Neuren (NEU) – ASX announcement **21 October 2021**

Neuren receives feedback on IND for Phelan-McDermid syndrome

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has received feedback from the US Food and Drug Administration (FDA) following the 30-day review of the Investigational New Drug (IND) application for its planned Phase 2 clinical trial of NNZ-2591 in children with Phelan-McDermid syndrome. Consistent with the feedback on the similar application for Angelman syndrome, which was reported by Neuren on 1 October, the IND has been placed on hold, with a formal hold letter to be issued by the FDA within the next 30 days. A third IND application for Pitt Hopkins syndrome is currently still under review by the FDA.

All three INDs are supervised by the FDA Office of Neuroscience, with Phelan-McDermid and Pitt Hopkins reviewed by the Division of Neurology 1 and Angelman reviewed by the Division of Psychiatry. After receiving the hold letters, Neuren will work with the Office of Neuroscience to resolve the common issues across these three planned clinical trials as quickly as possible. This may include requesting a Type A meeting to discuss amendments to the trial protocols.

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.

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 for 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.

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.