**Neuren (NEU) – ASX Announcement** **25 March 2022**

# Neuren receives FDA approval for Pitt Hopkins IND and Phase 2 trial

**Melbourne, Australia:** Neuren Pharmaceuticals (ASX: NEU) has received approval from the US Food and Drug Administration (FDA) to proceed with the Phase 2 trial of NNZ-2591 in Pitt Hopkins syndrome (PTHS). The Investigational New Drug application (IND) for the PTHS program is now active.

**Neuren CEO Jon Pilcher commented: "We are excited to proceed with this groundbreaking trial in Pitt Hopkins syndrome and look forward to working with the community in the US. Neuren now has three INDs active for NNZ-2591 and we are advancing our plan to address multiple neurodevelopmental disorders that have such high unmet need".**

The Phase 2 trial will be conducted at four hospitals in the United States, enrolling up to 20 children aged 3 to 17 years with PTHS to examine safety, tolerability, pharmacokinetics and efficacy over 13 weeks of treatment with NNZ-2591. Commencement is subject to ethics approval from the Institutional Review Boards (IRBs). Top-line results from the trial are anticipated in H1 2023.

Neuren also recently received FDA approval to proceed with Phase 2 trials of NNZ-2591 in Angelman syndrome and Phelan-McDermid syndrome. In parallel with the three Phase 2 trials, Neuren is executing the foundational work to prepare for Phase 3 development of NNZ-2591 across multiple indications.

Neuren is advancing plans for an IND and Phase 2 trial in a fourth neurodevelopmental disorder, Prader-Willi syndrome, targeting commencement in H2 2022 with results anticipated in H2 2023.

## About Pitt Hopkins syndrome

Pitt Hopkins syndrome (PTHS) is a neurodevelopmental condition affecting both males and females, 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 developmental delay 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. There is currently no treatment approved for PTHS.

**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, achieved positive results in a Phase 3 clinical trial for Rett syndrome and has also 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, Pitt Hopkins syndrome and Prader-Willi syndrome.

Recognising the urgent unmet need, all six 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.*