Neuren (NEU) - ASX Announcement **17 March 2020**

Neuren to capture substantially greater value by selecting best commercial outcome after US Phase 3 results in 2021

Highlights:

- **Excellent progress has been made by ACADIA in Rett syndrome US Phase 3 program:**
  - **First patients have completed placebo-controlled Lavender study and commenced open-label Lilac study**
  - **Lavender study results expected in 2021, with potential marketing approval in 2022**
  - **Rare Pediatric Disease designation from FDA recently granted**
  - **Neuren retains rights to all US data for registration and commercialization outside North America**
- **Neuren is now preparing for meetings with European regulatory authorities in 2020**
- **Value of trofinetide ex-North America rights will increase as development milestones are achieved**
- **Advised by Torreya, Neuren has considered a range of partnering proposals, and has concluded that selecting the optimum commercial outcome after Phase 3 results may capture substantially greater value and is in the best interests of shareholders**
- **Discussions with potential partners for Japan will continue in the interim**
- **Neuren making strong progress with NNZ-2591 for 3 neurodevelopmental disorders:**
  - **FDA granted Orphan Drug designation in each of Phelan-McDermid, Angelman and Pitt Hopkins syndromes**
  - **Efficacy demonstrated in all 3 mouse models and optimum dose clearly identified in Phelan-McDermid syndrome model to inform dose selection for Phase 2 trials**
  - **Preparation for Phase 1 trial and Phase 2 trials underway with support from patient advocacy groups and physicians**

Melbourne, Australia, 17 March 2020: Neuren Pharmaceuticals (ASX: NEU) today announced that it will defer partnering ex-North American rights to trofinetide in order to capture substantially greater value by selecting the optimum commercial outcome after Phase 3 trial results for Rett syndrome in the United States, which are expected in 2021. Under Neuren's licence agreement with North American partner ACADIA, Neuren has full and free access to use all US data for registration and commercialization of trofinetide outside North America. Neuren will now move forward with European regulatory authority meetings this year to discuss the Rett syndrome development program.

Advised by Torreya, Neuren has conducted a thorough process and considered a range of partnering proposals. Specific discussions initiated late in 2019 provided the basis for extending this process into

the first quarter of 2020. Notwithstanding the interest received, given the strong underlying progress made for both trofinetide and NNZ-2591, the Board has now concluded that it is in the best interests of shareholders to wait for additional development milestones, which if achieved may substantially increase the value of trofinetide and Neuren. Separate to this, discussions with potential partners for Japan are presently underway and will continue. The Board recognizes the specific benefits of working with an established partner to access the market in Japan.

ACADIA has made excellent progress in the United States, commencing the Phase 3 program for trofinetide in Rett syndrome in late 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 potential marketing approval in 2022.

ACADIA recently received Rare Pediatric Disease designation, which confirmed its eligibility upon marketing approval to receive a Priority Review Voucher. 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.

Whilst ACADIA has been advancing trofinetide, Neuren has continued to make strong progress with the development of NNZ-2591 for 3 neurodevelopmental disorders. The FDA granted Orphan Drug designation to Neuren for NNZ-2591 to treat each of Phelan-McDermid, Angelman and Pitt Hopkins syndromes. This was based on positive efficacy results in mouse models of all 3 conditions. In addition, Neuren recently reported compelling results in a dose-ranging study in the Phelan-McDermid syndrome model, which clearly identified the optimum dose to inform the dosing for clinical trials in patients. Neuren is now preparing for a Phase 1 trial in Australia and designing Phase 2 trials with support from patient advocacy groups and physicians.

Neuren Executive Chairman Richard Treagus commented: "Given the excellent progress made with both the trofinetide and NNZ-2591 programs and having carefully considered the partnering options, we believe the best outcome for shareholders will be achieved by carrying the positive momentum forwards into 2021 when the Rett syndrome phase 3 results are expected".

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.