

## Neuren Pursues New Drug for RTT

Pursuing the first Investigational New Drug (IND) for Rett syndrome:

Neuren Pharmaceuticals announces a program to the FDA about a proposed Rett syndrome clinical trial

- [Link to original Research Flash](#)

This month Neuren Pharmaceuticals Ltd held a pre-IND meeting with the FDA Division of Neurology Products to discuss clinical development plans for the NNZ-2566 Rett Syndrome program. The company requested the meeting to seek input and guidance from the FDA for the first clinical trial in older patients with Rett syndrome. The Investigational New Drug application is the precursory step before starting a clinical trial. Other than FDA staff, participants in the meeting included:

- Dr. Daniel Glaze, Professor, Baylor College of Medicine, and Medical Director of the Blue Bird Circle Rett Center at Texas Children's Hospital

- Dr. Jeffrey Neul, Associate Professor, Baylor College of Medicine, and Assistant Medical Director of the Blue Bird Circle Rett Center at Texas Children's Hospital

- Dr. Mike Snape, Chief Scientific Officer, Autism Therapeutics Ltd

- Dr. Steven Kaminsky, Chief Science Officer, International Rett Syndrome Foundation

- Dr. Joseph Horrigan, Assistant Vice President and Head of Medical Research, Autism Speaks

- David Zuchero, Chesapeake Regulatory Group (Neuren's US regulatory agent)

- Larry Glass, Neuren CEO

What is NNZ-2566?

NNZ-2566 is a drug that Neuren developed with the Department of Defense to treat mild Traumatic Brain Injury (mTBI) and has been shown to have great success in mTBI animal models. Neuren also tested NNZ-2566 in Rett mice and it has significant effects on lengthening neurons and maintaining synaptic connections in the Rett mice. These are very exciting studies that suggest there is a potential to increase the plasticity (or rewiring) of the Rett brain to reverse Rett symptoms.

Why is this exciting for the Rett community?

Neuren Pharmaceuticals has joined with the Baylor College of Medicine to test NNZ-2566 in a proposed Phase I and Phase IIa clinical trial. This proposed trial, along with the IGF-1 trial at the Children's Hospital in Boston, are trials designed to investigate whether these compounds can reverse some of the symptoms of Rett syndrome and modify the progression of the disorder in our Rett children. This announcement portrays the importance of partnerships between industry, academia, and private foundations like IRSF to accelerate bringing forward clinical trials specific to Rett syndrome.

For more information concerning their announcement please see the Neuren press release at [neurenpharma.com](http://neurenpharma.com).

If you have questions about this program please contact Paige Nues, Director of Family Support, or Jennifer Endres, Family Resource Manager, at The International Rett Syndrome Foundation (email: [admin@rettsyndrome.org](mailto:admin@rettsyndrome.org)).

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