

## Neuren holds pre-IND meeting on proposed Rett Syndrome clinical trials with FDA

**SYDNEY, Australia, 16 May 2012:** Neuren Pharmaceuticals Limited (ASX:NEU) announced today that it held a pre-IND meeting with the FDA Division of Neurology Products to discuss clinical development plans for the study of its lead drug NNZ-2566 in the treatment of Rett Syndrome. The Company requested the meeting to seek input and guidance from the FDA for the first clinical trial in patients with Rett Syndrome. In addition to FDA staff, represented at the meeting were:

- The Blue Bird Circle Rett Center at Texas Children's Hospital and Baylor College of Medicine by Dr Daniel Glaze, Professor and Medical Director, and Dr Jeffrey Neul, Associate Professor and Assistant Medical Director
- Autism Therapeutics Ltd by Dr Mike Snape, Chief Scientific Officer
- the International Rett Syndrome Foundation by Dr Steven Kaminsky, Chief Science Officer
- Autism Speaks by Dr Joseph Horrigan, Assistant Vice President and Head of Medical Research; and
- Neuren by Larry Glass, CEO.

Commenting on the meeting, Neuren CEO Larry Glass said: "We feel that the meeting was very productive in that we were able to explore with the FDA key, substantive issues affecting our regulatory and clinical development strategy. This will provide clarity as we move forward to finalize the protocols, submit the IND and prepare for the first trials in patients. The scientific and medical insight provided by Drs Glaze and Neul, who will lead the initial clinical studies, contributed immeasurably to what we feel was a very positive meeting. We are particularly appreciative of the willingness of the International Rett Syndrome Foundation and Autism Speaks to attend the meeting as advocates for patients and families affected by Rett Syndrome. "

Neuren plans to refine the clinical protocol and submit the IND within the next 60 days. Pending FDA review of the IND and completion of the ongoing Phase 1 trial of the NNZ-2566 oral formulation, we intend that this first Rett Syndrome trial will be initiated later this year. The initial trial will be designed to assess the safety and tolerability of NNZ-2566 in adolescent and adult Rett Syndrome patients and to obtain preliminary evidence of efficacy.

Families and caregivers with questions about this program are encouraged to contact the International Rett Syndrome Foundation (email: [admin@rettsyndrome.org](mailto:admin@rettsyndrome.org)).

### About Rett Syndrome

Rett Syndrome is a post-natal neurological disorder which occurs almost exclusively in females following apparently normal development for the first six months of life. Typically, between 6 to 18 months of age, patients experience a period of rapid decline with loss of purposeful hand use and spoken communication. Many patients have recurrent seizures. They experience a variety of motor problems including increased muscle tone (spasticity) and abnormal movements. They are never able to provide for their own needs. It is a

rare disorder and is believed to be second only to Down Syndrome as a cause of chronic neurological problems that include severe communication, motor disabilities and epilepsy. Rett Syndrome is caused by mutations on the X chromosome on a gene called MECP2. There are more than 200 different mutations found on the MECP2 gene. Rett Syndrome strikes all racial and ethnic groups, and occurs worldwide in 1 of every 10,000 to 23,000 female births.

#### **About the Blue Bird Circle Rett Center at Texas Children's Hospital**

The Blue Bird Circle Rett Center at Baylor College of Medicine is a multidisciplinary team of health professionals and involved in three major activities: care-giving, education, and research. The Rett Center at BCM is one of the largest Rett Centers in the United States and one of the few centers that specializes in the diagnosis and care of children and adults with Rett Syndrome. Since its founding in 1985, the Center has cared for more than 800 patients and is running the Rett Syndrome Natural History Study, an NIH-funded effort and the largest study of Rett Syndrome patients ever undertaken.

#### **About the International Rett Syndrome Foundation**

The International Rett Syndrome Foundation (IRSF) is a 501(c)(3) non-profit corporation established in July 2007 through the strategic merger of the Rett Syndrome Research Foundation and the International Rett Syndrome Association. The core mission of the IRSF is to fund research for treatments and a cure for Rett Syndrome while enhancing the overall quality of life for those living with Rett Syndrome by providing information, programs, and services. IRSF is the largest private source of funds for biomedical and clinical research on Rett Syndrome. IRSF believes the fastest way to a cure and treatments includes making significant and strategic investments in research while bolstering family support, public awareness, and advocacy programs. The goal of IRSF is to not only fund the highest quality research, but also to focus on projects that will accelerate the speed with which basic science discoveries are translated into clinical application for patients with Rett Syndrome.

#### **About Autism Speaks**

Autism Speaks was founded in 2005 and, since then, has grown into the nation's largest autism science and advocacy organization, dedicated to funding research into the causes, prevention, treatments and a cure for autism; increasing awareness of autism spectrum disorders; and advocating for the needs of individuals with autism and their families.

#### **About Neuren**

Neuren Pharmaceuticals is a biopharmaceutical company developing new therapies for brain injury, autism spectrum disorders, chronic neurological diseases and cancer. Neuren presently has two clinical-stage molecules, NNZ-2566 and Motiva®, in Phase 2 clinical trials largely funded by the US Army and the National Health and Medical Research Council, respectively. Through its subsidiary, Perseis Therapeutics Limited, Neuren is developing monoclonal antibodies against Trefoil Factors 1 and 3, proteins produced by cancer cells that are associated with cancer spread and reduced patient survival.

#### **For more information, please contact:**

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