Neuren (NEU) – ASX Announcement **7 January 2026**

NNZ-2591 PMS Phase 2 trial published in Neurology® Genetics

Highlights:

- **NNZ-2591 Phase 2 clinical trial in Phelan-McDermid syndrome (PMS) published in peer-reviewed medical journal, Neurology® Genetics**
- **Koala Phase 3 clinical trial in PMS recruiting in the US**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that results from its Phase 2 clinical trial of NNZ-2591 in children and adolescents with Phelan-McDermid syndrome (PMS) have been published in Neurology® Genetics, an open access peer-reviewed clinical and translational neurology journal of the American Academy of Neurology.

The full article, titled "NNZ-2591 in Children and Adolescents With Phelan-McDermid Syndrome" is available at: NNZ-2591 in Children and Adolescents With Phelan-McDermid Syndrome | Neurology Genetics.

The publication describes Neuren's previously completed Phase 2, 13-week, open-label clinical trial of NNZ-2591 in PMS patients aged 3 to 12 years, in which NNZ-2591 treatment was well-tolerated and both clinicians and caregivers reported meaningful improvements in important symptoms of PMS.

The Phase 2 trial results informed the design of Neuren's Phase 3 Koala trial, which is currently recruiting children and adolescents with PMS in the US. Koala has the same age range, length of treatment and target dosing as the Phase 2 trial.

Neuren CEO Jon Pilcher commented: "We are pleased that Neurology® Genetics has published our Phase 2 trial results in PMS, which provided the strong scientific basis to further investigate NNZ-2591 as a potential treatment for PMS in our ongoing Koala Phase 3 study. We are excited to be recruiting participants for Koala, which we hope is the next step towards a much-needed treatment for the PMS community."

About Koala

Koala is a Phase 3, randomized, double-blind, placebo-controlled clinical trial evaluating the safety and efficacy of NNZ-2591 in approximately 160 children aged 3 to 12 years with PMS. A screening period of up to 4 weeks is followed by treatment with NNZ-2591 or placebo for 13 weeks. The trial is currently recruiting participants in the US (Study Details | NCT07281079 | A Study of NNZ-2591 in Pediatric Participants With Phelan-McDermid Syndrome | ClinicalTrials.gov). The PMS program for NNZ-2591 has Fast Track, Rare Pediatric Disease and Orphan Drug designations from the US Food and Drug Administration (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) and DAYBUE STIX (trofinetide) are approved by the US Food and Drug Administration (FDA) 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 1.01, 117 Camberwell Road, Hawthorn East, VIC 3123

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.