Neuren (NEU) – ASX announcement **1 October 2021**

Neuren receives feedback on IND for Angelman syndrome and submits IND application for NNZ-2591 in Pitt Hopkins syndrome

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has submitted an Investigational New Drug (IND) application to the US Food and Drug Administration (FDA) for NNZ-2591 to treat Pitt Hopkins syndrome. This follows the earlier submissions of IND applications for NNZ-2591 to treat each of Angelman and Phelan-McDermid syndromes. Neuren has Orphan Drug designation from both the FDA and the European Medicines Agency for NNZ-2591 in all three syndromes, which are serious neurodevelopmental disorders with no approved medicines.

Neuren has also received feedback from the FDA following the 30-day review of the first IND application for Angelman syndrome. The Agency has specified additional clinical assessments to be added to the Phase 2 trial protocol to enhance safety monitoring during this first trial in patients. The IND has been placed on Clinical Hold until the changes to the protocol are agreed with the FDA. Formal notification of the Hold will be issued within 30 days, after which Neuren will likely seek a Type A meeting with the FDA to discuss and agree the necessary protocol amendment.

Neuren CEO Jon Pilcher commented: "This delay to the start of the Angelman syndrome trial is disappointing, however we will work with the FDA to amend the protocol as quickly as possible, as well as confirming any necessary amendment to the protocols for the Phelan-McDermid and Pitt Hopkins Phase 2 trials. We will also take this into account for our IND application and Phase 2 trial protocol in Prader-Willi syndrome, which we plan to submit in the first half of 2022."

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.

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.

Contact:
Jon Pilcher, CEO: jpilcher@neurenpharma.com; +61 438 422 271

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.