Neuren (NEU) – ASX announcement **1 September 2021**

DAFFODIL™ Rett syndrome clinical trial for trofinetide in girls aged 2 to 5 years

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today reported that its US partner for trofinetide, Acadia Pharmaceuticals (Nasdaq: ACAD), will be conducting DAFFODIL™, a clinical study of trofinetide in approximately 10 girls with diagnosed Rett syndrome, aged 2 to 5 years. DAFFODIL will be a 12-week, multicenter, open-label safety, tolerability and pharmacokinetics study in this younger age group, followed by a possible open-label extension for up to an additional 21 months. An update by Acadia to the Rett syndrome community in the United States is attached to this announcement.

About trofinetide for Rett syndrome

Acadia recently completed enrolment in the LAVENDER™ Phase 3 trial of trofinetide in Rett syndrome, remaining on track for top-line results in Q4 2021. LAVENDER is a randomised, double-blind, placebo-controlled Phase 3 trial testing treatment of approximately 180 patients aged 5 to 20 years for 12 weeks with trofinetide or placebo.

Rett syndrome is a debilitating neurodevelopmental disorder estimated to affect between 1 in 10,000 and 1 in 15,000 females worldwide. A range of severe impairments emerge in infancy, affecting nearly every aspect of the child's life: their ability to speak, walk, eat, and even breathe. There are currently no medicines approved for Rett syndrome. The trofinetide program has Fast Track, Orphan Drug and Rare Pediatric Disease designations from the US Food and Drug Administration (FDA).

The development and commercialisation of trofinetide in North America is fully funded by Acadia. Neuren is eligible to receive potential milestone payments of up to US$455 million, plus tiered escalating double-digit percentage royalties on net sales of trofinetide in North America, plus one third of the market value of a Rare Pediatric Disease Priority Review Voucher if awarded by the FDA upon approval of a New Drug Application for trofinetide. Further, Neuren has free and full access to all data for use in countries outside North America.

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 and Pitt Hopkins syndrome in H2 2021. Neuren is also planning a Phase 2 trial in Prader-Willi syndrome.

Because of the urgent unmet need, five programs have been granted "orphan drug" designation in both the United States and the European Union, a designation that provides incentives to encourage development of therapies for rare and serious diseases.

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.

August 30, 2021

Dear Rett Community,

Acadia will be conducting an additional clinical study of trofinetide. DAFFODIL™ will be a 12-week, multicenter, open-label safety, tolerability and pharmacokinetics study in girls with diagnosed Rett syndrome, 2 to 5 years of age, followed by a possible open-label extension for up to an additional 21 months.

Please recognize that the trial design criteria for DAFFODIL™ is limited to a small number (approximately 10 total) of female participants in specified weight ranges, to enable measurements of the blood levels of trofinetide, an investigational drug, and safety assessments across this age group.

Study information can be found on clinicaltrials.gov and at the study website: rettsyndromestudies.com. These will be updated with further information upon study and study site initiation.

Thank you to the International Rett Syndrome Foundation (IRSF) and the entire Rett community who help make this research possible. We appreciate your continued partnership on this journey to find a treatment to address the core symptoms of Rett syndrome.

COVID SAFETY PRECAUTIONS

The health of our volunteers and their families is our priority. We work 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.

FURTHER INFORMATION

If you have any questions about trofinetide or the clinical trials, please contact us at medicalinformation@acadiapharm.com

All our best,

The Acadia Rett Team

Acadia Pharmaceuticals Inc.
Tel. 858-558-2871
Fax. 858-558-2872

12830 El Camino Real, Suite #400
San Diego, CA 92130

www.acadia-pharm.com