

Neuren (NEU) – ASX Announcement

7 February 2025

Type C Meeting granted by FDA for Phelan-McDermid syndrome

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that the US Food and Drug Administration (FDA) has granted a Type C Meeting to discuss the primary efficacy endpoints in Neuren's planned pivotal Phase 3 clinical trial program for NNZ-2591 to treat Phelan-McDermid syndrome.

Neuren previously announced the positive outcomes from a Type B End of Phase 2 Meeting, at which alignment with FDA was reached on the other key features of the Phase 3 clinical trial program. A Type C Meeting was considered by FDA as the best forum for completion of the remaining efficacy endpoints discussion. The meeting will take place face-to-face in early April.

Neuren CEO Jon Pilcher commented: "We look forward to another constructive discussion with FDA. Having a confirmed Type C meeting now establishes a clear timetable and in parallel we are continuing all our preparations, planning for mid-year commencement of the first ever Phase 3 trial for children with Phelan-McDermid syndrome."

About Neuren

Neuren is developing new drug therapies to treat multiple serious neurological disorders that emerge in early childhood and have no or limited approved treatment options. Recognising the urgent unmet need, all programs have been granted "orphan drug" designation in the United States. Orphan drug designation provides incentives to encourage development of therapies for rare and serious diseases.

DAYBUE™ (trofinetide) is approved by the US Food and Drug Administration (FDA) and Health Canada for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren's second drug candidate, NNZ-2591, is in development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the Board 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.