



Neuren (NEU) – ASX Announcement

26 June 2025

US patent allowed for NNZ-2591 to treat Pitt Hopkins syndrome

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that the United States Patent and Trademark Office has allowed Neuren's patent application covering the use of NNZ-2591 to treat Pitt Hopkins syndrome (PTHS) for issuance as a patent. After issue the expiry date of the patent will be in April 2040. Related applications are pending in other territories.

There are currently no approved treatments for PTHS, which has a severely debilitating impact on the lives of patients, as well as their parents and siblings. The US Food and Drug Administration (FDA) has granted Orphan Drug designation and Fast Track designation to Neuren's development program for NNZ-2591 in PTHS.

In Neuren's multi-centre Phase 2 clinical trial of treatment with NNZ-2591 for 13 weeks, improvements were seen in clinically important aspects of PTHS, including communication, social interaction, cognition and motor abilities. 9 out of 11 children showed improvement assessed by clinicians and 8 out of 11 children showed improvement assessed by caregivers. Neuren plans to discuss the next steps in the development program with the FDA.

Neuren is also currently preparing to commence a Phase 3 clinical trial of NNZ-2591 in Phelan-McDermid syndrome, another serious neurodevelopmental disorder for which there are no approved treatments.

About Pitt Hopkins syndrome (PTHS)

Pitt Hopkins syndrome (PTHS) is a neurodevelopmental condition caused by the loss of one copy or a mutation of the TCF4 gene on chromosome 18. The incidence of PTHS has been estimated at between 1 in 34,000 and 1 in 41,000 people. Characteristics of PTHS are a range of developmental delays with moderate-to-severe intellectual disability and behavioral differences, hyperventilation and/or breath-holding while awake, seizures, gastrointestinal issues, lack of speech, sleep disturbance, stereotypic hand movements and distinctive facial features. Some individuals with PTHS are diagnosed with autism. Further information about PTHS is available at: www.pitthopkins.org.

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, programs have been granted "orphan drug" designation in the United States, which provides incentives to encourage the 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.

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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 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.