

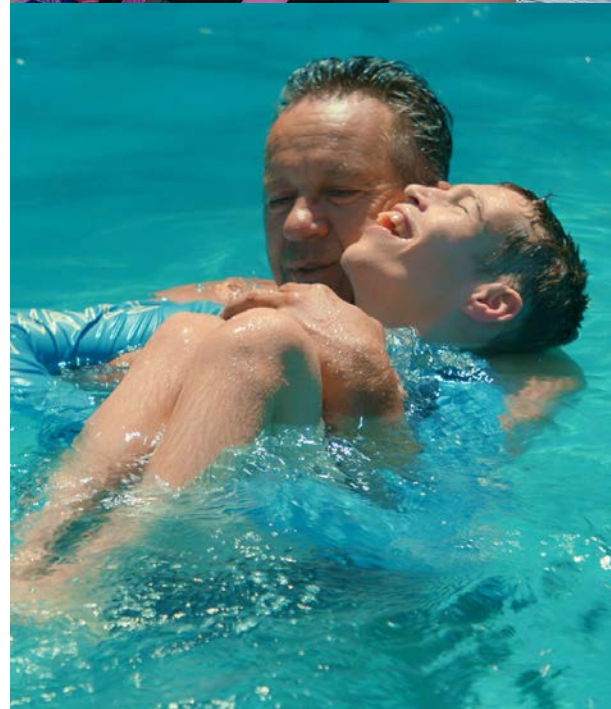
neuren

pharmaceuticals

2024 Full Year Results Webinar

28 Feb 2025

IMPROVING THE LIVES OF PEOPLE WITH
NEURODEVELOPMENTAL DISABILITIES



Forward looking statements

This presentation contains forward looking statements that involve risks and uncertainties. Although we believe that the expectations reflected in the forward looking statements are reasonable at this time, Neuren can give no assurance that these expectations will prove to be correct. Actual results could differ materially from those anticipated. Reasons may include risks associated with drug development and manufacture, risks inherent in the regulatory processes, delays in clinical trials, risks associated with patent protection, future capital needs or other general risks or factors.



Large potential upside for shareholders is enabled by financial strength

Maximise value of **NNZ-2591** as a multiple indication platform

- ✓ Positive Phase 2 results for **Phelan-McDermid syndrome**
- ✓ Positive Phase 2 results for **Pitt Hopkins syndrome**
- ✓ Positive Phase 2 results for **Angelman syndrome**

Long-term income growth from Acadia's successful global commercialization of



A\$359 million pro-forma cash¹

Value

¹ A\$222 million cash and short-term investments at 31 December 2024, adjusted to include receipt in Q1 2025 of PRV sale proceeds, sales milestone and Q4 2024 royalty and payment in Q1 2025 of Q4 2024 tax

The 2024 numbers – a record year of income for shareholders

Comprehensive income (CI)

A\$166m

CI margin

73%

NPAT

A\$142m

FX gain

A\$24m

Corporate & admin costs

A\$5m

R&D investment in NNZ-2591

A\$33m

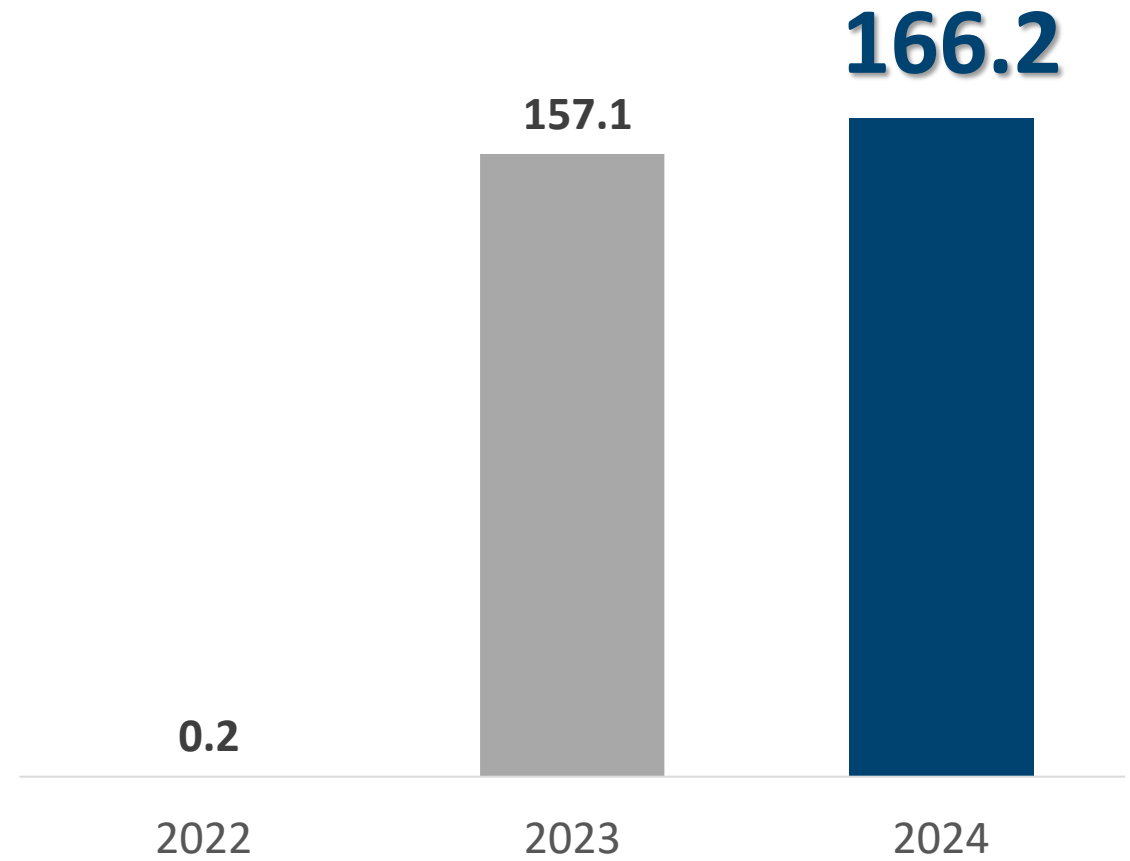
DAYBUE™ revenue 23 & 24

A\$445m

Pro-forma cash

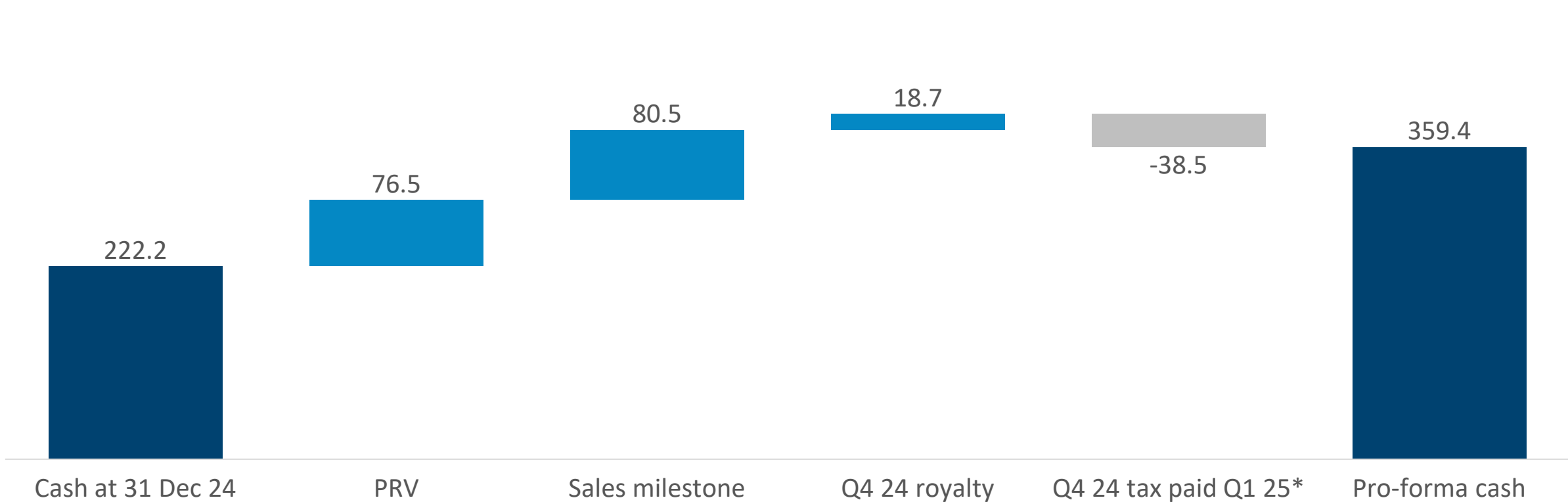
A\$359m

Total comprehensive income (A\$m)



Record high cash reserve organically generated to fund growth

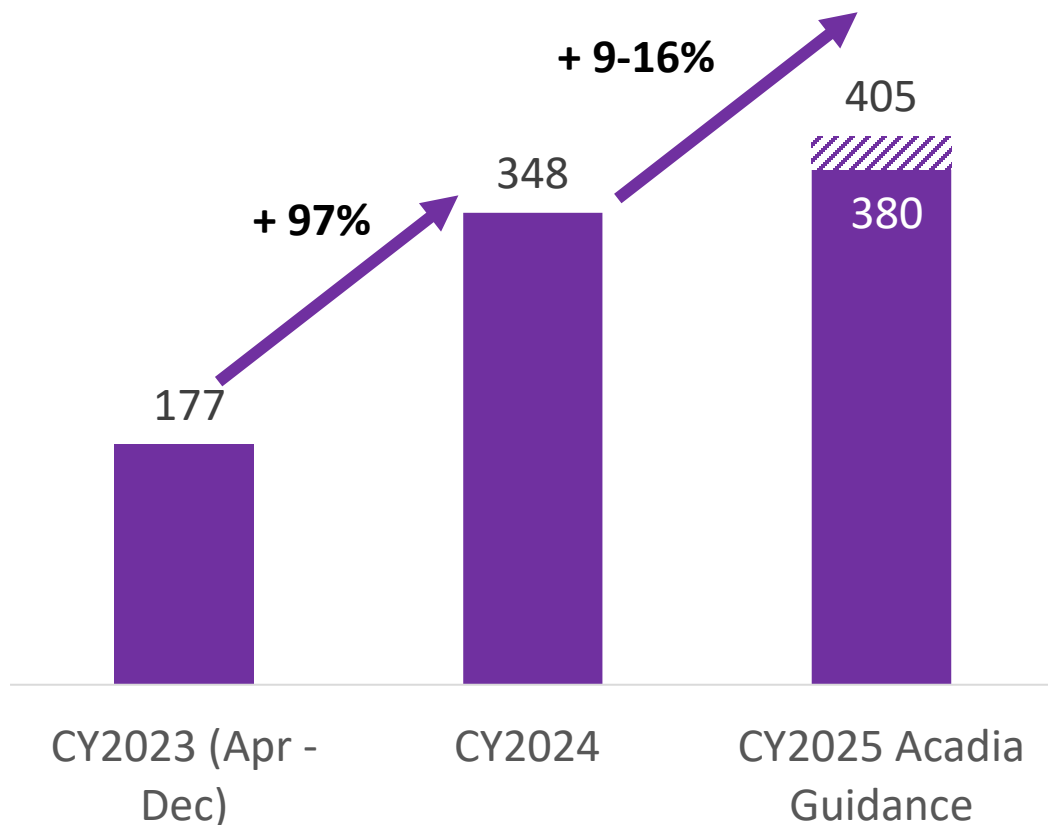
Cash and Pro-forma Cash (A\$m)



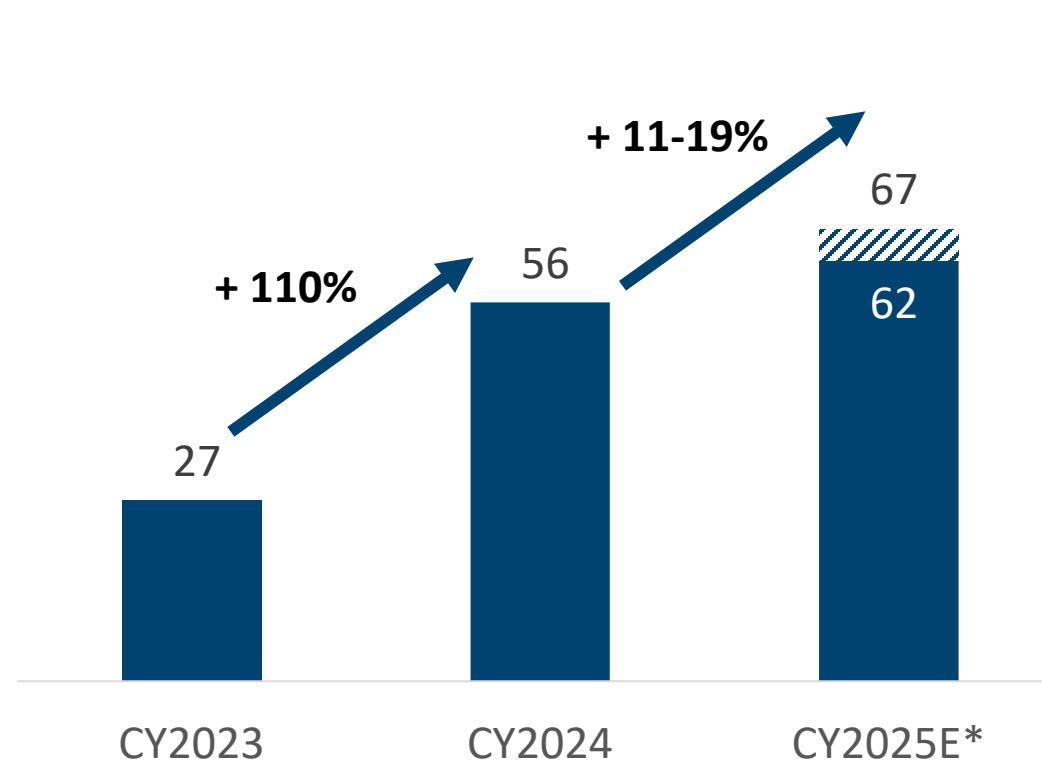
* Includes US withholding tax on Q4 24 royalty and sales milestone payment

Growing sustainable income for Neuren from DAYBUE™ in US

DAYBUE US Net Sales (US\$m)



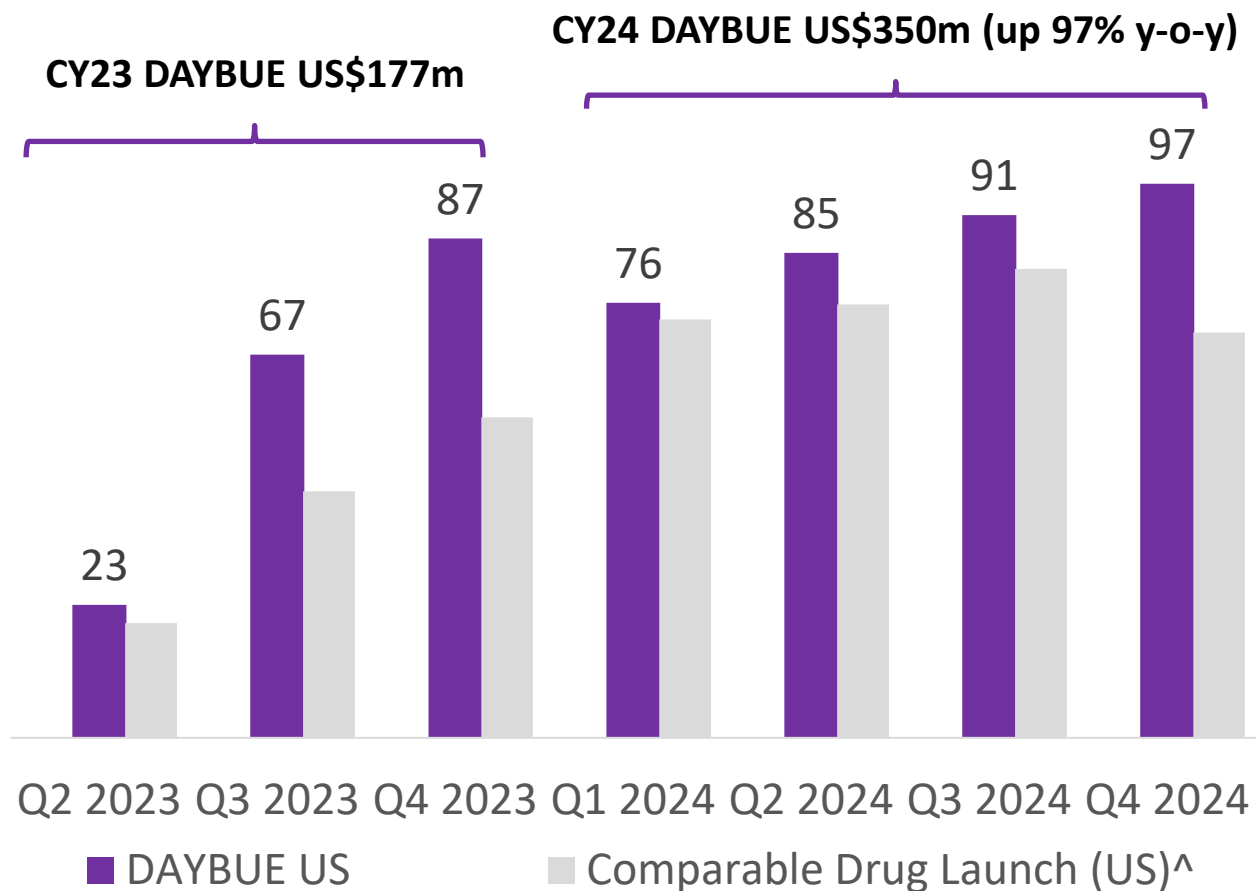
US Royalty to Neuren (A\$m)



* Based on CY25 Acadia DAYBUE US Net Sales Guidance of US\$380-405m, 10% of DAYBUE net sales up to US\$250m and 12% of DAYBUE net sales between US\$250m and US\$500m, and AUDUSD of 0.65

Q4 2024 was a record quarter of DAYBUE™ sales since launch in the US

US Net Sales (US\$m)



Significant further growth potential in the US¹:

- Diagnosed population expanding towards prevalence of 6,000-9,000
 - Currently 5,500–5,800 diagnosed patients in the US, grown from 4,500 at launch
- Continue growing new patient starts beyond 30% of diagnosed patients treated to date
 - Acadia increasing size of field force by ~30% and use of predictive analytics
 - Launching branded direct-to-consumer campaigns
 - Bringing positive experience to life, using growing body of real-world evidence, including LOTUS study, HCP peer-to-peer program, caregiver program series
- Maintaining stabilized persistency rate
 - 50% or higher after 12 months
 - 62% of active patients have been on treatment >12 months

[^] For illustrative purposes only. Comparable Orphan Drug has different patient/clinician experience, approval and distribution/logistical dynamics

¹ Latest stats based on Acadia 4Q and full year 2024 Earnings Presentation 26 February 2025 and 43rd Annual JP Morgan Healthcare Conference Presentation 14 January 2025

Long term growth opportunity for trofinetide through global expansion

Canada

600 - 900 Rett patients¹
Approved in Oct 2024
First sales in Q3 2025



US

6,000 - 9,000 Rett patients¹
Launched in Apr 2023



Europe

9,000 - 12,000 Rett patients¹
MAA filed with potential approval
Q1 2026
Initiation of Managed Access
Program Q2 2025
Acadia building EU leadership and
launch teams

Japan

1,000 - 2,000 Rett patients¹
PMDA discussions
ongoing; clinical study start
by Q3 2025 to support
marketing application

¹ Acadia estimates

Economics to Neuren from Acadia partnership

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)		Sales Milestones	
Annual Net Sales	Rates	Net Sales in one calendar year	US\$m
≤US\$250m	10%	≥US\$250m	✓ 50
>US\$250m, ≤US\$500m	12%	≥US\$500m	50
>US\$500m, ≤US\$750m	14%	≥US\$750m	100
>US\$750m	15%	≥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 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 **Mid-teens to low-20s %** of net sales

Leveraging trofinetide experience into multiple indications for NNZ-2591

Most advanced clinical program in PMS and PTHS, oral therapy competitive in AS

NNZ-2591

Indications	Orphan	Rare Pediatric	Fast Track	Positive Phase 2	Positive EoP2 Meeting	Commence Phase 3
Phelan-McDermid syndrome (PMS)						Plan for mid year 2025; type C meeting in April 2025
Pitt Hopkins syndrome (PTHS)						
Angelman syndrome (AS)						
Other pending						

Key milestones met and targeted

Milestones Achieved in 2024

- ✓ CY2024 DAYBUE net sales of ~US\$348m in the US, generated A\$56m royalties to Neuren and exceeded threshold for first sales milestone of US\$50m
- ✓ CY2024 Neuren total comprehensive income of A\$166m
- ✓ DAYBUE approved by Health Canada
- ✓ Trofinetide PIP accepted by EMA
- ✓ PRV sold for US\$150m, Neuren received 1/3
- ✓ Positive Phase 2 results for PMS, PTHS, AS
- ✓ Positive End of Phase 2 meeting with FDA for PMS
- ✓ A\$222m cash at 31 Dec 2024, A\$359m pro-forma cash¹

2025 Milestones

- ✓ Submission by Acadia of EU marketing application for trofinetide
 - First sales by Acadia of DAYBUE in Canada (Q3 2025)
 - Initiation by Acadia of Managed Access Program for trofinetide in Europe (Q2 2025)
- CY2025 DAYBUE US net sales guidance US\$380 – 405m, implying US\$62 – 67m US royalties to Neuren²
- Confirm alignment with FDA on primary efficacy assessment for PMS Phase 3 trial at Type C meeting in April
- Commence Phase 3 trial for PMS
- Advance PTHS and AS to registration trials subject to FDA consultation
- Advance Prader-Willi syndrome and/or undisclosed indications

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² Based on CY25 Acadia DAYBUE Net Sales Guidance of US\$380-405m, 10% of DAYBUE net sales up to US\$250m and 12% of DAYBUE net sales between US\$250m and US\$500m, and AUDUSD of 0.65

CONTACT

investorrelations@neurenpharma.com

Authorised by the CEO & Managing Director of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124