Neuren (NEU) – ASX Announcement **20 October 2025**

Fast Track granted by FDA for NNZ-2591 in Phelan-McDermid syndrome

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that the US Food and Drug Administration (FDA) has granted Fast Track designation for NNZ-2591 for the treatment of Phelan-McDermid syndrome (PMS). Fast Track is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Currently there are no FDA-approved treatments for PMS.

Neuren recently initiated "Koala", a Phase 3 randomized double-blind placebo-controlled clinical trial evaluating NNZ-2591 treatment in children aged 3 to 12 years with PMS. Alignment was reached with the FDA on the single Phase 3 trial design and endpoints to support a New Drug Application.

Neuren CEO Jon Pilcher commented: "As we approach Phelan-McDermid Syndrome Awareness Day on 22 October, we are very pleased to announce Fast Track designation for our NNZ-2591 program. Neuren's Koala trial is the first ever Phase 3 clinical trial for PMS, which we hope may lead to a much-needed treatment for this community. We encourage all initiatives to increase awareness and diagnosis of PMS and applaud the leadership of both the Phelan-McDermid Syndrome Foundation and CureSHANK."

Fast Track designation has also been granted for NNZ-2591 in Angelman syndrome (AS), which means that Neuren now has Fast Track for NNZ-2591 across all of PMS, Pitt Hopkins syndrome and AS.

About FDA Fast Track designation

Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier. A drug that receives Fast Track designation is eligible for some or all of the following:

- More frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval
- More frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers
- Eligibility for Accelerated Approval and Priority Review, if relevant criteria are met
- Rolling Review, which means that a drug company can submit completed sections of its New Drug Application (NDA) for review by FDA, rather than waiting until every section of the NDA is completed before the entire application can be reviewed. NDA review usually does not begin until the drug company has submitted the entire application to the FDA

About Phelan-McDermid syndrome

Phelan-McDermid syndrome (PMS) 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 neurons 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.

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. Recognising the urgent unmet need, each program has been granted "orphan drug" designation in the United States and the European Union. Orphan drug designation provides incentives to encourage development of therapies for rare and serious diseases.

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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.