



**Neuren (NEU) - ASX Announcement**

**7 April 2016**

## **First subject commences Neuren's pediatric Rett syndrome Phase 2 trial**

**Melbourne, Australia, 7 April 2016:** Neuren Pharmaceuticals (ASX: NEU) today announced that the first subject has commenced its Phase 2 clinical trial of trofinetide in children and adolescents with Rett syndrome. Neuren's program is receiving valuable support from rettsyndrome.org, including grant funding towards the cost of this pediatric trial.

The randomized, double-blind, placebo-controlled, dose-ranging study in subjects aged 5 to 15 years is being conducted at up to 12 sites across the United States, led by clinicians experienced in Rett syndrome including the experts who led Neuren's first trial in adolescents and adults. Neuren plans to enroll approximately 64 subjects and to complete the trial by the end of 2016.

The trial will evaluate the safety, efficacy and pharmacokinetics of three dose levels of trofinetide, 50mg/kg, 100mg/kg and 200mg/kg each taken orally twice daily. Efficacy outcome measures include the Motor Behavior Assessment, a measure that captures key clinical symptoms of Rett syndrome. The trial duration for each subject including follow-up is approximately 10 weeks.

Neuren's previous Phase 2 clinical trial demonstrated clinical benefit from treatment with trofinetide in subjects aged 16 to 45 years at a dose level of 70mg/kg twice daily. The current trial will evaluate the safety and efficacy of trofinetide in a younger age group, at higher doses and for a longer duration of treatment, as well as confirming the optimum dose levels for the subsequent Phase 3 trial in children, adults and adolescents.

Neuren has received Orphan Drug designation in the United States and the European Union for trofinetide in Rett syndrome.

Neuren Executive Chairman Richard Treagus commented: "We are excited as we take this next step in the development of trofinetide as a potential treatment for Rett syndrome. We are very grateful to rettsyndrome.org and to the families whose participation and support make this trial possible."

### **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 Phase 2 development for 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 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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