



Neuren (NEU) - ASX Announcement

16 February 2015

Neuren receives Orphan Drug designation from FDA for trofinetide in Rett syndrome

Melbourne, Australia, 16 February 2015: Neuren Pharmaceuticals (ASX: NEU) today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug designation to Neuren's drug trofinetide for treatment of Rett syndrome.

Orphan Drug designation is a special status that the FDA may grant a drug intended to treat a rare disease or condition. Orphan Drug designation qualifies the sponsor of the drug for 7 years of marketing exclusivity following marketing authorisation. The FDA granted Orphan Drug designation to Neuren for trofinetide in Fragile X syndrome in October 2013.

Neuren has commenced the process of Orphan Drug applications to the European Medicines Agency for trofinetide in both Rett syndrome and Fragile X syndrome. Orphan Drug designation in the European Union qualifies the sponsor of the drug for 10 years of marketing exclusivity following marketing authorisation.

"This further validates a key part of our strategy and the grant of Orphan Drug designation for Rett syndrome is another important commercial milestone for Neuren", commented Neuren Executive Chairman, Richard Treagus. "The marketing exclusivity periods that apply to Orphan Drugs in the United States and Europe are extremely valuable in relation to the potential commercialisation of trofinetide."

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. An application for Breakthrough Therapy has been submitted to the FDA. Neuren expects to meet with the FDA in the first half of 2015 to discuss the remaining requirements for the development of trofinetide in Rett syndrome.

Responding to the growing interest in its key clinical programs, Neuren is releasing a video on its website www.neurenpharma.com that describes the mechanism of action of trofinetide.

About trofinetide (proposed INN)

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.

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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