

Neuren (NEU) – ASX Announcement

6 February 2026

First patient dosed in Neuren's Phelan-McDermid syndrome Phase 3 trial

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today reported that the first participant has completed the 4-week screening period and commenced the 13-week period of twice-daily dosing in its Koala Phase 3 clinical trial of NNZ-2591 for Phelan-McDermid syndrome (PMS). This is the first Phase 3 trial ever to be conducted in PMS, for which there are no approved treatment options.

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 program has Fast Track, Rare Pediatric Disease and Orphan Drug designations from the US Food and Drug Administration.

More participants are scheduled to start dosing or start screening during February and March. To date 25 families have been referred to the two activated trial sites. At this early stage, 37 more families are already on waitlists for a trial site in a convenient geographical location, with two sites expected to activate in February and 20 more across the US progressing towards activation. In parallel, Neuren continues its commitment to the PMS community as the Presenting Sponsor of the 2026 Phelan-McDermid Syndrome Foundation Family Conference, "The Climb We Make Together", which will take place in Colorado in July 2026.

Neuren CEO Jon Pilcher commented: "We are excited to have started the treatment phase of our Koala Phase 3 study and are very encouraged by the level of interest in the PMS community. We are proud to be the presenting sponsor of the PMSF Family Conference in July and we anticipate strong momentum for Koala as trial sites around the US progressively activate during the first half of this year."



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Further study details can be accessed at: [Study Details](#) | [NCT07281079](#) | [A Study of NNZ-2591 in Pediatric Participants With Phelan-McDermid Syndrome](#) | [ClinicalTrials.gov](#)

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.

Contact:

investorrelations@neurenpharma.com

Jon Pilcher, CEO: +61 438 422 271

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.