Neuren (NEU) – ASX Announcement **13 May 2025**

Neuren receives FDA minutes and re-confirms primary endpoints for Phase 3 trial

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today confirmed that it has received the official minutes of its Type C Meeting with the US Food and Drug Administration (FDA), held on 8 April to discuss efficacy endpoints for its upcoming Phase 3 clinical trial of NNZ-2591 to treat Phelan-McDermid syndrome.

As announced last month by Neuren following the Meeting, the co-primary endpoints for the single Phase 3 pivotal trial will be the change from baseline in the Receptive Communication sub-domain of the Vineland Adaptive Behavior Scales, Third Edition (VABS-3 Receptive-Raw Score) and the overall score in the Phelan-McDermid Syndrome Assessment of Change (PMSA-C, previously referred to as CGI-I in Neuren's Phase 2 trial). Both measures were robustly positive with clinically meaningful improvement in Neuren's Phase 2 open-label clinical trial. 16 out of 18 children showed improvement measured by the VABS-3 Receptive-Raw Score, with mean improvement of 7.5 from a mean baseline of 29.0 (Wilcoxon signed rank test $p=0.0001$) and 16 out of 18 children showed improvement from baseline measured by the PMSA-C with a mean score of 2.4 (Wilcoxon signed rank test $p<0.0001$).

Neuren remains on-track to commence the Phase 3 trial mid-year 2025, subject to FDA review of the final version of the trial protocol. Neuren's financial strength means that no additional funding is required to execute the program.

About Phelan-McDermid syndrome (PMS)

Phelan-McDermid syndrome is caused by a deletion or other change in the 22q13 region of chromosome 22, which includes the SHANK3 gene, or a mutation of the gene. PMS is also known as 22q13 deletion syndrome. The SHANK3 gene codes for the shank3 protein, which supports the structure of synapses between nerve cells in the brain. It is estimated that between 1 in 8,000 and 1 in 15,000 people have PMS. There are no medications, drugs, or therapies specifically for PMS, which has an overwhelming unmet medical need. PMS has severe quality of life impacts on those living with it, as well as on parents and siblings. The most common characteristics are moderate to severe developmental and intellectual impairment and developmental delay, delayed or absent speech, symptoms of autism, low muscle tone, motor delays, mild to severe epilepsy, behavioural problems and difficulties with socialization, activities of daily living and self-care. Further information about PMS is available at: www.pmsf.org and www.cureshank.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, all programs have been granted “orphan drug” designation in the United States. Orphan drug designation provides incentives to encourage 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.