Neuren (NEU) - ASX Announcement **16 June 2020**

Temporary enrolment pause ended in Phase 3 trial in the US

Melbourne, Australia, 16 June 2020: Neuren Pharmaceuticals (ASX: NEU) today reported that its US partner ACADIA Pharmaceuticals has re-initiated enrolment in the Phase 3 LAVENDER study of trofinetide in Rett syndrome. Enrolment of new patients was paused temporarily in March 2020, due to the measures taken in the US to combat the COVID-19 pandemic. An update issued by ACADIA to the Rett syndrome community is attached.

Neuren CEO Jon Pilcher commented: "We are very pleased that enrolment of patients can now continue in this very important clinical trial for all stakeholders and we look forward to the results next year."

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.

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.

June 15, 2020

Dear Rett Community,

We hope you and your families are doing well during this challenging time. We appreciate your patience as we navigate the coronavirus (COVID-19) pandemic.

As the nation begins to reopen its doors, we remain committed to the health and safety of all patients, families, caregivers and clinical staff participating in the ongoing LAVENDER and LILAC studies. We are working closely with clinical sites and study investigators to take all necessary precautions identified through local and national guidance. In addition to protecting the health of study participants and clinical staff, we are also taking appropriate measures to safely and effectively collect patient data to ensure the integrity of study results.

In light of these collaborative and protective measures, as well as the easing of restrictions throughout the country, we are pleased to announce that we are re-initiating enrollment in the Phase 3 LAVENDER study. Each site will begin enrolling again on its own timetable based on the availability of staff, their site-specific policies, and an agreement on the site's plan for the safe conduct of clinical study visits.

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.

To learn more about the LAVENDER study and determine interest in participating, you can visit the study website at www.rettsyndromestudies.com or the LAVENDER study page on clinicaltrials.gov. If you have already registered, your information has been appropriately recorded and passed on to the closest clinical study site available.

If you have any questions about trofinetide or enrolling into the LAVENDER study, please contact us at medicalinformation@acadiapharm.com.

Thank you for your continued support and we hope you are your families are healthy and safe. We will continue to provide updates as appropriate.

All our best,
The ACADIA Rett Team