

US IND for Neuren's Phase II trial of NNZ-2566 in Rett Syndrome opened

SYDNEY, Australia, 24 December 2012: Neuren Pharmaceuticals Ltd (ASX:NEU) is pleased to announce that the US Food and Drug Administration has informed the company that it may proceed with the Phase II clinical trial to assess the safety and efficacy of NNZ-2566 oral solution in adolescents and adults with Rett Syndrome.

Following a discussion with the FDA, the dosing period for this initial study in patients with Rett Syndrome will be 14 days. The study will utilize a randomized, double-blind, placebo-controlled design. There will be a low dose and a high dose NNZ-2566 cohort in the study; the high dose cohort will be initiated following completion of the low dose cohort and review by the independent Data and Safety Monitoring Committee. A total of 60 subjects will be randomized 2:1 to NNZ-2566 versus placebo. Assessments will include pharmacokinetics, safety and tolerability, objective physiological measurements such as EEG and cardiac and respiratory function as well as standardized evaluations of symptom severity and global functional status. The study is expected to be completed within approximately one year. Patient screening and enrollment are planned to begin in January 2013.

Commenting on the FDA's decision, Larry Glass, CEO, said: "We are very pleased with this outcome and welcome the opportunity to begin our assessment of whether NNZ-2566 will provide meaningful benefit for Rett Syndrome patients and their families. We are most grateful for the contributions of the many medical and scientific experts and advocates who have made this possible as well as for the advice and guidance provided by the FDA."

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 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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