Neuren (NEU) - ASX Announcement **7 May 2020**

Neuren commences first clinical trial for NNZ-2591

Melbourne, Australia, 7 May 2020: Neuren Pharmaceuticals (ASX: NEU) today announced the commencement of its first clinical trial of NNZ-2591. The Phase 1 trial, conducted in Australia, will assess safety, tolerability and pharmacokinetics in healthy adult volunteers.

The first stage of the open label trial will test a single oral dose of NNZ-2591 at 3 ascending dose levels. Subject to review of the data from this first stage and from non-clinical studies currently in progress, the second stage of the trial will test twice daily oral dosing for 14 days at 2 ascending dose levels. 30 subjects will be enrolled (6 in each of the 5 dosing cohorts), with analysis of adverse events, physical and laboratory measurements and pharmacokinetic parameters. The second stage of the trial is scheduled to conclude in the 4th quarter of 2020.

Data from the trial will form part of the Investigational New Drug (IND) application to the US Food and Drug Administration (FDA) in order to proceed with Phase 2 trials in children with neurodevelopmental disorders. Based on its mechanism of action and positive results in animal models, NNZ-2591 has received Orphan Drug designation from the FDA for each of Phelan-McDermid, Angelman and Pitt Hopkins syndromes, three debilitating disorders that currently have no approved treatments.

Neuren Executive Chairman Richard Treagus commented: “We are very pleased to be commencing clinical trials of NNZ-2591. We believe this therapy has the potential to make a real difference to the treatment of these debilitating disorders, which currently have no approved therapies. Having compiled a compelling package of non-clinical data, we now look forward to commencing the clinical program which is a very important step forwards in confirming the potential of NNZ-2591.”

About Neuren

Neuren is developing new therapies for debilitating neurodevelopmental disorders that emerge in early childhood and are characterized by impaired connections and signalling between brain cells. The therapies utilize synthetic analogs of neurotrophic peptides that occur naturally in the brain. Trofinetide is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs have each received Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America, whilst retaining all rights outside North America. Neuren is advancing the development of NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, each of which has received Orphan Drug designation in the United States.

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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.