Neuren (NEU) – ASX Announcement **10 March 2021**

Neuren successfully completes drug substance manufacturing for NNZ-2591 Phase 2 trials

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that manufacturing of the drug substance for Phase 2 trials of NNZ-2591 has been successfully completed on schedule. Neuren is preparing to submit Investigational New Drug (IND) applications to the US Food and Drug Administration for trials in children with Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome.

Neuren CEO Jon Pilcher commented: "We have successfully developed a proprietary process for large scale manufacturing with exceptional purity and high yield. This is a key part of the strong foundations we have built for NNZ-2591, which can now be leveraged across multiple valuable indications. As well as supplying the upcoming trials in Phelan-McDermid, Angelman and Pitt Hopkins, the campaign has produced enough drug substance at no extra cost to supply a Phase 2 trial in Prader-Willi syndrome."

Today's announcement means that four of the milestones for 2021 set out in Neuren's recently released corporate presentation and reproduced below have already been achieved. Five important milestones remain, leading up to the Rett syndrome Phase 3 trial topline results expected in Q4 2021.

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 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. Both programs have been granted Fast Track designation by 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 plans 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 2021. Neuren is also preparing for 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 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.