Neuren (NEU) - ASX Announcement **20 September 2019**

Neuren approaching key milestones in Q4 2019

Melbourne, Australia, 20 September 2019: Neuren Pharmaceuticals (ASX: NEU) Executive Chairman Richard Treagus today commented on the outlook for Q4 2019:

"Neuren is approaching an exciting fourth quarter of 2019, with some important milestones for the business anticipated.

Firstly, our North American partner ACADIA plans to commence the "LAVENDER" Phase 3 trial of trofinetide in Rett syndrome. The trial aims to achieve the outcome seen in Neuren's successful Phase 2 pediatric trial, enhanced by incorporating twice the treatment duration, an optimized dosing regimen and a higher statistical powering associated with a much larger sample size. The partnership with ACADIA is working very well and importantly is delivering in respect of the execution and funding of the many preparatory activities necessary for the Phase 3 program. More broadly, ACADIA continues to demonstrate its capabilities in the development and commercialization of innovative therapies to address unmet needs in central nervous system disorders and its market capitalization now stands in excess of US$6 billion.

Secondly, we expect the US Food and Drug Administration (FDA) to complete its review of the three applications for Orphan Drug designation for NNZ-2591 in each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome that Neuren submitted at the end of July. Orphan Drug designation is an important commercial milestone which if granted will add significant value and momentum in respect of our plans to move into clinical trials in 2020.

Our process to evaluate potential corporate transactions, advised by Torreya a global investment bank specializing in life sciences, will continue in the fourth quarter given the importance of these near term milestones."

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Neuren completed Phase 2 development of trofinetide 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.

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.

Contact:

Jon Pilcher, CFO & Company Secretary: jpilcher@neurenpharma.com; +61 438 422 271