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

Q1 2020 Activity Report

Summary of cash flows and related activity

At 31 March 2020 Neuren held cash and cash equivalents of $13.4 million, compared with $13.8 million at 31 December 2019. Net cash of $1.5 million was used in operating activities, offset by gains of $1.1 million in the value of US dollar cash holdings following a significant strengthening of the US dollar against the Australian dollar. During the quarter the non-clinical studies and preparation for a Phase 1 trial in Australia that Neuren is undertaking for the planned Investigational New Drug Application (IND) for NNZ-2591 in the United States continued, along with preparation for European regulatory authority meetings to discuss the Rett syndrome development program. R&D payments of $0.9 million mainly comprised payments for non-clinical studies and manufacture of NNZ-2591 for those studies and the Phase 1 trial.

Key events in the quarter

- The US Food and Drug Administration (FDA) granted Rare Pediatric Disease Designation to trofinetide for Rett syndrome. On marketing approval of a product with this 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. Vouchers were sold in April 2019 and July 2019 for US$105 million and US$95 million respectively.
- Neuren announced compelling results from a dose ranging study of NNZ-2591 in a model of Phelan-McDermid syndrome:
    - Clear dose response demonstrated with 4 escalating dose levels
    - Optimum dose identified, informing dose selection for planned Phase 2 clinical trials
    - Better efficacy achieved after 6 weeks treatment compared with 3 weeks at the same dose
- Neuren decided to 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. Under Neuren's licence agreement with ACADIA, Neuren has full and free access to use all US data for registration and commercialization of trofinetide outside North America.
- Due to the COVID-19 pandemic, ACADIA temporarily paused the enrolment of new patients in the Phase 3 LAVENDER study until it believes it has the ability to collect data from new patients while ensuring their safety. This modification did not impact patients already enrolled in the LAVENDER study. In addition, Neuren deferred the commencement of Phase 2 trials of NNZ-2591 and associated expenditure, with the intention to accelerate this when it is able to do so.

- The international and institutional make-up of Neuren's share register was enhanced significantly by the acquisition of approximately 14% of Neuren's shares by funds managed by Karst Peak Capital Limited, an international investment management firm focused on equity investments in healthcare and technology.

Listing Rule 4.7C.3

In item 6 of the Appendix 4C cash flow report for the quarter, payments to Related Parties of approximately $150,000 comprised fees to executive and non-executive directors.

About Neuren

Neuren is developing new therapies for debilitating neurodevelopmental disorders that emerge in early childhood and are characterized by impaired connections and signalling between brain cells. The therapies utilize synthetic analogs of neurotrophic peptides that occur naturally in the brain. 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 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 NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, 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.