Neuren (NEU) – ASX Announcement **22 December 2025**

Health Canada approves Clinical Trial Application for NNZ-2591

Highlights:

- **Health Canada has approved Neuren's Clinical Trial Application (CTA) for the Koala Phase 3 clinical trial of NNZ-2591 in Phelan-McDermid syndrome**
- **CTA approval enables the inclusion of clinical trial sites in Canada**
- **Second site in the US now activated and enrolling**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that Health Canada has approved its Clinical Trial Application (CTA) for the Koala Phase 3 clinical trial evaluating NNZ-2591 for the treatment of Phelan-McDermid syndrome (PMS) in children aged 3 to 12 years. Approval of the CTA enables the potential initiation of clinical trial sites in Canada.

The Koala Phase 3 clinical trial previously received Investigational New Drug (IND) approval from the US Food and Drug Administration (FDA) and enrolment is ongoing (Study Details | NCT07281079 | A Study of NNZ-2591 in Pediatric Participants With Phelan-McDermid Syndrome | ClinicalTrials.gov). A second US site has now been activated and is enrolling in California.

Neuren CEO Jon Pilcher commented: "We are pleased to see a second regulator provide clearance for Neuren's Koala trial, which is the first ever Phase 3 clinical trial in PMS. We look forward to working with investigators and the PMS community in Canada. Meanwhile we now have sites enrolling on both East and West coasts in the US, with many more working through the process of activation."

About Neuren

Neuren is developing new drug therapies to treat multiple serious neurological disorders that emerge in early childhood and have no or limited approved treatment options.

DAYBUE® (trofinetide) and DAYBUE STIX (trofinetide) are approved by the US Food and Drug Administration (FDA) for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren's second drug candidate, NNZ-2591, is in development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome. Recognising the urgent unmet need, each program has been granted "orphan drug" designation in the United States and the European Union. Orphan drug designation provides incentives to encourage development of therapies for rare and serious diseases.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the Board of Neuren Pharmaceuticals Limited, Suite 1.01, 117 Camberwell Road, Hawthorn East, VIC 3123

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.