



Neuren (NEU) - ASX Announcement

16 August 2019

New patent granted for Neuren's NNZ-2591 in Japan

Melbourne, Australia, 16 August 2019: Neuren Pharmaceuticals (ASX: NEU) today announced the grant by the Japan Patent Office of a new patent for NNZ-2591. After issue the patent entitled "Bicyclic compounds and methods for their use in treating autism spectrum disorders and neurodevelopmental disorders" will expire in July 2034. A patent derived from the same international application has already been issued in the United States and others are pending in the United States and in Europe. These patents supplement issued composition of matter patents in the United States, Europe and Japan.

Neuren recently announced positive results with NNZ-2591 in mouse models of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome, three debilitating neurodevelopmental disorders for which there are currently no approved drug therapies. 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 has submitted applications to the US Food and Drug Administration (FDA) for Orphan Drug designation in each of these three indications.

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.

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.



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.

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