provider is consulted by phone before the first clinic visit to anticipate medical needs and maximize the benefits of an appointment at Gillette. Gillette works closely with families to develop a care plan that's appropriate for their child, based on their experience and the latest information and research about Rett syndrome. Because they believe that care shouldn't end when a child reaches adulthood, they have developed specific clinics for adults with childhood-onset disorders, like Rett syndrome.

Gillette's current research initiatives are focused on how girls with Rett syndrome both interpret and communicate about pain. As new biological and chemical therapies are developed, Gillette hopes to be a leader in understanding their effects and benefits in both the clinical and community setting. The staff at Gillette and their colleagues at the University of Minnesota look forward to future collaboration with other university medical centers that wish to research treatment and care of children who have Rett syndrome.

Rettsyndrome.org is proud to have designated Gillette Children's Specialty Hospital a Clinical Research Center of Excellence in 2017. As one of only 14 clinics to achieve this, Gillette continues to lead the way with innovative and cutting-edge care for children with Rett syndrome.

# RettEd

OUR FREE WEBINAR SERIES



Registration for webinars, recordings of all webinars and a PDF of the Question & Answer portion of each are available at <https://www.rettsyndrome.org/RettEd>

## Upcoming RettEd Webinars

All Webinars will be held from 1-2:30 PM EST unless stated otherwise

- 5/8/18** IEP: Strategies for Rett Success  
*Valerie Owen, PhD*
- 6/12/18** Movement Disorders & Motor Issues in Rett Syndrome: Definition, Intervention and Clinical Research  
*Bernhard Suter, MD,*
- 7/10/18** Communication - Making it Count  
*Judy Lariviere, M.Ed., OTR/L, UCSF*
- 8/14/18** Understanding Autonomic Dysfunction in Rett Syndrome  
*Jeff Neul, MD, PhD, Vanderbilt University*
- 9/11/18** Epilepsy: Incidence, Treatments & Research  
*Eric Marsch, MD, PhD, CHOP*
- 10/9/18** Special Update on ABLE Accounts  
*Mary Anne Ehlert, CFP, Protected Tomorrows, Inc*

**RettEd will present an IEP Summer Series!**  
**Check website for registration.**





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# Thank YOU!

It will take a united army of dedicated, passionate people to win the battle against Rett syndrome. We are extremely grateful for strong partnerships with other organizations and are also indebted to the incredible individuals that fundraise on our behalf, moving us closer to our common goal of curing Rett syndrome. This support allows us to invest in critical research and provide much needed support for families. We would like to thank our many donors for your generosity and support.