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

Neuren submits IND and Ethics applications for NNZ-2591 in Angelman 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 Angelman syndrome. An Ethics Committee application has also been submitted to conduct the planned Phase 2 clinical trial at three hospitals in Australia. Neuren is preparing to commence the trial after approval of both applications.

Neuren is also completing IND applications for NNZ-2591 to treat Phelan-McDermid syndrome and Pitt Hopkins syndrome. All three conditions are serious neurodevelopmental disorders with no approved medicines. Neuren conducted three pre-IND meetings with the FDA Office of Neuroscience to discuss the proposed Phase 2 clinical trials in patients with each syndrome. The clear and constructive guidance from the FDA enabled Neuren to proceed with compiling the IND applications.

Neuren CEO Jon Pilcher commented: "Filing the first IND application for NNZ-2591 is a very important milestone in our plan to develop NNZ-2591 for multiple serious neurological conditions. We are eager to start the Phase 2 trial in Angelman syndrome, which we hope will demonstrate the potential for NNZ-2591 to provide an urgently needed treatment option."

Angelman syndrome (AS) is caused by a loss of function of the *UBE3A* gene in chromosome 15. AS 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.

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.

Because of the urgent unmet need, five programs have been granted "orphan drug" designation in both the United States and the European Union, a designation that 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.