

Neuren completes NNZ-2566 Oral Phase I safety study and files IND for Rett Syndrome

SYDNEY, Australia, 22 November 2012: Neuren Pharmaceuticals (ASX:NEU) is pleased to announce the successful completion of a Phase I study of the oral formulation of NNZ-2566 in healthy subjects. As previously announced, the Phase I protocol was amended to enable dosing at a higher level than originally envisioned. The study was completed successfully and the oral solution of NNZ-2566 appears to be safe and well-tolerated when administered at the highest dose in the Phase I trial (100 mg/kg twice daily for five days).

The availability of an oral formulation opens the way for a number of studies in autism spectrum and neurodevelopmental disorders and brain injury. The first of these will be a safety and efficacy study of NNZ-2566 in adolescents and adults with Rett Syndrome. Neuren has today filed an Investigational New Drug (IND) application for this study and patient screening and enrolment will begin following approval by the FDA and the clinical study site Institutional Review Board (IRB). The proposed dose regimen in this Phase II protocol builds on the apparent safety profile from the Phase I study with a top dose well above that initially envisioned. The IND is expected to be open 30 days from submission.

The lead clinical site for the Phase II study will be the Blue Bird Circle Rett Center at Texas Children's Hospital and the Baylor College of Medicine which will be led by Daniel Glaze, MD, and Jeffrey Neul, MD, PhD, Director and Assistant Director, respectively. An application to initiate the trial also has been submitted to the Texas Children's Hospital Institutional Review Board. Patient screening and enrolment will begin pending approval by the FDA and the Texas Children's IRB. Part of the cost of the Phase II trial in Rett Syndrome patients is being underwritten by a grant from the International Rett Syndrome Foundation to Baylor.

Commenting on submission of the IND, Joe Horrigan, MD, Vice President of Clinical Development and Medical Affairs, said: "We are very pleased to submit this application which represents the culmination of tremendous effort by so many individuals who are committed to improving the quality of life for patients with Rett Syndrome and their families."

Once the IND for NNZ-2566 in Rett Syndrome is open, it will support additional studies of the oral solution in other autism spectrum and neurodevelopmental disorders. Neuren plans to initiate a Phase II trial in paediatric patients with Rett Syndrome in 2013 and is planning studies in additional disorders.

The completed Phase I study of NNZ-2566 oral also supports initiation of the Phase II study in concussion for which there already is an open IND in place. The concussion study is part of the clinical studies program supported by the US Army and will make NNZ-2566 the only proprietary drug in development for all TBI patients irrespective of the severity of the injury. Concussion or mild TBI is a significant problem in both civilian and military populations including athletes.

About NNZ-2566

NNZ-2566 is a synthetic analogue of a naturally occurring neuroprotective and neurotrophic molecule derived from IGF-1, a growth factor produced by brain cells as well as in other parts of the body. The intravenous form of NNZ-2566 is presently in a Phase II clinical trial in patients with moderate to severe traumatic brain injury which has received Fast Track designation from the US FDA. The company is currently undertaking final preparations to initiate two additional Phase II trials with the oral form of NNZ-2566 – one in patients with concussion or mild TBI and one in patients with Rett Syndrome.

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 of 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 up to 1 of every 10,000 female births and affects some 15,000 girls and women in the U.S. alone.

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 Neuren

Neuren Pharmaceuticals is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders 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.

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