

The International Rett Syndrome Foundation (IRSF) is the leading research, advocacy, and family empowerment organization for Rett syndrome. We're on a mission to transform the lives of all those affected by Rett syndrome by accelerating research toward treatment and a cure while empowering families with the information, programs, and services they need to thrive.

Over nearly 40 years, IRSF has invested **more than \$58 million** to fund breakthrough discoveries and life-changing advancements in research while supporting **thousands of individuals and families** around the world at all stages in their Rett journey.

Our vision is to create a world without Rett syndrome.

MISSION STATEMENT

To accelerate full spectrum research to cure Rett syndrome and empower families with information, knowledge, and connectivity.

ABOUT RETT SYNDROME

Rett syndrome is a rare genetic neurological disorder that occurs mostly in females (1 in 10,000 births), with a growing number of males being identified, and leads to severe impairments that affect nearly every aspect of life. Rett syndrome is usually recognized in children between 6 to 18 months as they begin to miss developmental milestones or lose abilities they had gained, including their ability to speak, walk, eat, and even breathe. The hallmark of Rett syndrome is near constant repetitive hand movements while awake, and individuals with Rett may experience seizures, scoliosis, breathing issues, GI issues, and more. Rett syndrome is not a degenerative disorder; individuals can live to middle age or beyond.

IRSF RESEARCH IMPACT

- Funded the research that identified the gene that causes Rett syndrome (MECP2) and proved that it can be reversed in mice.
- Funded the first gene therapy research in Rett syndrome and the first clinical trials on medications to treat Rett.
- Funded early-phase clinical trials of trofinetide, now the first-ever FDA-approved treatment for Rett syndrome known as DAYBUE™ in the United States.
- Created the infrastructure for industry and pharmaceutical partners to invest in Rett research, including the Natural History Study, Rett Syndrome Registry, and Clinical Trial Committee.
- Established a clinical network of 18 U.S. Rett Syndrome Centers of Excellence to ensure everyone with Rett has access to high-quality clinical care.

IRSF FAMILY EMPOWERMENT IMPACT

- Established a network of Family Empowerment Representatives that provide 24/7 support and individualized support for diagnosed families in every state.
- Developed a robust Rett Education program that offers families online, expert-led tutorials on various Rett-related research and care topics, reaching families in 60+ countries.
- Created the IRSF Rett Research Ready™ Program and myRett Trial Finder tool to prepare families for clinical trial involvement.
- Published the first-ever research-based Rett Syndrome Primary Care Guidelines, Rett Syndrome Communication Guidelines, and The Rett Syndrome Handbook, to enhance quality of care and help those living with Rett thrive.
- Created opportunities for families to connect, share, and learn by hosting signature events, including the family RettAway, Raise a Glass for Rett Gala, regional Strollathons, and ASCEND National Summit.

