Neuren (NEU) – ASX Announcement **20 October 2022**

Neuren receives US$10 million milestone payment

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has now received the milestone payment of US$10 million from its US partner Acadia Pharmaceuticals (Nasdaq: ACAD). The payment was due following the US Food and Drug Administration (FDA) acceptance for review of Acadia's New Drug Application (NDA) of trofinetide for the treatment of Rett syndrome. The FDA granted a Priority Review and assigned a Prescription Drug User Fee Act (PDUFA) action date of 12 March 2023.

Acadia has exclusive rights to develop and commercialize trofinetide in North America. Neuren retains all rights to trofinetide for all countries outside North America and has a fully paid-up, irrevocable licence for use in those countries to all data generated by Acadia. The development and commercialisation of trofinetide in North America is fully funded by Acadia.

If the NDA is approved by the FDA, the next potential milestone payment to Neuren would be US$40 million (A$62 million at an assumed exchange rate of 0.65), payable following the first commercial sale of trofinetide in the United States. Subsequently, Neuren is eligible to receive double-digit percentage royalties on net sales of trofinetide in North America, plus milestone payments of up to US$350 million (A$538 million) on achievement of a series of four thresholds of total annual net sales, plus one third of the market value of a Rare Pediatric Disease Priority Review Voucher if awarded by the FDA upon approval of the NDA, with the one third share estimated by Neuren as US$33 million (A$51 million).

About Neuren

Neuren is developing two new drug therapies to treat multiple serious neurological disorders that emerge in early childhood, none of which have any approved medicines.

A New Drug Application for the lead compound, trofinetide, to treat Rett syndrome is under Priority Review by the US Food and Drug Administration (FDA), with a PDUFA action date of 12 March 2023. 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 conducting Phase 2 trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome, Pitt Hopkins syndrome and Prader-Willi syndrome.

Recognising the urgent unmet need, all six 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.

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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.