



Neuren (NEU) - ASX Announcement

2 March 2015

Neuren receives FDA response to request for Breakthrough Therapy

Melbourne, Australia, 2 March 2015: Neuren Pharmaceuticals (ASX: NEU) today announced that the US Food and Drug Administration (FDA) has responded to Neuren's request for Breakthrough Therapy designation for trofinetide to treat Rett syndrome. The FDA agreed that Rett syndrome meets the criteria for a serious or life-threatening condition. However, Breakthrough Therapy will not be granted at this time because the FDA considered that data from Neuren's exploratory Phase 2 trial provide insufficient evidence to demonstrate substantial improvement over existing therapies using conventional statistical methods.

Neuren will proceed as planned under the Fast Track designation that has already been granted by the FDA under its expedited programs for serious conditions. Neuren expects to meet with the FDA in the first half of 2015 to agree the remaining requirements for the development of trofinetide in Rett syndrome.

"We maintain that Neuren submitted a strong case for Breakthrough Therapy. Nevertheless we look forward to meeting with the FDA and advancing the development of trofinetide for Rett syndrome under the Fast Track expedited program", commented Neuren Executive Chairman, Richard Treagus.

The FDA recently granted Orphan Drug designation to trofinetide for Rett syndrome and Neuren has commenced the process of an Orphan Drug application to the European Medicines Agency.

About Orphan Drug, Fast Track and Breakthrough Therapy designations

Orphan Drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition. Amongst other incentives, Orphan Drug designation qualifies the sponsor of the drug for seven years of marketing exclusivity and various development incentives including waiver of the prescription drug user fee for a marketing application.

A drug may be designated as a Fast Track product if it is intended for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. Fast Track designation is intended to facilitate development and expedite review of drugs to treat serious and life-threatening conditions so that an approved product can reach the market expeditiously.

Breakthrough Therapy is intended to streamline drug development and regulatory review of innovative new medicines that address unmet medical needs for serious diseases or conditions. The criteria for Breakthrough Therapy require preliminary clinical evidence indicating that the drug may demonstrate a



substantial improvement over existing therapies on at least one clinically significant endpoint. Breakthrough Therapy designation conveys all of the Fast Track program features, as well as a commitment that FDA will work closely with the sponsor on an efficient drug development program.

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. 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 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 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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