Neuren Shareholder Update, January 2016

Highlights:

- **Neuren achieves significant progress during 2015:**
  - **Successful proof of concept clinical trials for trofinetide in Rett syndrome and Fragile X syndrome**
  - **Orphan drug designation for Rett syndrome and Fragile X syndrome granted in US and EU**
  - **New US patent issued, covering use of trofinetide in Rett syndrome**
  - **Intrepid (TBI) clinical trial enrolment completed**
  - **Cash reserves strengthened ensuring the company is fully funded for 2016**
- **Neuren sets out a clear strategy for 2016:**
  - **Clinical trial in young girls with Rett syndrome scheduled to commence in Q1 2016**
  - **Meeting with FDA in H1 2016 to discuss further development plans for Fragile X syndrome**
  - **Intrepid (TBI) clinical trial top-line results expected in April 2016**
  - **Completion of commercial manufacturing process optimisation**
  - **Leading US healthcare investment bank Leerink Partners appointed to advise Neuren's board**

Melbourne, Australia, 4 January 2016: Neuren Pharmaceuticals (ASX: NEU) today provided an update for shareholders on the Company's progress to date, as well as the key objectives for 2016.

Clinical Development

Over the last 18 months, Neuren has focused on the execution of four Phase 2 clinical trials of trofinetide in neurodevelopmental disorders and traumatic brain injury. The trials in Rett syndrome and Fragile X syndrome were successfully completed and produced highly encouraging results.

The challenges encountered in enrolling subjects at critical care hospitals for the "Intrepid" Phase 2 trial in moderate-to-severe traumatic brain injury were effectively addressed during 2015 and this allowed full subject enrolment to be completed in October. The enrolment rate in the concussion trial currently being undertaken with the US Army continues to be slower than originally anticipated and as such those trial timelines remain under review.

Regulatory and patents

Important regulatory and commercial progress was made during the year. Neuren received orphan drug designation from the United States Food and Drug Administration (FDA) and the European Medicines Agency

(EMA) for both Rett syndrome and Fragile X syndrome. A productive meeting was held with the Division of Neurology of the FDA, which provided Neuren with guidance on the remaining development required for Rett syndrome.

Additional patent protection was achieved in the United States with the grant of a new patent for the use of trofinetide to treat Rett syndrome. Further international applications covering neurodevelopmental disorders are currently pending.

Financing

Neuren continued to receive strong support for its development programs in Rett syndrome and Fragile X syndrome from the leading patient advocacy organizations rettsyndrome.org, FRAXA Research Foundation and the National Fragile X Foundation. Rettsyndrome.org has agreed to provide support and funding of up to US$1 million towards the cost of Neuren's next clinical trial. Additionally, Neuren raised $6.3 million through a share placement in November 2015, thereby strengthening cash reserves and ensuring the Rett syndrome development program can proceed without delay through 2016.

Strategy for 2016

Neuren is therefore pleased to report that it is in a strong position and is approaching 2016 with a clearly defined strategy.

Firstly, the Phase 2 clinical trial in girls aged 5 to 15 with Rett syndrome will commence in the first quarter and is expected to be complete in the fourth quarter of 2016. In this younger population, the trial will test higher doses of trofinetide for a longer treatment period of 6 weeks. Further dialogue with the FDA will seek to confirm the requirements for a registration Phase 3 trial planned for 2017.

Secondly, Neuren expects to meet with the Division of Psychiatry of the FDA in the first half of 2016 to seek input on the further development of trofinetide for Fragile X syndrome.

Thirdly, top-line results from the Intrepid trial are expected to be available in April 2016. This trial has been supported and funded through an extensive collaboration with the US Army.

Fourthly, Neuren's investment in optimizing and scaling up the commercial manufacturing process for trofinetide is due to complete in 2016. This investment has been required to support future New Drug Applications as well as commercial product supply for all trofinetide indications.

Corporate

Neuren's board believes that trofinetide holds significant value as a potential new medical treatment, but also recognizes the extent of the resources required to ensure that full value can be achieved across all clinical indications. The board therefore considers it appropriate to examine fully all available options to ensure that trofinetide is developed and commercialized as quickly as possible for the benefit of all stakeholders. A number of international pharmaceutical companies have expressed interest in the trofinetide development programs over the last 12 months following the release of the clinical trial results. Neuren has therefore engaged Leerink Partners (www.leerink.com), a leading US investment banking firm specializing in healthcare, as its sole corporate adviser to assist the board in evaluating all future options available to the Company.

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 and correcting deficits in synaptic function. Trofinetide is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The intravenous form of trofinetide is presently in a Phase 2 clinical trial in patients with moderate to severe traumatic brain injury. The oral form of trofinetide is in Phase 2 development in Rett syndrome, Fragile X syndrome and mild traumatic brain injury (concussion). Three programs have received Fast Track designation from the US FDA and the Rett syndrome and Fragile X syndrome programs have also received Orphan Drug designation in the United States and 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.

For more information, please contact:
Dr Richard Treagus, Executive Chairman: rtreagus@neurenpharma.com ; +61 417 520 509