International Rett Syndrome Foundation Celebrates FDA Approval of Trofinetide for Treatment of Rett Syndrome

CINCINNATI, OHIO, UNITED STATES, March 11, 2023 /EINPresswire.com/ -- The International Rett Syndrome Foundation (IRSF) today joined with the entire Rett syndrome community to celebrate the U.S. Food and Drug Administration (FDA) approval of DAYBUE™ (trofinetide), the first drug ever approved for the treatment of Rett syndrome. This momentous approval is the result of many things, including over a decade of IRSF's investment in research that identifies potential treatment options for Rett syndrome and the community's willingness to be active research partners.

"This is a truly historic day for the Rett syndrome community," said Melissa Kennedy, Chief Executive Officer of the International Rett Syndrome Foundation (IRSF). "We are grateful to every family that bravely participated in the clinical trials, the scientific research teams that spearheaded the development, the medical teams that served as vital partners in the clinical trials, and the donors who believed in our mission. We celebrate every individual that was involved in getting our community to this day."

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> *Melissa Kennedy, IRSF CEO*

Trofinetide was originally considered as a possible treatment for traumatic brain injury before the potential benefit was identified for Rett syndrome by former IRSF Chief Science Officer, Dr. Steve Kaminsky. Since then, IRSF has provided nearly $2 million in funding for Phase 2 clinical trials and worked closely with Trofinetide's US commercial licensee, Acadia Pharmaceuticals, to facilitate Phase 3 study enrollment and participation at IRSF's Center of Excellence network of clinical trial sites.

Bill Farnum, a former IRSF board member, and parent to a daughter with Rett, expressed his excitement for the approval of Trofinetide. "I am proud to have been on the board that supported research investment in Trofinetide, and, as a parent, I am overjoyed to see this day finally come," Farnum said. "This approval brings hope to families and represents a major step forward in the fight against 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, affecting nearly every aspect of life. Rett syndrome first becomes apparent between 6 to 18 months as children begin to miss developmental milestones or lose abilities they had gained, including their ability to speak, walk, eat, and even breathe. Trofinetide has shown promising results in clinical trials, improving breathing irregularities and reducing hand movements and body movements in Rett syndrome patients.

FDA approval is just the first step in ensuring the treatment benefits those living with Rett syndrome. IRSF is committed to partnering with families to advocate at the state and federal levels for affordable access to Trofinetide. The approval will further efforts to raise awareness for Rett syndrome and secure federal commitment to annually fund Rett research and drug development.

The approval of Trofinetide marks a pivotal moment for families and will create many questions about what approval means for those impacted by the condition. “This is a milestone moment for not only the scientific community but for every family with a loved one that has held on to hope that one day there would be a treatment for Rett syndrome,” said Dominique Pichard, MD, IRSF Chief Scientific Officer. “This is a critically important approval; however, we know that this drug will not be the answer for every individual with Rett syndrome. We must continue to champion greater pharmaceutical focus on drug development to address the variation within the entire Rett community.”

With the Rett community at the center of the foundation, IRSF is dedicated to empowering families with the resources, support, and education they need on treatments like Trofinetide and ensuring they have access to best-in-class care through IRSF’s Center of Excellence Rett syndrome clinical network. IRSF is also committed to accelerating Rett syndrome research until there are treatments including cures for all Rett syndrome patients, male and female, and with different presentations of this disease.

“Rett is not one-size-fits-all; because Rett syndrome has a broad range of presentations, we need a broad range of treatments, including cures,” Kennedy said. “With this unprecedented announcement comes an opportunity to reiterate our commitment that we will press harder for more research, more trials, and more FDA-approved therapeutics to help in overcoming Rett. Our mission of accelerating research continues until we can bring treatments to ALL individuals with Rett syndrome.”

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, affecting 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. There is no treatment currently available for Rett syndrome.

About International Rett Syndrome Foundation (IRSF)
As the leading Rett syndrome research and advocacy organization, the International Rett Syndrome Foundation (IRSF) builds upon its nearly 40-year commitment to breakthrough discoveries and life-changing advancements in research toward a cure while supporting families affected by Rett syndrome. Through its legacy foundation pioneers, IRSF has invested over $58M in research leading to identifying Rett syndrome's cause, demonstrating Rett syndrome is reversible in mice, and supporting the clinical trials that led to the first FDA-approved treatment. IRSF fights for families living with Rett syndrome and a world without it. Learn more at rettsyndrome.org.

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