Neuren (NEU) – ASX announcement **20 December 2023**

Completion of enrolment in Neuren's Angelman syndrome Phase 2 trial

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that the last subject to be screened this week will complete the enrolment of subjects into its Phase 2 clinical trial of NNZ-2591 in Angelman syndrome. Top-line results from the trial are expected to be available in Q3 2024.

Neuren CEO Jon Pilcher commented "We are grateful to the Angelman community in Australia including both FAST Australia and the ASAA, together with the trial site teams, who have enabled this important milestone to be achieved. 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 Angelman syndrome."

The Phase 2 trial in children aged 3 to 17 years at three hospitals in Australia 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 has announced positive top-line results from a Phase 2 trial in children with Phelan-McDermid syndrome and is also conducting Phase 2 clinical trials of NNZ-2591 in children with Pitt Hopkins syndrome and Prader-Willi syndrome. Top-line results from the Pitt Hopkins syndrome trial are expected in Q2 2024. 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 Angelman syndrome

There are currently no approved medicines for AS, which is characterized by severe developmental delay and learning disabilities that become noticeable by the age of 6 – 12 months. Children and adults with AS typically have balance issues, motor impairment and can have debilitating seizures. Some individuals never walk, most do not speak and disruptive sleep also can be a serious challenge. Individuals have a normal life expectancy, but they require continuous care and are unable to live independently. AS is caused by a loss of function of the UBE3A gene on chromosome 15, with incidence estimated at between 1 in 12,000 and 1 in 24,000 people. Further information about AS is available at: https://cureangelman.org.au/

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. 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.

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's second drug candidate, NNZ-2591 is in Phase 2 development for each of Phelan-McDermid syndrome, Angelman syndrome, Pitt Hopkins syndrome and Prader-Willi syndrome.

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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.