

Press Release:
New Research Trust Dedicated to
Furthering Treatment of Rett Syndrome
Launched in the United Kingdom

July 21, 2010

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New Research Trust Dedicated to Furthering Treatment of Rett Syndrome Launched in the United Kingdom

A new non-profit organisation, the Rett Syndrome Research Trust UK (RSRT UK) launches today. Set up by the families of girls and women with Rett Syndrome, the new trust has a sole focus – namely to fund research and capitalise on recent scientific advances in order to ensure Rett Syndrome becomes the first ever reversible brain disorder. RSRT UK will work closely with a US partner, the Rett Syndrome Research Trust.

Rett Syndrome is an autism spectrum disorder which manifests in apparently healthy little girls, just as they are beginning to speak and walk. Rett Syndrome robs them of those emerging skills, stagnating their development and leaving them with a myriad of disabilities and complex medical needs for the rest of their lives. There is no cure.

But researchers know that Rett Syndrome is caused by mutations in a gene called MECP2. This gene makes a protein which is required for proper maintenance of neuronal function. Most astoundingly, recent research has demonstrated that if proper levels of the protein are restored, the symptoms of Rett Syndrome are reversed in mouse models of even late-stage disease.

The organisation's inaugural event, a gala reception is planned for November 18, 2010. The event will be held at the Wellcome Trust Headquarters in London, with guest of honour, Professor Adrian Bird of the University of Edinburgh, the world's leading expert in MECP2, Trustee and scientific advisor to the Rett Syndrome Research Trust and author of the landmark paper in the journal *Science*, which established the reversibility of Rett Syndrome in mice.

Rachael Bloom, Chairman of the Board of Trustees of RSRT UK and mother of a 14-year-old daughter who suffers with the disorder, said: "The Rett Syndrome Research Trust UK formed when a group of UK families came together with the fervent belief that parents must take an active role in the fight against Rett Syndrome. We want to see this research driven to its ultimate conclusion, replicating the results of the reversal experiments not in mice, but in girls and women living with Rett Syndrome today.

Time is not on our side. A cure that is decades away may come too late for families of adult girls, and condemns little girls, newly diagnosed, to a childhood burdened by the staggering array of neurological and orthopaedic symptoms which arise as Rett Syndrome progresses. Our partnership with the US-based Rett Syndrome Research Trust, our shared name and common goal, puts us in a prime position to aggressively impact research and the speed at which treatment can be delivered for all of our daughters, no matter where in the world they may be."

Commenting on the launch of RSRT UK, Executive Director of the US-based RSRT Monica Coenraads said: "This syndrome devastates the lives of children all over the world. The determined commitment of a global network of families is crucial if the promise of reversal is to be achieved in our lifetimes. We applaud the dedication, energy and foresight of the parents spearheading RSRT UK and look forward to working with like-minded families everywhere who share our urgency, focus and determination to reverse Rett."

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About Rett Syndrome Research Trust UK

The Rett Syndrome Research Trust UK is a newly formed charitable organisation which has been established to work in synergy with the US-headquartered Rett Syndrome Research Trust, compounding a global effort to drive research into tangible treatment for girls and women living with Rett Syndrome today.

For more information visit: ReverseRett.org.uk

About the Rett Syndrome Research Trust

RSRT is a non-profit organization with a highly focused and urgent mission: eradicate Rett Syndrome and related *MECP2* disorders. In search of a cure and effective treatment options, RSRT operates at the center of global scientific activity, funding bold projects that are unlikely to be supported by the NIH or other more traditional funding agencies. RSRT refutes the conventional practice of labs working in isolation, instead seeking out, promoting and funding collaborations and consortia in which scientists work across multiple disciplines. These relationships enable the development and execution of a research agenda that neither academia nor industry could achieve alone. Since 2008, RSRT has provided \$25 million of financial support to: 4 clinical trials testing 3 compounds, 33 scientists in 27 academic institutions and 3 biotech firms. To learn more about the Trust, please visit www.ReverseRett.org

About the MECP2 Duplication Syndrome Fund at RSRT

In an effort to leverage RSRT's deep knowledge base and well-established global scientific networks the *MECP2* Duplication Syndrome Fund at RSRT was created in late 2010. The Fund exclusively supports projects devoted to the study and means of treatment of *MECP2* Duplication Syndrome. 100% of every dollar contributed is invested in research – not a single penny goes to overhead.