



**Neuren (NEU) - ASX Announcement**

**3 December 2019**

## **NNZ-2591 patent granted in Europe to 2034 for neurodevelopmental disorders**

**Melbourne, Australia, 3 December 2019:** Neuren Pharmaceuticals (ASX: NEU) today announced that the European Patent Office has issued a notice of allowance for a new patent covering NNZ-2591 to treat neurodevelopmental disorders. After issue the patent will expire in July 2034. Neuren is developing NNZ-2591 to treat three debilitating childhood disorders for which currently there are no approved drug therapies.

The new patent is titled “Neuroprotective bicyclic compounds and methods for their use in treating autism spectrum disorders and neurodevelopmental disorders”. Patents derived from the same international application were previously granted in the United States and in Japan. These patents supplement issued composition of matter patents in the United States, Europe and Japan.

Following positive results in mouse models, Neuren recently received Orphan Drug designations from the US Food and Drug Administration (FDA) for NNZ-2591 to treat each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome. Neuren is currently undertaking the manufacturing development and non-clinical studies required before submitting an Investigational New Drug (IND) Application to the FDA and commencing clinical trials in 2020.

These three debilitating neurodevelopmental disorders currently have no approved drug therapies. Each is caused by a mutation or deletion in a different gene or chromosomal region; however they share an underlying impairment in the connections and signaling between brain cells. The aim of treatment with NNZ-2591 is to restore normal functional connectivity and signaling.

### **About Neuren**

Neuren is developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Neuren’s lead drug candidate trofinetide is currently in a Phase 3 clinical trial 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 received 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 outside North America. Neuren is advancing the development of its second drug candidate NNZ-2591 for Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, each of which has Orphan Drug designation in the United States.

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*Forward-looking Statements*

*This 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.*

**ASX Listing Rules information**

This announcement was authorized to be given to the ASX by the board of directors of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124