



Neuren (NEU) – ASX announcement

5 December 2023

Neuren completes enrolment in Pitt Hopkins syndrome Phase 2 trial

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that enrolment of subjects into its Phase 2 clinical trial of NNZ-2591 in Pitt Hopkins syndrome has been completed. Top-line results from the trial are expected to be available in Q2 2024.

Neuren CEO Jon Pilcher commented “We are grateful to all the people who have contributed to achieving this important milestone as we strive to develop a potential first therapy for Pitt Hopkins syndrome, including the Pitt Hopkins community and the trial site teams in the United States. We now look forward to the remaining patients completing the trial and to releasing the first results of treatment with NNZ-2591 in children with Pitt Hopkins syndrome.”

The Phase 2 trial in children aged 3 to 17 years at five hospitals in the United States is examining safety, tolerability, pharmacokinetics and efficacy over 13 weeks of treatment with NNZ-2591. All subjects receive NNZ-2591 as an oral liquid dose twice daily.

Neuren is also conducting Phase 2 clinical trials of NNZ-2591 in children with three other neurodevelopmental disorders – Phelan McDermid syndrome, Angelman syndrome and Prader-Willi syndrome. All four programs have been granted Orphan Drug designation by the US Food and Drug Administration (FDA) and are being developed under Investigational New Drug (IND) applications. Each syndrome is a seriously debilitating neurological disorder that emerges in early childhood and has no or limited approved treatment options.

About Pitt Hopkins syndrome

Pitt Hopkins syndrome (PTHS) is a neurodevelopmental condition caused by the loss of one copy or a mutation of the *TCF4* gene on chromosome 18. The incidence of PTHS has been estimated at between 1 in 34,000 and 1 in 41,000 people. Characteristics of PTHS are a range of developmental delay with moderate-to-severe intellectual disability and behavioral differences, hyperventilation and/or breath-holding while awake, seizures, gastrointestinal issues, lack of speech, sleep disturbance, stereotypic hand movements and distinctive facial features. Some individuals with PTHS are diagnosed with autism. Further information about PTHS is available at: www.pitthopkins.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) for the treatment of Rett syndrome in adult and pediatric patients two years of age and older. Neuren has granted an



exclusive worldwide licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren is conducting Phase 2 trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome, Pitt Hopkins syndrome and Prader-Willi syndrome.

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.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the board of directors 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.