



Neuren (NEU) - ASX Announcement

22 September 2020

Neuren initiates manufacture of NNZ-2591 for Phase 2 trials

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has initiated the manufacture of NNZ-2591 to supply the planned Phase 2 clinical trials. The contract manufacturer that supplied drug substance for the ongoing Phase 1 trial will carry out this larger scale manufacturing campaign.

Neuren CEO Jon Pilcher commented: "We have made excellent progress in the optimisation and scale up of the manufacturing process for NNZ-2591. This investment is another important stepping-stone towards the Phase 2 trials in 2021, in which we aim to confirm the potential of NNZ-2591 to address the urgent unmet need in three serious childhood disorders."

Neuren plans to commence Phase 2 trials in patients with each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome in 2021. Neuren has received Orphan Drug designation from the FDA for NNZ-2591 to treat all three of these debilitating disorders that currently have no approved medicines.

About Neuren

Neuren is developing two new drug therapies to treat 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.