Neuren (NEU) – ASX Announcement **8 August 2025**

Neuren adds *SYNGAP1*-related disorder to NNZ-2591 pipeline

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced the addition of *SYNGAP1*-related disorder (SRD) into its neurodevelopmental disorders pipeline for NNZ-2591, following positive results in a pre-clinical model. There are currently no approved treatments for SRD. The incidence of the genetic cause of SRD has been reported as 1 per 16,000 individuals.

SRD is caused by a variant on the *SYNGAP1* gene located on Chromosome 6, which is responsible for producing the SYNGAP1 protein. The protein acts as a regulator in the synapses and insufficient production leads to impaired communication between neurons. This results in the many neurological issues seen in *SYNGAP1* patients, including intellectual disability, low muscle tone, global development delay, epilepsy, sensory processing disorder, gross and fine motor skill delays, coordination disorder, speech delay, sleep and behavior disorder and autism spectrum disorder.

In an in-vitro model of SRD in human iPSC-derived neurons, treatment with NNZ-2591 reversed the neuronal dysfunction caused by *SYNGAP1* haploinsufficiency.

Neuren is developing NNZ-2591 to treat multiple neurodevelopmental disorders for which there are no approved treatments and is preparing to commence a Phase 3 clinical trial in Phelan-McDermid syndrome.

More information about SRD can be viewed on the website of the SYNGAP Research Fund: https://curesyngap1.org/

About Neuren

Neuren is developing new drug therapies to treat multiple serious neurological disorders that emerge in early childhood and have no or limited approved treatment options. Recognising the urgent unmet need, programs have been granted “orphan drug” designation in the United States, which provides incentives to encourage the development of therapies for rare and serious diseases.

DAYBUE™ (trofinetide) is approved by the US Food and Drug Administration (FDA) and Health Canada for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren’s second drug candidate, NNZ-2591, is in development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the Board 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.