



**Neuren (NEU) - ASX Announcement**

**5 October 2017**

## **Presentations and publication for Neuren's Rett syndrome clinical trials**

**Melbourne, Australia, 5 October 2017:** Neuren Pharmaceuticals (ASX: NEU) today announced upcoming presentations and a publication for its two completed clinical trials of trofinetide in Rett syndrome.

Poster presentations of Neuren's latest Phase 2 trial in girls aged 5 to 15 years, titled "Trofinetide, a novel IGF-1 related treatment, demonstrates efficacy for children and adolescents with Rett syndrome", will be given at three conferences in October 2017:

- The Child Neurology Society Annual Meeting in Kansas City, 4-7 October, presented by Alan Percy MD.
- The Rare Disease and Orphan Products Breakthrough Summit of the National Organization for Rare Disorders in Washington D.C., 16-17 October, presented by Walter Kaufmann MD.
- The American Academy of Child & Adolescent Psychiatry Annual Meeting in Washington D.C., 23-28 October, presented by Walter Kaufmann MD.

Neuren's previous Phase 2 trial in women aged 15 to 45 years has been published in the journal *Pediatric Neurology* (DOI: <http://dx.doi.org/10.1016/j.pediatrneurol.2017.07.002>). The article, co-authored by Daniel G. Glaze MD et al, is titled "A Double-Blind, Randomized, Placebo-Controlled Clinical Study of Trofinetide in the Treatment of Rett Syndrome".

### **About Neuren and trofinetide**

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. Neuren presently has trofinetide in Phase 2 clinical trials as well as NNZ-2591 in pre-clinical development. 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, correcting deficits in synaptic function and regulating oxidative stress response. The most advanced trofinetide program is for Rett syndrome, supported by [rettsyndrome.org](http://rettsyndrome.org). Both the Rett syndrome and Fragile X syndrome programs have been granted Fast Track designation by the US Food and Drug Administration and have Orphan Drug designation in both the United States and the European Union.



*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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