



2023 IRSF Talking Points

Thanks to your generous support, the horizon is very bright. Events like this have made a significant difference in advancing Rett syndrome research toward treatments and ultimately a cure. Because of you:

- IRSF committed to fund more than **\$4.4M in research investments** to set up the next wave of Rett breakthroughs, including research that:
 - Investigates genetic treatments as curative approaches
 - Paves the path to new drugs to treat Rett syndrome
 - Ensures clinical trial readiness and success
- The FDA approved Trofinetide as the **first-ever FDA-approved treatment for Rett syndrome**, now known in the U.S. as Daybue™. IRSF invested nearly \$2M to help to fund Phase 2 adult and pediatric clinical trials of this compound and supported the Phase 3 trials through identifying clinical trial sites and promoting participant recruitment.
- The **first adult patient with Rett syndrome was dosed in a gene therapy clinical trial** in Canada, made possible by discoveries by an IRSF-funded researcher. The patient showed clinical improvements at 4-weeks post-treatment, clearing the way for the launch a pediatric study in the U.S. and U.K. soon. And **recruitment began for a gene therapy pediatric clinical trial** at 3 U.S.-based clinical trial sites that are part of IRSF's Center of Excellence clinic network.
- In 2022, 85% of expenses support our core mission, and with income from investments and IRSF reserves, we committed **100% of every dollar donated to accelerating research and empowering families**. We're proud to be a top-rated charity. (FY22)
- There are nearly **40 active Rett syndrome research projects** around the world funded thanks to IRSF donor and supporters.
- More than **20 pharmaceutical companies** are currently working on treatments that we hope could bring us even closer to a world without Rett. Fifteen years ago, there were none.
- IRSF supports a thriving Rett syndrome community, connecting **thousands of families** with the resources they need wherever they are on their Rett journey.
- Experts and families around the world have access to educational resources to help individuals with Rett syndrome thrive. Our RettEd Webinar series has been viewed by families in **more than 200 countries and territories**.
- IRSF just launched **My Rett Ally**, a web-based app to support U.S. parents and caregivers. The free tool helps families simplify, organize, and share their loved one's most important medical and care information all in one secure place.