Neuren (NEU) - ASX Announcement **13 October 2015**

Rettsyndrome.org confirms financial support for Neuren's clinical trials of trofinetide

Melbourne, Australia, 13 October 2015: Neuren Pharmaceuticals (ASX: NEU) today announced that the International Rett Syndrome Foundation (Rettsyndrome.org) has made a financial commitment of up to US$1 million, to continue its support of Neuren's clinical trials of trofinetide for the treatment of Rett syndrome.

Rettsyndrome.org previously supported Neuren's Phase 2 clinical trial in adults and adolescents with Rett syndrome, which successfully demonstrated clinical benefit from treatment with trofinetide. The benefit observed in the trial encompassed many of the core symptoms of Rett syndrome and was observed in both clinician and caregiver assessments. Neuren has since received orphan drug designation in the United States and the European Union for trofinetide in Rett syndrome.

The US Food and Drug Administration (FDA) has granted Fast Track designation for the development program in the United States. Neuren recently met with the FDA to discuss the remaining development requirements, which provided meaningful guidance for Neuren in all areas of the development program.

In 2016, Neuren will conduct a pediatric clinical trial in which higher doses of trofinetide will be tested in children below the age of 16 with Rett syndrome. The pediatric trial will enable Neuren to confirm the optimum dose levels for the subsequent Phase 3 trial, as well as generating information on the treatment of children and younger adolescents. Neuren is working with the FDA to agree on the Phase 3 study design that will support potential approval of a New Drug Application in the United States.

"We are delighted to continue Neuren's partnership with Rettsyndrome.org and work together to accelerate the development of trofinetide for Rett syndrome", commented Neuren's Chief Science Officer, Larry Glass. "To date, trofinetide has demonstrated an excellent safety and tolerability profile, with evidence of a pattern of clinical benefit across the broad phenotype of Rett syndrome, which is consistent with its normalising effects on brain biology".

Dr Steven Kaminsky, Chief Science Officer of Rettsyndrome.org, commented: "This is a very exciting time for us at Rettsyndrome.org. The work by Neuren provides one of the brightest opportunities to change the quality of life for those suffering with Rett syndrome. We are hopeful that the upcoming clinical trials are successful and lead to new avenues for treatment of Rett syndrome as well as for other synaptic disorders of the central nervous system."

About trofinetide

Trofinetide is a synthetic analogue of a naturally occurring neurotrophic peptide derived from IGF-1, a growth factor produced by brain cells. In animal models, trofinetide exhibits a wide range of important effects including inhibiting neuroinflammation, normalizing the role of microglia and correcting deficits in synaptic function. trofinetide is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The intravenous form of trofinetide is presently in a Phase 2 clinical trial in patients with moderate to severe traumatic brain injury. The oral form of trofinetide is in Phase 2 development 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 Rett syndrome and Fragile X syndrome programs have also received Orphan Drug designation in the United States and the European Union.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing 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, trofinetide in 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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