

Neuren highlights participation in the PMSF Family Conference

Melbourne, Australia: Neuren Pharmaceuticals Limited (Neuren; ASX: NEU) was honoured to support and participate in the biannual Phelan-McDermid Syndrome Foundation Family Conference as the Presenting Sponsor. The conference titled “The climb we make together” was held in Colorado from 15 July to 19 July, with more than 800 community members attending.

Neuren CEO Jon Pilcher addressed the conference in both the opening and closing sessions and Chief Medical Officer Liza Squires presented Neuren’s development program for its investigative drug NNZ-2591, culminating in the ongoing Koala study - the first ever Phase 3 clinical trial for Phelan-McDermid syndrome (PMS). The Neuren team of 12 people hosted both a Neuren corporate booth as well as a booth dedicated to the Koala study, which were visited by many families.



Neuren CEO Jon Pilcher commented: “The PMSF Family Conference was inspiring and energising for the Neuren team, with so many conversations with parents and siblings caring for their loved ones with PMS. The conference demonstrated how far the PMS community has come in the four years since Neuren started the first ever multi-site clinical trial. As we strive to achieve a potential first approved treatment for PMS, the courage, determination and support of PMS families and continuing the productive collaboration with clinicians, researchers, PMSF and CureSHANK will be critical.”

Robbie Baker, CEO of the Phelan-McDermid Syndrome Foundation (PMSF), commented: “We are grateful to Neuren for serving as the Presenting Sponsor of the 2026 PMSF Family Conference. Neuren's generous support has helped bring together families, researchers, and clinicians to learn from one another and strengthen our shared commitment to advancing research and care for individuals with PMS. We are in an unprecedented era of research and therapeutic development in PMS, including Neuren’s Phase 3 Koala study evaluating NNZ-2591. This landmark study is the first-ever Phase 3 clinical trial conducted specifically for PMS. For a community navigating a rare disorder with no currently approved treatments, this milestone represents both meaningful scientific progress and hope for the future.”

The conference also featured an overview by Tess Levy (Icahn School of Medicine, Mt Sinai, New York), the lead author of the paper recently published in the Autism Research journal of a study estimating the prevalence of PMS to be 1 in 7,300 people (<https://onlinelibrary.wiley.com/doi/full/10.1002/aur.70297>) This is significantly higher than previous estimates, reinforcing the magnitude of the unmet need and the importance of ongoing initiatives to accelerate diagnosis and improve genetic testing. The study was supported by Neuren.

Neuren’s Koala Phase 3 clinical study in children aged 3 to 12 years with PMS now has 13 trial sites activated to enrol participants, including the first site in Canada. Four more sites are scheduled to



activate in August. More than 100 potential participants have been referred by Neuren to active sites or await activation of closer sites. The first participants have completed 13-weeks of treatment in the randomised placebo-controlled study and are continuing in the 52-week open-label extension study. In addition, the first patient from Neuren's previously completed Phase 2 open-label clinical study in paediatric patients with PMS has now been able to re-commence treatment by enrolling in the open-label extension study.

Neuren Chief Medical Officer Liza Squires commented: "Neuren's Koala Phase 3 study continues to gain momentum. 13 sites across the US and Canada are now actively enrolling, and four additional sites are scheduled to open next month. We are excited and proud to partner with the PMS community to advance this important trial."

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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 1 in 7,300 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 Pharmaceuticals is developing new drug therapies to treat multiple serious neurological disorders caused by genetic abnormalities or brain injury, that have no or limited approved treatment options. Neuren's therapies target the critical role of Insulin-like growth factor 1 (IGF-1) in the brain, using orally administered analogs of naturally occurring peptides.

DAYBUE® (trofinetide) oral solution is approved by the US Food and Drug Administration (FDA), Health Canada and the Ministry of Health in Israel and DAYBUE STIX (trofinetide) powder is approved by the FDA for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide license to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.



Neuren's investigative drug candidate, NNZ-2591 (ercanetide), is in clinical development as an oral solution treatment for multiple neurodevelopmental disorders, including Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome. Each of these programs has been granted "orphan drug" designation in the United States and the European Union as well as Fast Track and Rare Pediatric Disease designations from the FDA. Neuren is also developing NNZ-2591 for the treatment of hypoxic ischemic encephalopathy (HIE), a serious condition caused by brain injury before or shortly after birth.

Currently, Neuren is conducting a Phase 3, randomized, double-blind, placebo-controlled clinical trial ("Koala") evaluating the safety and efficacy of NNZ-2591 in children aged 3 to 12 years with Phelan-McDermid syndrome and a 52-week open-label extension study.

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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.