Neuren (NEU) - ASX Announcement **22 August 2018**

Neuren receives initial payment of US$10 million from ACADIA

Melbourne, Australia, 22 August 2018: Neuren Pharmaceuticals (ASX: NEU) today announced that it has received the initial payment of US$10 million (approximately A$13.6 million) under the recently announced license agreement with ACADIA Pharmaceuticals Inc.

Under the agreement, ACADIA has been granted exclusive rights to develop and commercialise trofinetide for all clinical indications in North America. Neuren has retained all commercial rights to trofinetide outside North America and has free access and rights to use all the technical, clinical and regulatory data that will be generated by ACADIA in the US.

Neuren is completing certain in-progress manufacturing activities and the remainder of the second non-clinical chronic toxicology study. ACADIA has assumed responsibility for funding and executing all remaining development activities for trofinetide in all indications, including the single Phase 3 trial for Rett syndrome which is due to commence in the second half of 2019. Neuren is already working closely with ACADIA's clinical, regulatory and manufacturing teams, as drug supplies for the Phase 3 trial are manufactured and clinical protocols, sites and logistics are prepared. ACADIA's investment for Rett syndrome alone prior to a US marketing application is expected to be approximately US$55 million.

Rett syndrome has been estimated to occur in 1 in 10,000 to 15,000 live female births worldwide. For North America, ACADIA has indicated the peak annual sales potential for trofinetide in Rett syndrome as more than US$500 million. ACADIA will pay to Neuren double-digit percentage royalties on net sales of trofinetide across all indications in North America. These royalties provide the largest component of value to Neuren from the payments to be received from ACADIA.

Trofinetide in Rett syndrome and Fragile X syndrome is covered by issued patents to 2032 as well as by orphan drug exclusivity periods following marketing approval of up to 7.5 years in the US and up to 12 years in the EU.

In addition to the royalties on trofinetide sales in North America, Neuren is eligible to receive:

- Up to US$105 million on achievement of US development milestones for Rett syndrome and Fragile X syndrome.
- Up to US$350 million on achievement of thresholds of total annual net sales of trofinetide in North America, all of which Neuren expects ACADIA to achieve if trofinetide is approved for Rett syndrome and Fragile X syndrome.
- One third of the value of any Rare Pediatric Disease Priority Review Voucher awarded by the FDA on approval of a New Drug Application for trofinetide.

With the ACADIA partnership in place and the initial payment of US$10 million received, Neuren is now well placed to consider development and commercialization alternatives for trofinetide outside North

America, including in Europe and Japan, as well as being able to advance the development of Neuren's second patented drug compound, NNZ-2591.

Neuren Executive Chairman Richard Treagus commented: "ACADIA has demonstrated strong strategic intent and commitment to trofinetide and we have full confidence in their development and commercialization capabilities, as well as their funding capacity. The partnership has secured the capabilities and funding required to bring trofinetide to market, while retaining for Neuren a very significant participation in the value of trofinetide in North America, all the value of trofinetide outside North America and the ability to advance NNZ-2591."

About Rare Pediatric Disease Priority Review Vouchers

Under Section 529 to the Federal Food, Drug, and Cosmetic Act (FD&C Act), the US Food and Drug Administration (FDA) will award priority review vouchers to sponsors of rare pediatric disease product applications that meet certain criteria. A sponsor may choose to request rare pediatric disease designation from the FDA prior to submitting a New Drug Application (NDA), however an application for the voucher itself must be submitted with the NDA and the voucher is awarded on approval of the NDA. A voucher can be redeemed to receive a priority review of a subsequent marketing application for a different product, which can reduce the target review time for that product to 6 months, compared with the standard 10 months. A voucher can be sold to another sponsor. The market value of the voucher depends on supply and demand. The value derives from three potential factors: shifting sales earlier, longer effective patent life due to earlier entry, and competitive benefits from earlier entry relative to competitors. Earlier market entry can be worth hundreds of millions of dollars to the voucher holder. In 2014, the first voucher was sold for US$67.5 million. In 2015, a voucher was purchased for US$350 million. In 2017, five vouchers sold for prices between US$110 million and US$150 million.

About Neuren and trofinetide

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. Neuren has completed Phase 2 development of trofinetide for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs for trofinetide in Rett syndrome and Fragile X syndrome have each been granted Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Trofinetide is a synthetic analogue of a naturally occurring neurotrophic peptide derived from IGF-1, a growth factor produced by brain cells. In animal models, trofinetide exhibits a wide range of important effects including inhibiting neuroinflammation, normalizing the role of microglia, correcting deficits in synaptic function and regulating oxidative stress response.

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Forward-looking Statements
This ASX-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.