



Neuren (NEU) - ASX Announcement

14 August 2015

“trofinetide” confirmed as the International Nonproprietary Name (INN) for Neuren’s NNZ-2566

Melbourne, Australia, 14 August 2015: Neuren Pharmaceuticals (ASX: NEU) today announced that in the World Health Organization’s latest publication of International Nonproprietary Names for Pharmaceutical Substances (INN), **trofinetide** has been confirmed as the INN for glycyl-2-methyl-L-prolyl-L-glutamic acid, which was previously designated by Neuren as NNZ-2566.

Neuren is advancing the development of trofinetide as a novel therapy for a range of neurological conditions with unmet need. Phase 2 clinical development is in progress for Rett syndrome, Fragile X syndrome, moderate to severe traumatic brain injury and concussion. Three of these programs have received Fast Track designation from the US Food and Drug Administration (FDA) and the Rett syndrome and Fragile X syndrome programs have also received Orphan Drug designation in the United States and the European Union.

Neuren expects to announce top-line results in December 2015 from its Phase 2 trial of trofinetide in Fragile X syndrome and is continuing with preparations for the remaining development in Rett syndrome, following its recent meeting with the FDA.

About International Nonproprietary Names

Since its inception, the aim of the INN system has been to provide health professionals with a unique and universally available designated name to identify each pharmaceutical substance. The existence of an international nomenclature for pharmaceutical substances, in the form of INN, is important for the clear identification, safe prescription and dispensing of medicines to patients, and for communication and exchange of information among health professionals and scientists worldwide. The final stage of the selection process is the recommended INN. Once a name has been published as a recommended INN it will not normally be modified further and is ready for use in labelling, publications, on drug information. It will serve to identify the active pharmaceutical substance during its life-time worldwide.

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. In November 2014, Neuren announced top-line results from its Phase 2 clinical trial in 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.

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 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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