Neuren (NEU) – ASX Announcement **11 March 2022**

Neuren receives Ethics approval for Angelman Phase 2 trial in Australia

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has received approval from the Human Research Ethics Committee for its Phase 2 clinical trial of NNZ-2591 in Angelman syndrome (AS). The approval covers the three sites in Australia that will conduct the trial: Queensland Children's Hospital/Centre for Children's Health Research, South Brisbane, Sydney Children's Hospital and Austin Health, Victoria. Separate research governance authorisation is now required from each site before they can be initiated and commence enrolment of subjects.

Neuren CEO Jon Pilcher commented: "This is another important step achieved for the first clinical trial of NNZ-2591 in patients and Neuren's first in Australia. We are very excited to be working closely with the local Angelman syndrome community and are eager to accelerate development of this potential therapy which has shown such promise to date."

The AS Phase 2 trial will enroll up to 20 children aged 3 to 17 years to examine safety, tolerability, pharmacokinetics and efficacy over 13 weeks of treatment with orally administered NNZ-2591. Results from the trial are expected in H1 2023.

Neuren is also awaiting clearance from the US Food and Drug Administration (FDA) of Investigational New Drug (IND) applications for Phase 2 trials of NNZ-2591 in each of Phelan-McDermid syndrome and Pitt Hopkins syndrome. Both of those trials will be conducted in the United States.

In parallel with the Phase 2 trials, Neuren is executing the foundational work to prepare for Phase 3 development of NNZ-2591 across these neurodevelopmental disorders and Prader-Willi syndrome, all of which have urgent need for an approved treatment.

About Angelman syndrome

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.

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.