Neuren (NEU) – ASX Announcement **03 March 2026**

Acadia confirms plan to request CHMP re-examination

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) advised that its partner, Acadia Pharmaceuticals (Nasdaq: ACAD), today confirmed it plans to request re-examination of the opinion adopted by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) regarding the Marketing Authorization Application for trofinetide for the treatment of Rett syndrome in patients two years of age and older. As anticipated, the previously announced CHMP trend vote was confirmed in the formal vote.

Trofinetide is approved in the United States, Canada and Israel, where it represents the first and only treatment approved for Rett syndrome.

In Acadia's announcement Acadia's Chief Executive Officer Catherine Owen Adams commented: "While we are disappointed by the CHMP's recommendation to refuse approval, we continue to be encouraged by the meaningful benefits trofinetide has demonstrated for people living with Rett syndrome. The strong engagement and positive feedback we have seen from patients, caregivers, and clinicians in the Rett community reinforce our belief in the treatment's clinical value. We remain committed to working constructively with EU regulators to explore next steps and to bring this therapy to patients."

Markus Schulze, caregiver and member of the Rett Syndrome Society Nordrhein-Westfalen from Germany said: "Our family and others who play an important role in the delivery of care know first-hand the challenges that individuals living with Rett syndrome face every day. It is our hope that this important therapy will be approved to help the EU Rett community better navigate life with Rett syndrome."

While the pivotal LAVENDER™ clinical trial successfully met its co-primary and key secondary endpoints, the CHMP issued a refusal based on perceived deficits including: the treatment effect observed with trofinetide after 12 weeks, while measurable, was viewed as limited in magnitude; the study did not capture all core symptoms of Rett syndrome; and that assessment of longer-term outcomes was influenced by patient discontinuations over time. Acadia believes this feedback provides important information as it considers the intended re-examination.

Neuren CEO Jon Pilcher commented: "Neuren fully supports a re-examination of the CHMP opinion. Trofinetide has been making a difference for patients for nearly three years in approved markets and the unmet medical need in Europe remains substantial and urgent."

About Neuren

Neuren Pharmaceuticals is developing new drug therapies to treat multiple serious neurological disorders caused by genetic abnormalities or brain injury, that have no or limited approved treatment options. Neuren's therapies target the critical role of Insulin-like growth factor 1 (IGF-1) in the brain, using orally administered analogs of naturally occurring peptides.

DAYBUE® (trofinetide) oral solution is approved by the US Food and Drug Administration (FDA), Health Canada and the Ministry of Health in Israel and DAYBUE STIX (trofinetide) powder is approved by the FDA for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide license to Acadia Pharmaceuticals Inc. for the development and commercialization of trofinetide.

Neuren's second drug candidate, NNZ-2591, is in clinical development as an oral solution treatment for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome. Each of these programs has been granted "orphan drug" designation in the United States and the European Union as well as Fast Track and Rare Pediatric Disease designations from the FDA. Neuren is also developing NNZ-2591 for the treatment of hypoxic ischemic encephalopathy (HIE), a serious condition caused by brain injury before or shortly after birth.

Currently, Neuren is conducting a Phase 3, randomized, double-blind, placebo-controlled clinical trial ("Koala") evaluating the safety and efficacy of NNZ-2591 in children aged 3 to 12 years with Phelan-McDermid syndrome and a 52-week open-label extension study.

Contact:
investorrelations@neurenpharma.com
Jon Pilcher, CEO: +61 438 422 271

ASX Listing Rules information
This announcement was authorized to be given to the ASX by the CEO & Managing Director of Neuren Pharmaceuticals Limited, Suite 1.01, 117 Camberwell Road, Hawthorn East, VIC 3123

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.