



**Neuren (NEU) - ASX Announcement**

**10 March 2020**

## **Additional information**

The following is additional information regarding the compelling results that were reported on 6 March 2020 from a dose ranging study of NNZ-2591 in a model of Phelan-McDermid syndrome (PMS). The results will be used to inform dose selection for Neuren's planned clinical trials in patients.

- The number of mice tested in the study was 100 (10 per group).
- The statistical design of the study was that for each of the 8 behavioral tests, each dose group was compared with the Wild Type plus vehicle group (i.e. normal mice receiving placebo) in order to assess whether there was a statistically significant difference.
- All of the results for the Knockout mice plus placebo group and the Knockout mice plus each of the two lower dose groups were statistically significantly different when compared with the Wild Type plus vehicle group (i.e. the p value was less than 0.05).
- All of the results for the Knockout mice plus each of the two higher dose groups were not statistically significantly different when compared with the Wild Type plus vehicle group (i.e. the p value was greater than 0.05), thereby indicating that the treated Knockout mice in these 2 groups were indistinguishable from the normal mice.

### **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 received Orphan Drug designation in the United States.

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### **ASX Listing Rules information**

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