

Neuren completes enrolment in Phase 2 trial of NNZ-2566 in Rett Syndrome

Melbourne, Australia, 26 June 2014: Neuren Pharmaceuticals (ASX: NEU) announced today that enrolment of subjects in its Phase 2 clinical trial of NNZ-2566 in Rett Syndrome has been completed. This will enable top-line results from the trial to be released by Neuren in the 4th quarter of 2014, as planned.

The double-blind, placebo-controlled trial, initiated in April 2013, is the first commercial multi-site clinical trial in Rett Syndrome, for which currently there is no approved therapy available. The trial has been supported by the International Rett Syndrome Foundation (IRSF) and will be featured at the 2014 IRSF Symposium in the Washington D.C. area, June 24th-26th.

Two dose levels of orally administered NNZ-2566 are being tested in female subjects aged 16 to 45 years. The trial duration for each subject is approximately ten weeks, including the screening and follow-up periods. To date, 47 subjects have completed the trial, with 7 more in progress. The total number of subjects completing the trial therefore may be up to 54.

As well as the primary endpoint of safety and tolerability, a number of different measures will be analysed for signs of clinical efficacy. To date there have been four meetings of the independent Drug Safety Monitoring Committee and no safety concerns have been identified.

Neuren Executive Chairman Richard Treagus commented; "We are pleased to be able to announce that our three clinical trial sites have successfully completed the enrolment of subjects by 30 June as planned. We now look forward to completing the trial and analysing the data to assess the potential for NNZ-2566 to help Rett Syndrome sufferers and their physicians."

About Rett Syndrome

Rett Syndrome is a post-natal neurological disorder that 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. These individuals 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 genetically-determined cause of chronic neurological problems in females 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 approximately 1 in every 10,000 live female births.

About NNZ-2566

NNZ-2566 is a synthetic analogue of a naturally occurring neurotrophic peptide derived from IGF-1, a growth factor produced by brain cells. In animal models, NNZ-2566 exhibits a wide range of important effects including inhibiting neuroinflammation, normalising the role of microglia and

correcting deficits in synaptic function. In the Fragile X model, these actions resulted in statistically significant improvement in all core anatomic and behavioural features of the disorder that were assessed. NNZ-2566 is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The intravenous form of NNZ-2566 is presently in a Phase 2 clinical trial in patients with moderate to severe traumatic brain injury. The oral form of NNZ-2566 is in Phase 2 trials in Rett Syndrome and Fragile X Syndrome. All three programs have received Fast Track designation from the US FDA and the Fragile X Syndrome program has also received Orphan Drug designation. Neuren intends to implement a Phase 2 clinical trial with the oral form of NNZ-2566 in patients with concussion (mild traumatic brain injury).

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a publicly listed biopharmaceutical company focusing on the development of new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions such as Rett Syndrome and Fragile X Syndrome as well as acute neurological injuries. Neuren presently has a clinical stage molecule, NNZ-2566 in three Phase 2 clinical trials as well as NNZ-2591 in pre-clinical development.

Forward-looking Statements

This ASX-announcement contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks and important factors that may cause the actual results, performance or achievements of Neuren to be materially different from the statements in this announcement.

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