Neuren (NEU) - ASX Announcement **25 August 2016**

Neuren expands Phase 2 Rett syndrome pediatric clinical trial

Highlights:

- **Neuren has completed a detailed evaluation of a range of strategic options.**
- **Strong commercial interest was registered in the Rett syndrome and Fragile X syndrome programs, with the outcome of the current Phase 2 Rett syndrome pediatric clinical trial confirmed as an important and valuable milestone.**
- **Neuren will consider partnering options in the major markets once results of the pediatric trial are available.**
- **Rett syndrome pediatric trial update:**
    - Enrolment rate is running ahead of schedule.
    - No safety or tolerability signals identified in the blinded trial to date.
    - Faster enrolment and good subject availability provides an opportunity to expand trial numbers beyond the original target of 64 completers.
    - Additional subjects will be randomized on 1:1 basis between 200mg/kg (highest dose) and placebo in order to maximize the power of the trial.
    - Top-line results from expanded trial still expected in Q1 2017.
- **Rett syndrome Phase 3 timelines revised, with anticipated commencement in 2018**.
- **Neuren will seek shareholder approval required under the New Zealand Takeovers Code to give the board the flexibility to issue a limited number of additional shares to interests of its major shareholder, Mr Lang Walker.**

Melbourne, Australia, 25 August 2016: Neuren Pharmaceuticals (ASX: NEU) today provided an update on the development of trofinetide for the treatment of Rett syndrome. Neuren is currently conducting a randomized, double-blind, placebo-controlled Phase 2 clinical trial for girls aged 5 to 15 years with Rett syndrome.

Neuren has undertaken and now completed a wide-ranging evaluation of strategic options, including partnering, assisted in this process by Leerink Partners, a leading US investment banking firm specializing in healthcare. A number of international pharmaceutical companies expressed interest in Neuren's Rett syndrome and Fragile X syndrome programs. Detailed discussions with those companies have indicated that the results of the Rett syndrome trial, due in the first quarter of 2017, will be an important and valuable component of the trofinetide development package. Neuren recognizes the potential commercial value this could represent and therefore has determined that partnering in the major markets is best considered once the pediatric trial results are available.

The trial is in progress at 12 sites in the United States, led by clinicians experienced in the diagnosis and treatment of Rett syndrome. The total duration of the trial, from screening through to follow-up, is eleven weeks. Enrolment of girls into the trial has progressed ahead of schedule. To date, 49 subjects have been randomized and 5 are in screening. 18 subjects are scheduled to enter screening before the end of September and the trial sites have confirmed that more are available, which means that Neuren has the opportunity to expand the trial beyond the original target of 64 completing subjects, whilst still delivering top-line results in the first quarter of 2017.

The initial 64 subjects are being randomized into four dose groups: 50mg/kg, 100mg/kg, 200mg/kg and placebo. To date in the blinded trial, no safety or tolerability issues have emerged. Neuren therefore has confidence in randomizing the additional subjects into only two groups, being the highest dose and placebo, on a 1:1 basis. This will maximize the sample size and increase the statistical power of the comparison between the highest dose and placebo groups.

In order to fund the expansion of the Rett syndrome trial, Neuren has elected to defer further investment in certain other trofinetide development activities, including chronic toxicity studies and manufacturing, until the results of the pediatric trial are available. This will result in the anticipated commencement of a Phase 3 trial in Rett syndrome being revised from late 2017 to 2018.

The Neuren board wishes to increase the Company's funding flexibility during this important period. Neuren's largest shareholder Mr Lang Walker, who currently has an interest in approximately 19% of Neuren's shares, has expressed a willingness to support that aim. Under the New Zealand Takeovers Code, Neuren may not issue shares that would increase Mr Walker's interests above 20%, unless approved by the other shareholders. Therefore the board intends in coming weeks to seek shareholder and regulatory approvals that would enable Neuren to issue a limited number of additional shares to interests of Mr Walker, if it is considered by the board to be both necessary and in the best interests of all shareholders. Full details of the proposal for approval will be contained in a notice of meeting.

Neuren Executive Chairman Richard Treagus commented: "The Rett syndrome community's support for our pediatric clinical trial is evidenced by the strong enrolment rate and excellent study compliance. We believe the opportunity to enroll additional girls into the trial while still delivering top-line results in Q1 2017 is in the best interests of all Neuren's stakeholders. We look forward to making the pediatric study results available early in 2017 and at that point assessing the optimum path to market for trofinetide."

About trofinetide

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. Trofinetide is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The most advanced program is for Rett syndrome, supported by rettsyndrome.org. Both the Rett syndrome and Fragile X syndrome programs have been granted Fast Track designation by the US Food and Drug Administration (FDA) and have orphan drug designation in both the United States and the European Union. Following marketing authorization, orphan drug designation provides a market exclusivity period of 7 years in the United States and 10 years in the European Union.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions as well as acute neurological injuries. Neuren presently has a clinical stage molecule, trofinetide in Phase 2 clinical trials as well as NNZ-2591 in pre-clinical development.

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.

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