



Neuren (NEU) – ASX Announcement

29 September 2020

Neuren submits Orphan designation applications for NNZ-2591 in Europe

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has submitted Orphan designation applications to the European Medicines Agency (EMA) for NNZ-2591 in each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome. Neuren received Orphan Drug designation from the US Food and Drug Administration (FDA) for all three indications last year.

Under the EMA's Orphan procedure timetable, a decision is expected in January or February 2021.

Orphan designation in the EU enables sponsors to benefit from incentives including free protocol assistance, fee reductions and 10 years of market exclusivity plus 2 additional years if approved for paediatric use. During that exclusivity period, the EMA and the EU Member States shall not accept another marketing authorisation application for a similar medicinal product in the same therapeutic indication.

Neuren plans to commence Phase 2 trials in patients with each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome in 2021 to confirm the potential of NNZ-2591 to address the urgent unmet need in these three serious childhood disorders.

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.