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

Neuren receives FDA approval for Angelman 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 Angelman syndrome (AS). The Investigational New Drug application (IND) for the AS program is now active.

Neuren CEO Jon Pilcher commented: "FDA approval of the first IND for NNZ-2591 is a huge step forward in Neuren's plan to develop NNZ-2591 for four serious neurodevelopmental disorders. The Angelman syndrome Phase 2 trial in Australia has been keenly awaited by the Angelman community and we are very excited to be able to proceed. We also now anticipate receiving clearance next month of our IND applications for similar Phase 2 trials in Phelan-McDermid and Pitt Hopkins syndromes, subject to completion of those FDA reviews."

The AS Phase 2 trial will be conducted at three hospitals in Australia, enrolling up to 20 children aged 3 to 17 years to examine safety, tolerability, pharmacokinetics and efficacy over 13 weeks of treatment with NNZ-2591. Commencement of the trial is subject to Ethics Committee approval. Results from the trial are anticipated in H1 2023. In parallel with the trial, Neuren is also executing the foundational work to prepare for Phase 3 development.

There are currently no approved medicines for AS, which is characterized by severe developmental delay and learning disabilities that become noticeable by the age of 6 – 12 months. Children and adults with AS typically have balance issues, motor impairment and can have debilitating seizures. Some individuals never walk, most do not speak and disruptive sleep also can be a serious challenge. Individuals have a normal life expectancy, but they require continuous care and are unable to live independently. AS is caused by a loss of function of the *UBE3A* gene on chromosome 15.

Investor Zoom Webinar 11:00am AEDT Friday 25 February

You are invited to register using this link:

https://zoom.us/webinar/register/WN_N-CpWr0dTcuRaONTtcg2VQ

Participants may submit questions during registration or during the session

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.