



Neuren (NEU) – ASX Announcement

24 February 2025

Neuren receives Rare Pediatric Disease Designation from FDA

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has received Rare Pediatric Disease Designation from the US Food and Drug Administration (FDA) for NNZ-2591 in each of Pitt Hopkins syndrome (PTHS) and Angelman syndrome (AS). Neuren has also previously received Rare Pediatric Disease Designation from the FDA for NNZ-2591 in Phelan-McDermid syndrome (PMS).

With this designation in place for PMS, PTHS and AS, Neuren may be awarded a priority review voucher (PRV) if the Rare Pediatric Disease PRV program is reauthorized by the US Congress and NNZ-2591 receives marketing authorisation for any of these indications. The Rare Pediatric Disease PRV program is designed to incentivize drug development for serious rare pediatric diseases. If awarded, a PRV can be redeemed to receive priority review for a different product or sold to another sponsor. As previously reported, Neuren’s partner Acadia Pharmaceuticals received a PRV for DAYBUE™ (trofinetide) in Rett syndrome and sold the PRV for US\$150 million.

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 development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome.

Contact:

investorrelations@neurenpharma.com

Jon Pilcher, CEO: +61 438 422 271

ASX Listing Rules information

This announcement was authorized to be given to the ASX by the Board 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.