

Neuren (NEU) – ASX Announcement

19 July 2024

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 Phelan-McDermid syndrome. A sponsor who receives an approval for a drug with this designation may qualify for a priority review voucher (PRV) that can be redeemed to receive priority review for a different product or sold to another sponsor. The rare pediatric disease PRV program aims to incentivize drug development for serious rare pediatric diseases.

Neuren is preparing for an End of Phase 2 Meeting with FDA in Q3 2024 to discuss the development program for NNZ-2591 in Phelan-McDermid syndrome. Neuren previously reported results from a Phase 2 trial in which significant improvement was assessed by both clinicians and caregivers across multiple efficacy measures.

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) for the treatment of Rett syndrome in adult and pediatric patients two years of age and older. 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 and Pitt Hopkins syndrome.

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ASX Listing Rules information

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