

2023



Rett GAZETTE

Accelerating Research.
Empowering Families.



**HELP
FOR TODAY.**
Hope
FOR TOMORROW.



International
Rett Syndrome
Foundation

The First FDA-Approved
Treatment **p4**

Take Action
for Rett **p10**

New Family
Resources **p14**

SAVE *The* DATE!



2024 Rett Syndrome National Summit

Mark your calendar for ASCEND 2024!

Come join us in the Mile High City as we ascend to greater heights at The Westin Westminster. This location offers sweeping views of the Rocky Mountains and is conveniently situated just 15 minutes from both Denver and Boulder.

Featuring a lineup of expert speakers, updates on Rett syndrome research, and opportunities to connect with your Rett community, ASCEND 2024 is not to be missed. Look out for information on early bird registration, discounted room rates, and more later this year!

**ASCEND 2024
Rett Syndrome
National Summit**

**June 19-22, 2024
Westminster, CO**



An Incredible Year for the Rett Community

Since our last issue, we have seen an unprecedented acceleration in the Rett syndrome treatment landscape, giving us so much reason to hope. In 2023 alone, two gene therapy clinical trials began enrolling, more than 20 companies continue to invest in treatments, and IRSF investments funded nearly 40 active research projects to pave the path to new drug treatments, investigate even more genetic approaches, and ensure their success in clinical trials. Most excitingly, this year witnessed a historic milestone for the Rett syndrome community with the FDA approval of Trofinetide, the first-ever treatment for Rett syndrome. We are thrilled that parents now have a treatment option that may help their children do more and struggle less.

But this is just the first of many treatments needed. Rett is not one-size-fits-all, and with your help, we will continue to invest broadly and smartly in the most promising research at every stage.

So, as we continue to aggressively invest in research that promises to bring us closer to a world without Rett syndrome, our Foundation is proud to continue to empower families with the resources they need today to thrive. In the following pages, you'll learn how we've expanded our offering of tools to help navigate the care of a loved one with Rett, from the *My Rett Ally* web application to our new Adult Transition of Care Toolkit. We've created new ways to connect healthcare providers to our Center of Excellence network of best-in-class care clinics. And we've made it even easier to advocate for federal research funding and accessible and affordable care for your loved one.

Help for today and hope for tomorrow. We are committed to ensuring every family facing Rett syndrome has the resources they need now and the hope for a bright future. No matter where you are on your Rett syndrome journey, know that IRSF is with you until there are solutions for ALL individuals with Rett syndrome. We are ALL IN for you.

With Hope and Gratitude.

Melissa Kennedy,
Chief Executive Officer



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Scan this QR code to learn more about our Gazette features.

A First for Rett!

Trofinetide is the first-ever FDA-approved treatment for Rett syndrome.



March 10, 2023, was a milestone day for our Rett syndrome community. A few days shy of the official PDUFA action date, Acadia Pharmaceuticals announced that its investigational drug, Trofinetide, was approved by the U.S. FDA for the treatment of Rett syndrome. The FDA's approval of the drug, now known commercially in the U.S. as DAYBUE™, was broad, including all individuals with Rett — girls and boys — aged two and older with no upper limit.

This approval did not happen overnight. It represents the culmination of a journey that began more than a decade ago (see below) and succeeded thanks to the perseverance and commitment of the researchers, donors, and families who participated in every phase of the clinical trials. More than 350 girls and women participated in the clinical trials, and we cannot thank each participant enough. Your selfless sacrifice of time, energy, blood, sweat, and tears has provided our community with so much hope — hope for a better life for our children and hope that even more treatments will soon cross the finish line.

Like any drug or treatment, Trofinetide will not be effective for every individual with Rett. But it is a FIRST and paves the way for future therapies and treatments. Forty years ago, no one was investing in Rett syndrome research. Now that landscape has dramatically changed, positioning us for more breakthroughs just like this. There is so much reason to hope.

Learn more about DAYBUE (trofinetide)

For IRSF's complete list of resources, including links to our video panels with parents and physicians, scan the QR code. You and your HCP should also visit daybue.com for prescribing information, FAQs, and more.



TROFINETIDE: FROM DISCOVERY TO FDA APPROVAL



2012

Dr. Steve Kaminsky, IRSF's then-CSO, identifies the potential benefit of the compound for Rett syndrome and connects with leaders at Neuren Pharmaceuticals for further investigation.



2013

Neuren begins an adult Phase 2 clinical trial of Trofinetide (then called NNZ-2566), with nearly \$800k of funding from IRSF.



2016

Neuren begins a pediatric Phase 2 clinical trial of Trofinetide with \$1M of funding from IRSF.



2017

Neuren reports positive results from the Phase 2 clinical trial.



2018

Acadia Pharmaceuticals and Neuren enter into a North American License Agreement for the continued development and commercialization of Trofinetide.

HOPE: What Parents are Saying About this Milestone



"We realize this may not be the answer for everyone, but it gives hope to many and it brings awareness to Rett and the possibility that there will be more. More research, more trials, more treatments, and eventually a cure."

- Patty Mevis, mom to Kira

"Part of me wants to light a bonfire and have a celebratory street party for days. The other part of me wants to cry hysterically because there is a glimmer of hope. One more tool. One more next right thing to try. We never stop trying. We never stop doing the next right thing. As part of this community, we all have this in common; and this drug is our next right thing."

- Kristi Young, mom to Faith



"I've waited three decades for this moment. The hope we now have can't be measured in words. It's a momentous moment in history. I'm so grateful to [everyone] involved in the trials. The dedication and commitment to a better quality of life for families living with Rett Syndrome will be appreciated in every dose."

- Sheri Howells, mom to Chelsea



OCT 2019

Acadia begins the Phase 3 LAVENDER study in girls aged 5-20 with support from IRSF to identify clinical trial sites and promote participant recruitment.



SEPT 2021

The first patient enrolls in the DAFFODIL study of girls aged 2-5.



DEC 2021

Acadia reports positive top-line results from the LAVENDER study.



Summer 2022

Acadia submits a New Drug Application (NDA) to the FDA. The NDA is accepted and granted priority review.



MAR 2023

Trofinetide, now called DAYBUE™, becomes the first FDA-approved treatment for Rett syndrome!

The First Crucial Step to Global Access: In July, Acadia acquired the international rights to make Trofinetide available worldwide!



Clinical Trial Update

Research continues to accelerate towards our goal of having multiple treatments, including cures, for Rett syndrome. Here are some of the most promising updates since our last issue.

TAYSHA

GENE THERAPIES

Taysha Gene Therapies has initiated the Phase 1/2 clinical trial study of TSHA-102, a gene replacement therapy for adult women with Rett syndrome. The first adult woman with Rett syndrome was dosed with TSHA-102 through an injection into her spinal canal. TSHA-102 is intended to deliver a functioning copy of *MECP2* to the cells in the brain. The injection into the spinal canal includes the AAV-9 virus, which carries the *miniMECP2* gene product to the cells for the body to make more MeCP2 protein. Taysha is encouraged by the initial clinical observations from the first patient, and began screening of their second potential patient at the end of June. They will share early results from the first patient(s) quarterly.



Neurogene has announced that recruitment has begun for the clinical trial of NGN-401, a gene replacement therapy, for children with Rett syndrome. The 3 clinical trial sites are Texas Children's Hospital, Children's Hospital Colorado, and Boston Children's Hospital. This gene replacement therapy is delivered by an injection directly into a ventricle in the brain where the AAV-9 virus carries the *MECP2* gene to cells in the brain. There will be 5 females ages 4-10 enrolled.



Anavex Life Sciences has concluded a Phase 2/3 study in girls ages 5-17 with Rett syndrome in Australia, Canada, and the UK as well as a Phase 3 study in women over 18 years of age with Rett syndrome in Australia and the UK. They plan to share the results of these 2 studies in the 2nd half of 2023. Anavex has also shared data from an open-label extension study for participants in the U.S.-based Phase 2 clinical trial, which demonstrated improvement in Rett symptoms over 36 weeks of treatment. Anavex 2-73 (blarcamesine) is an activator of the sigma-1 receptor and is taken as a liquid once a day.



NeuroTech International has initiated a Phase 2 clinical trial of their medical cannabinoid product, NT1164, in females with Rett ages 5-18 in Australia.

For the most up-to-date news and status on Rett syndrome clinical trials:



\$4.4M in Rett Syndrome Research Grants Awarded

Our commitment to setting up the next wave of Rett research breakthroughs continues with the largest annual investment in IRSF's history.

At the end of 2022, the IRSF Board of Directors approved funding for an exciting array of new grants to support Rett syndrome research. Coming from scientists and researchers across the globe, this critical research will make inroads toward our goal of more drugs and genetic treatments to help our loved ones with Rett.

With over \$4 million committed this year alone (and \$58M to date!), IRSF is building on a strong foundation to change the landscape. Because Rett syndrome is a complex disorder that requires multiple approaches, we are investing in ALL the essential components that will pave the path to success: lab researchers, clinical researchers, clinicians, clinical trials, and beyond.

The investment in these projects, made possible by the many generous donors and families who believe in our Foundation's mission, will get us closer to new drug options and genetic therapies, and improve clinical trial readiness and success. We are committed to a full-spectrum approach to provide solutions for everyone living with Rett syndrome.

Our strategy is simple but powerful: *improve care today and create treatments for tomorrow.*

Learn more about this year's funded research and all research funded by IRSF:



MEET THE FUTURE OF RETT SYNDROME RESEARCH

IRSF's Independence Award is granted to current postdocs to support their early and exciting MeCP2 research as they transition to research independence. This year's recipients join an esteemed group of previous awardees, including Dr. Steven Gray whose discoveries led to Taysha's gene therapy clinical trial. We can't wait to see how these recipients will change the game too.



Sameer Bajikar, PhD
Baylor College of Medicine

Understanding the function of 2 genes misregulated due to MeCP2 mutations and testing drugs to restore the imbalance



Julian Halmaj, PhD
UC Davis

Studying a CRISPR-based strategy to restore MeCP2 through X-chromosome reactivation



Yi Liu, PhD
Whitehead Institute

Screening for drugs that can potentially fix defects due to MeCP2 mutations in RTT

2023 IRSF Rett Syndrome Scientific Meeting

At the beginning of June, nearly 200 scientists and researchers came together in Nashville for the annual IRSF Rett Syndrome Scientific Meeting. Over the course of 3 days, attendees shared their latest research advances and discussed ways to leverage learning in the lab to be rapidly deployed in the clinic.

The meeting included more than 40 speakers, including 6 distinguished keynotes and a thoughtful caregiver panel, and featured more than 75 posters covering the molecular and cellular function of MECP2, therapeutic approaches for Rett syndrome, and updates on the treatments on the horizon.



Landmark work from the lab of **Dr. Huda Zoghbi**, shown during her keynote address on day 1, identified mutations in MeCP2 as the underlying cause of Rett syndrome, a discovery that catapults the field forward to this day.



One of the meeting highlights was a **caregiver panel** on what it's like for families to live with Rett syndrome. Many researchers — who don't often get to hear firsthand from parents — shared that this session renewed their commitment to finding the treatments including cures that will impact every individual with Rett.



Dr. Nathaniel Heintz delivers his keynote to open the final session of day 1 that featured researchers discussing MeCP2 outside of its comfort zone.



During one of the evening poster sessions, **Dr. Jeffrey Neul** discusses his poster on the results of the Trofinetide open-label LILAC study with a meeting attendee.



Co-Founder of Spark Therapeutics and leader in the gene therapy field, **Dr. Katherine High**, speaks to a packed room on day 2.



Before her keynote on how data informs drug development, the FDA's **Dr. Michelle Campbell** from the CDER asks a question from the audience.



Dr. James Youakim of Acadia Pharmaceuticals, pictured in discussion during a poster session, kicked off day 3's session on treatments on the horizon for Rett. The session featured updates from 7 pharmaceutical companies actively researching Rett syndrome.



Keynote speaker **Dr. Rudolf Jaenisch** answers questions during a Q&A panel to kick off day 2.



Dr. Jeannie Lee, a day 2 keynote speaker and recipient of an Innovation Award in IRSF's most recent grant cycle, shares her insights during a meeting break.



IRSF is grateful to this year's planning committee: (pictured from L-R): Zhaolan (Joe) Zhou, PhD; Colleen Niswender, PhD; Eric Marsh, MD, PhD (not pictured): James Eubanks, PhD; Jean-Christophe Roux, PhD

"I thoroughly enjoyed the meeting because of the breadth of the science presented and exciting new developments in Rett research. I particularly enjoyed the panel discussions that provided a wonderful forum to hear from experts, discuss challenges openly, and think collectively about new ideas and possible solutions. I am grateful to the organizers for all their efforts to ensure the meeting was a great success."

- Dr. Huda Zoghbi



Make Your Voice Heard

Advocating for your loved one with Rett syndrome is easier than ever before with our new Advocacy Tool.

Now more than ever, it's critical that your elected officials hear from you and learn about Rett syndrome. Your voice — joined by other Rett parents across your state — can help mobilize officials to advocate for access to treatments and approve research funding that will bring more treatments and cures.

With our new Advocacy Tool, using your voice to take action is quick and easy! Just choose the action, enter your address, and you'll get everything you need to send your story to your state and federal officials in seconds.

Use your voice now to raise awareness and advocate for someone you love with Rett syndrome.

Visit rettsyndrome.org/advocacy or scan the QR code to get started.



Did you know?

Thanks to your recent efforts, Rett researchers received more than \$663K to advance their critical work from the FY22 DoD Bill!

Take Action for Rett!

STATE-LEVEL ADVOCACY

FDA approval is just the beginning. State-elected officials often play a role in granting access to FDA-approved treatments, ensuring the availability of care, and making sure treatments are affordable for all who need them. You can raise awareness of the needs of Rett syndrome patients in your state now.

STATE PROCLAMATIONS

Join the effort to get your state government to proclaim October as Rett Syndrome Awareness Month. Use our Advocacy Tool to write your governor today!

SUPPORT FEDERAL FUNDING FOR RESEARCH

It's critical to write your members of Congress every year to ensure that Rett syndrome is listed as a topic eligible for federal Department of Defense (DoD) research funding through the Peer Reviewed Medical Research Program (PRMRP) in the final Defense Appropriations bill. As of July 2023, Rett syndrome is listed in the FY24 Senate Defense funding report, but it is critical that it be listed in the final FY24 bill negotiated between the House and Senate. Write your members of Congress with our tool and urge them to include Rett syndrome as a topic eligible for federal funding!



In July, IRSF Family Empowerment Rep, Vanessa Peace, advocated for her daughter, Ella, and the hundreds of families in her state at the Texas Drug Utilization Review Board Meeting. During her public testimony, Vanessa shared her experience as a parent, giving the board a glimpse of Ella's daily life and sharing how hopeless she felt until now. She urged the Board to list FDA-approved treatments for Rett syndrome, like Daybue™, on the state's formulary to be covered by Medicaid. Though she only had 3 minutes to speak, her story made an impact — the Board voted in her favor!



ADVOCATES IN ACTION



This summer, a panel of experts including IRSF Board members Leslie Mehta and Mary Forrest Engel, headed to Washington, D.C. to brief members of Congress, staffers, and aides on Rett syndrome. The briefing, sponsored by U.S. Reps Brad Wenstrup (R-OH-2) and Abigail Spanberger (D-VA-7), helped raise awareness of Rett syndrome and the need to advance research to develop treatments and a cure for the disease.



In May, the Otis family joined New York Assemblyman John McDonald as he introduced a resolution to raise awareness of Rett syndrome on the Assembly floor. Rick and Alyssa Otis are strong advocates for their son with Rett syndrome, Barrett. This year, they established The Barrett Otis Research Fund for Males with Rett exclusively to support research on the growing numbers of males with Rett syndrome.

Don't forget — October is Rett Syndrome Awareness Month! This October, there are so many ways you can join IRSF to help increase awareness of Rett syndrome around the globe. More awareness means more support for families and more funding for critical research.

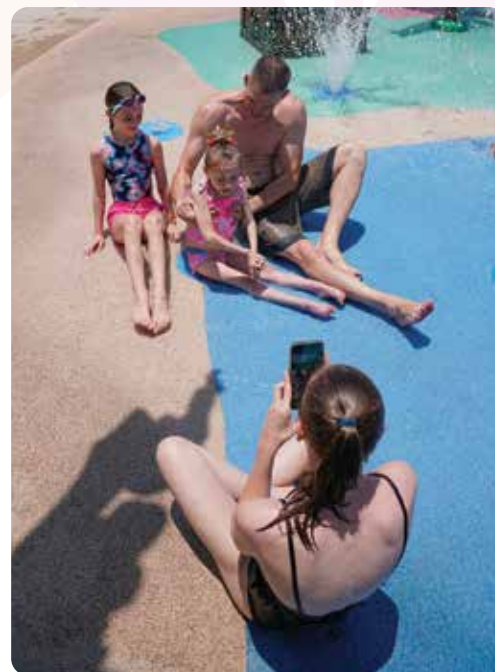
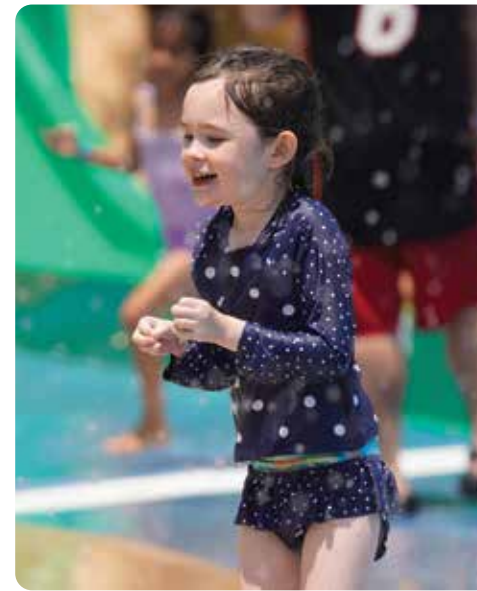
Find out more at rettsyndrome.org/October.



2023 RettAway

At the end of June, families came together for the RettAway, a destination family vacation last held in 2017. An exclusive opportunity for families to have fun together in a location that understands the challenges and joys of caring for a loved one with Rett syndrome, this year's RettAway brought families back to Morgan's Wonderland, an accessible theme park in San Antonio, Texas. While fun was had all weekend long, the true highlight of the weekend was an afternoon at Morgan's Inspiration Island Splash Experience reserved just for Rett families!

It was amazing to see the Rett community out in full force with nearly 70 families from 23 states braving the Texas heat — one of the largest gatherings of Rett families EVER!



With Families Every Step of the Way

IRSF empowers you with the resources and tools you need to thrive, no matter where you are on your Rett syndrome journey.

Rett Syndrome Registry™

Help unlock treatments including cures for Rett syndrome by participating in the Rett Syndrome Registry™. Building upon the success of the Natural History Study, the Registry is a longitudinal study of individuals with Rett syndrome and other MECP2 loss of function variant disorders. Designed together with IRSF Center of Excellence Network medical directors, this study collects data on the signs and symptoms of Rett syndrome as reported by both clinicians and the individual's caregiver.

It's easy to participate! Caregiver reports can be filled out online at each family's convenience and COE clinicians will collect the clinical data as part of normal clinic visits. There are no examinations or tests to undergo beyond the practices of current best-in-class clinical care.



Are you ready to help ensure clinical trial success, develop consensus-based care guidelines, and so much more? Contact your Center of Excellence clinic to learn more, and enroll in the Rett Syndrome Registry at rettsyndrome.org/registry.

Center of Excellence Clinic Network



18 Rett syndrome clinics across the country have been designated as IRSF Centers of Excellence, offering expert multi-disciplinary, best-in-class care specific to Rett syndrome that meets or exceeds our Foundation's rigorous requirements. Learn more about the COE designation and find a Rett clinic near you at rettsyndrome.org/COE.

myRett Trial Finder



myRett Trial Finder makes it easy to find and participate in Rett syndrome clinical trials listed on clinicaltrials.gov and beyond, matching specific criteria and location. The tool is updated as new studies begin recruiting volunteers. Get Rett Research Ready™ and view clinical trials enrolling now at rettsyndrome.org/findatrial.



Adult Transition of Care Toolkit

IRSF's new *Rett Syndrome Transition of Care Toolkit* is a must for any individual with Rett syndrome nearing adulthood. The Toolkit is designed to empower parents and guide healthcare providers through the conversations necessary to transition a loved one to adult medical care. Every journey to adulthood is different, but with this Toolkit, families can face the challenges of aging with Rett head-on thanks to the insights and learnings of Rett caregivers who have been there before. Developed by IRSF in collaboration with the Child Neurology Foundation, the Toolkit is available to download for free at rettsyndrome.org/TOCToolkit.

Life After Rett

IRSF's "Life After Rett" Facebook group is a place where anyone who has lost a child, grandchild, or family member to Rett syndrome can find support and sympathy from those who have experienced the same heartbreak and grief. Members discuss ways to celebrate and remember their loved ones as they navigate a new normal and commemorate them through continued involvement with IRSF. If you wish to join this private Facebook group, please search "Life After Rett" on Facebook.



SPOTLIGHT ON: MIDWEST RETT SYNDROME FOUNDATION

The MRSF just hosted its 2023 Moose Summer Classic for Rett Syndrome, a 16-team adult recreational charity hockey tournament. Players hit the ice in Minnesota for the tournament, all to raise funds to support Midwest families affected by Rett syndrome!

IRSF is proud to work in partnership with regional Rett syndrome associations like the MRSF who play a key role in connecting families with services, education, and more at the local level.



My Rett Ally

Your Care Companion

My Rett Ally is a better way to simplify, organize, and share your child's most important medical and care information all in one web app.

Every individual with Rett syndrome is different, and so is their medical journey. My Rett Ally, powered by mejo®, is a new tool that helps caregivers organize and track information specifically tailored to their loved one with Rett.

CREATE YOUR MEDICAL JOURNAL

- Use our guided Rett-specific questionnaire to capture the information most important to you.

GET ORGANIZED

- You'll have all of your child's most important information and documents organized in one place — no more binders!

SAFELY SHARE

- Send the right care info through text or email to medical professionals, babysitters, and anyone involved in the care of your loved one.

The My Rett Ally tool is built for caregivers, by caregivers, exclusively for Rett syndrome. Learn more at rettsyndrome.org/myrettally or scan the QR code now to get started for FREE!



Resources for Your Healthcare Providers

Not every family is able to travel to a specialized Rett syndrome clinic, but their loved one still deserves the best care. Share the educational resources below with your pediatrician, neurologist, or any professional involved in the treatment of your loved one.

Continuing Education Resources

IRSF works with experts to create educational programming for HCPs, SLPs, and any professional hoping to learn more about Rett syndrome.

CME Opportunities

Global Education Group and PlatformQ Health Education, in collaboration with NORD and IRSF, produce free CME activities throughout the year to educate caregivers on Rett syndrome. Featuring a panel of experts, families, and more, these on-demand CME opportunities cover a wide range of topics, including the criteria for diagnosis of Rett syndrome, managing symptoms, genetic testing, treatment options, and more. Learn more at rettsyndrome.org/cme and look out for new opportunities in the coming year.

Communication Professionals

Our “Communication in Rett Syndrome Program” provides professionals with strategies to help individuals with Rett syndrome reach their full potential. Guided by the research-based *Rett Syndrome Communication Guidelines*, this 3-part course provides therapists, educators, and families with information on assessment, intervention, and long-term management of communication in individuals with Rett syndrome. Learn more: rettsyndrome.org/for-communication-professionals.



Join the NACN Mailing List



The North American Clinic Network (NACN) is open to all clinicians involved in the care of someone with Rett syndrome. Group members will receive quarterly e-newsletters, featuring resources, news, educational opportunities, and more to expand their knowledge of Rett syndrome and the care of Rett patients. Join the NACN today at rettsyndrome.org/nacn.

DAYBUE™ Prescriber Group

All prescribers of DAYBUE for the treatment of Rett syndrome are invited to join a new, private group on Phlox focusing on this first-ever FDA-approved treatment for Rett. In this group, any prescriber can ask questions in the interactive Q&A feed, access resources like the epic dot phase template and sample insurance appeal packets, and collaborate with COE directors on the prescription and usage of DAYBUE by their patients. This group is built through Hive Networks to be a safe and HIPAA-compliant environment. To register and join, email Carmen Luna at cluna@rettsyndrome.org.

Natural History Study (NHS) Database



More than 1,000 individuals participated in the Rett Syndrome NHS to provide detailed information on how Rett progresses over time and affects an individual's health. With 15 years of clinical data across 7,000+ data fields, this Rett syndrome clinical database is the most comprehensive in the world. To learn how you can use this database to boost your treatment development efforts, contact us at research@rettsyndrome.org.



RETT BESTIES

Isla and Nat have been “besties” since they met at barely 12 months old, and their friendship just solidified when they discovered they are sisters in Rett. Now they are inseparable, whether they’re playing at the park, using their talkers, swimming, or even doing horse therapy! “Their bond is deep,” shared Isla’s mom, Emily. “They calm each other down and remind each other that they are never, ever alone.”



ART IS LIFE

Hannah works tirelessly all year long with her art instructor to create beautiful pieces of art that are transformed into calendars, notecards, and print items. And that hard work pays off — last December, Hannah hosted her 11th(!) annual art show in North Carolina to share her work and raise funds for IRSF!



OUR SUGAR PLUM FAIRY

One of Clara’s favorite things to do is be ON STAGE! This past year she got to be a toy in *The Nutcracker* and danced in her recital with all her dance friends. Her mom, Amanda, wants to express her gratitude to their community dance academy for being so inclusive and making Clara’s dance dreams come true!



A NEW SET OF WHEELS

Dena’s family has been working on customizing her chair to her exact needs, what they lovingly refer to as her “Cadillac.” Finally, on Dena’s 43rd birthday, she got to take the chair out for a spin! For Dena’s first ride, her niece, Aryelah, shared that “the skies cleared just for [us] so that we could take her for a walk and tilt her up so she could see the clouds and trees for the first time in years!”



HITTING THE SLOPES

The years after Gwynie’s diagnosis were difficult for her family, especially her neurotypical twin sister. Gwynie had been the dominant twin, but slowly started to fall behind and ultimately lose abilities. But this winter, Gwynie and her sister got to experience a first together: a ski trip! It was wonderful to see Gwynie ski the same slope as her sister, and her family hopes to make many more memories just like this.

MAKING WAVES

Ever since Lucy was a baby, she has always been attracted to water, but her parents have worried about having her in the pool. But when her parents learned that Lucy’s kindergarten teacher is also an experienced swimmer who gives swimming lessons to kids with different levels of abilities, Lucy was enrolled immediately! After 4 years of lessons, Lucy is a master swimmer — she can hold her breath underwater, float on her back, use her legs to kick, and even jump off the diving board for fun!



TAKING IT TO THE HILL

In May, 19-year-old Maddy visited Washington, D.C. for the first time to raise awareness of Rett syndrome. Using her eye-gaze device to communicate, Maddy spent time with the entire Colorado congressional delegation over the course of her 2-day trip, sharing her story and highlighting issues that are important to her.



Help give hope to families all over the world with Rett syndrome. Share your story at rettsyndrome.org/share.



We Are Closer Than Ever to a World without Rett

Thanks to your generosity, IRSF is funding the promising, innovative research that will get us there.

Your past financial support has allowed IRSF to execute our powerful research strategy that is focused on improving care today and creating treatments, including cures for tomorrow. Thank you!

Your generosity makes a difference every day in the lives of those with Rett and their families.

With Trofinetide becoming the first FDA-approved drug for the treatment of Rett syndrome and numerous drug companies advancing gene therapy studies, we are on the edge of more breakthroughs and more treatments. One day our sons and daughters will do more and struggle less. One day we will live in a world without Rett.

At this critical time in Rett research, would you consider making a gift?

Everything counts in our fight against Rett. For more information on ways to give, visit rettsyndrome.org/give or contact *Tim Frank* at tfrank@rettsyndrome.org.



WAYS TO GIVE:

- Make a one-time gift
- Become a monthly donor
- Ask family and friends to give in honor of your child
- Donate stocks or a car
- Host a fundraising event
- Leave IRSF a bequest in your will

ALL IN:

Using their passion to raise funds for a world without Rett.



POUND® is a high-energy workout that allows you to release your inner rock star while getting in shape. Cindy Henry is a POUND® instructor in St. Louis and created **POUND Out Rett!** to support her friend's son Gio, who has Rett syndrome.



Wrestling for Rett? Why not! Eli McCausland runs MAW Midwest All-Star Wrestling in Minnesota. He's also dad to Natalie (15) who is his daily inspiration. He combined his two loves to go ALL IN for a cure.



Friends James and Shane are bourbon aficionados. They selected a barrel of bourbon from the **Nashville Barrel Company** and had it bottled and branded in honor of Madi, who has Rett. Their fundraiser was a great success, including one bottle auctioned off at last year's Raise a Glass gala for \$26,000.



Brittany Shannon participated in Rare Disease Day on February 28 by starting a **personal page** in honor of her daughter, Brielle, who has Rett syndrome. Sharing Brielle's story with family and friends and their desire for a cure, Brittany raised over \$4,000 — much of which was doubled thanks to a matching gift!



PRE-ORDER NOW!

Reserve your copy of the new *Raising a Hand for Rett, Vol. 3* today at raisingahand.com! Proceeds from Vols 1-3 benefit IRSF and our vision of a world without Rett!

Save the Date!

Join the community this fall at a signature event near you!

Stroll for a Cure



Ready...set...STROLL! This fall, it's time to walk, run, or stroll with your community to raise crucial funds to treat and cure Rett. Since 2004, these family-friendly events powered by dedicated volunteers, sponsors, donors, and Rett families have raised more than \$15M to advance IRSF's mission to empower families and accelerate research toward a world without Rett.

This year, it's even easier to find a Strollathon near you with 13 locations to choose from:

- September 9** Nebraska & Pittsburgh
- September 10** Michigan
- September 23** Raleigh
- September 30** Philadelphia
- October 1** Minnesota
- October 7** Maryland, New Jersey & St. Louis
- October 21** So Cal
- October 22** Dallas
- November 4** Florida & Nor Cal

Register for the Stroll nearest you:
rettsyndrome.org/strollathon



Presented nationally by



Get Ready to Raise Your Glass

Raise a Glass for Rett is IRSF's newest signature event that brings the entire Rett syndrome community together on one night for Rett. Heading into its 4th year, the gala can be experienced either virtually from the comfort of your own home or at one of our exciting in-person locations across the U.S.

Raise a Glass for Rett Saturday, October 28th

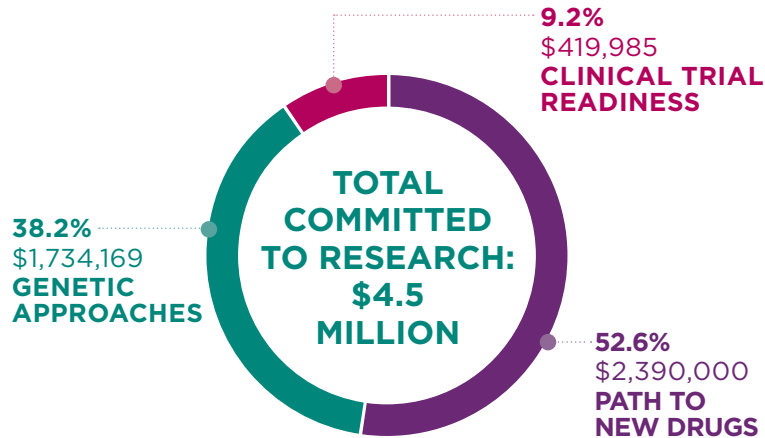
Streaming live at 8 pm ET with in-person events in Grand Rapids, Houston, and Cincinnati!

[Learn more at rettsyndrome.org/RAG](https://rettsyndrome.org/RAG)

2022 IMPACT REPORT

INTERNATIONAL RETT SYNDROME FOUNDATION is laser-focused on creating a world without Rett syndrome. We are ALL IN for a cure and families. Your generous support accelerates the best research and breakthrough discoveries in Rett syndrome while helping families thrive through IRSF resources, advocacy, and empowerment.

ACCELERATING RESEARCH



DESIGNATED 18 U.S. RETT CLINICS AS IRSF CENTERS OF EXCELLENCE

HOSTED EL-PFDD MEETING: 586 CAREGIVERS SHARED THEIR EXPERIENCE DIRECTLY WITH FDA

156 ATTENDEES AT SCIENTIFIC MEETING



43 SCIENTIFIC MEETING POSTERS

EMPOWERING FAMILIES



219 NEW FAMILIES SUPPORTED THROUGH IRSF

SUPPORTING FAMILIES FROM OVER 200+ COUNTRIES + TERRITORIES AROUND THE WORLD



599,719 UNIQUE WEBSITE VISITS

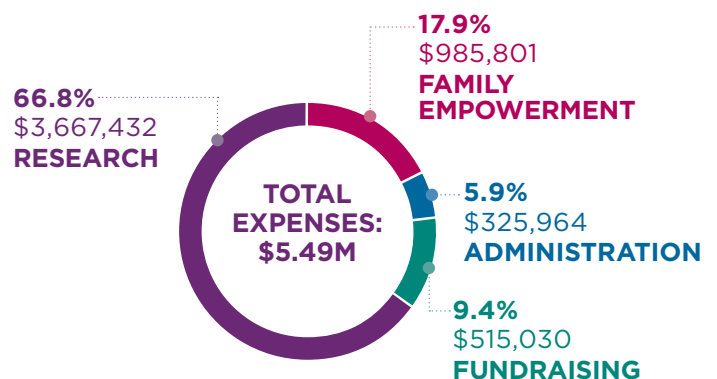
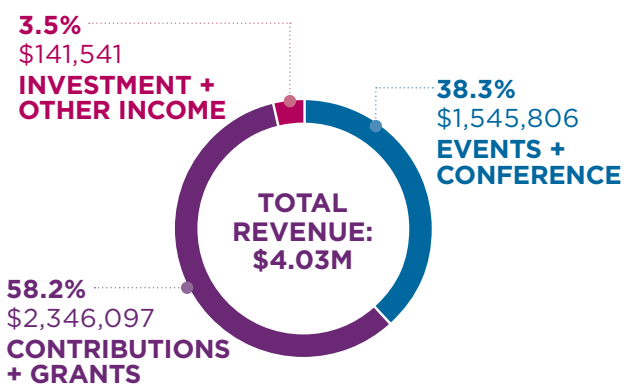


550 REGISTRANTS
MORE THAN 150 1ST-TIME ATTENDEES



COMMUNICATION CE COURSE
84 SLPS TRAINED
857 HOURS VIEWED

FINANCIALS



85% of expenses supported our core mission, and with income from investments and IRSF reserves, we committed 100% of every dollar donated to accelerating research and empowering families in 2022.



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Rett Syndrome Doesn't Stop.

NEITHER DO WE.

Because of you, we continue to provide critical care and support to families affected by Rett syndrome, while accelerating research toward life-changing treatments 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!

