Neuren (NEU) – ASX Announcement **27 February 2026**

2025 royalty income A$65 million, profit after tax A$30 million

Highlights:

Financial and capital management

- **A$65 million royalty income from DAYBUE® (trofinetide) in 2025, up 15% from 2024**
- **2025 profit after tax A$30 million**
- **A$296 million cash and short-term investments at 31 Dec 2025**
- **A$510 million cumulative income earned from DAYBUE since launch in 2023**
- **A$50 million on-market share buy-back completed, new buy-back initiating on 2 March 2026**

DAYBUE

- **2025 DAYBUE net sales US$391 million, up 12% from 2024**
- **New powder formulation DAYBUE STIX approved by US FDA and launched during H1 2026**
- **Acadia guidance for 2026 net sales growth to US$460-490 million implying royalties to Neuren of A$70-77 million**
- **Acadia to request a re-examination of the opinion by the CHMP of the European Medicines Agency**
- **Results of Acadia's trial to support marketing application in Japan expected Q4 2026/Q1 2027**

NNZ-2591

- **Neuren's Koala Phase 3 clinical trial in Phelan-McDermid syndrome commenced, with Fast Track designation and FDA alignment on single Phase 3 trial to support New Drug Application**
- **Rare Pediatric Disease priority review voucher program reauthorised; recent voucher sales for US$200 million and US$205 million**
- **Fast Track designation granted by FDA for Pitt Hopkins syndrome (PTHS) and new US patent issued to 2040 covering NNZ-2591 to treat PTHS**
- **Development for hypoxic ischemic encephalopathy (HIE) initiated, partnering with Hope for HIE, the worldwide voice of families who have children with HIE**
- ***SYNGAP-1* related disorder (SRD) added to development pipeline**

Melbourne, Australia: Neuren Pharmaceuticals Limited (ASX: NEU) announced financial results for 2025, reporting royalty income of A$65 million and profit after tax of A$30 million.

Neuren CEO Jon Pilcher commented: "In 2025 we achieved a critical milestone for Neuren's value creation strategy with the commencement of our Koala Phase 3 clinical trial of NNZ-2591 in Phelan-McDermid syndrome. There is so much to look forward to this year as we continue to execute that program towards a New Drug Application and in parallel advance NNZ-2591 for Pitt Hopkins syndrome and HIE. All of this is self-funded by our growing revenue from DAYBUE, which has now reached A$510 million since launch in 2023. We are very excited to watch the impact of the recent launch of DAYBUE STIX in the US as a potentially attractive new option for Rett syndrome patients and their families."

Financial commentary

	2025 A$m	2024 A$m
Royalty income	65	56
Interest income	12	11
	77	**67**
One-time revenue from first sales milestone	-	80
One-time revenue from sale of RPD Priority Review Voucher	-	77
Foreign currency gain	8	4
Total income	**85**	**228**
R&D expenditure	(36)	(33)
Corporate & administration expenditure	(6)	(5)
Foreign currency loss	(3)	(7)
Profit before tax	**39**	**183**
Income tax expense	(9)	(41)
Profit after tax	**30**	**142**

In 2025 royalty revenue of A$65 million was earned under the license agreement with Acadia, up 15% from A$56 million in 2024. Interest income was A$12 million, up from A$11 million in 2024. Revenue from Acadia in 2024 also included one-time sales milestone revenue of A$80 million, as DAYBUE net sales for the year in North America exceeded US$250 million, and one-time revenue of A$77 million from Neuren's share of Priority Review Voucher sale proceeds.

Other income in 2025 included a foreign currency gain of A$8 million mainly due to the translation of cash and short-term investments held in Australian dollars to the US dollars functional currency (2024: A$7 million loss). This was partially offset by a loss of A$3 million on the fair value of outstanding forward contracts to sell Australian dollars and buy US dollars (2024: A$4 million gain).

Research and development costs increased by A$3 million to A$36 million for the year ended 31 December 2025, driven by commencement of the Phelan-McDermid syndrome Phase 3 trial.

Corporate and administrative costs of A$6 million in 2025 increased by A$1 million from the prior period, mainly due to increased share-based payments expense relating to new share options issued during 2025. Income tax expense for 2025 was A$9 million (2024: A$41 million).

Net profit after income tax for 2025 was A$30 million (2024: A$142 million).

Total cash and short-term investments at 31 December 2025 were A$296 million (31 December 2024: A$222 million). Net cash generated from operating activities in 2025 was A$125 million, compared with net cash used of A$11 million in 2024. Neuren made tax payments of A$54 million in 2025, which included A$43 million for 2024 tax and A$11 million instalments for 2025 tax, compared with tax payments of A$37 million in 2024. Net cash used in financing activities was A$33 million, comprising A$39 million of payments for the share buy-back, offset by A$6 million of proceeds received on conversion of loan funded shares and exercise of share options.

Capital management

During 2025 Neuren completed the on-market share buy-back program commenced in 2024, in which A$50 million was deployed to buy back shares at an average price of A$12.27 per share. A new share buy-back program is being initiated on 2 March 2026, reflecting the Board's view that the current share price materially undervalues Neuren's assets, relative to internal analyses and the range of recently published analyst valuations. Neuren has a very strong cash position supported by growing cash flows from the DAYBUE franchise. Neuren's NNZ-2591 development programs for PMS, PTHS and HIE all remain well-funded alongside the buy-back, which will occur at Neuren's discretion and will not exceed 5% of the total shares on issue 12 months prior to the commencement of the buy-back. Throughout the buy-back period of up to 12 months, Neuren will continue to assess market conditions, the prevailing share price, operational performance, available investment opportunities and all other relevant considerations, and may vary, suspend, or terminate the buy-back program at any time.

DAYBUE® (trofinetide)

In April 2023 Neuren's exclusive worldwide licensee for trofinetide, Acadia Pharmaceuticals (NASDAQ: ACAD), launched DAYBUE® (trofinetide) in the United States as the first approved treatment for Rett syndrome. In the period since launch, Neuren has earned cumulative income from DAYBUE of A$510 million.

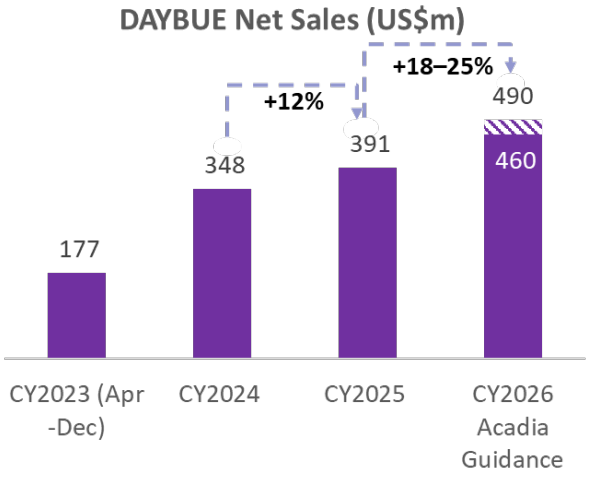
DAYBUE net sales for the year ended 31 December 2025 were US$391 million, delivering royalties of A$65 million to Neuren. The number of unique patients receiving a DAYBUE shipment continued to grow in 2025, exceeding 1,000 for the first time. The persistency rate after 12 months of treatment increased to approximately 55%. There is substantial potential for further growth in the US with two-thirds of the 6,000 diagnosed Rett patients yet to try DAYBUE. During the year Acadia completed an expansion of its DAYBUE field force in the US by ~30% to accelerate future growth in the community outside the Rett syndrome centers of excellence. In Q4 2025 momentum continued to build with 76% of new prescriptions originating from community physicians outside centers of excellence.

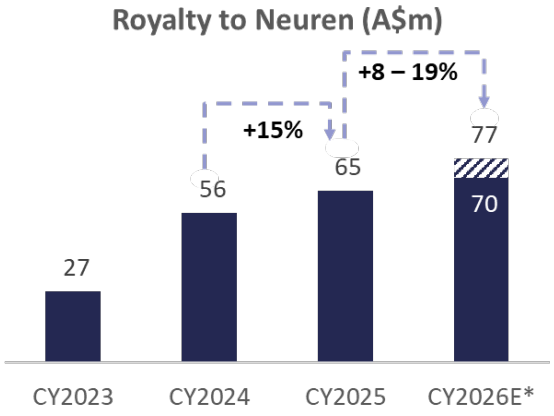
In December 2025 Acadia received US Food and Drug Administration (FDA) approval of DAYBUE STIX (trofinetide) for oral solution, a dye- and preservative-free powder formulation of trofinetide for the treatment of Rett syndrome in adult and pediatric patients two years of age and older. The new powder formulation offers children and adults living with Rett syndrome new flexibility and choice regarding the dose volume and taste of their DAYBUE treatment, potentially facilitating treatment of a significant number of new patients from families who had declined to try or discontinued the liquid formulation. DAYBUE STIX is being launched on a limited basis in Q1 2026 and more broadly early in Q2 2026. The existing oral solution formulation will remain available.

In January 2025 Acadia submitted a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for trofinetide for the treatment of Rett syndrome in adults and pediatric patients two years of age and older. In February 2026 Acadia was informed by the Committee for Medicinal Products for Human Use (CHMP) of the EMA of a negative trend vote on the MAA. Acadia intends to request a re-examination of the opinion by the CHMP following its formal adoption in late February, with the CHMP opinion on the re-examination likely at the end of Q2 2026.

Acadia announced the approval of DAYBUE oral solution by the Israel Ministry of Health in January 2026. In Japan trofinetide received Orphan Drug Designation and Acadia commenced a small clinical trial to support a marketing application, with results anticipated in Q4 2026 or Q1 2027, facilitating the application in 2027. Named patient supply programs are active and growing across multiple regions including Europe, the Middle East and Latin America.

Acadia has provided guidance for full-year net sales in 2026 of US$460-490 million. The guidance comprises sales only from the US and international named patient programs, with no inclusion of EU commercial sales from any potential EU marketing authorisation. Assuming this guidance is met and an exchange rate range of 0.70-0.72, Neuren anticipates earning full-year royalties of A$70-77 million.





* Based on Acadia full year 2026 DAYBUE Net Sales Guidance of US$460-490m, conservatively assuming North America royalty rates only (10% of DAYBUE net sales up to US$250m and 12% of DAYBUE net sales between US$250m and US$500m), and AUDUSD of 0.70 to 0.72

Neuren's entitlement to royalties and milestone payments from development and commercialisation of trofinetide in North America and outside North America are summarised in the following tables:

North America

✓	**US$10m**	**upfront in 2018**
✓	**US$10m**	**in 2022 following acceptance of NDA for review**
✓	**US$40m**	**in 2023 following 1st commercial sale in the US**
✓	**US$50m**	**In 2024 one third share of Priority Review Voucher awarded to Acadia (sold for US$150m)**
	US$55m	Milestone payments related to Fragile X

Tiered Royalty Rates (% of net sales)[1]

Annual Net Sales	Rates
≤US$250m	10%
>US$250m, ≤US$500m	12%
>US$500m, ≤US$750m	14%
>US$750m	15%

Sales Milestones[1]

	Net Sales in one calendar year	US$m
✓	≥US$250m	50
	≥US$500m	50
	≥US$750m	100
	≥US$1bn	150

Outside North America

✓	**US$100m**	**upfront in 2023**
	US$35m	following 1st commercial sale in Europe
	US$15m	following 1st commercial sale in Japan
	US$10m	following 1st commercial sale of a 2nd indication Europe
	US$4m	following 1st commercial sale of a 2nd indication Japan
	Sales milestones[1]	On achievement of escalating annual net sales thresholds: Europe: up to **US$170m** Japan: up to **US$110m** RoW: up to **US$83m**
	Tiered royalties[1]	**Mid-teens to low-20s %** of net sales

[1] Royalty rates payable on the portion of annual net sales that fall within the applicable range. Each sales milestone payment is payable once only.

NNZ-2591 (ercanetide)

In 2024 Neuren achieved positive top-line results from the Phase 2 clinical trials of NNZ-2591 in children with Phelan-McDermid syndrome (PMS), Pitt Hopkins syndrome (PTHS) and Angelman syndrome (AS).

PMS

In April 2025 Neuren announced that the primary endpoints for a single Phase 3 pivotal clinical trial of NNZ-2591 in PMS had been agreed with the FDA. Alignment with FDA was previously reached on the other key features of the Phase 3 program at an End of Phase 2 Meeting. In the second half of 2025 Neuren initiated the first two sites in the United States for the Koala Phase 3 trial and the first two participants commenced dosing in February 2026. Koala is a Phase 3, randomised, double-blind, placebo-controlled clinical trial evaluating the safety and efficacy of NNZ-2591 in approximately 160 children aged 3-12 years with PMS. A screening period of up to 4 weeks is followed by treatment with NNZ-2591 or placebo for 13 weeks. All participants may be eligible to continue treatment with NNZ-2591 for 12 months in an open-label extension trial.

The Koala trial co-primary endpoints will be the change from baseline in the Receptive Communication sub-domain of the Vineland Adaptive Behavior Scales, Third Edition (VABS-3 Receptive-Raw Score) and the overall score in the Phelan-McDermid Syndrome Assessment of Change (PMSA-C, previously referred to as CGI-I in Neuren's Phase 2 trial). Both measures were robustly positive with clinically meaningful improvement in Neuren's Phase 2 open-label clinical trial. The endpoints pair the caregiver's assessment of change in one of the most impactful health concerns in PMS with the clinician's assessment of change across multiple aspects of PMS.

In October 2025 Neuren was granted Fast Track designation by the FDA for the PMS program. Neuren's financial strength means that no additional funding is required to execute the program to support a New Drug Application.

In February 2026 the United States Congress reauthorised the Rare Pediatric Disease Priority Review Voucher (PRV) program to 30 September 2029. The program provides for the award of a PRV to drug developers that receive FDA approval for a drug for a designated rare pediatric disease. The voucher entitles the holder to priority review of a different drug or may be transferred or sold to another drug developer. Recently two drug developers announced the sale of vouchers for US$200 million and US$205 million respectively. Neuren currently holds Rare Pediatric Disease designation for NNZ-2591 in PMS, which means that marketing approval by FDA would qualify Neuren for a voucher, of which Neuren would retain 100% ownership and proceeds of any sale.

PTHS

During 2025 Neuren was granted Fast Track designation by the FDA for the PTHS program and a new patent covering NNZ-2591 to treat PTHS was also granted by the US Patent and Trademark Office, with expected expiry in 2040.

In early 2026 Neuren received feedback from the FDA regarding its clinical development plans for PTHS, which indicated that in a controlled trial to demonstrate efficacy of NNZ-2591, a PTHS-specific clinical

global impression (CGI) scale may be used as a co-primary endpoint if it is accompanied by an observer-reported functional outcome measure. This is similar to the approach that was agreed and is being implemented in Neuren's ongoing Phase 3 trial in Phelan McDermid syndrome (PMS). Neuren is currently assessing alternative trial designs and endpoint analysis methodologies to accommodate that PTHS is significantly rarer and generally more profoundly disabling than PMS. Further interaction with the FDA will likely be required to finalise this assessment.

HIE

During 2025 Neuren initiated the development of NNZ-2591 for hypoxic-ischemic encephalopathy (HIE), a devastating type of brain injury caused when a baby's brain does not receive enough oxygen or blood flow before or shortly after birth. Many thousands of babies and children experience HIE every year. It is one of the leading causes of neonatal death and neurodevelopmental disability worldwide.

Neuren believes NNZ-2591 can potentially provide a highly differentiated form of treatment continuing beyond acute treatment in the neonatal intensive care unit to target both the acute effects and chronic impairments resulting from HIE. In September 2025 Neuren commenced a formal partnership with Hope for HIE supporting development of NNZ-2591 to treat HIE. Hope for HIE is the global organisation connecting families, researchers, clinicians, biotech and more to improve the quality of life for children and families impacted by HIE.

In February 2026 Neuren received feedback on its plan to submit an IND application for the treatment of HIE and the proposed initial clinical study of the pharmacokinetics, tolerability and safety of NNZ-2591 for one month in neonates and infants with HIE to open the IND. FDA generally accepted this IND-opening clinical study and the doses of NNZ-2591 to be evaluated, providing some guidance on the inclusion/exclusion criteria and safety monitoring. FDA requested that Neuren provides additional juvenile animal study data to support NNZ-2591 dosing in neonatal participants prior to initiating the clinical study. Neuren plans to generate this data before submitting the IND application and commencing the clinical study. In parallel Neuren is continuing to advance the logistical requirements for study execution. FDA also encouraged Neuren to submit a future meeting request to discuss appropriate endpoints, study population and safety monitoring for a subsequent study, which Neuren intends will support registration.

SRD

In 2025 Neuren also added *SYNGAP1*-related disorder (SRD) into its neurodevelopmental disorders pipeline for NNZ-2591. SRD is caused by a variant on the *SYNGAP1* gene located on Chromosome 6, which is responsible for producing the SYNGAP1 protein. The protein acts as a regulator in the synapses and insufficient production leads to impaired communication between neurons. This results in the many neurological issues seen in SRD patients including intellectual disability, low muscle tone, global development delay, epilepsy, sensory processing disorder, gross and fine motor skill delays, coordination disorder, speech delay, sleep and behavior disorder and autism spectrum disorder. In an in-vitro model of SRD in human iPSC-derived neurons, treatment with NNZ-2591 reversed the neuronal dysfunction caused by *SYNGAP1* haploinsufficiency.

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.

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

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.