Neuren (NEU) – ASX Announcement **15 February 2021**

Successful Phase 1 trial for Neuren's NNZ-2591

Highlights:

- **Twice daily oral dosing for seven days was well tolerated at all dose levels tested**
- **No Serious Adverse Events (SAEs)**
- **No clinically significant findings from safety lab tests, vital signs, or cardiac tests**
- **All adverse events (AEs) were mild or moderate and resolved during the trial**
- **All subjects completed dosing, apart from one subject on lowest dose**
- **PK analyses are ongoing**
- **Neuren preparing for FDA meeting, IND Applications and Phase 2 trials**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today reported that in its Phase 1 trial twice daily oral dosing of NNZ-2591 for seven days was safe and well tolerated in healthy volunteers.

Data from the Phase 1 trial will form part of Neuren's planned Investigational New Drug (IND) Applications to the US Food and Drug Administration (FDA), in preparation for Phase 2 trials in Phelan McDermid, Angelman and Pitt Hopkins syndromes in 2021.

Neuren CEO Jon Pilcher commented: "This trial was the first human dosing for NNZ-2591 and we are very pleased with the outcome. Twice daily oral dosing for seven days was safe and well tolerated at doses we expect to be within the effective therapeutic range, which gives us confidence for dosing patients in our planned Phase 2 trials."

Top-line results from the trial

The trial was conducted under GCP at commercial clinical trial facilities in Perth and Sydney. The primary objective was to evaluate safety and tolerability, with a secondary objective to evaluate pharmacokinetic (PK) parameters. Two double-blind placebo-controlled cohorts of eight healthy adult volunteers were dosed orally twice per day for seven days. In each cohort, six subjects received NNZ-2591 and two subjects received placebo. Each cohort was titrated up to the target dose, with the target dose in the second cohort double the target dose in the first cohort. These two cohorts were preceded by preliminary testing of single oral doses of NNZ-2591, which enabled modelling of potential multiple dosing regimens.

Adverse Events (AEs) were recorded and blood samples were analysed for safety parameters. Cardiac tests and neurological examinations were also carried out. Blood samples were collected for the PK analyses, which are currently ongoing.

No Serious Adverse Events (SAEs) were reported. All reported AEs were mild or moderate and resolved during the trial. There were no clinically significant findings from safety laboratory tests, vital signs, or cardiac tests.

In the seven days' dosing cohorts, the most common AE reported was drowsiness. In the higher dose cohort only one of the reported AEs was moderate, the remainder were mild. All subjects completed the scheduled dosing, apart from one of the eight subjects in the lower dose cohort, who ceased dosing after receiving the first starting dose following moderate drowsiness and incoordination.

About Neuren

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

The lead drug compound, trofinetide, is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. Both programs have been granted Fast Track designation by 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 plans 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 2021.

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