



4600 Devitt Drive
Cincinnati, Ohio 45246
+1 513 874 3020 phone
+ 513 874 2520 fax
+1 800 818 RETT (7388) toll free
www.rett syndrome.org

International Rett Syndrome Foundation
4600 Devitt Drive
Cincinnati, OH 45246
1-800-818-7388
For Immediate Release- September 21, 2009
Contact: Kathryn Kissam, 804-519-6231 mobile

IRSF Announces Funding of the First Clinical Trial with a Disease Modifying Therapy for Rett Syndrome

(Cincinnati, OH) - Today, the International Rett Syndrome Foundation announced that it will provide \$200,000 in 2009 to support a newly proposed clinical trial with a growth-factor based treatment for Rett Syndrome. The study will be carried out by a team of clinical trials specialists at the Boston Children's Hospital, led by Dr. Omar Khwaja MD, PhD and will be the first potentially disease modifying therapy ever to be tested in Rett syndrome patients. Investigators will test a drug called Increlex[®]. This is an engineered form of the human protein Insulin-like Growth Factor-1, an FDA approved drug for children with short stature due to severe Primary IGF-1 deficiency. Preliminary evidence in animal models of Rett syndrome has recently suggested that targeting the IGF-1 signaling axis may provide a potential avenue for therapy.

"We are pleased to announce the funding of this groundbreaking new clinical study which presents a novel therapeutic strategy for the treatment of Rett syndrome" said Dr. Antony Horton Chief Scientific Officer at the International Rett Syndrome Foundation. "This study will be carried out by a collaborative network of leading clinicians and neuroscientists in some of the World's most prestigious research institutes at the cutting edge of biomedical research. Through our funding of this study we demonstrate our commitment to moving forward the most advanced treatments to prevent, treat and ultimately reverse Rett syndrome".

Dr. Omar Khwaja, principal investigator on the study commented further "*Since the 2007 studies which demonstrated potential reversal of Rett syndrome in animal models, there has been a paradigm shift in our thinking about ways to treat this devastating condition. Our pilot study will utilize an existing FDA approved medication, seeking to boost growth of synapses in the brain of patients with Rett syndrome*".

Increlex[®] will be provided gratis by Tercica Inc., A Subsidiary of the IPSEN Group, who have reviewed and approved the trial through their internal scientific review board.

Dr. Khwaja and his team have also secured additional funding for this clinical trial through a grant provided by Harvard University's Catalyst Pilot Awards for Clinical Translational Research

IRSF provides careful due diligence on all clinical studies involving patients with Rett syndrome since safety is of paramount importance. We accomplish this through a rigorous peer-review process, drawing on the external advice and expertise of leading clinical trials specialists in the fields of Rett syndrome, Autism, Pediatric Neurology and other relevant disciplines as appropriate.

About Rett Syndrome

Rett syndrome (RTT), a brain disorder affecting development in childhood, has been identified almost exclusively in females. RTT results in severe movement and communication problems following apparently normal development for the first six months of life. The characteristic features include loss of speech and purposeful hand use, occurrence of repetitive hand movements, abnormal walking, abnormal breathing, and slowing in the rate of head growth. Current treatment for girls with RTT includes physical and occupational therapy, speech therapy, and medication for seizures. No cure for Rett syndrome is known. In 2008, researchers heralded a major breakthrough by reversing RTT symptoms in mouse models. Rett syndrome is recognized as the "Rosetta Stone" of other neurological disorders, with genetic links to other disorders like autism and schizophrenia.

About the International Rett Syndrome Foundation

IRSF is the world's leading private funder of basic and clinical Rett syndrome research, funding over \$18M in research grants and quality research programs to date. Annually, IRSF hosts the world's largest gathering of global Rett researchers and clinicians to establish research direction and priorities while exchanging ideas and the most recent information. IRSF is the most comprehensive non-profit organization dedicated to providing thorough and accurate information about Rett syndrome, offering informational and emotional family support, and stimulating research aimed at accelerating treatments and a cure for Rett syndrome and related disorders. To learn more about IRSF and Rett syndrome, visit www.rett Syndrome.org or call IRSF at 1-800-818-RETT.

About Boston Children's Hospital

Children's Hospital Boston is home to the world's largest research enterprise based at a pediatric medical center, where its discoveries have benefited both children and adults since 1869. More than 500 scientists, including eight members of the National Academy of Sciences, 11 members of the Institute of Medicine and 12 members of the Howard Hughes Medical Institute comprise Children's research community. Founded as a 20-bed hospital for children, Children's Hospital Boston today is a 396-bed comprehensive center for pediatric and adolescent health care grounded in the values of excellence in patient care and sensitivity to the complex needs and diversity of children and families. Children's also is the primary pediatric teaching affiliate of Harvard Medical School. For more information about the hospital and its research visit: www.childrenshospital.org/newsroom.