



**Neuren (NEU) - ASX Announcement**

**13 August 2015**

## **Neuren confirms orphan drug designation in Europe for Rett syndrome and Fragile X syndrome**

**Melbourne, Australia, 13 August 2015:** Neuren Pharmaceuticals (ASX: NEU) today reported that the European Commission has formally adopted the decision to grant Orphan Designation to Neuren's trofinetide for both Rett syndrome and Fragile X syndrome in the European Union (EU).

Orphan designation in the EU enables sponsors to benefit from a number of incentives, including 10 years of market exclusivity once the medicine is on the market. During that exclusivity period, the EMA and the EU Member States shall not accept another application for a marketing authorisation, for the same therapeutic indication, in respect of a similar medicinal product.

Neuren's programs for Rett syndrome and Fragile X syndrome have previously been granted Orphan Drug designation by the US Food and Drug Administration (FDA), which provides incentives including 7 years of market exclusivity in the United States.

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 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 the United States and the European Union. 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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