Neuren (NEU) - ASX Announcement **4 March 2020**

Commentary on potential benefit to Neuren of Rare Pediatric Disease designation

Highlights:

- **Marketing approval of a product with Rare Pediatric Designation confers eligibility to receive a Priority Review Voucher from FDA**
- **Voucher may be sold – sale price of vouchers in April 2019 and July 2019 was US$95-105 million[1]**
- **Under Licence Agreement, Neuren receives from ACADIA one third of the market value of a voucher**
- **Phase 3 trial enrolling, first patients have completed LAVENDER and commenced LILAC**
- **LAVENDER results expected in 2021, with potential for marketing approval in 2022**

Melbourne, Australia, 4 March 2020: Neuren Pharmaceuticals (ASX: NEU) and ACADIA Pharmaceuticals (Nasdaq: ACAD) earlier today reported that ACADIA's program to develop trofinetide for Rett syndrome in the United States has received Rare Pediatric Disease designation from the US Food and Drug Administration (FDA).

On marketing approval of a product with Rare Pediatric Disease designation, the sponsor is eligible to receive a Priority Review Voucher, which can be used to obtain FDA review of a New Drug Application for another product in an expedited period of 6 months. The voucher may also be sold for use by another company. Under the terms of the Licence Agreement between Neuren and ACADIA, Neuren will receive from ACADIA one third of the market value of a Priority Review Voucher. In January 2020, the Report to Congressional Committees on FDA's Priority Review Voucher Programs noted that vouchers were sold in April 2019 and July 2019 for US$105 million and US$95 million respectively[1].

ACADIA initiated the Rett syndrome Phase 3 program in the United States in October 2019. The program involves treatment of approximately 180 females aged 5 to 20 with trofinetide or placebo for 12 weeks to evaluate efficacy and safety (the "LAVENDER" study), following which patients are eligible to continue treatment with trofinetide for 40 weeks to provide longer-term safety data (the "LILAC" study). The first patients enrolled have completed LAVENDER and commenced LILAC. Results from the LAVENDER study are expected in 2021, with the potential for marketing approval in 2022.

The Rare Pediatric Disease Priority Review Voucher Program is designed to incentivize companies to develop treatments for diseases or conditions that are serious or life-threatening in children under the age of 18 and that affect less than 200,000 persons in the United States.

[1] Source: GAO-20-251 Report to Congressional Committees, January 2020

About Neuren

Neuren is developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Neuren's lead drug candidate trofinetide is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs in Rett syndrome and Fragile X syndrome have each received Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America, whilst retaining all rights outside North America. Neuren is advancing the development of its second drug candidate NNZ-2591 for Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, each of which has received Orphan Drug designation in the United States.

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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.