Neuren (NEU) - ASX Announcement **24 March 2020**

Temporary pause in new enrolments for LAVENDER trial in the US

Melbourne, Australia, 24 March 2020: Neuren Pharmaceuticals (ASX: NEU) today provided an update on the Phase 3 LAVENDER study of trofinetide in Rett syndrome, due to the current measures in the United States to combat the COVID-19 pandemic. At this time, Neuren's US partner ACADIA Pharmaceuticals is not planning to enroll new patients in the LAVENDER study until ACADIA believes it has the ability to collect data from new patients while ensuring their safety. This modification does not impact patients already enrolled in the LAVENDER study. An update issued by ACADIA to the Rett syndrome community is attached.

Separately, current conditions make the planning and commencement by Neuren of Phase 2 trials for NNZ-2591 in the United States before the end of 2020 extremely challenging, therefore the target date for commencement and the associated expenditure have been deferred. The situation will be closely monitored and when conditions improve Neuren will accelerate the commencement. In the meantime, the NNZ-2591 non-clinical studies that Neuren is undertaking for the planned Investigational New Drug Application (IND) in the United States remain ongoing and on schedule.

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 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.

March 23, 2020

Dear Rett Community,

As a result of the many uncertainties surrounding the coronavirus (COVID-19) pandemic, we wanted to provide you with an update on the trofinetide Phase 3 LAVENDER study.

The health and safety of clinical study patients, caregivers, investigator staff and all study partners are paramount. ACADIA is taking proactive steps to advance our research programs considering guidance from the U.S. Food and Drug Administration (FDA) and local policies during this public health crisis to protect the safety of patients and the integrity of the clinical trial data. At this time, we are not planning to enroll new patients in the LAVENDER study until we believe we have the ability to collect data from new patients while ensuring their safety. This modification does not impact patients already enrolled in the LAVENDER study.

As we have shared before, both LAVENDER and LILAC are progressing, and most study sites are open. LAVENDER is a 12-week study that will evaluate the efficacy and safety of trofinetide and placebo in approximately 180 girls and young women aged 5 to 20 years with Rett syndrome. All girls and young women completing the LAVENDER trial are eligible to enroll in the LILAC study, a 40-week extension study in which all participants receive trofinetide and are followed to evaluate long term tolerability, safety, and effectiveness of the drug.

If you have visited the LAVENDER study web site (www.rettsyndromestudies.com) and registered interest in participating, your information has been appropriately recorded and passed on to the closest clinical study site available.

If you have any questions about trofinetide, please contact us at medicalinformation@acadia-pharm.com.

Thank you for your continued support, and we wish you and your families well during this unprecedented time. For additional information on how ACADIA is responding to the COVID-19 pandemic, please visit www.acadia-pharm.com.

All our best,
The ACADIA Rett Team