



**Neuren (NEU) - ASX Announcement**

**12 December 2019**

## **2019 YEAR-END UPDATE AND 2020 OUTLOOK**

**Melbourne, Australia, 12 December 2019:** Neuren Pharmaceuticals (ASX: NEU) Executive Chairman Richard Treagus today provided an update on the strong position of the business approaching the end of 2019 and the positive outlook heading into 2020:

“In September we highlighted two very important milestones that were anticipated for the 4<sup>th</sup> quarter of 2019. Each of those milestones was achieved; firstly our US partner ACADIA commenced the LAVENDER Phase 3 trial of trofinetide in Rett syndrome and secondly the US Food and Drug Administration granted all three of our Orphan Drug designation applications for NNZ-2591 to treat each of Phelan-McDermid, Angelman and Pitt Hopkins syndromes.

In addition a new patent was granted in Europe for NNZ-2591 treating neurodevelopmental disorders, which means we now have patents to 2034 in each of the key markets of the US, Europe and Japan.

Over the course of 2019 Neuren, assisted by Torreyia, has actively engaged with third parties both in and outside the US to evaluate potential transactions. We have received a number of proposals to date which have led to more detailed discussions. Throughout these discussions Neuren has remained clear on what it considers to be an acceptable range of possible outcomes. In recent weeks the process has advanced further, with a level of interest that the Neuren Board regards as meaningful and appropriate. Whilst there remains no certainty as to the outcome or timing, the current specific discussions form a good basis to extend the Torreyia process into the new year.

Neuren is approaching the end of the year in a strong position, advancing 2 valuable drugs to treat 5 debilitating childhood disorders which currently have no approved therapies. The lead program is in Phase 3 in the US, fully funded and executed by ACADIA, and we believe it should satisfy the FDA’s criteria for a Rare Pediatric Disease designation. Meanwhile the Neuren team is continuing to make very good progress towards clinical trials of NNZ-2591 in 2020, building on the highly encouraging results in animal models that were reviewed by the FDA before granting Orphan Drug designation.

In summary, there are 3 significant future value drivers for the Neuren business; firstly the opportunity to realise Neuren’s share of trofinetide value in the US through ACADIA’s Phase 3 results and New Drug Application, secondly appointing a commercial partner for trofinetide in the major ex-US markets using the US data for registration, and thirdly demonstrating the clinical benefit of NNZ-2591 in 3 valuable indications. These elements have been comprehensively described in the reports and current valuations<sup>1</sup> recently published by Bell Potter and MST Access, the analysts that currently cover Neuren.”



## **About Neuren**

Neuren is developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Neuren's lead drug candidate 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 in Rett syndrome and Fragile X syndrome 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 its second drug candidate NNZ-2591 for Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, each of which has 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.*

<sup>1</sup>*Disclaimer: Neuren does not endorse, confirm, or express a view as to the accuracy of the analyst valuations.*