Neuren (NEU) – ASX Announcement **15 December 2025**

US FDA approves DAYBUE® STIX (trofinetide) for oral solution, a new powder formulation, for Rett syndrome

Highlights:

- **DAYBUE STIX is a new dye- and preservative-free powder formulation of trofinetide**
- **DAYBUE STIX provides Rett syndrome patients and caregivers with new flexibility and choice**
- **DAYBUE and DAYBUE STIX are the only US FDA-approved treatments for Rett syndrome**
- **Neuren receives royalties on net sales of trofinetide**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today announced that its partner, Acadia Pharmaceuticals (Nasdaq: ACAD), has received US Food and Drug Administration (FDA) approval of DAYBUE® STIX (trofinetide) for oral solution, a dye- and preservative-free powder formulation of trofinetide for the treatment of Rett syndrome in adult and pediatric patients two years of age and older. The new powder formulation offers children and adults living with Rett syndrome new flexibility and choice regarding the dose volume and taste of their DAYBUE treatment.

Neuren CEO Jon Pilcher commented: "The Neuren team is excited about the approval of this new treatment option for Rett syndrome families and the continued investment and innovation for trofinetide by our global partner, Acadia. Caregivers can mix DAYBUE STIX with a variety of water-based liquids providing flexibility to modify the taste and volume of their loved-one's dose. We look forward to seeing the impact as DAYBUE STIX becomes more broadly available during 2026.

The approval of this new formulation was informed by the results of a bioequivalence study, which demonstrated that both original DAYBUE (trofonetide) oral solution and the new DAYBUE STIX (trofinetide) for oral solution powder formulation provide comparable exposure. This confirmed bioequivalence means patients can expect the same efficacy and safety established by the oral solution formulation when using DAYBUE STIX.

Acadia expects DAYBUE STIX to be available on a limited basis starting in the first quarter of 2026 and more broadly early in the second quarter of 2026. The current oral solution formulation will remain available.

The announcement by Acadia, including indication and important safety information for DAYBUE® (trofinetide) and DAYBUE® STIX (trofinetide) can be viewed at: https://ir.acadia.com/news-releases/news-release-details/acadia-pharmaceuticals-announces-fda-approval-daybuer-stix. Full Prescribing Information is also available at DAYBUEhcp.com.

Acadia has an exclusive worldwide licence for the development and commercialisation of trofinetide. Neuren receives royalties on all net sales of trofinetide and is eligible to receive additional payments on achievement of commercial and development milestones. Further details are available on page 7 in Neuren's Investor Presentation released on 10 November 2025: https://www.neurenpharma.com/Investor-Presentation-10-November-2025.pdf.

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 CEO & Managing Director 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.