



**Neuren (NEU) - ASX Announcement**

**17 May 2019**

**Neuren validates product pipeline for NNZ-2591 in neurodevelopmental disorders**

**Melbourne, Australia, 17 May 2019:** Neuren Pharmaceuticals (ASX: NEU) announced earlier today that NNZ-2591 has demonstrated positive results in pre-clinical models of two additional neurodevelopmental disorders – Angelman syndrome and Pitt Hopkins syndrome. This follows the announcement in February 2019 of positive effects in the *Shank3* model of Phelan-McDermid syndrome. Neuren anticipates that each of these indications meets the criteria for Orphan Drug designation.

Neuren’s Executive Chairman Richard Treagus made the following statement following these results:

“We are excited that NNZ-2591 has clearly demonstrated positive and wide-ranging effects in all three of these disorders. We look forward to submitting the results for journal publication in due course. These promising results further confirm the potential of this unique neurotrophic drug across a number of neurodegenerative and neurodevelopmental conditions. Neuren has chosen to focus the development on neurodevelopmental disorders with a high unmet need, where NNZ-2591 is most likely to have the greatest impact on patient outcomes and which can leverage the Neuren team’s experience and expertise gained from the development of trofinetide.

These disorders can often be misdiagnosed as autism, and can also be co-diagnosed with autism. Each of them is caused by a mutation or deletion in a different specific gene or chromosomal region; however typically they share many common symptoms and an underlying impairment in the connections and signaling between individual brain cells. Restoring the normal connectivity and signaling between brain cells is considered to be a central property of NNZ-2591.

Neuren is now expediting the remaining tasks including the manufacturing development, toxicity studies and a Phase 1 clinical study that are required before commencing Phase 2 clinical studies with NNZ-2591. We are targeting filing an Investigational New Drug Application (IND) with the US Food and Drug Administration and commencing Phase 2 studies in the second half of 2020.

With trofinetide scheduled to commence Phase 3 in Q42019, the Neuren team is fully committed to working with physicians and families to progress the development of NNZ-2591 for this promising pipeline of neurodevelopmental indications.”

**About Neuren and NNZ-2591**

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for neurodevelopmental disorders with high unmet need. Neuren completed Phase 2 development of its lead drug candidate trofinetide for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs in Rett syndrome and Fragile X syndrome have each been granted Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc.



for the development and commercialization of trofinetide in North America, whilst retaining all rights to trofinetide outside North America.

Neuren is advancing the development of its second drug candidate NNZ-2591, a synthetic analog of the neurotrophic peptide, cyclic glycine proline (cGP), which occurs naturally in the brain. NNZ-2591 has demonstrated efficacy in pre-clinical models of Parkinson's disease, stroke, traumatic brain injury, peripheral neuropathy, Fragile X syndrome, Phelan-McDermid syndrome, Angelman syndrome, Pitt Hopkins syndrome, memory impairment and multiple sclerosis.

**Contact:**

Dr Richard Treagus, Executive Chairman: [rtreagus@neurenpharma.com](mailto:rtreagus@neurenpharma.com); +61 417 520 509

*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.*