Neuren (NEU) – ASX announcement **3 September 2021**

FDA grants Orphan Drug Designation for NNZ-2591 to treat Prader-Willi syndrome

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today reported that the US Food and Drug Administration (FDA) has granted Orphan Drug designation to Neuren's NNZ-2591 for the treatment of 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. A range of other problems can include intellectual and learning disabilities, growth hormone deficiency, sleep disturbances, speech difficulties, obsessive-compulsive symptoms, gastrointestinal complications, and difficulty controlling emotions.

Neuren previously announced 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.

Neuren CEO Jon Pilcher commented: "We were excited by the strong pre-clinical efficacy of NNZ-2591, which clearly demonstrated the potential for the mechanism of action to have a positive impact on Prader-Willi syndrome. We are now delighted to receive Orphan Drug designation from the FDA following review of our rationale and data. This underpins the commercial opportunity and follows Orphan Drug designation already granted for Phelan-McDermid, Angelman and Pitt Hopkins syndromes."

Orphan Drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition. Amongst other incentives, Orphan Drug designation qualifies the sponsor of the drug for 7 years of marketing exclusivity, plus 6 months if approved for pediatric use, as well as waiver of the prescription drug user fee for a marketing application.

About Neuren

Neuren is developing two new drug therapies to treat multiple serious neurological disorders that emerge in early childhood, none of which have any approved medicines.

The lead compound, trofinetide, is currently in a Phase 3 clinical trial for Rett syndrome with top-line results expected in Q4 2021 and has completed a Phase 2 clinical trial in Fragile X syndrome. Both programs have Fast Track designation from the US Food and Drug Administration (FDA). 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 preparing to initiate Phase 2 trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome in H2 2021. Neuren is also planning a Phase 2 trial in Prader-Willi syndrome.

Because of the urgent unmet need, five programs have been granted "orphan drug" designation in both the United States and the European Union, a designation that 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 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.