Neuren (NEU) – ASX Announcement **3 February 2026**

Acadia provides update on regulatory submission for trofinetide for the treatment of Rett syndrome in the European Union

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) advised that its licensee Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) has today provided an update on its marketing authorization application (MAA) for trofinetide for the treatment of Rett syndrome in the European Union (EU). Acadia was informed by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) of a negative trend vote on its MAA for trofinetide for the treatment of Rett syndrome, following its recent CHMP oral explanation. Subject to the outcome of the CHMP vote in February, Acadia intends to request a re-examination of the opinion by the CHMP upon its formal adoption.

"While the negative trend vote is disappointing and not what we hoped for, we believe the strong data that supported the approval of trofinetide for the treatment of Rett syndrome in the United States, Canada, and Israel speak to the meaningful benefits that trofinetide can deliver," said Catherine Owen Adams, Acadia's Chief Executive Officer. "We now have more than 1,000 patients on active treatment globally, from newly diagnosed 2-year-olds to adults who have lived with their disease for decades. Our ongoing real-world experience study in the U.S. continues to show outcomes that closely mirror the impact observed in rigorous randomized clinical trials conducted across a broad age range. We look forward to working with the EMA and other stakeholders to advance trofinetide as an important potential treatment option in the EU. Our commitment to the Rett syndrome community in the EU remains steadfast, and we are fully dedicated to making trofinetide available to individuals and families who urgently need a new therapeutic option."

Pursuant to EU legislation, an applicant has the right to request a re-examination of a CHMP opinion within 15 calendar days of receipt of the opinion, followed by submission of the grounds for the request for re-examination within 60 calendar days of receipt of the opinion. The CHMP has up to 60 days after receipt of these grounds to re-examine its opinion.

Neuren CEO Jon Pilcher commented: "Given the totality of experience with trofinetide in clinical trials and real world use over many years, this negative trend vote is frustrating for us and the Rett syndrome community in the EU. We fully support Acadia's intention to seek re-examination of the CHMP opinion in February, if necessary."

About Neuren

Neuren is developing new drug therapies to treat multiple serious neurological disorders that emerge in early childhood and have no or limited approved treatment options.

DAYBUE® (trofinetide) and DAYBUE STIX (trofinetide) are approved by the US Food and Drug Administration (FDA) for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide licence to Acadia Pharmaceuticals Inc. for the development and commercialisation of trofinetide.

Neuren's second drug candidate, NNZ-2591, is in development for multiple neurodevelopmental disorders, with positive results achieved in Phase 2 clinical trials in Phelan-McDermid syndrome, Pitt Hopkins syndrome and Angelman syndrome. Recognising the urgent unmet need, each program has been granted "orphan drug" designation in the United States and the European Union. Orphan drug designation provides incentives to encourage development of therapies for rare and serious diseases.

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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 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.