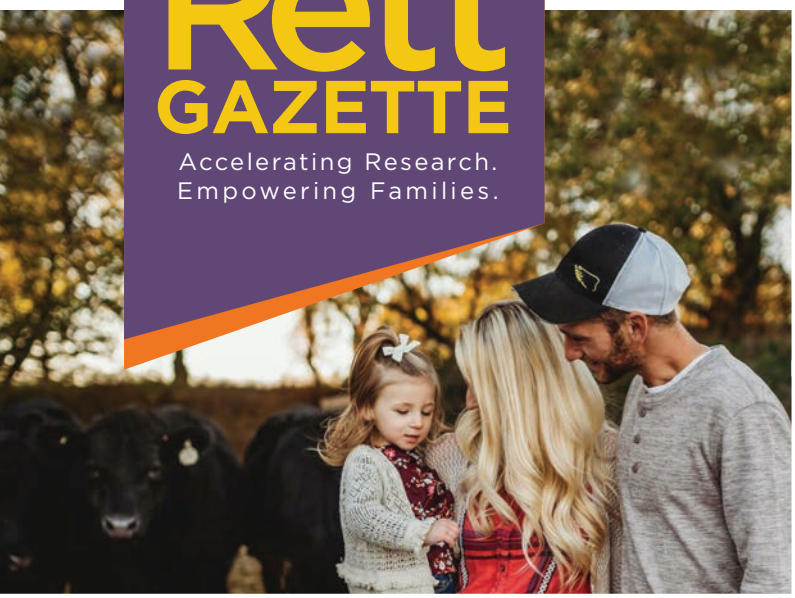
SUMMER 2020

Rett GAZETTE

Accelerating Research.
Empowering Families.



Confronting Challenges Head On

SERVING THE RETT COMMUNITY IN UNPRECEDENTED TIMES

Rettsyndrome.org
ACCELERATING RESEARCH. EMPOWERING FAMILIES.

Melissa Kennedy
Executive Director

As I write this, we are feeling the impact of COVID-19 on our world. Uncertainty remains about how this virus will continue to affect the Rett syndrome community.

We are being tested. Like you, I try to focus on the hundreds of stories of love, grace, kindness, and support in the face of adversity. Things may not be "normal" for a while, so we will continue to look for beauty in each day. Our resilience is what will get us through. And the Rett syndrome community understands what it means to be resilient. It is our honor to serve you every single day.

At times like this, it becomes crystal clear that the two-pronged mission of our Foundation — accelerating research and empowering families — is what's needed. **Our team continues to work hard to fulfill our mission in new and different ways. We have developed 30 hours of online and virtual educational programs to help keep our families active and engaged while staying at home.** These resources have had over 35,000 views. We are working with researchers to identify ways to continue important research because they have had to close their labs to all but essential personnel. We launched a secure platform so that physicians caring for Rett syndrome patients can communicate with one another securely and privately. It is critical that something learned in one Rett clinic is shared with the nationwide network of providers immediately.

I know we will grow stronger through this trial. I'm honored to share this Gazette with you, which shows how we are serving you during this challenging time.

And as always, with gratitude and hope,

Dominique Pichard, MD
Chief Science Officer

I am wrapping up an exhilarating first nine months with Rettsyndrome.org! I have attended rare disease meetings, met with leaders at the NIH and FDA, attended the Rett Science Symposium organized by Drs. Huda Zoghbi and Adrian Bird, and met with Rett syndrome families. I have been laying the groundwork to move the needle toward treatments for our loved ones with Rett syndrome. Every day I look into the eyes of my daughter, Catalina, and I feel the urgency to find an end to the struggles and the suffering from Rett syndrome. Every passing day that she struggles with Rett syndrome, and its effects on her precious body, is one day longer than I want. It is with this urgency and this passion that I work hard every day to change the course of the lives of our loved ones.

In 2020, with the guidance of our new Scientific Advisory Board, we will continue to seek the best science to invest in while continuing the critical scientific activities we have been funding to bring treatments to our children. Since 2012, we have had a collaboration with Psychogenics, which tested 34 drugs on a mouse model of Rett syndrome. From this work, three drugs have gone on to clinical trials and a fourth one is close to starting. That is a phenomenal translation rate for drug development! **We are welcoming pharmaceutical companies to the field of Rett syndrome and helping them create Rett syndrome drug development programs so that our loved ones will have treatments sooner.** Through close collaboration with our medical experts, we have also generated the first Primary Care Guidelines, published the first Communication Guidelines, and are working on developing best practices for clinical care at our Rett syndrome clinics.

As a fellow Rett syndrome parent, I am humbled to be serving our community as the Chief Science Officer. You have my commitment to work hard every day to bring the best science forward, to keep open lines of communication with key decision-makers like the NIH and the FDA, and to continue to work towards treatments for our children living with Rett syndrome.

Our Newly Funded Research

We're excited to announce that we have awarded $1.3 million in international research — focused on unlocking the many mysteries of Rett syndrome as we develop treatments and work toward a cure. Rett syndrome is an incredibly complex brain disorder; no two affected individuals are the same. Our Foundation focuses on multiple research and treatment approaches. View full descriptions of their Rett syndrome research on our website: www.rettsyndrome.org/research/our-research/funded-research/

MEET OUR NEW CLASS OF RESEARCHERS

GENE-TARGETED THERAPIES

Thorsten Stafforst, PhD
Harnessing RNA editing to treat Rett syndrome

Crystal Zhao, PhD
Functional analysis of an enhancer-like element required for Xist expression

TRANSLATIONAL RESEARCH

Nicoletta Landsberger, PhD
Cellular and molecular drug screening system for Rett syndrome therapy

Louise Dickson, PhD
Investigating a novel treatment for Rett syndrome targeting mGluR7

BASIC SCIENCE

Lotje De Witte, MD, PhD
Investigation of microglia, an immune cell, in Rett syndrome

Kerry Delaney, PhD
Brain cell communication in a mouse model of Rett syndrome

Ferdinando Fiumara, MD, PhD
Pathophysiological structure, interactomics, and function of the N-terminus of MeCP2-E1 and its disease-related mutants

Hilde van Esch, MD, PhD
Brain development in a humanized mouse model of Rett syndrome and Rett-related disorders

NEUROHABILITATION THERAPY

Meir Lotan, PhD
A physical therapy-based scoliosis intervention program for patients with Rett syndrome

Confronting COVID-19 Challenges Head On

RETTSYNDROME.ORG HAS RELEASED NEW RESOURCES TO EMPOWER & EQUIP YOU DURING COVID-19

By Paige Nues, Director of Family Empowerment, Mom to Katie

The shelter-in-place guidelines have created unique challenges for our families caring for a loved one with Rett syndrome. Many face-to-face therapies have been stopped. Most therapy has shifted to parents and caregivers. And we have all experienced the loss of well-established daily routines and the added burden of trying to maintain a safe, healthy environment that protects our vulnerable children.

Here are three key ways our Foundation is helping to meet the challenges facing Rett syndrome families right now.

COVID-19 COMMUNITY UPDATES

We know that the rapid and recent spread of COVID-19 has many of you understandably concerned for the welfare of your families. We hear you. And we are still here for you.

Our leadership team is actively monitoring the development of the coronavirus outbreak and updating our practices as needed to protect our community. We will walk with you through this outbreak and will help keep you informed of news and science-supported precautionary actions you can use to help keep your family safe and healthy. Please visit our website regularly for updates from Dr. Tim Benke, Rettsyndrome.org Medical Advisor.

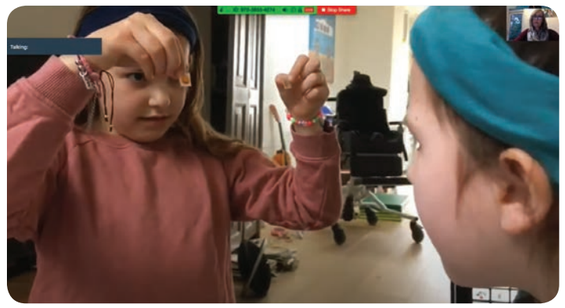
PHYSICAL THERAPY AND COMMUNICATION TRAININGS

We have mobilized our global network of Rett experts, researchers, partners, and parents to develop trainings for you to use at home. A list of home-bound trainings can be found on our website. These are some of our most popular Facebook Live events.

Communication Intervention Strategies, Parts 1-3, led by Dr. Gill Townend and Dr. Theresa Bartolotta, expands on information found in our Communications Guidelines. Expect practical advice and strategies backed up by solid research and illustrated with examples.

Moves & Stretches for Children, Tweens, and Adults, Parts 1-3, led by Dr. Meir Lotan, demonstrates techniques you can use to keep your loved one active and moving while staying at home.

Communicating & Connecting While at Home, led by Judy Lariviere, M.Ed., OTR/L shows how to support your loved one with Rett syndrome in their communication through engagement and participation in some fun family activities.



AT-HOME RESOURCES AND ACTIVITIES

An international group of therapists, advocates, and parents who are passionate about supporting children with Rett syndrome put this resource together. It contains ideas that may be helpful for you and your child while you are spending more time at home. The ideas are based on the concept of participation, that is, meaningful involvement in activities of daily life, with focus on the home setting. There are sections on household chores, personal care, learning and education, exercise and activity, and indoor play and games. You will also find online resources, including where to purchase switches and switch-operated toys and where to go for additional help.

In addition to our At-Home Online Trainings, we have re-released past RettEd webcasts that are relevant to families during this time. As always, we continue to host monthly RettEd webcasts with top researchers and experts in the field of Rett syndrome.

HERE'S WHAT SOME PARENTS HAD TO SAY ABOUT OUR TRAININGS:

"Very interesting ideas that I will take along and implement."

"It's such an awesome resource to have and makes me feel a bit of normalcy during this. [One] big take-away I had from [Facebook] live with Dr. Lotan was to have Viv walk to various activities and have her help as much as she can. I've been trying to remember to do that instead of just carrying her everywhere... even though it's hard to remember!"

"Thank you for this! It is so motivating and valuable!"

"It is a great session for me. It's an emergency for me to learn and teach my daughter how to communicate! She is crying, and crying daily and constantly and I don't have any picture book or device."

"Great seminar!! Loved the information and ability to connect with others on FB live! Can't wait for the next!"

"Thanks for a great session. Lots of ideas here for me in UK with my son."

RettEd HERE'S WHAT'S COMING UP:

RettEd Webcast: WEDNESDAY, JULY 15, 1PM (EDT):
Longevity in Rett Syndrome, Myths and Facts with Dr. Alan Percy

RettEd Webcast: TUESDAY, AUGUST 11, 1PM (EDT):
What Hurts? Understanding Pain and Rett Syndrome with Dr. Frank Symons

RettEd Webcast: TUESDAY, SEPTEMBER 8, 1PM (EDT):
Holiday Life Hacks for Rett Syndrome

RettEd Webcast: TUESDAY, OCTOBER 13, 1PM (EDT):
Clinical Trial Update

RettEd Webcast: TUESDAY, NOVEMBER 10, 1PM (EDT):
Communication Guidelines and Workbook for Rett Syndrome with Theresa Bartolotta, PhD

Register online: rettsyndrome.org/education

Serving & Equipping Families with Rett

By Samantha Brant, Family & Community Engagement Manager, Mom to Macy

When most families find out their child has Rett syndrome, it's the first time they've ever heard of the disease. Often they're overwhelmed with emotions and fear and questions as they try to figure out what to do next, and many families search the internet looking for answers.

That's usually where my relationship with them begins. They find Rettsyndrome.org and register on our "Newly Diagnosed" webpage. When I receive their registration, I immediately connect them with resources and a community that's ready to embrace them.

I remember how I felt when my daughter, Macy, was diagnosed at 17 months, so I know they're probably feeling scared and alone. I take their hand and tell them it's going to be ok. Most parents are grateful, but some are not ready. And that's okay. We'll be there — no matter where they are in this journey — with resources, information, and a community they can easily connect to.

When I first reach out to a family with a new diagnosis, I recommend they visit the closest Rett syndrome clinic so that their child can get a full evaluation and begin finding the best strategies, devices, and caregivers. I also connect them to valuable tools — such as the First 100 Days Pathway, the Rett Syndrome Primary Care Guidelines, our Rett Syndrome Handbook, and the newly released Rett Syndrome Communication Guidelines — to help them prepare for that visit by familiarizing themselves with what to expect.

I also give them the contact information for their family empowerment state representative — a person who has experienced Rett syndrome personally and can provide a listening ear and specific recommendations to resources in their home state. These volunteers are a wealth of information and are energized by helping families find the best care and community for their children. The range of their backgrounds — caregivers, fathers, grandmothers, mothers, and others — provides a robust wealth of knowledge to families. They're available by phone or email (see www.rettsyndrome.org/for-families/connect), and they position themselves in their communities and online as leaders. Each state has their own Facebook group as well.

In addition, the Family Advisory Council supports these state representatives, oversees the entire program, and makes recommendations on what the Rett community needs. This allows our state representative program to adjust to the changing needs of our Rett community.

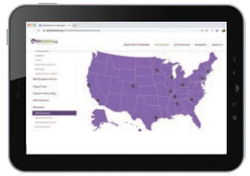
I'm so grateful for all the volunteers in our Rett community because we know that wherever a family is on their Rett journey, they will always have access to the best, most reliable, up-to-date information and resources, as well as a much-needed listening ear. And as always, any family can reach out directly to me; if I'm not able to answer your questions, I assure you I will get you connected with someone who is.

Families usually have several urgent questions following a diagnosis:

- "What should I do next?"
- "How will my child communicate with me?"
- "Why should I go to a clinic?"
- "What do I ask when I go to a clinic?"

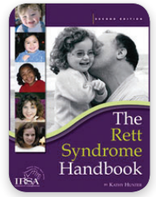
Our Family & Community Engagement Manager Samantha Brant is ready with answers. Contact her at sbrant@rettsyndrome.org.

NEED A RESOURCE? HERE'S A LIST OF SOME OF WHAT WE HAVE AVAILABLE:



State Resources Page

This interactive online map provides a list of family empowerment representatives, treatment and resource centers, state agencies and resources, and parent recommended resources.



Rett Syndrome Handbook

This comprehensive book covers everything from family issues to common problems to therapeutic avenues and more.

Primary Care Guidelines

This is an invaluable tool to use for doctors' visits so that areas of assessment and assessment details are addressed in a timely and consistent manner.

Monthly Webcasts

These monthly webcasts on various Rett syndrome related topics include Newly Diagnosed, Epilepsy, Nutrition, Sleep, and more!

Communications Guidelines

The first book of its kind, it gives parents, caregivers, communication professionals, therapists, school educators — in short, anyone who interacts or provides care to your child with Rett syndrome — access to the best information and strategies to facilitate your child's communication.

Individual Education Plans (IEP)

This resource includes IEP examples from preschool age to high school, webinars on how to build an effective team, and suggestions on how to make the plan inclusive.

Nutritional Guide

This beneficial tool is designed to help improve the health outcomes in Rett syndrome by learning about nutritional options, the potential difficulties that may come with the diagnosis, and resources to make the best choices for your child.

"Tonight… I'm finally processing Hayden's diagnosis and trying to work through all of the initial appointments for therapies and getting to a Rett Clinic and overwhelming doesn't begin to describe it. It is also hard because Hayden is part of the 20% of kiddos who are atypical, making it even harder to get everything in order. This book [provided by Rettsyndrome.org], now that I'm digging in, is like a roadmap of Rett and becoming very helpful."

~ Amie, parent

Everyone with Rett Deserves to be Heard

INTRODUCING THE RETT SYNDROME COMMUNICATION GUIDELINES

People often underestimate the communication capabilities of individuals with Rett syndrome. This means that our loved ones are often left out of meaningful school, play, and social interactions. To address this critical issue, Rettsyndrome.org funded an Angel Grant in 2015 to create The Rett Syndrome Communications Guidelines — a global endeavor that included 650 Rett syndrome experts and parents from 43 countries.

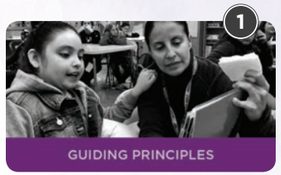
In it, you'll find the recommended guidelines for the assessment, intervention, and longer-term management of communication in individuals with Rett syndrome, as well as relevant background information for caregivers and professionals.

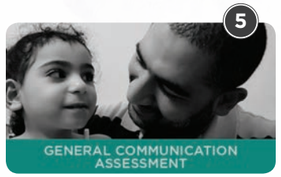
It's never too early or too late to start. In fact, wherever you are on your Rett communication journey, this handbook can help guide your next steps — whether you have a child who is newly diagnosed or an older adult child.

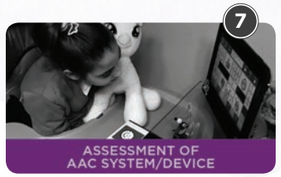
To get started today, download Section 4: Strategies to Optimize Engagement for free, and the entire book is available to purchase as a download or a hard copy at our website: Rettsyndrome.org.

HOW IT'S ORGANIZED

The Rett Syndrome Communication Guidelines is divided into these eight easy-to-navigate sections:







EARLY PRAISE

"The Clinical Guidelines for the Management of Communication in Individuals with Rett Syndrome handbook will be a valuable manual to families of individuals with Rett Syndrome after initial diagnosis and in the years to come. These families can use the handbook when meeting with doctors (neurologists, pediatricians, gastroenterologists, ophthalmologists, etc.) so that parents and caregivers are more knowledgeable in advocating for therapies for their [children]."

~ Andrea Etkie, Speech-Language Pathologist, M.A., CCC-SLP

"This is an incredible resource for parents, communication partners, and ultimately for children/adults with Rett Syndrome. I've read through it twice. I wish I had it two years ago when first starting AAC for our daughter, Ann. It's comprehensive yet concise. After working with three highly trained SLPs over that two year period, I can't think of any relative material that can be added or that has been left out."

~ Jack Kuwik, parent and Family Advisory Council member

"I just had an IEP [meeting] today and want to send it to our speech and language therapist right now. I want to send to my extended family and all therapists that work with Jaidyn [daughter] for ideas, consistency, and understanding for communication."

~ Daniel Warner, parent and Family Advisory Council member

"Learning how to use augmentative and alternative communication (AAC) and learning how to facilitate their loved ones to maximize their potential, takes belief in their abilities, a great deal of effort, and a lot of support. Seeking knowledge and skills on how to adjust assessments and how to manage communication, parents (and professionals) find themselves in need of expert support, in need of a shared vision of how to approach these aspects... Our beautiful daughters and sons with Rett syndrome may be unable to use their own voices to speak, but surely these guidelines will help them move forward in communicating their needs and opinions and in speaking their minds."

~ Mariëlle van den Berg, Chair, Rett Syndrome Association Netherlands

ORDER YOUR COPY TODAY AT RETTSYNDROME.ORG

Rett Research Ready™

Exploring the Journey Toward a World Without Rett

As the number of research studies and clinical trials for Rett syndrome grows, there is hope in the search for treatment. Getting your child involved in research can help develop treatments for our loved ones affected by Rett syndrome.

Deciding to enroll your child in a clinical trial is not easy. It takes knowledge, courage, and hope. When making this decision, you need to be research ready. That means fully understanding how clinical trials work and what participation means for your loved one and family.

OUR CLINICAL TRIAL READINESS PROGRAM

At Rettsyndrome.org, we recognize that many families in our community need more accurate information to be able to understand the current research opportunities available and how you and your child may be able to participate.

Our team developed the **Rett Research Ready™ program** to help you learn about Rett syndrome research and get all the knowledge you need to make smart decisions for your family.

"We want to build the foundation to help people feel comfortable and informed as they consider enrolling their child in a study," says Dr. Dominque Pichard, Chief Science Officer.

To do this, our online clinical trial readiness program provides:

***my*RETT TRIAL FINDER —** High quality, accurate information about all clinical trial studies, regardless of trial sponsor

EDUCATION MATERIALS — Simplified information about the technical aspects of study design, study phases, and other details you should understand

RESOURCES — Information to help families:

- Review details about [relevant] studies
- Ask questions
- Discuss options with their care team

SUPPORT — Assist families, researchers, and sponsors to facilitate a positive impact on living with Rett and its eventual cure

HOW THE SEARCH TOOL WILL HELP YOU

To help you access all of this information, Rettsyndrome.org created an easy-to-use tool, called ***my*Rett Trial Finder**, that will help you gather patient and family-focused research information all in one place.

The online *my*Rett Trial Finder will:

- Always be up-to-date.
- Explain the research process in easy-to-understand language.
- Gather all information about Rett syndrome clinical trials and other research activities, such as surveys or rehabilitative studies, in one place. This lets you compare and contrast the details of each one.
- Give you a tool to use when talking with your child's doctors and care team.
- Provide an educational platform to learn about the latest research tools and findings.
- Help you get started on your clinical trial journey.

ARE YOU RESEARCH READY?

To learn more, visit our website where you can access accurate, timely, and high-quality information about research and clinical trials. Rettsyndrome.org will provide ongoing support to ensure you have the resources you need to make informed decisions with your care team and be research ready.

Visit trials.rettsyndrome.org to get started

Gene Modifying Therapy Research: Accelerating Toward a Cure

By Dominique Pichard, MD

WE WANTED TO EXPLAIN THIS INCREDIBLY COMPLICATED WORK AND AT THE SAME TIME LET YOU KNOW THAT WE'VE GOT YOU COVERED.

We are working hard to provide a better life for your loved ones with Rett syndrome. It is believed that the greatest life improvements will come from treatments that address Rett syndrome at its root cause: mutations in the Methyl-CpG Binding Protein 2 (*MECP2*) gene. Gene modifying therapies, a class of treatments that work by altering genes inside the body's cells, may do just that. By fixing the *MECP2* defect, these treatments could permanently convert the diseased cells of a patient with Rett syndrome into healthy ones. Hopefully, this would stop disease progression, eliminate symptoms, and possibly provide a curative therapy for Rett syndrome. Today, gene modifying therapies are becoming a reality. Many in our community may be aware of the recent success of a Spinal Muscular Atrophy (SMA) gene therapy. This therapy overcame one of the major barriers to Rett syndrome gene modifying therapy: delivering genetic changes to brain cells.

This achievement brings us much closer to a gene modifying therapy for Rett syndrome. Yet, much work remains to be done to ensure the safety and efficacy of such a treatment. We still don't know the consequences of modifying a patient's mutant *MECP2* gene. Additionally, we know that while *MECP2* mutations cause Rett syndrome, too much good *MECP2* can result in another disorder. Thus, modifying *MECP2* requires great care.

Fortunately, there are many ways to approach gene modifying therapy, including gene replacement (the method used to treat SMA), gene activation, and RNA editing. Rettsyndrome.org currently funds research on all these approaches.

Gene replacement provides a working *MECP2*

One way to treat Rett syndrome is to simply give cells a working copy of *MECP2*. Gene replacement accomplishes this task using an adeno-associated virus (AAV) vector. This non-pathogenic AAV vector functions as a delivery service. It travels from the site of its injection to the cells in the brain where it adds its normal *MECP2* gene "package" to these cells' contents, providing a replacement for the mutant *MECP2*.

Before this type of therapy can be attempted in humans, it must be thoroughly tested in an animal model. Toward this end, Dr. Steven Gray at UT Southwestern, one of our previously funded researchers, has helped develop AAV vectors for the delivery of *MECP2* in mouse models of Rett syndrome. Some of our currently funded researchers are also making advancements in this space, including Dr. Sarah Sinnett, a next-generation researcher and former post-doctoral researcher in Dr. Gray's lab. Dr. Sinnett at UT Southwestern is working to find out if AAV-mediated gene replacement combined with an enriched environment or a physical exercise program results in better treatment outcomes in a mouse model than gene replacement alone.

Meanwhile, Dr. Colleen Niswender's lab at Vanderbilt is addressing the *MECP2* dosing issue. Since a variety of *MECP2* mutations can cause Rett syndrome, her lab is testing if the optimal dose of *MECP2* depends on which mutation is present using a mouse model. The results of these investigations will critically inform the design of the safest and most beneficial gene replacement treatment for humans.

Gene activation uses existing good *MECP2*

Another way to treat Rett syndrome involves using the normal *MECP2* gene that female cells already have. You may have heard that Rett syndrome is an X-linked disorder. This is because *MECP2* is located on the X-chromosome. Females have two X-chromosomes, whereas males have one X-chromosome and one Y-chromosome. Only one X-chromosome can be active in each cell. This means that in females with Rett syndrome, the cells in the body are mosaic: some have the working *MECP2* gene turned on and some have the nonfunctional *MECP2* gene turned on. The other X-chromosome is still in each cell, but it has been turned off.

Several of our researchers are working to get the good *MECP2* gene females with Rett syndrome already have turned on again. Dr. Jeannie Lee's lab at Massachusetts General Hospital is using a combination of drugs to turn on the entire otherwise inactive X-chromosome. Dr. Kyle Fink's lab at UC Davis is taking advantage of recent breakthroughs in gene-editing technology, CRISPR, to turn on just the silent *MECP2* gene. Meanwhile, Dr. Chrystal Zhao at Sanford Burnham Prebys Medical Discovery Institute will determine whether blocking a regulator of X-chromosome silencing results in reactivation. All three researchers are testing their approaches in mouse models and cell cultures derived from Rett syndrome patients to accelerate any successes to the clinic. Their important work will help identify the best methods for unlocking the potential of a female patient's good *MECP2*.

RNA editing corrects the course of defective *MECP2*

While *MECP2* mutations cause Rett syndrome, it is the protein product of the *MECP2* gene (rather than the gene itself) that creates all the problems. The path to making this protein requires a *MECP2* RNA intermediate, which offers the opportunity for another Rett syndrome intervention.

Dr. Thorsten Stafforst at the University of Tübingen, one of Rettsyndrome.org's new awardees, is taking advantage of this opportunity. The goal of his research is to edit mutant *MECP2* RNA so that it can make a normal protein. Cells already contain RNA editing machinery. Dr. Stafforst's research aims to simply direct that machinery to mutant *MECP2* RNA as a new and different way to treat Rett syndrome.

Hope for a better life on the horizon

It will take time to evaluate these different approaches. However, **recent successes in gene modifying therapy research make us cautiously optimistic that a transformative treatment for Rett syndrome is on the horizon.** To assist our loved ones as soon as possible, we at Rettsyndrome.org will continue seeking treatments to alleviate symptoms and improve quality of life while we wait for this research to help create a world without Rett syndrome.

"We continue to explore approaches to treat Rett syndrome by gene therapy, and that future is looking brighter. It was a fellowship from RSO in 2007 that started me working on gene therapy for Rett syndrome, for which I'll always be thankful."

~ Dr. Steven Gray

Mission Sparks a Million Dollars in Gifts

Roehr humbly explains, "The little bit that we all do is the most rewarding thing that means so much to us..." (Pictured Alvin Roehr and Gordy Rich)

Twenty-three years ago, Alvin Roehr was introduced to Rettsyndrome.org through a close relationship with Gordy and Anne Rich and their daughter, Kelly, who has Rett syndrome. Moved by our mission, Roehr shared with his friends and colleagues at the Roehr Agency his hope for Kelly and his passion to support Rettsyndrome.org. Then he led by example, and he and his team decided to play for Rettsyndrome.org in the Chubb Charity Challenge each year.

Alvin has become an invaluable advocate for our work and leading others, such as the Robert and Adele Schiff Foundation, to support Rettsyndrome.org themselves. Roehr's advocacy has contributed immeasurable increases in awareness of Rett syndrome and procured over a million dollars in financial donations, much of which was key in helping bring trofinetide and other compounds into clinical trials.

Amy Kendall, senior VP of Chubb Insurance and CCC Cincinnati tournament leader, has watched Roehr's dedication to Rettsyndrome.org and now shares his passion to help find a cure. "Watching Alvin interact with Kelly and the love and care he has is unparalleled," explains Amy. "Being such a small part of a revolutionary breakthrough [trofinetide clinical trial] for the Rett syndrome foundation is unmatched ... and that's why we do what we do."

Inspired by Roehr, Chubb Insurance created four videos showcasing his dedication to Rett syndrome and his relationship with Kelly. These have been shown across the country, introducing thousands to our foundation's mission and focus.

Gordy is incredibly grateful to Alvin for his commitment in supporting the foundation's mission. "It's indescribable! From the bottom of my heart, I am very thankful to the Roehrs for the impact they have had. We are on the precipice of treatments and a cure for our loved ones, and that's what Anne and I live for every day."

Thank you, Alvin Roehr, for your friendship, partnership, and focus on helping us find treatments and hopefully one day a cure. We're inspired by your heart and your generosity.

UPCOMING EVENTS

Our event calendar has shifted quite a bit, but we are adapting to continue to raise awareness for Rett syndrome and funds for Rett syndrome research. For a complete list of events, visit our online event calendar at **www.rettsyndrome.org/events.**

**Please note all events are subject to change.*

8/11
Samantha Corpus Golf Tournament, CA

9/12
Nebraska Strollathon: Virtual

9/19
Run for Rett, IL

9/26
Cincinnati Strollathon: Virtual

10/3
Rett Ride Across America

10/4
Minnesota Strollathon: Virtual

10/17
SoCal Strollathon, CA

10/24
South Carolina Strollathon, SC

10/31
Florida Strollathon: Virtual

11/7
San Antonio Strollathon, TX

Rettsyndrome.org

2019 IMPACT REPORT

Our Foundation works with researchers, clinics, pharmaceutical companies, associations, and families to relentlessly accelerate research while empowering families. Together, with your support, we will create a world without Rett syndrome. Thank you!

$49.8M INVESTED IN RESEARCH THROUGH 2019

89% OF EVERY DOLLAR FUNDS RESEARCH AND FAMILY EMPOWERMENT
**CHARITY NAVIGATOR 3-YEAR AVERAGE*

$1.7M RAISED FROM 88 EVENTS

5K NEW DONORS IN 2019

522K UNIQUE VISITS TO RETTSYNDROME.ORG

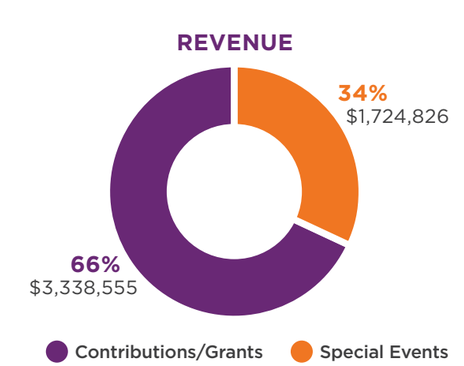
322 NEW FAMILIES JOINED RETTSYNDROME.ORG

275 INDIVIDUALS ATTENDED RSO ED DAYS

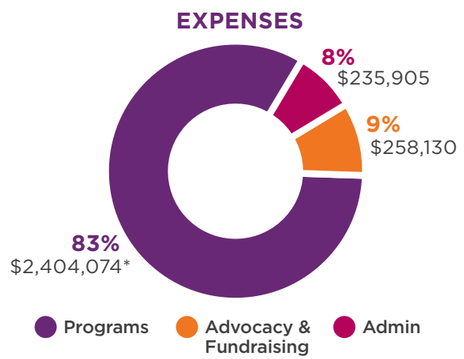
GLOBAL EDUCATION EFFORT:
2.6K INDIVIDUALS FROM 59 COUNTRIES TUNED IN TO OUR WEBCASTS

AWARDED HIGHEST RATINGS:

REVENUE



EXPENSES



**Lower than typical program expense due to timing of grant payments.*

Rettsyndrome.org
ACCELERATING RESEARCH. EMPOWERING FAMILIES.
4600 Devitt Drive | Cincinnati, OH 45246-1104

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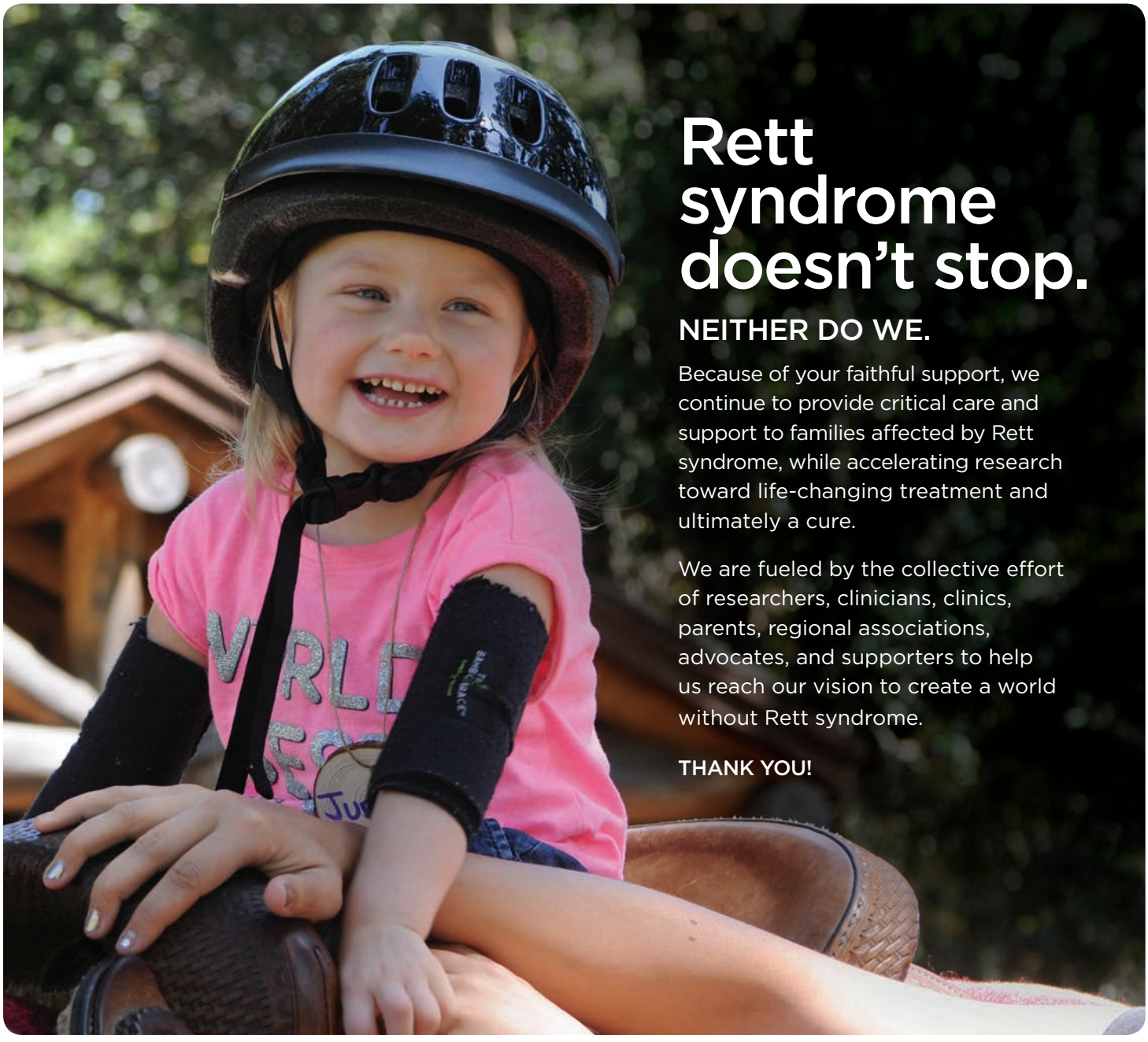

Rett syndrome doesn't stop.

NEITHER DO WE.

Because of your faithful support, we continue to provide critical care and support to families affected by Rett syndrome, while accelerating research toward life-changing treatment and ultimately a cure.

We are fueled by the collective effort of researchers, clinicians, clinics, parents, regional associations, advocates, and supporters to help us reach our vision to create a world without Rett syndrome.

THANK YOU!