

Neuren (NEU) - ASX Announcement

14 July 2020

Scientific publications and presentations for trofinetide and NNZ-2591

Melbourne, Australia, 14 July 2020: Neuren Pharmaceuticals (ASX: NEU) today provided an update on recent scientific journal publications and conference presentations for the trofinetide and NNZ-2591 development programs.

Neuren's Phase 2 clinical trial of trofinetide in Fragile X syndrome has been published in the scientific journal Pediatric Neurology. The article *"Berry-Kravis E et al., A Double-Blind, Randomized, Placebo-Controlled Clinical Study of Trofinetide in the Treatment of Fragile X Syndrome, Pediatric Neurology"* is now freely available online via the link: <u>https://doi.org/10.1016/j.pediatrneurol.2020.04.019</u>. A PDF copy is also available in the Scientific Presentations section of Neuren's website.

A poster on the design of the LAVENDER Phase 3 clinical trial of trofinetide in Rett syndrome was presented by ACADIA at the 72nd Annual Meeting of the American Academy of Neurology. A PDF copy of the poster *"Neul JL, Percy AK, Benke TA, Berry-Kravis EM, Glaze DG, Jones NE, Youakim JM. Trofinetide: a novel approach to Rett syndrome"* is available in the Scientific Presentations section of Neuren's website.

The trofinetide program was also presented by Neuren at the virtual meeting of "Rett Syndrom I Sverige" (the Swedish Rett syndrome association).

The NNZ-2591 program was presented by Neuren at the virtual symposium of the Pitt Hopkins Research Foundation and later this month will be presented at the virtual educational sessions of the Phelan-McDermid Syndrome Foundation.

About Neuren

Neuren is developing new therapies for neurodevelopmental disorders with high unmet need, utilizing synthetic analogs of neurotrophic peptides that occur naturally in the brain. Trofinetide is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs have each received 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 outside North America. Neuren is advancing the development of NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, each of which has received Orphan Drug designation in the United States.



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ASX Listing Rules information

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