Neuren (NEU) – ASX Announcement **23 January 2023**

Prader-Willi syndrome IND for NNZ-2591 approved by FDA

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that the US Food and Drug Administration (FDA) has reviewed Neuren's Investigational New Drug (IND) application for NNZ-2591 in Prader-Willi syndrome (PWS) and given approval for Neuren to proceed with the planned Phase 2 clinical trial in children with PWS.

Neuren is developing NNZ-2591 for four serious neurological disorders that emerge in early childhood. Phase 2 trials are currently ongoing in children with each of Angelman, Phelan-McDermid and Pitt Hopkins syndromes, for which there are no approved medicines. All four programs have been granted Orphan Drug designation by the FDA.

Neuren previously reported positive results in the *Magel2*-null mouse model of Prader-Willi syndrome, in which treatment with NNZ-2591 for 6 weeks normalized fat mass, insulin levels, IGF-1 levels and all behavioural deficits.

About Prader-Willi syndrome

Prader-Willi syndrome (PWS) is a highly debilitating neurodevelopmental disorder, caused by defects in the 15q11-q13 region of chromosome 15. The estimated incidence is 1 in 10,000 – 30,000 males and females across all races and ethnicities. Infants with PWS have very low muscle tone and suffer from feeding difficulties. An unregulated appetite and easy weight gain characterize the later stages of PWS, which can lead to morbid obesity. The range of other challenges for individuals with PWS can include intellectual and learning disabilities, growth hormone deficiency, sleep disturbances, speech difficulties, obsessive-compulsive symptoms, gastrointestinal complications, and difficulty controlling emotions.

About Neuren

Neuren is developing two new drug therapies to treat multiple serious neurological disorders that emerge in early childhood and have no or limited approved treatment options.

A New Drug Application for the lead compound, trofinetide, to treat Rett syndrome is under Priority Review by the US Food and Drug Administration (FDA), with a PDUFA action date of 12 March 2023. Neuren has granted an exclusive licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide in North America, while retaining all rights outside North America.

Neuren is conducting Phase 2 trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome, Pitt Hopkins syndrome and Prader-Willi syndrome.

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.

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ASX Listing Rules information
This announcement was authorized to be given to the ASX by the board of directors 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.