



Neuren (NEU) - ASX Announcement

13 October 2017

Neuren confirms Phase 3 plan for Rett syndrome at FDA Meeting

Melbourne, Australia, 13 October 2017: Neuren Pharmaceuticals (ASX: NEU) today announced that at its End of Phase 2 Meeting, the US Food and Drug Administration agreed with Neuren's proposal for the key elements of its clinical development program to support a New Drug Application for trofinetide to treat children and adults with Rett syndrome.

Neuren will conduct a single pivotal Phase 3 trial, using the Rett Syndrome Behaviour Questionnaire (RSBQ) and the Clinical Global Impression of Improvement (CGI-I) as co-primary efficacy endpoints. The double-blind, randomized, placebo-controlled trial will test one active dose group with a treatment duration of 6 months. The dosing regimen has been designed to achieve consistent drug exposure in subjects regardless of their weight.

Neuren Executive Chairman Richard Treagus commented: "We are pleased to have held a very constructive meeting with the FDA Division of Neurology Products. It has provided necessary confirmation on the key issues relating to our proposed Phase 3 trial in Rett syndrome. We are now able to progress the final stages of development with full confidence."

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. 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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