



Neuren (NEU) - ASX Announcement

17 May 2019

NNZ-2591 demonstrates positive effects in Angelman syndrome pre-clinical model

Melbourne, Australia, 17 May 2019: Neuren Pharmaceuticals (ASX: NEU) today announced that treatment with its drug candidate NNZ-2591 has normalized all deficits in a pre-clinical model of Angelman syndrome (AS). Neuren anticipates that AS meets the criteria for Orphan Drug designation. There is currently no treatment specifically for AS.

NNZ-2591 was tested in the *ube3a* knockout mouse model, which resembles features of Angelman syndrome in humans and includes motor deficits, learning problems and alterations in synaptic connectivity and plasticity.

The study compared normal mice (“wild type”) and mice with a disrupted gene (“knockout”). In the knockout mice, treatment with NNZ-2591 for 6 weeks normalized the deficits in all the tests of anxiety, daily living, sociability, motor performance and cognition as well as eliminating seizures. All positive confirmatory measures were statistically significant.

AS is a neurodevelopmental condition estimated to affect 1 in 15,000 people, both males and females. AS is caused by a deletion or mutation in the ubiquitin protein ligase E3A (*UBE3A*) gene on chromosome 15.

Characteristics of Angelman syndrome (AS) are delayed development, intellectual disability, anxiety and hyperactivity, severe speech impairment, problems with movement and balance, seizures and sleep disorders. AS has often been misdiagnosed as autism. Many symptoms persist into adulthood and people with AS require lifelong care and support from clinicians and caregivers.

About Neuren and NNZ-2591

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for neurodevelopmental disorders with high unmet need. Neuren has completed Phase 2 development of its lead drug candidate trofinetide for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs in Rett syndrome and Fragile X syndrome have each been granted Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America, whilst retaining all rights to trofinetide outside North America.

Neuren is advancing the development of its second drug candidate NNZ-2591, a synthetic analog of the neurotrophic peptide, cyclic glycine proline (cGP), which occurs naturally in the brain. NNZ-2591 has demonstrated efficacy in pre-clinical models of Parkinson’s disease, stroke, traumatic brain injury, peripheral neuropathy, Fragile X syndrome, Phelan-McDermid syndrome, Angelman syndrome, memory impairment and multiple sclerosis.



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Forward-looking Statements

This ASX-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.