Neuren (NEU) - ASX Announcement **29 July 2019**

Neuren submits 3 Orphan Drug applications to FDA for NNZ-2591

Melbourne, Australia, 29 July 2019: Neuren Pharmaceuticals (ASX: NEU) announced today that it has submitted 3 applications for Orphan Drug designation to the US Food and Drug Administration. The applications are for its drug candidate NNZ-2591 to treat each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, for which there are currently no approved drug therapies. Neuren recently announced positive results with NNZ-2591 in separate mouse models of each of these debilitating neurodevelopmental disorders.

Each disorder is caused by a mutation or deletion in a different gene or chromosomal region; however they share many common symptoms and an underlying impairment in the connections and signaling between brain cells. The aim of treatment with NNZ-2591 is to restore normal functional connectivity and signaling.

Neuren is currently undertaking the manufacturing development and non-clinical studies required before submitting an Investigational New Drug (IND) Application in the United States and commencing Phase 2 clinical trials in the second half of 2020.

Neuren Executive Chairman Richard Treagus commented: “These 3 Orphan Drug applications are an important first interaction with the FDA as Neuren progresses the development of NNZ-2591 for these debilitating disorders. We are focused on being ready to start trials in patients next year.”

Orphan Drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition. Amongst other incentives, Orphan Drug designation qualifies the sponsor of the drug for 7 years of marketing exclusivity, plus 6 months if approved for pediatric use, as well as waiver of the prescription drug user fee for a marketing application.

About Neuren and NNZ-2591

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.

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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.