



Neuren (NEU) – ASX announcement

30 August 2021

Neuren receives approval of R&D Tax Incentive Advance Overseas Finding

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that AusIndustry has approved an Advance and Overseas Finding under the R&D Tax Incentive for the development of NNZ-2591 as a novel therapy for neurodevelopmental disorders. Neuren may receive R&D Tax Incentive cash rebates of up to \$6.5 million for eligible research and development expenditure incurred over 2020, 2021 and 2022.

Neuren is in the final stage of preparation to commence a Phase 2 clinical trial of NNZ-2591 for children in Australia with Angelman syndrome, a serious neurodevelopmental disorder with no approved medicines.

Neuren CEO Jon Pilcher commented: “We appreciate this support from the Australian government for our mission to develop NNZ-2591 for multiple serious neurological conditions. It is timely as we approach the start of our Phase 2 trial in Angelman syndrome, which we hope will demonstrate the potential for NNZ-2591 to provide a treatment option for the families impacted by this debilitating condition.”

Angelman syndrome (AS) is caused by a loss of function of the *UBE3A* gene in chromosome 15. AS 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.

About Neuren

Neuren is developing two new drug therapies to treat multiple serious neurological disorders that emerge in early childhood, none of which have any approved medicines.

The lead compound, trofinetide, is currently in a Phase 3 clinical trial for Rett syndrome with top-line results expected in Q4 2021 and has completed a Phase 2 clinical trial in Fragile X syndrome. Both programs have Fast Track designation from the US Food and Drug Administration (FDA). Neuren has granted an exclusive licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide in North America, while retaining all rights outside North America.



Neuren is preparing to initiate Phase 2 trials of its second drug candidate, NNZ-2591, for each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome in H2 2021. Neuren is also planning a Phase 2 trial in Prader-Willi syndrome.

Because of the urgent unmet need, five programs have been granted “orphan drug” designation in both the United States and the European Union, a designation that provides incentives to encourage development of therapies for rare and serious diseases.

Contact:

Jon Pilcher, CEO: jpilcher@neurenpharma.com; +61 438 422 271

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.