



Neuren (NEU) – ASX Announcement

11 October 2021

US patent to 2034 granted for Neuren’s NNZ-2591 in autism

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) has received a Notice of Allowance from the US Patent and Trademark Office for a new patent that covers NNZ-2591 to treat autism. The term of the patent, titled “Bicyclic compounds and methods for their use in treating autism” extends to July 2034. Similar claims have previously been granted in Europe and Japan.

Neuren is currently developing NNZ-2591 to treat Phelan-McDermid, Angelman, Pitt Hopkins and Prader-Willi syndromes, four serious lifelong neurological disorders that emerge in early childhood.

Neuren CEO Jon Pilcher commented: “We have seen that the mechanism of action of NNZ-2591 appears to have broad utility in neurodevelopmental disorders. Given that autism is estimated to affect one in 160 children, the grant of this important US patent could potentially enable a very large future opportunity as more becomes known about the causes of autism.”

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, Pitt Hopkins syndrome and Prader-Willi syndrome.

Recognising the urgent unmet need, all six 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.

Contact:

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



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.

ASX Listing Rules information

This announcement was authorized to be given to the ASX by the CEO of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124