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

Neuren Presentations at the ACMG Annual Clinical Genetics Meeting 2025

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced the acceptance of three abstracts for presentation at the 2025 American College of Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting, being held on 18–22 March 2025, in Los Angeles.

Details of the presentations are as follows:

"NNZ-2591, a Synthetic IGF-1 Metabolite Analog: Phase 2 Clinical Trial Results for Children and Adolescents with Pitt Hopkins Syndrome"

- Presentation Type: Platform (O38)
- Date/Time: Thursday, 20 March – 1:30 PM (Platform Session 2)

"The Evolving Genetic Landscape of Phelan-McDermid Syndrome and Implications for Diagnostics"

- Presentation Type: Poster (P672)
- Date/Time: Friday, 21 March– 10:30-11:30 AM – Exhibit Hall A

"Safety, Efficacy, and Exposure-Response of NNZ-2591, a Synthetic Analog of an IGF-1 Metabolite, for Phelan-McDermid Syndrome in Children and Adolescents"

- Presentation Type: Poster (P166)
- Date/Time: Friday, 21 March – 10:30-11:30 AM – Exhibit Hall A

All ACMG Annual Clinical Genetics Meeting content to be presented or published is embargoed until the time of the actual conference presentation. Posters will be embargoed until Wednesday, 19 March at 5:00 pm PT.

Neuren Chief Medical Officer Liza Squires M.D. commented: "We are very pleased to have three presentations at ACMG this year which highlight our programs to develop NNZ-2591 as a much-needed treatment for Phelan-McDermid syndrome and Pitt Hopkins syndrome. The presentations address the evolving diagnostic landscape in Phelan-McDermid syndrome, and the encouraging results achieved in our Phase 2 trials."

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. Recognising the urgent unmet need, all programs have been granted "orphan drug" designation in the United States. Orphan drug designation provides incentives to encourage development of therapies for rare and serious diseases.

DAYBUE™ (trofinetide) is approved by the US Food and Drug Administration (FDA) and Health Canada 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 Phase 2 development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the CEO & Managing Director of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124

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.