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J.P.Morgan Healthcare Conference 2025

16 Jan 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.

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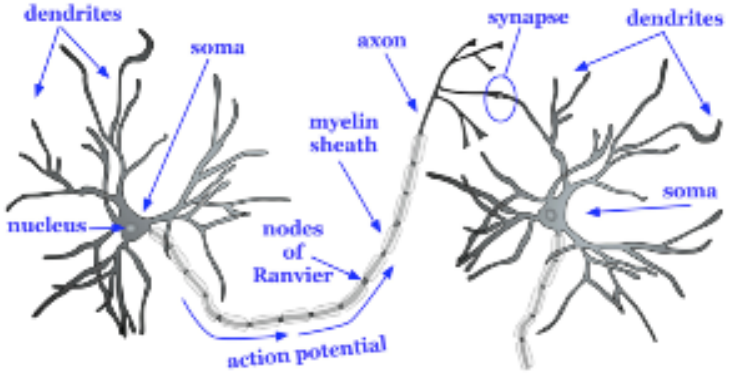
Seeking a ground-breaking impact on neurodevelopmental disorders

Rett	Fragile X	Phelan-McDermid	Angelman	Pitt Hopkins	Prader-Willi
MECP2	*FMR1*	*SHANK3*	*UBE3A*	*TCF4*	*15q11-q13*



Impaired communication between neurons, abnormal formation/pruning of dendrites & chronic inflammation

Neuren's drugs

target the critical role of IGF-1 in this upstream process, using analogs of peptides that can be taken orally as liquids

Severe impact on nearly every aspect of life		
walking and balance issues	anxiety and hyperactivity	seizures
Impaired communication	intellectual disability	Impaired social interaction
impaired hand use	sleep disturbance	gastrointestinal problems

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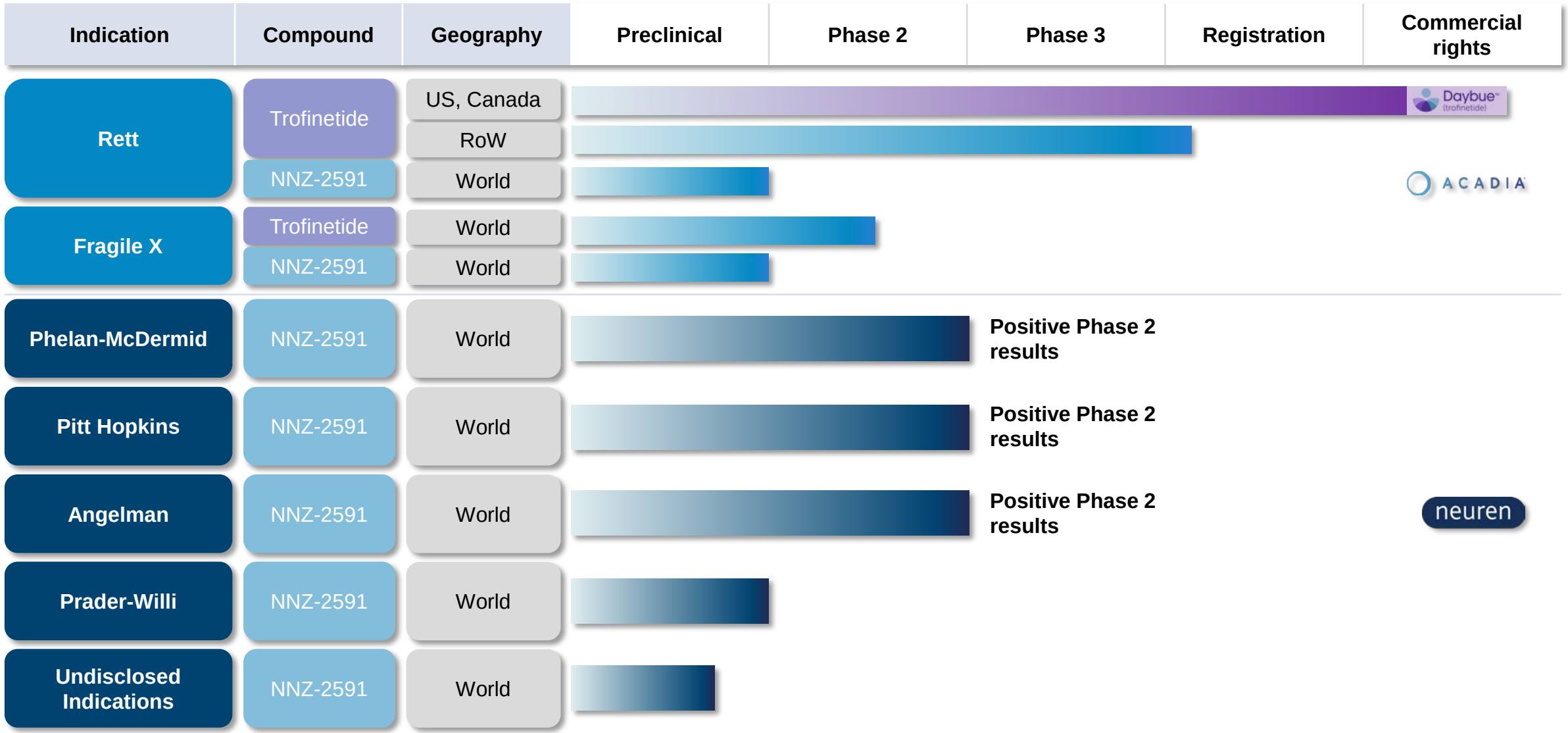
Commercial and late-stage pipeline



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Large potential upside supported by robust financial foundation

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**

Strong sustainable income growth from Acadia's successful global commercialization of Daybue™ (trofinetide)

A$210m cash as at 30 Sep 2024

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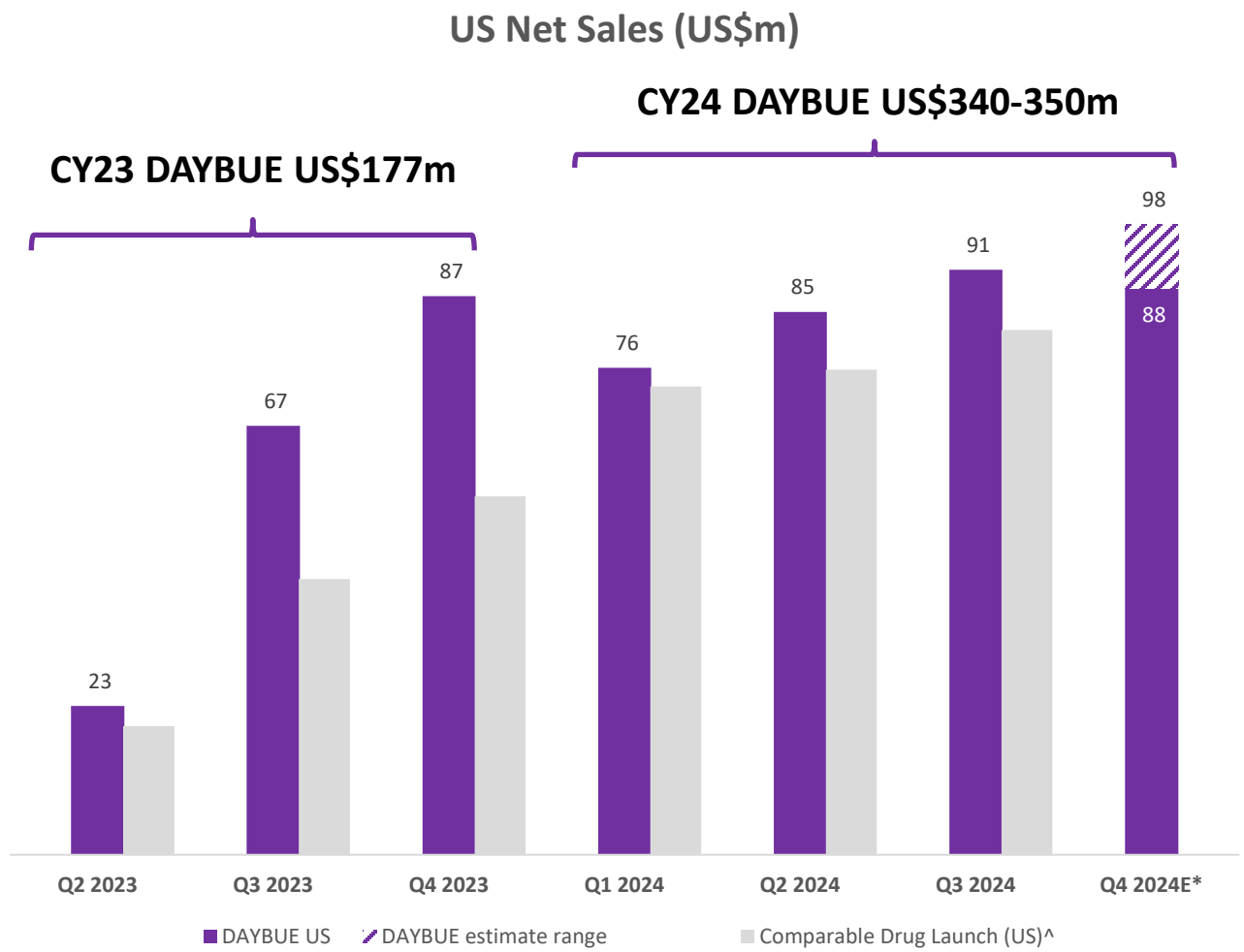
DAYBUE is a highly successful rare disease launch in the US



Significant further growth potential in the US[1]:

- 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 1,600 patients treated to date
  - Acadia substantially increasing size of field force 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
  - 66% of active patients have been on treatment >10 months

* Implied by the difference between Acadia's full year CY2024 net sales guidance and actual net sales for 9 months to date

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

1. Latest stats based on Acadia 43rd Annual J.P. Morgan Healthcare Conference Presentation 14 January 2025 and Acadia 3Q24 Earnings Presentation 6 November 2024

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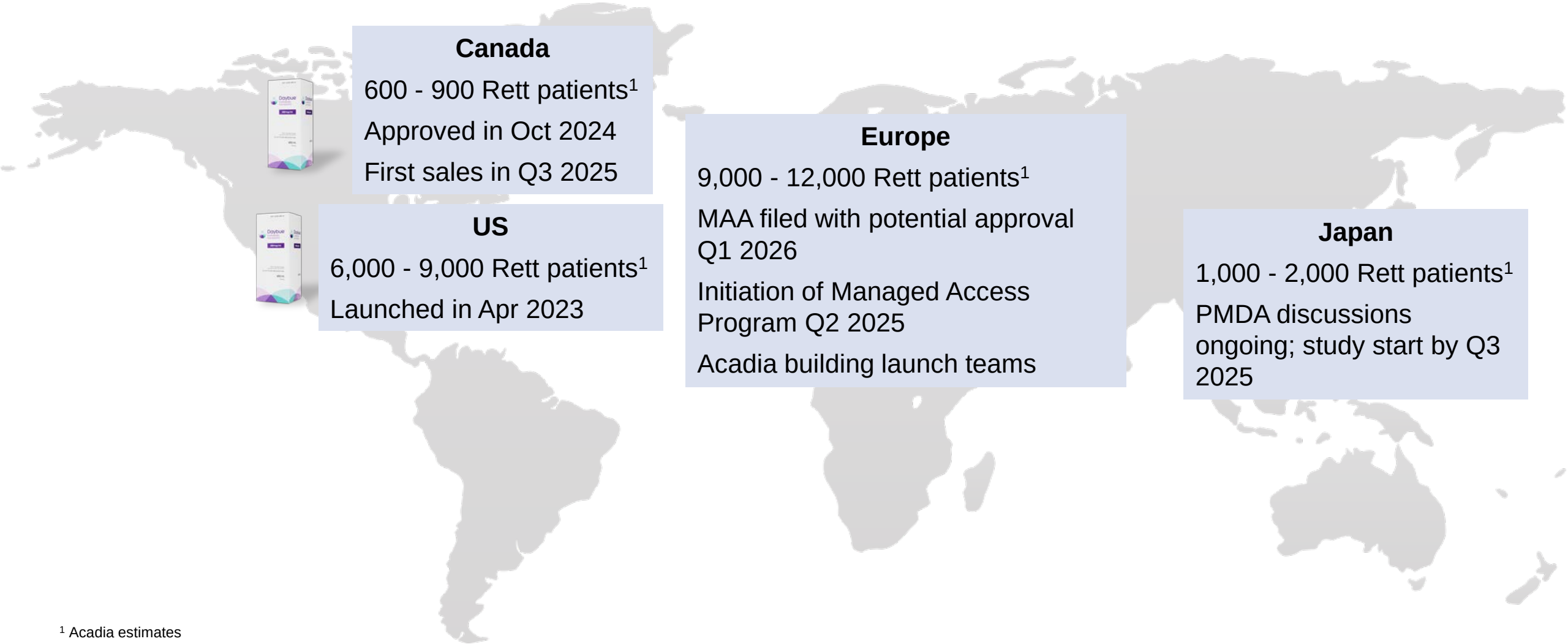
Long term growth opportunity through global expansion



Canada

600 - 900 Rett patients[1]

Approved in Oct 2024

First sales in Q3 2025

US

6,000 - 9,000 Rett patients[1]

Launched in Apr 2023

Europe

9,000 - 12,000 Rett patients[1]

MAA filed with potential approval Q1 2026

Initiation of Managed Access Program Q2 2025

Acadia building launch teams

Japan

1,000 - 2,000 Rett patients[1]

PMDA discussions ongoing; study start by Q3 2025

[1] Acadia estimates

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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)

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

Sales Milestones

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	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

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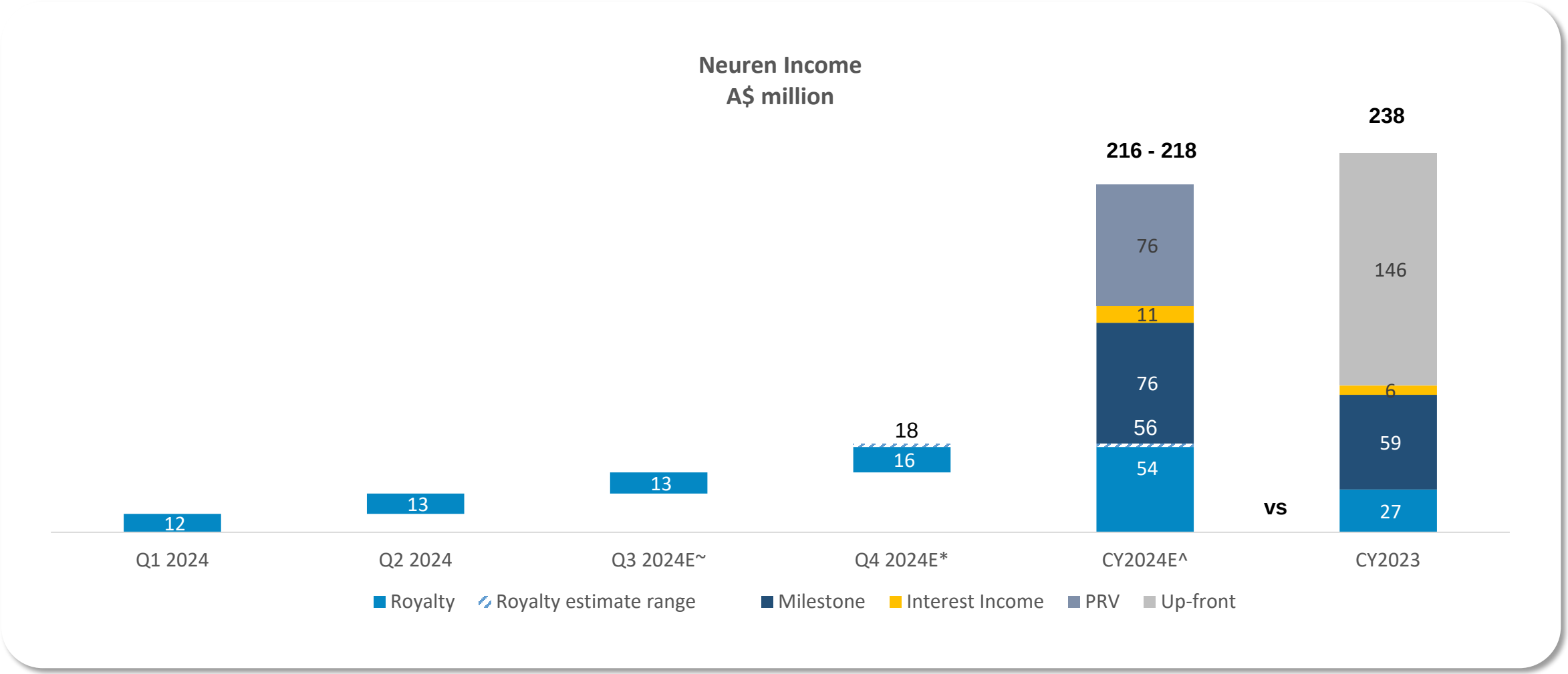
Growing sustainable income to Neuren from DAYBUE



~ Based on 10% of DAYBUE net sales of US$89.5, 12% of DAYBUE net sales of US$1.7 and AUDUSD of 0.692842
* Based on 12% of DAYBUE net sales and assumed AUDUSD of 0.66
^ PRV based on US$50m, which is 1/3 share of the US$150m total sale price, before applicable cost, and assumed AUDUSD of 0.66; milestone payment based on US$50m and assumed AUDUSD of 0.66

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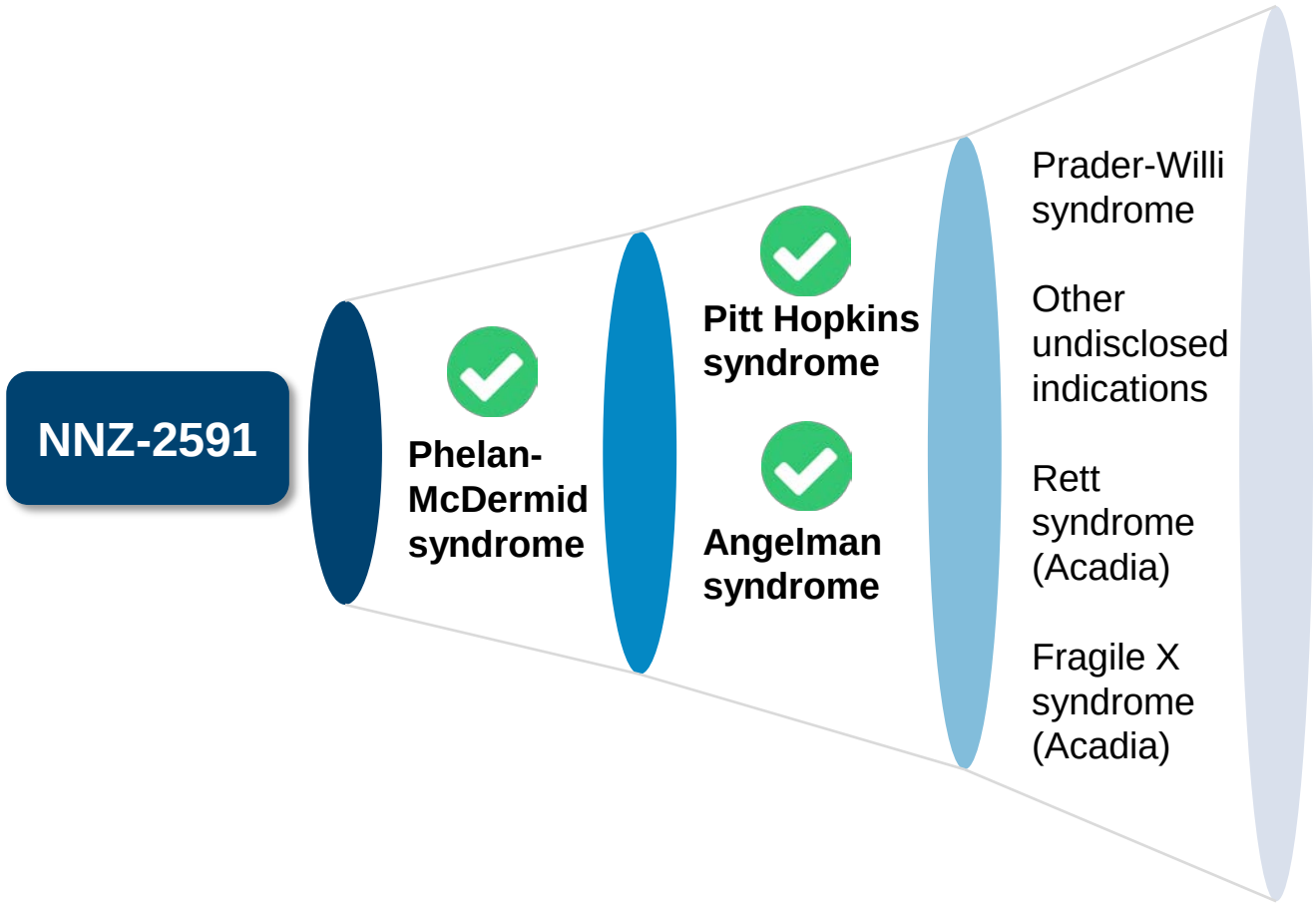
Multiple indications opportunity for NNZ-2591



- **Positive results in PMS, PTHS and AS syndrome Phase 2 trials**
- **Most advanced clinical program in PMS and PTHS, oral therapy competitive in AS**
- **Positive End of Phase 2 meeting with FDA for PMS - alignment on single Phase 3 trial, same dose, population and duration as Phase 2, with negotiation of primary efficacy endpoints continuing**
- US IND open for Prader-Willi syndrome
- Advancing non-clinical studies in multiple undisclosed indications
- Trofinetide and Rett syndrome experience directly transferable to NNZ-2591 programs
- Rett and Fragile X syndromes are licensed to Acadia, with same economics to Neuren as trofinetide; Neuren retains worldwide rights to all other indications

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Phase 2 trial results validating multi-indication platform

	Phelan-McDermid syndrome (PMS) N=18, 13 weeks	Pitt Hopkins syndrome (PTHS) N=11, 13 weeks	Angelman syndrome (AS) N=13, 13 weeks	
Safety & tolerability	Safe and well tolerated, with no meaningful trends in laboratory values or other safety parameters during treatment			
Efficacy			*All*	*3-12 years*
Clinician Global Impression of Improvement mean score (% of patients improved)	2.4 (89%)	2.6 (82%)	3.0 (85%)	*2.8 (100%)*
Caregiver Impression of Change mean score (% of patients improved)	2.7 (83%)	3.0 (73%)	3.2 (67%)	*2.6 (100%)*
# patients with Clinical Global Impression of Severity improvement (% of patients)	7 (39%)	6 (55%)	4 (31%)	
Consistent improvement in clinically important aspects	Communication, behavior, cognition, social	Communication, social, cognition, motor	Communication, behavior, cognition, motor	

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Significant market opportunity

No approved treatment for PMS, PTHS or AS

	Competitive Position	Estimated Prevalence	Potential Patients in the US[4]
Phelan-McDermid syndrome (PMS)	Most advanced clinical program	1/8,000 to 1/15,000 males and females[1]	17,000 - 32,000
Pitt Hopkins syndrome (PTHS)	Most advanced clinical program	1/34,000 to 1/41,000 males and females[2]	6,000 - 7,000
Angelman syndrome (AS)	Two RNA therapies (spinal injections) commencing Phase 3	1/10,000 to 1/20,000 males and females[3]	12,000 - 25,000

[1] Phelan McDermid Syndrome Foundation (PMSF) (www.pmsf.org)
[2] Pitt Hopkins Research Foundation (PHRF) (pitthopkins.org)
[3] Angelman Syndrome Foundation (ASF) (www.angelman.org), Facts About Angelman Syndrome
[4] Estimates based on United Nations population data 2022, derived by applying the estimated prevalence range to the populations under 60 years

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Key milestones

Milestones Achieved in 2024

- ✓ 9 months to date CY2024 DAYBUE net sales of ~US$252m in the US, generated A$38m royalties to Neuren and exceeded threshold for first sales milestone of US$50m
- ✓ DAYBUE approved by Health Canada
- ✓ Trofinetide PIP accepted by EMA
- ✓ PRV sold for US$150m, Neuren to receive 1/3
- ✓ Positive Phase 2 results for PMS, PTHS, AS
- ✓ Positive End of Phase 2 meeting with FDA for PMS
- ✓ A$210m cash as at 30 Sep 2024

2025 Milestones

- ✓ Submission by Acadia of EU marketing application for trofinetide
- CY2024E Neuren total income of A$216 – 218m[1]
- First sales by Acadia of DAYBUE in Canada (Q3 2025)
- Initiation by Acadia of Managed Access Program for trofinetide in Europe (Q2 2025)
- Confirm alignment with FDA on primary efficacy assessment for PMS Phase 3 trial
- 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

[1] Includes: Q1 and Q2 actual royalties, Q3 royalty estimate based on 10% of DAYBUE net sales of US$89.5 plus 12% of DAYBUE net sales of US$1.7 and AUDUSD of 0.692842, Q4 royalty estimate based on 12% of DAYBUE net sales implied by Acadia guidance and assumed AUDUSD of 0.66; PRV based on US$50m, which is 1/3 share of the US$150m total sale price, before applicable cost, and assumed AUDUSD of 0.66; milestone payment based on US$50m and assumed AUDUSD of 0.66; estimated interest income of A$11m

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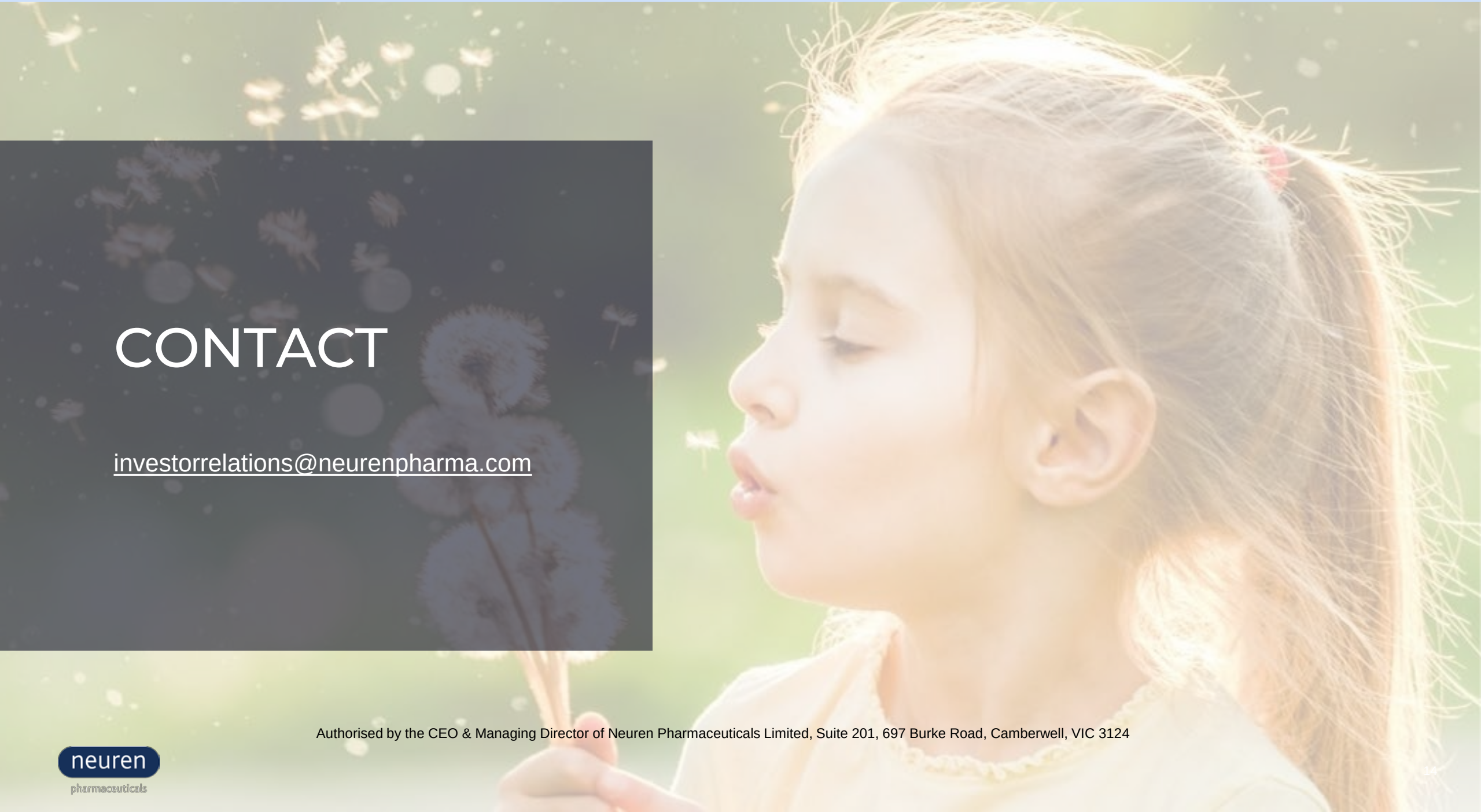

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