Neuren (NEU) – ASX Announcement **29 June 2026**

DAYBU® (trofinetide) recommended for approval in the European Union

Highlights:

- **The Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion recommending European Union (EU) marketing authorisation for DAYBU® following a re-examination procedure**
- **If granted by the European Commission, DAYBU® would become the first treatment for neurobehavioral symptoms of Rett syndrome in the EU**
- **If marketing authorisation is granted and DAYBU® is launched, Neuren would be entitled to receive US$35 million following first commercial sale in the EU, as well as royalties on net sales and potential sales milestone payments**

Melbourne, Australia: Neuren Pharmaceuticals Limited (Neuren; ASX: NEU) welcomes the announcement by its partner Acadia Pharmaceuticals Inc. (Acadia; Nasdaq: ACAD) that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion following a re-examination procedure, recommending the granting by the European Commission (EC) of a marketing authorisation for DAYBU® (trofinetide) for the treatment of neurobehavioral symptoms of Rett syndrome in adults and pediatric patients aged five years and older. If granted, DAYBU® would be the first therapy approved for this indication in the EU. Acadia's announcement, including comment by the President of Rett Syndrome Europe, can be accessed in the Investors section of the Acadia website www.acadia.com.

Neuren CEO Jon Pilcher commented: "I am so delighted for all stakeholders to see this positive outcome from the CHMP re-examination process recommending marketing authorisation for DAYBU® in the EU. With no approved treatment currently available in the EU, approval of DAYBU® would represent an important step forward for patients, caregivers and the wider Rett syndrome community profoundly impacted by this devastating condition."

Following the CHMP recommendation, the EC will review the opinion and is expected to issue a final decision in the coming months. If DAYBU® is approved, the marketing authorisation would apply to all 27 EU member states, as well as Iceland, Liechtenstein and Norway. More information on the CHMP opinion and EMA review process for DAYBU® can be found on the EMA website: https://www.ema.europa.eu/en/medicines/human/EPAR/daybu.

Neuren has granted an exclusive worldwide licence to Acadia for the development and commercialisation of trofinetide. Under the licence agreement, for Europe, Neuren is entitled to receive US$35 million following first commercial sale, sales milestone payments of up to US$170 million on achievement of escalating annual net sales thresholds, and tiered royalties from mid-teens to low-20s % of net sales.

Click **here** to view this announcement on our Investor Hub.

About Rett Syndrome

Rett syndrome is a rare, complex, neurodevelopmental disorder and occurs in approximately one of every 10,000 to 15,000 female births worldwide.[1,2,3] A child with Rett syndrome generally exhibits an early period of apparently normal development until six to 18 months, when many of their skills seem to slow down or stagnate. This is typically followed by a regression phase when the child loses acquired communication skills and purposeful hand use. The child may then experience a plateau period in which they could show mild recovery in cognitive interests, but body movements remain severely diminished. As they age, those individuals living with Rett may continue to experience a stage of motor deterioration, which can last the rest of the patient's life.[2] Rett syndrome is typically caused by a genetic mutation on the MECP2 gene.[4] In preclinical studies, deficiency in MeCP2 function is thought to lead to impairment in synaptic communication and brain plasticity, and the deficits in synaptic function may be associated with Rett manifestations.[4,5,6]

Symptoms of Rett syndrome may also include development of hand stereotypies, such as hand wringing and clapping, and gait abnormalities.[7] Most individuals living with Rett syndrome typically live into adulthood and require intense round-the-clock care.[1,8]

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

[1] Fu C, Armstrong D, Marsh E, et al. Consensus guidelines on managing Rett syndrome across the lifespan. BMJ Paediatrics Open. 2020;4:e000717.
[2] Kyle SM, Vashi N, Justice MJ. Rett syndrome: a neurological disorder with metabolic components. Open Biol. 2018; 8:170216.
[3] May DM, Neul JL, Satija A, et al. Real-world clinical management of individuals with Rett syndrome: a physician survey. J Med Econ. 2023;26(1):1570-1580.
[4] Amir RE, Van den Veyver IB, Wan M, et al. Rett syndrome is caused by mutations in X-linked MECP2, encoding methyl-CpG-binding protein 2. Nat Genet. 1999; 23(2):185-188.
[5] Fukuda T, Itoh M, Ichikawa T, et al. Delayed maturation of neuronal architecture and synaptogenesis in cerebral cortex of Mecp2-deficient mice. J Neuropathol Exp Neurol. 2005; 64(6):537-544.
[6] Asaka Y, Jugloff DG, Zhang L, et al. Hippocampal synaptic plasticity is impaired in the Mecp2-null mouse model of Rett syndrome. Neurobiol Dis. 2006; 21(1):217-227.
[7] Neul JL, Kaufmann WE, Glaze DG, et al. Rett syndrome: revised diagnostic criteria and nomenclature. Ann Neurol. 2010; 68(6):944-950.
[8] Tarquinio DO, Hou W, Neul JL, et al. The changing face of survival in Rett syndrome and MECP2-related disorders. Pediatr Neurol. 2015; 53(5):402-411.

FDA for the treatment of Rett syndrome. Neuren has granted an exclusive worldwide license to Acadia Pharmaceuticals Inc. for the development and commercialisation 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.

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ASX Listing Rules information

This announcement was authorized to be given to the ASX by the board of directors 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.