**Neuren (NEU) – ASX Announcement** **20 January 2025**

# J.P. Morgan Healthcare Conference recap

Neuren Pharmaceuticals (ASX: NEU) was pleased to participate in the 43rd Annual J.P. Morgan Healthcare Conference, held from 13 to 16 January 2025, in San Francisco (**Conference**).

Neuren delivered a presentation at the Conference and engaged in one-on-one meetings with investors and industry peers, showcasing its development pipeline for NNZ-2591 and robust financial growth profile from DAYBUE™ (trofinetide). A copy of Neuren's presentation slides was released last week and an audio recording of the presentation by Neuren CEO Jon Pilcher is now available on Neuren's company website (https://www.neurenpharma.com/news-media/media).

2025 is shaping up to be a defining year for Neuren, as NNZ-2591 progresses into late-stage development as a multi-indication platform following positive Phase 2 trial results in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome. Neuren is actively preparing to commence a Phase 3 trial in Phelan-McDermid syndrome. Alignment with the US Food and Drug Administration (FDA) has been reached regarding a single Phase 3 trial, with the same dose, population and duration as the Phase 2 trial, while negotiations with FDA continue on the primary efficacy endpoints to be used in the trial. Neuren also plans to advance Pitt Hopkins syndrome and Angelman syndrome to registration trials, subject to FDA consultation. Furthermore, Neuren is progressing non-clinical studies in multiple undisclosed indications, with an update to be announced in due course.

Neuren's global partner for trofinetide, Acadia Pharmaceuticals (Acadia), also presented at the Conference, announcing the submission of a Marketing Authorisation Application to the European Medicines Agency, as well as initiatives for the next wave of growth of DAYBUE in the US. Potential approval in Europe is expected in Q1 2026, with earlier initiation of Managed Access Programs anticipated in Q2 2025. For DAYBUE in the US, Acadia announced a substantial expansion of its field force, plans to launch direct-to-consumer campaigns and an omni-channel strategy to bring DAYBUE clinical experience to life. An audio recording of Acadia's Conference presentation and the presentation slides are available on its website (https://ir.acadia.com/events-and-presentations).

**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.*