Neuren (NEU) – ASX announcement **5 August 2021**

Rett syndrome Phase 3 trial enrolment successfully completed – results in Q4 2021

Highlights:

- **Acadia has successfully completed the planned enrolment of patients into the LAVENDER Phase 3 trial of trofinetide in Rett syndrome**
- **Top-line results from this pivotal Phase 3 trial are on track for Q4 2021**
- **The randomised, double-blind, placebo-controlled Phase 3 trial is testing treatment of approximately 180 patients for 12 weeks**
- **Rett syndrome is a severely debilitating neurodevelopmental disorder for which there are currently no approved medicines**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that its US partner for trofinetide, Acadia Pharmaceuticals (Nasdaq: ACAD), has successfully completed enrolment in the LAVENDER Phase 3 trial of trofinetide in Rett syndrome, remaining on track for top-line results in Q4 2021. LAVENDER is a randomised, double-blind, placebo-controlled Phase 3 trial testing treatment of approximately 180 patients for 12 weeks with trofinetide or placebo.

Rett syndrome is a debilitating neurodevelopmental disorder estimated to affect between 1 in 10,000 and 1 in 15,000 females worldwide. A range of severe impairments emerge in infancy, affecting nearly every aspect of the child's life: their ability to speak, walk, eat, and even breathe. There are currently no medicines approved for Rett syndrome. The trofinetide program has Fast Track, Orphan Drug and Rare Pediatric Disease designations from the US Food and Drug Administration (FDA).

Neuren CEO Jon Pilcher commented: "This successful completion of enrolment is a very important milestone for Neuren and we are now excited to await results from the Phase 3 trial before the end of 2021. Given the many challenges presented by the pandemic over the last 18 months, this is a great achievement by Acadia and reflects the remarkable commitment and determination of the Rett syndrome community in the United States."

The development and commercialisation of trofinetide in North America is fully funded by Acadia. Neuren is eligible to receive potential milestone payments of up to US$455 million, plus tiered escalating double-digit percentage royalties on net sales of trofinetide in North America, plus one third of the market value of a Rare Pediatric Disease Priority Review Voucher if awarded by the FDA upon approval of a New Drug Application for trofinetide. Further, Neuren has free and full access to all data for use in countries outside North America.

Near-term potential financial impact on Neuren

If the results of the Phase 3 trial are positive, a New Drug Application is approved by the FDA and trofinetide is launched in the US, Neuren would earn revenue over 2022 and 2023 for Rett syndrome in the US alone of A$111 million plus double-digit percentage royalties on net sales. This assumes a USD/AUD exchange rate of 0.75 and that Neuren receives US$33 million as its share of the market value of a Rare Pediatric Disease Priority Review Voucher awarded on approval of a New Drug Application. There is no cost associated with this revenue. Positive results from the Phase 3 trial should also enable Neuren to commercialise trofinetide in Europe and Asia.

2021 milestones progress

- ✓ *EU Orphan designations for Phelan-McDermid, Angelman, and Pitt Hopkins*
- ✓ *Successful Phase 1 trial results for NNZ-2591*
- ✓ *Prader-Willi syndrome added to NNZ-2591 pipeline*
- ✓ *Complete drug substance manufacturing for NNZ-2591 Phase 2*
- ✓ *Pre-IND meetings with FDA to agree NNZ-2591 Phase 2 plans*
- ✓ ***Acadia completes enrolment in trofinetide Rett syndrome Phase 3***
- **Submit NNZ-2591 INDs to FDA**
- **Commence NNZ-2591 Phase 2 trials**
- **Orphan designation for Prader-Willi syndrome**
- **Trofinetide Rett syndrome Phase 3 top-line results**

About Neuren

Neuren is developing two new drug therapies to treat multiple serious neurological disorders that emerge in early childhood, none of which have any approved medicines.

The lead compound, trofinetide, is currently in a Phase 3 clinical trial for Rett syndrome with top-line results expected in Q4 2021 and has completed a Phase 2 clinical trial in Fragile X syndrome. Both programs have Fast Track designation from the US Food and Drug Administration (FDA). 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 preparing to initiate Phase 2 trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome in H2 2021. Neuren is also planning a Phase 2 trial in Prader-Willi syndrome.

Because of the urgent unmet need, five programs have been granted "orphan drug" designation in both the United States and the European Union, a designation that 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.