Neuren (NEU) – ASX Announcement **23 March 2022**

Neuren receives FDA approval for Phelan-McDermid 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 Phelan-McDermid syndrome (PMS). The Investigational New Drug application (IND) for the PMS program is now active.

Neuren CEO Jon Pilcher commented: "We are excited to be able to proceed with this eagerly anticipated trial following FDA approval of our second IND for NNZ-2591 and we look forward to advancing in partnership with the Phelan-McDermid community in the US. We also anticipate a response from the FDA for our third IND in Pitt Hopkins syndrome in coming days".

The Phase 2 trial will be conducted at four hospitals in the United States, enrolling up to 20 children aged 3 to 12 years with PMS to examine safety, tolerability, pharmacokinetics and efficacy over 13 weeks of treatment with NNZ-2591. Commencement is subject to ethics approval from the Institutional Review Boards (IRBs). Top-line results from the trial are anticipated in H1 2023.

In parallel with the Phase 2 trials in PMS, Angelman syndrome and Pitt Hopkins syndrome, Neuren is executing the foundational work to prepare for Phase 3 development of NNZ-2591 across multiple indications.

Neuren is also now able to move forward with plans for an IND and Phase 2 trial in a fourth neurodevelopmental disorder, Prader-Willi syndrome, targeting commencement in H2 2022 with results anticipated in H2 2023.

About Phelan-McDermid syndrome

PMS is caused by a deletion or other change in the 22q13 region of chromosome 22, which includes the *SHANK3* gene, or a mutation of the gene. PMS is also known as 22q13 deletion syndrome. The *SHANK3* gene codes for the shank3 protein, which supports the structure of synapses between nerve cells in the brain. It is estimated that between 1 in 8,000 and 1 in 15,000 people have PMS. The most common characteristics are intellectual disability, delayed or absent speech, symptoms of autism (approximately 75% are diagnosed with autism spectrum disorder), low muscle tone, motor delays, and epilepsy. There is currently no cure or treatment specifically for PMS.

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.

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.