



Neuren (NEU) – ASX Announcement

7 June 2023

Neuren receives US\$40m milestone payment

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today reported that it has received the milestone payment of US\$40 million earned following the first commercial sale of DAYBUE™ (trofinetide) by Neuren’s North America partner Acadia Pharmaceuticals (NASDAQ: ACAD). DAYBUE was approved by the US Food and Drug Administration (FDA) on 10 March 2023 and is the first and only drug approved by the FDA for the treatment of Rett syndrome.

Neuren and Acadia have an exclusive license agreement for Acadia to develop and commercialise trofinetide for the treatment of Rett syndrome and other indications in North America. Neuren is eligible to receive ongoing royalties on net sales of trofinetide in North America, plus milestone payments of up to US\$350 million on achievement of a series of four thresholds of total annual net sales, plus one third of the market value of the Rare Pediatric Disease Priority Review Voucher that was awarded to Acadia by the FDA upon approval of the NDA, with the one third share estimated by Neuren as US\$33 million. No royalties or similar costs are payable by Neuren to third parties, which means that Neuren’s revenue from Acadia will flow through to pre-tax profit.

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.

DAYBUE™ (trofinetide) is approved by the US Food and Drug Administration (FDA) for the treatment of Rett syndrome in adult and pediatric patients two years of age and older. 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 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.