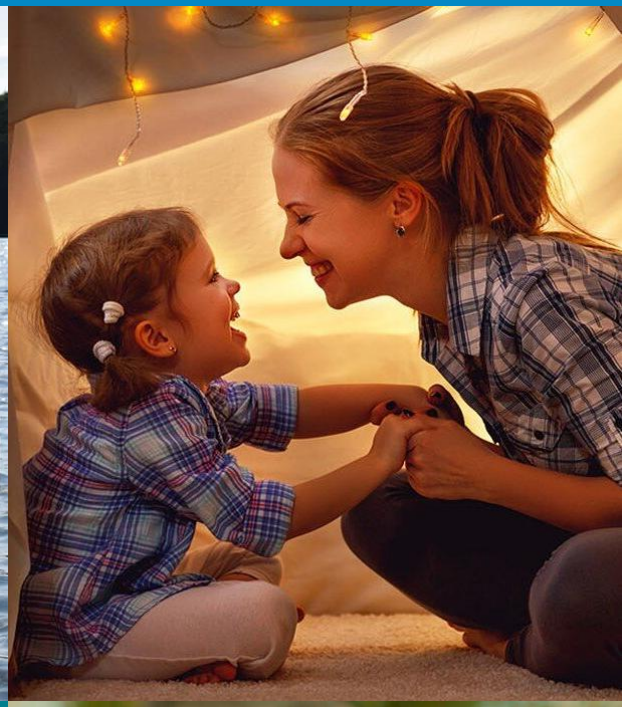
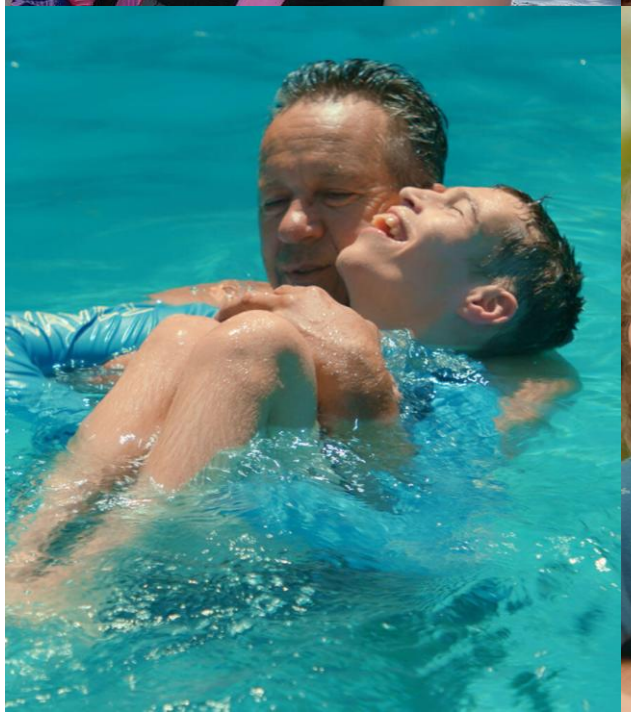
neuren pharmaceuticals

Investor Presentation

15 Nov 2024

IMPROVING THE LIVES OF PEOPLE WITH NEURODEVELOPMENTAL DISABILITIES

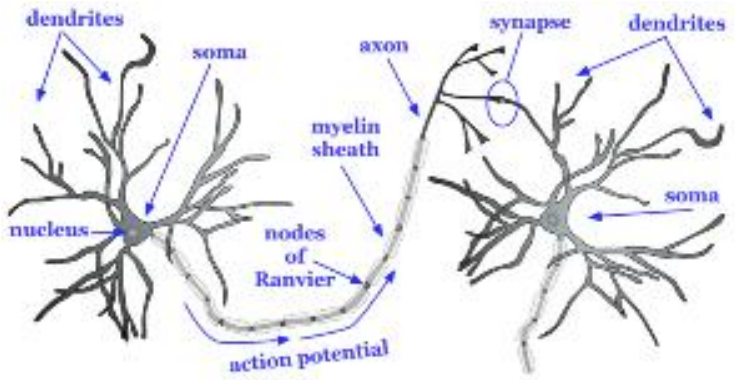

Forward looking statements

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.

neuren
pharmaceuticals

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

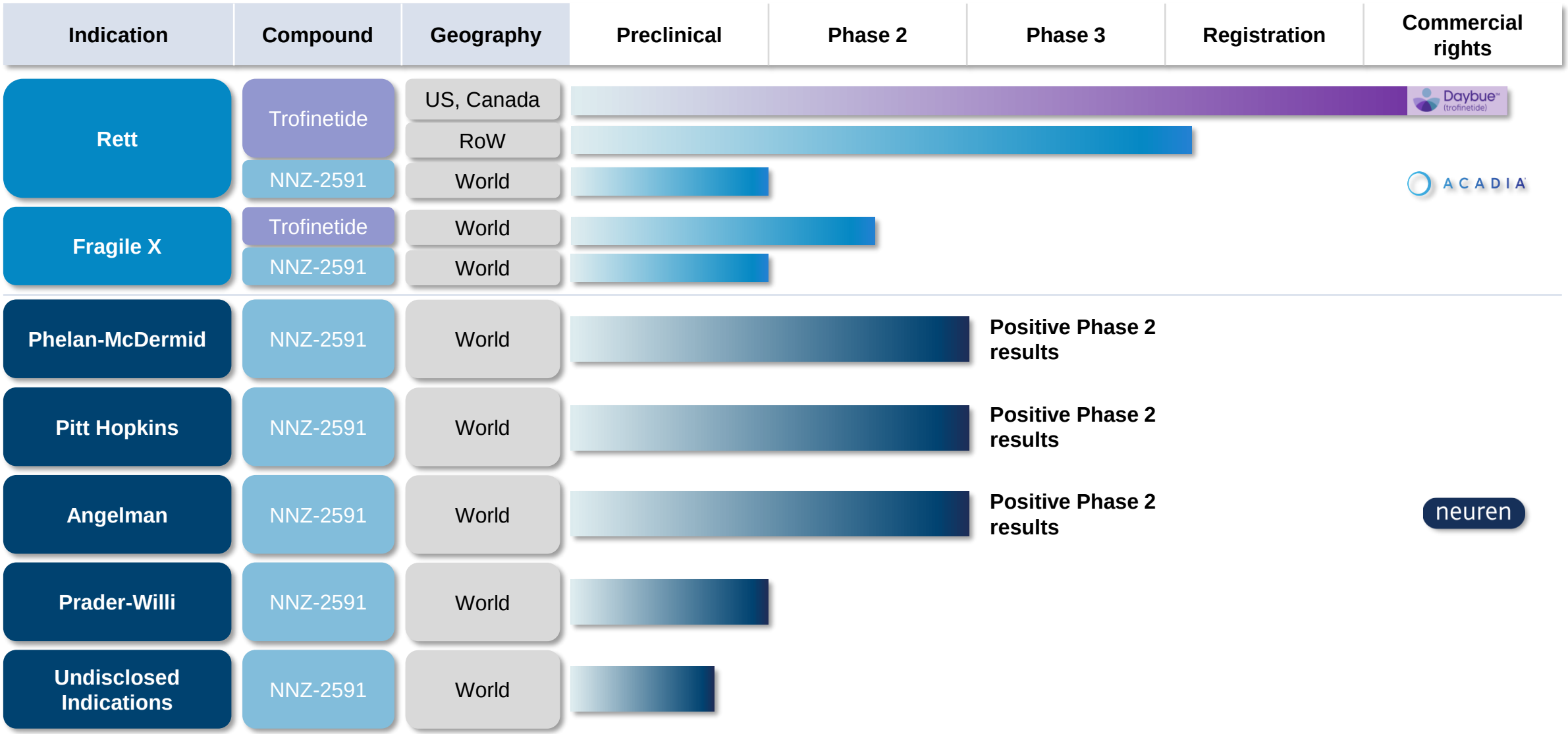
Neuren's drugs

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

neuren pharmaceuticals

Commercial and late-stage pipeline



neuren pharmaceuticals

Large potential upside supported by robust financial foundation

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

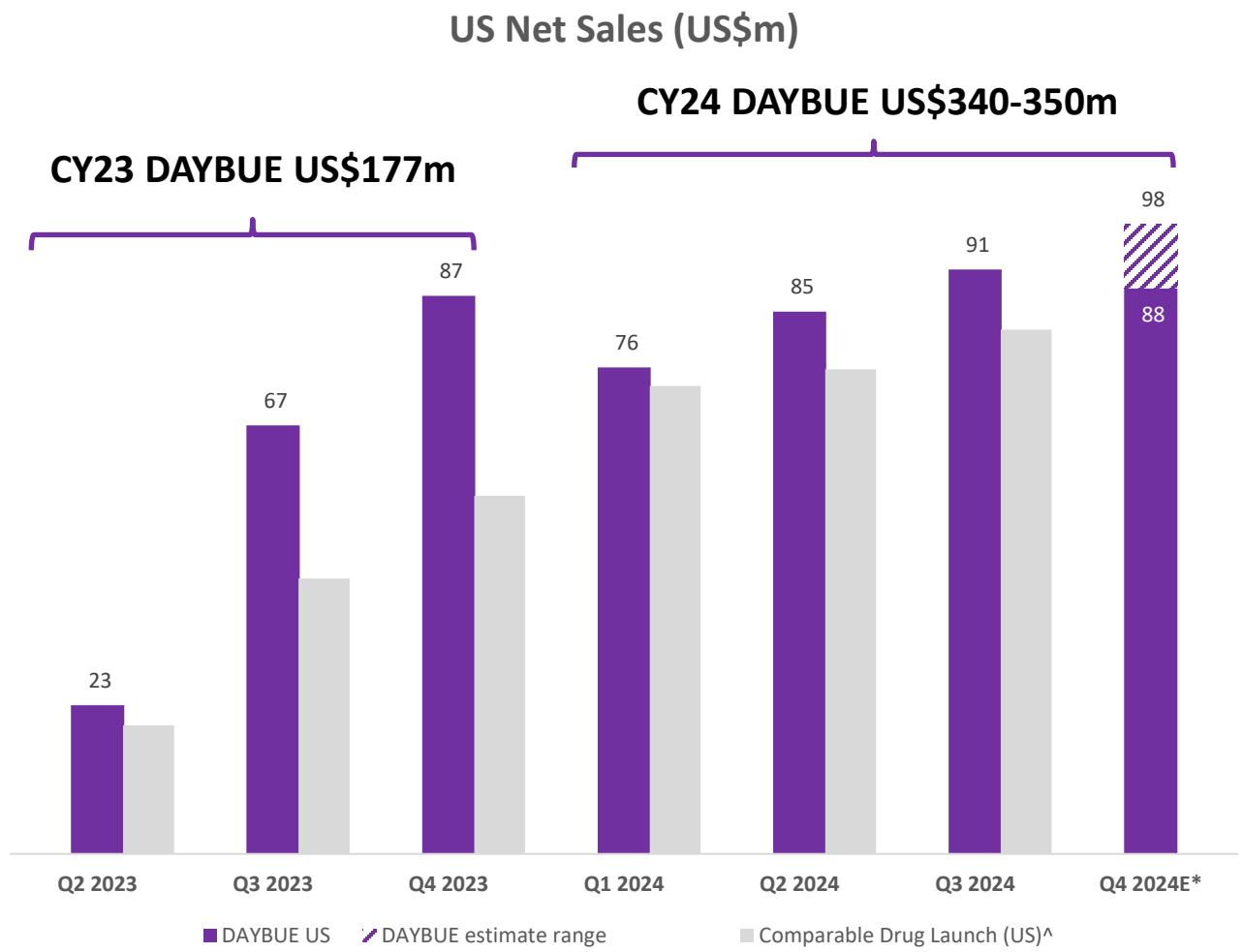
- ✓ Positive Phase 2 results for **Phelan-McDermid syndrome**
- ✓ Positive Phase 2 results for **Pitt Hopkins syndrome**
- ✓ Positive Phase 2 results for **Angelman syndrome**

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

A$210m cash as at 30 Sep 20204

neuren pharmaceuticals

DAYBUE is one of the most successful rare disease launches in the US



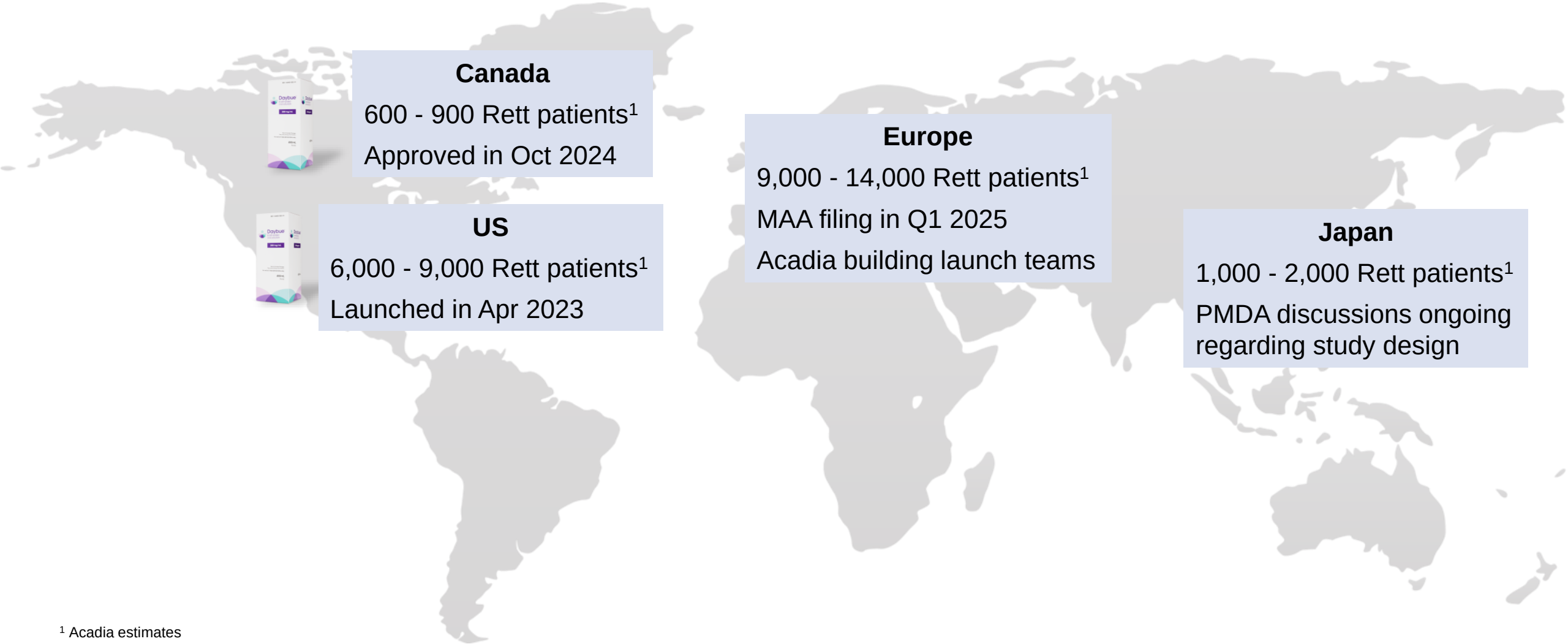
Further growth potential in the US:

- Continued increasing penetration:
  - Currently >30% of 5,000 diagnosed patients have initiated DAYBUE therapy
- Expanding diagnosed & addressable population towards prevalence of 6,000-9,000
  - ~10% increase in diagnosed patients since DAYBUE launch
- Stabilized persistency rate
  - 50% or higher after 12 months
  - 60% of all patients have now been on treatment >10 months
- Growing real-world evidence of positive experience
  - LOTUS study, HCP peer-to-peer program, caregiver program series

* 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

neuren pharmaceuticals

Global expansion driving long term growth



Canada

600 - 900 Rett patients[1]

Approved in Oct 2024

US

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

Launched in Apr 2023

Europe

9,000 - 14,000 Rett patients[1]

MAA filing in Q1 2025

Acadia building launch teams

Japan

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

PMDA discussions ongoing regarding study design

[1] Acadia estimates

neuren pharmaceuticals

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 Q2 2023 following 1st commercial sale in the US**
	US$50m	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**

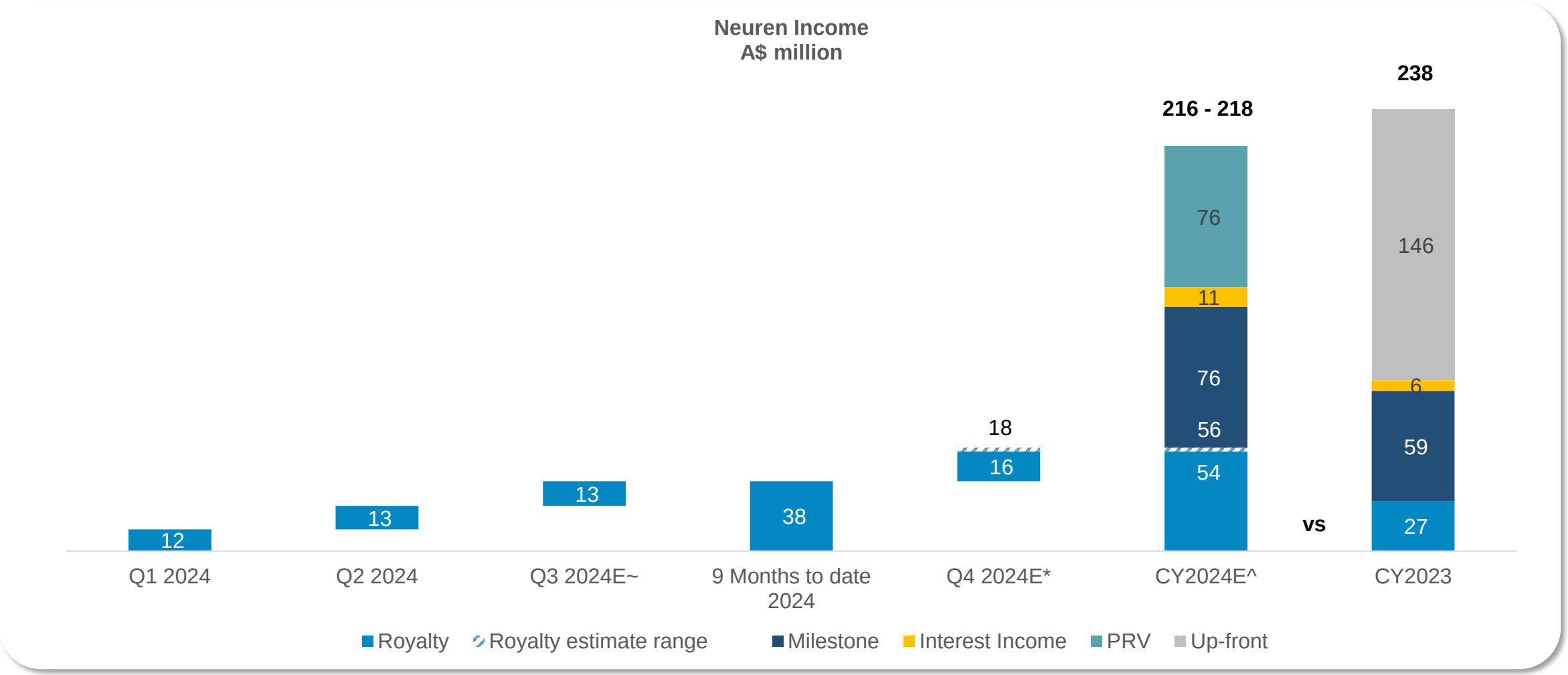
Outside North America

✓	**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

neuren pharmaceuticals

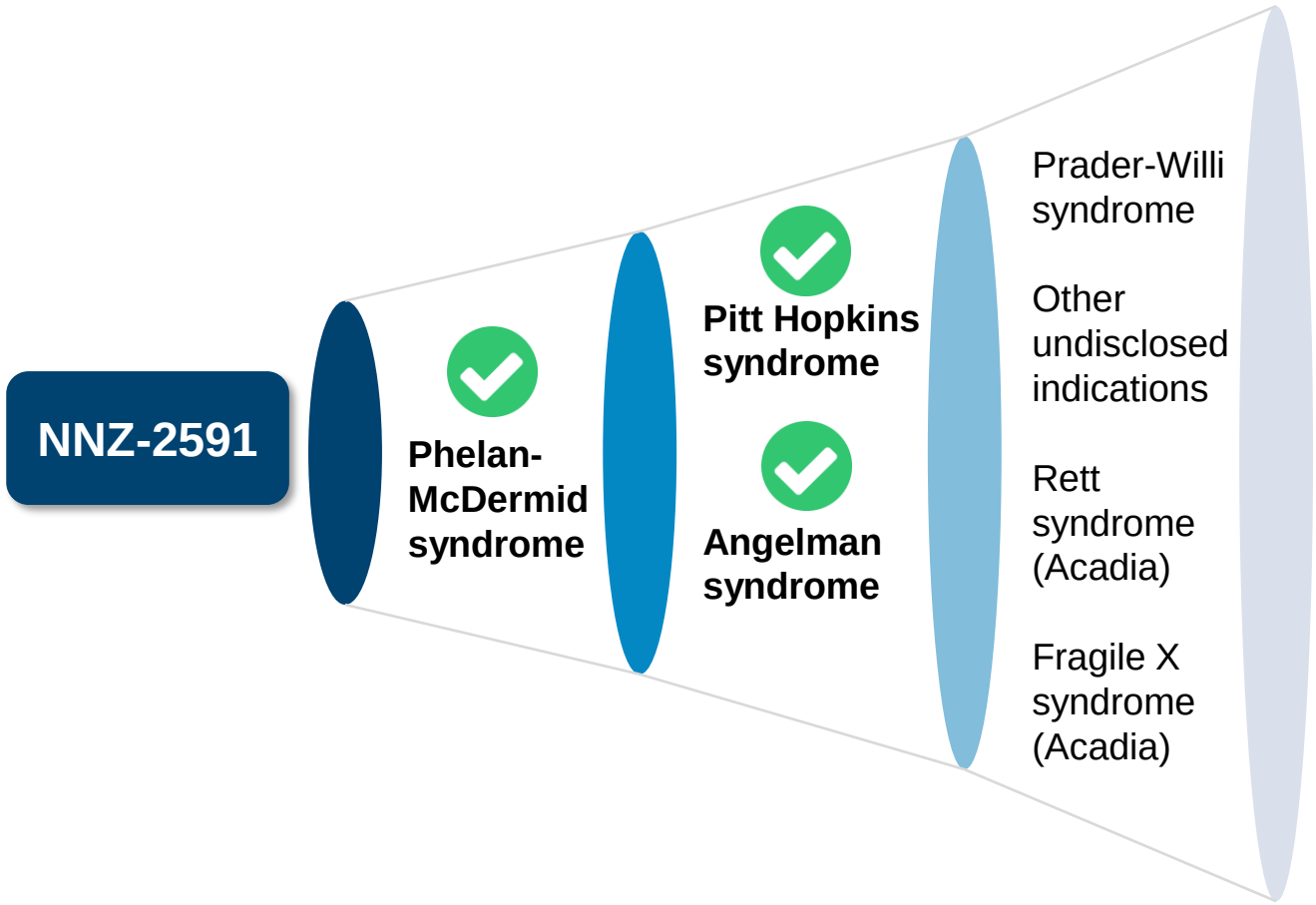
Growing sustainable income to Neuren from DAYBUE



~ 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

neuren pharmaceuticals

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**
- 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

neuren pharmaceuticals

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	

neuren pharmaceuticals

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

neuren pharmaceuticals

Key milestones

Milestones Achieved in 2024

- ✓ Positive Phase 2 results for PMS, PTHS, AS
- ✓ Positive End of Phase 2 meeting with FDA for PMS
- ✓ 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
- ✓ PRV sold for US$150m, Neuren to receive 1/3
- ✓ A$210m cash as at 30 Sep 20204
- ✓ DAYBUE approved by Health Canada
- ✓ Trofinetide PIP by Acadia accepted by EMA

Upcoming Milestones

- CY2024E Neuren total income of A$216 – 218m[1]
- Launch by Acadia of DAYBUE in Canada
- Submission by Acadia of EU marketing application for trofinetide
- Confirm alignment with FDA on primary efficacy assessment for PMS Phase 3 trial
- Commence Phase 3 program for PMS
- Advance PTHS and AS subject to FDA consultation
- Advance Prader-Willi syndrome and/or undisclosed indications

1 Include: 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

neuren pharmaceuticals

CONTACT

investorrelations@neurenpharma.com

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