

Neuren to release results from Rett Syndrome Phase 2 trial in November 2014

Melbourne, Australia, 24 September 2014: Neuren Pharmaceuticals (ASX: NEU) announced today that it expects to release top-line results from its Phase 2 clinical trial of NNZ-2566 in Rett syndrome in November 2014. 53 subjects have now completed the double-blind, placebo controlled trial, which evaluates orally administered treatment with NNZ-2566 for up to one month in adolescent and adult females (ages 16-45 years).

Neuren's trial is the first multi-site, sponsor-led clinical trial in Rett syndrome, for which there are currently no approved medicines. Rett Syndrome is a severe neurological disorder caused by mutations of the *MeCP2* gene on the X chromosome. The disorder has an onset in early childhood and is often progressive into adolescence and adulthood.

The primary endpoint for the trial is the safety and tolerability of two dose levels of NNZ-2556 as compared to placebo. However the trial is also enriched with a number of outcome measures that provide insight into efficacy and seek to establish whether there is a pattern of clinical benefit evident from treatment with NNZ-2566. These outcome measures can be classified into four "Efficacy Domains":

- 1) Rating scales specific to Rett Syndrome signs and symptoms (e.g. Motor Behavior Assessment and Clinical Severity Scale), completed by the clinician;
- 2) Global assessments keyed to the specific signs and symptoms of Rett Syndrome (e.g. Clinical Global Impression of Improvement), completed by the clinician;
- 3) Rating scales keyed to specific Rett Syndrome signs and symptoms (e.g. Top 3 Concerns) and general rating scales (e.g. Aberrant Behavior Checklist) completed by caregivers; and
- 4) Physiological measures (e.g. assessments that evaluate breathing abnormalities).

The clinical responses compared to baseline in the four domains will be examined for each subject individually, as well as for each treatment group as a whole. The analysis of the results will determine, for individuals and for the group, whether there is a systematic pattern of benefit from treatment with NNZ-2566.

"We are grateful to the patients and families affected by Rett Syndrome that have made this ground-breaking clinical trial possible" commented Neuren Executive Chairman, Richard Treagus. "Neuren is approaching a very important milestone in November, which has the potential to materially increase the value of NNZ-2566. If the trial results establish a clinical benefit in Rett Syndrome, we will move quickly to apply to the FDA for Orphan Drug designation and potentially for Breakthrough Therapy designation".

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 clinical decline that stabilizes later in life. 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. There are approximately 20,000 females with Rett Syndrome in the United States and more than 50,000 worldwide.

The young women enrolled in Neuren's trial are in the late stage of the disease (Stage 4) which is characterized by an inability to speak, walk and use one's hands. Additional signs and symptoms in young women with Rett Syndrome include autonomic dysfunction (which can affect the rate and rhythm of the heart), breathing abnormalities, seizures, abnormal curvature of the spine, irritability, unusual behaviors such as prolonged bouts of crying and screaming, and muscle rigidity or spasticity. On the whole, older individuals with Rett Syndrome tend to have more severe symptoms and a greater degree of overall debilitation relative to children with this disorder.

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, normalizing the role of microglia and correcting deficits in synaptic function. 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, Fragile X Syndrome and mild traumatic brain injury (concussion). Three programs have received Fast Track designation from the US FDA and the Fragile X Syndrome program has also received Orphan Drug designation.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company focusing on the development of new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions as well as acute neurological injuries. Neuren presently has a clinical stage molecule, NNZ-2566 in four 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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