Neuren (NEU) - ASX Announcement **19 November 2020**

Neuren receives approval for final stage of NNZ-2591 clinical trial

Highlights:

- **Phase 1 trial in healthy adult volunteers set to report in January 2021**
- **Phase 2 trials planned for 2021 in children with 3 serious neurodevelopmental disorders that have no approved medicines**
- **NNZ-2591 has 3 Orphan Drug designations from FDA, with applications in Europe pending decisions in January 2021**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that it has received Ethics Committee approval to move forward with the final stage of its first clinical trial of NNZ-2591. The Phase 1 trial in Australia is evaluating the safety, tolerability and pharmacokinetics (PK) of NNZ-2591 in healthy adult volunteers before advancing to Phase 2 trials in patients in 2021.

The Phase 1 trial is scheduled to conclude in January 2021. Data from the trial will form part of Investigational New Drug (IND) applications to the US Food and Drug Administration (FDA) for Phase 2 trials in children with neurodevelopmental disorders. Based on its mechanism of action and clear efficacy 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 medicines. Decisions on applications submitted for Orphan designation in Europe are expected in January 2021.

The updated protocol for the final stage of the Phase 1 trial will test 16 subjects across two double-blind placebo-controlled dosing cohorts. Adverse events, physical and laboratory measurements and PK parameters will be recorded during twice daily oral dosing for 7 days. Ethics Committee approval to proceed with the final stage was received after review of data from non-clinical safety studies and the safety, tolerability and PK data from the first stage of the trial, in which single doses of NNZ-2591 were administered.

Neuren CEO Jon Pilcher commented: “This first clinical trial is generating valuable information about the characteristics of NNZ-2591. It is an important stepping-stone to commencing Phase 2 trials in patients in 2021, in which we aim to confirm the potential of NNZ-2591 to address the urgent unmet need for three serious childhood disorders.”

About Neuren

Neuren has two new drug therapies in clinical development for five 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. Because of the urgent unmet need, the programs have each been granted Fast Track designation by the US Food and Drug Administration (FDA) and "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.

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 advancing the development of its second drug candidate NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, each of which has received orphan drug designation in the United States. Neuren has commenced a Phase 1 clinical trial of NNZ-2591 and plans to initiate Phase 2 trials in all three disorders in 2021.

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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.