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**Neuren (NEU) – ASX Announcement** **2 November 2026**

# FDA grants Orphan Drug Designation for NNZ-2591 to treat HIE

**Melbourne, Australia:** Neuren Pharmaceuticals (ASX: NEU) today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation to Neuren's NNZ-2591 for the treatment of neonatal hypoxic-ischemic encephalopathy (HIE).

Orphan Drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition with fewer than 200,000 patients in the United States. Amongst other incentives, Orphan Drug designation qualifies the sponsor of the drug for 7 years of marketing exclusivity, as well as waiver of the prescription drug user fee for a marketing application.

HIE is a potentially devastating type of brain injury caused when a baby's brain does not receive enough oxygen or blood flow before or shortly after birth. Many thousands of children experience HIE every year. It is one of the leading causes of neonatal death and neurodevelopmental disability worldwide. HIE can lead to a range of symptoms in surviving children, including developmental delays, cognitive impairment, cerebral palsy, and seizures. Some children develop serious long-term complications that can affect them well into adulthood.

Neuren is currently conducting a juvenile animal safety study required by the FDA prior to initiating a clinical study in HIE. Neuren plans to submit an Investigational New Drug (IND) application in H1 2027.

Neuren CEO Jon Pilcher commented: "We are very pleased to receive the important commercial milestone of Orphan Drug designation. We now look forward to discussing the development path with the FDA and preparing the IND application for a clinical study in HIE, a devastating condition with serious need."

Neuren is developing NNZ-2591 for multiple indications, including Phelan-McDermid syndrome, Pitt Hopkins syndrome, HIE and Angelman syndrome, all of which have Orphan Drug designation.

**About Neuren**

Neuren Pharmaceuticals is developing new drug therapies to treat multiple serious neurological disorders caused by genetic abnormalities or brain injury, that have no or limited approved treatment options. Neuren's therapies target the critical role of Insulin-like growth factor 1 (IGF-1) in the brain, using orally administered analogs of naturally occurring peptides.

DAYBUE® (trofinetide) oral solution and DAYBUE STIX (trofinetide) powder are approved for the treatment of Rett syndrome by the US Food and Drug Administration (FDA). The oral solution is also approved by the European Medicines Agency, Health Canada and the Ministry of Health in Israel. Neuren has granted an exclusive worldwide license to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren's investigative drug candidate, NNZ-2591 (ercanetide), is in clinical development as an oral solution treatment for multiple neurodevelopmental disorders, including Phelan-McDermid syndrome,

Pitt Hopkins syndrome and Angelman syndrome. Each of these programs has been granted "orphan drug" designation in the United States and the European Union as well as Fast Track and Rare Pediatric Disease designations from the FDA. NNZ-2591 has also been granted orphan drug designation for the treatment of hypoxic ischemic encephalopathy (HIE), a serious condition caused by brain injury before or shortly after birth.

Currently, Neuren is conducting a Phase 3, randomized, double-blind, placebo-controlled clinical trial ("Koala") evaluating the safety and efficacy of NNZ-2591 in children aged 3 to 12 years with Phelan-McDermid syndrome and a 52-week open-label extension study.

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**ASX Listing Rules information**

This announcement was authorized to be given to the ASX by the CEO of Neuren Pharmaceuticals Limited, Suite 1.01, 117 Camberwell Road, Hawthorn East, VIC 3123

***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.*

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