Neuren (NEU) – ASX Announcement **7 December 2020**

Neuren receives notice of positive opinion on all three Orphan designation applications for NNZ-2591 in Europe

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has received notice from the European Medicines Agency (EMA) of positive opinions for all three Orphan designation applications that were submitted for NNZ-2591 in Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome. Under the Orphan procedure timetable, the European Commission is scheduled to issue the decisions in January.

Orphan designation in the EU enables sponsors to benefit from incentives including free protocol assistance, fee reductions and 10 years of market exclusivity plus two additional years if approved for paediatric use. During that exclusivity period, the EMA and the EU Member States shall not accept another marketing authorisation application for a similar medicinal product in the same therapeutic indication.

Neuren plans to commence Phase 2 trials in patients with each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome in 2021 to confirm the potential of NNZ-2591 to address the urgent unmet need in these three serious childhood disorders.

Neuren CEO Jon Pilcher commented: "We are pleased to have received no questions following the EMA review of our compelling pre-clinical results and the rationale for treatment with NNZ-2591. We move forward to Phase 2 trials with increased confidence now that we have Orphan designation for all three indications in the two largest markets. We look forward to engaging with both the FDA and the EMA as we strive to develop an effective treatment for patients and families living with these debilitating conditions."

About Neuren

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

The lead drug compound, trofinetide, is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. Because of the urgent unmet need, the programs have each been granted Fast Track designation by the US Food and Drug Administration (FDA) and "orphan drug" designation in both the United States and the European Union, a designation that provides incentives to encourage therapies for rare and serious diseases.

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 advancing the development of its second drug candidate NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, each of which has received orphan drug designation in the United States and the European Union. Neuren has commenced a Phase 1 clinical trial of NNZ-2591 and plans to initiate Phase 2 trials in all three disorders in 2021.

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

ASX Listing Rules information
This announcement was authorised 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.