



Neuren (NEU) - ASX Announcement

26 April 2016

Neuren Shareholder Update

Melbourne, Australia, 26 April 2016: Neuren Pharmaceuticals (ASX: NEU) today provided an update to shareholders following the announcement of top-line results from the Phase 2 clinical trial of trofinetide in moderate to severe traumatic brain injury (the "INTREPID" trial).

Further development in the brain injury indications will be determined following additional analysis of the INTREPID data and discussions with the US Army on the funding and execution of future trials. This review is to include the concussion trial being conducted at Fort Bragg, and until this has been completed, all expenditure related to the concussion program has been placed on hold.

Premised on the acute anti-inflammatory effects of trofinetide, the INTREPID clinical trial was conceived and co-designed with the US Army in 2009. Subsequently, via collaborations with leading academic laboratories, Neuren has expanded the understanding of trofinetide's actions and it is now recognized that more fundamental effects on cellular and molecular processes are key to its potential therapeutic potential. Following positive results from testing in preclinical animal models of Rett syndrome and Fragile X syndrome, in 2012 Neuren made the decision to progress trofinetide into clinical development for these indications. This was a significant change in strategy and focus for the business.

Since that time, the majority of the value of trofinetide has been, and it remains, in chronic therapies for neurodevelopmental disorders. This has been reinforced by Neuren's discussions with pharmaceutical companies, which have centered for the most part on these Orphan Drug programs.

Neuren's business activities and financial resources are focused on the development programs for Rett syndrome and Fragile X syndrome. Significant investments are being made this year on the optimization of manufacturing processes for commercial supply of trofinetide. Neuren is also investing in chronic toxicity studies for the specific purpose of enabling Phase 3 trials and New Drug Applications for these indications.

The Phase 2 clinical trial in children and adolescents with Rett syndrome is underway, with 3 subjects randomized and 6 more scheduled for their first visit. In this latest trial, following discussion with the FDA Division of Neurology, higher doses of up to 200 mg/kg twice daily are being tested for a longer treatment period compared with the first Rett syndrome trial. The excellent safety profile of trofinetide, which was confirmed in the INTREPID trial, is an important attribute of the drug, enabling the dose to be increased with confidence to target further clinical benefit.

Neuren is scheduled to meet with the FDA Division of Psychiatry prior to 30 June to discuss the development plan for Fragile X syndrome, which Neuren expects will assist in planning the next stages of this program.



Neuren Executive Chairman Richard Treagus commented: “Working with Leerink as our appointed US corporate adviser, we are evaluating a range of alternatives that we expect will allow Neuren to accelerate the development and commercialization of trofinetide. The Orphan Drug indications remain the key focus for Neuren.”

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, correcting deficits in synaptic function and regulating oxidative stress response. Trofinetide is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The intravenous form of trofinetide is 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 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 a clinical stage molecule, trofinetide in Phase 2 clinical trials as well as NNZ-2591 in preclinical 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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