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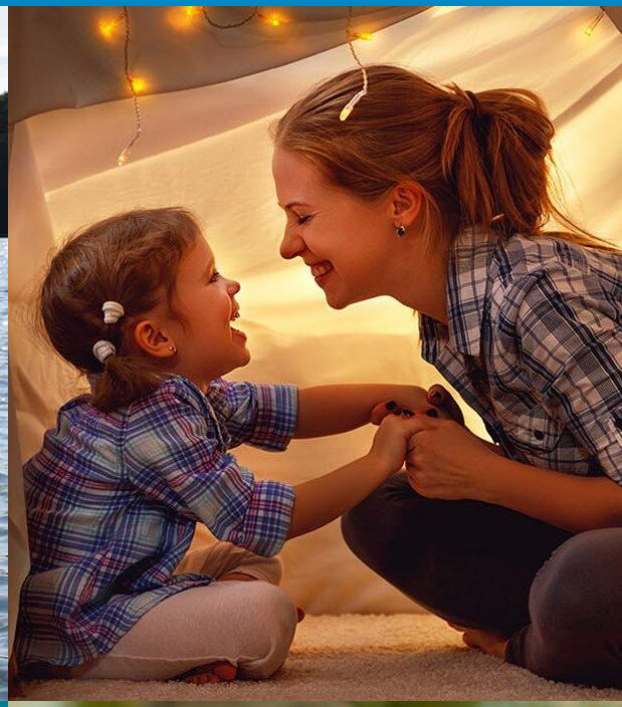
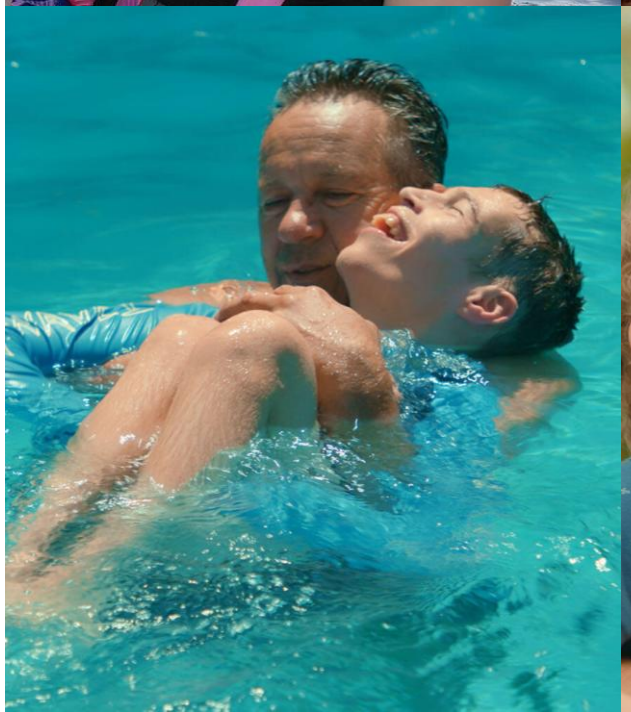
Investor presentation

27 February 2026

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.

Ground-breaking impact on pediatric neurological Orphan indications

Neurodevelopmental disorders

Rett *(MECP2)*

Phelan-McDermid *(SHANK3)*

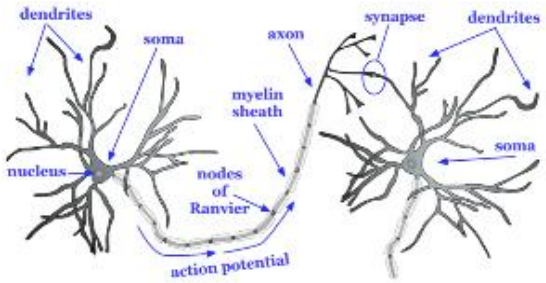
Pitt Hopkins *(TCF4)*

Fragile X *(FMR1)*

Angelman *(UBE3A)* | **Prader-Willi** *(15q11-q13)* | ***SYNGAP1*-related disorder** *(SYNGAP1)*

Brain injury

Hypoxic-Ischemic Encephalopathy
(lack of oxygen or blood flow to the brain before, during or shortly after birth)



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 naturally occurring peptides that can be taken orally as liquids

Excitotoxicity, mitochondrial dysfunction, and acute & chronic inflammatory processes

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**

Long-term impact on survivors

Developmental delays	**Seizures**
Cognitive impairment	
Cerebral palsy	

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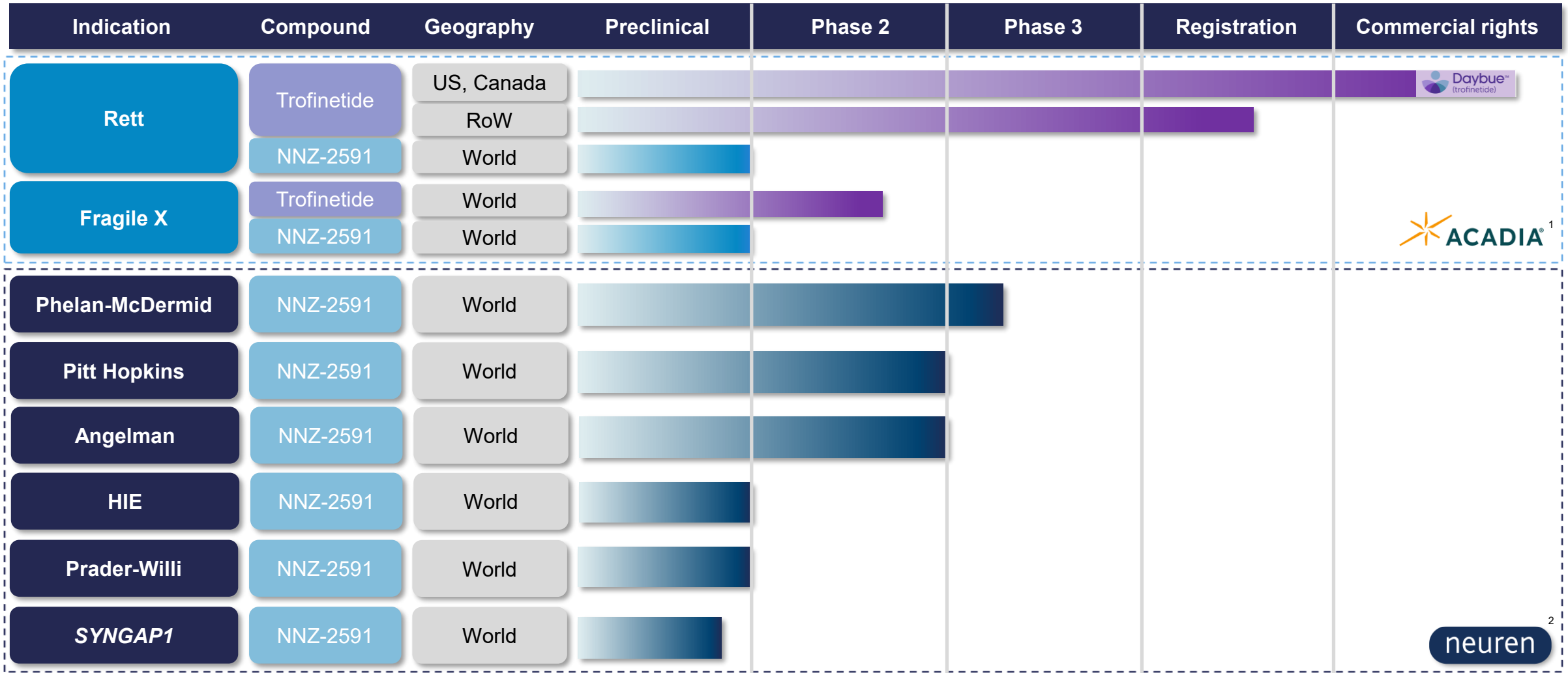
Multiple late-stage opportunities supported by commercial product



[1] Exclusive license for Trofinetide and NNZ-2591 (Rett and Fragile X only) globally [2] Wholly owned by Neuren

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Large potential upside for shareholders is enabled by financial strength

A$296 million cash as at 31 Dec 2025

Long-term income growth from DAYBUE® (trofinetide)

A$510m income from DAYBUE since launch in 2023

NNZ-2591 indications prioritised for maximum commercial impact

Phelan-McDermid syndrome

Pitt Hopkins syndrome

Hypoxic Ischemic Encephalopathy (HIE)

Angelman syndrome, Prader-Willi Syndrome, *SYNGAP1*, Rett syndrome (Acadia)*, Fragile X syndrome (Acadia)*

*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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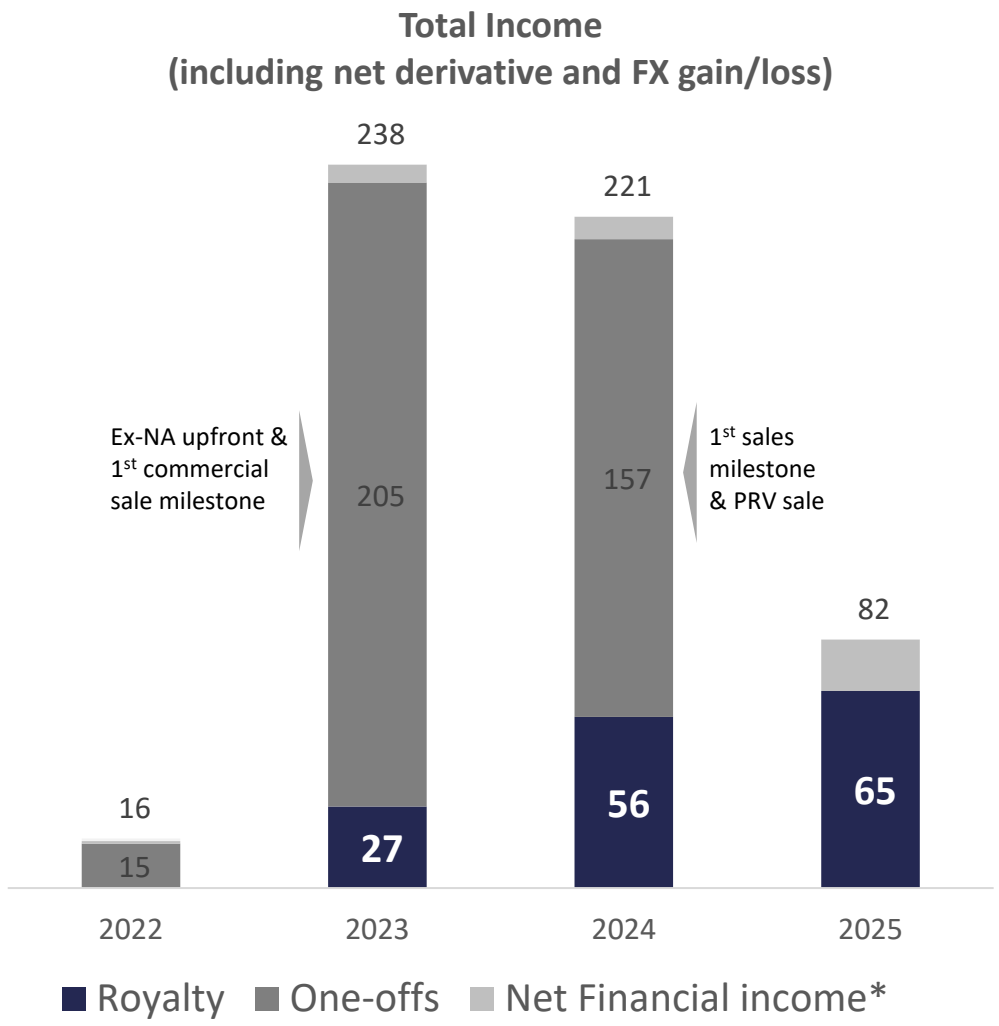
The 2025 numbers – growing sustainable income



- **A$65m** royalty income up 15% YoY
- Interest income **A$12m**
- **A$30m** NPAT without any one-time revenue
- Corporate & admin costs only **A$6m**
- R&D investment in NNZ-2591 **A$36m**
- **A$510m** DAYBUE revenue since launch in 2023

* Finance Income + net gain / (loss) on financial derivatives + net FX gain / (loss), excluding FX translation

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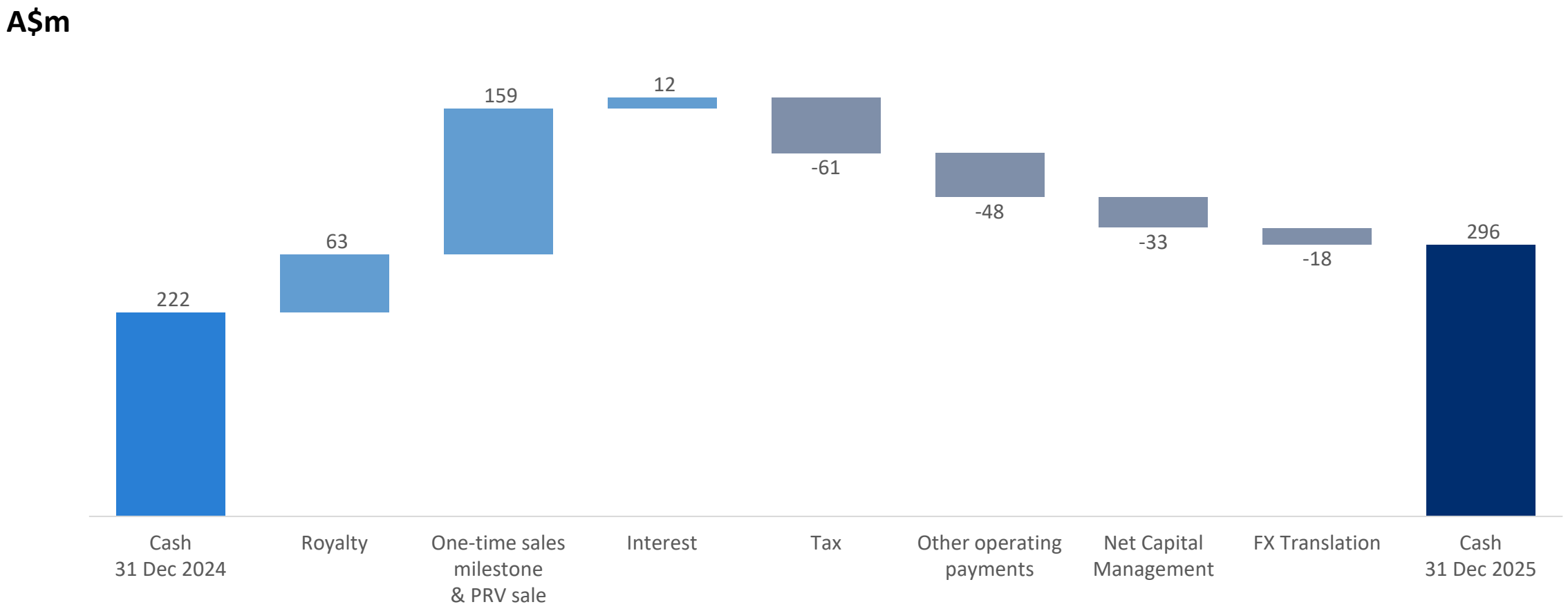
Organically generated cash flow continues to fund growth



DAYBUE®
(trofinetide)

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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 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

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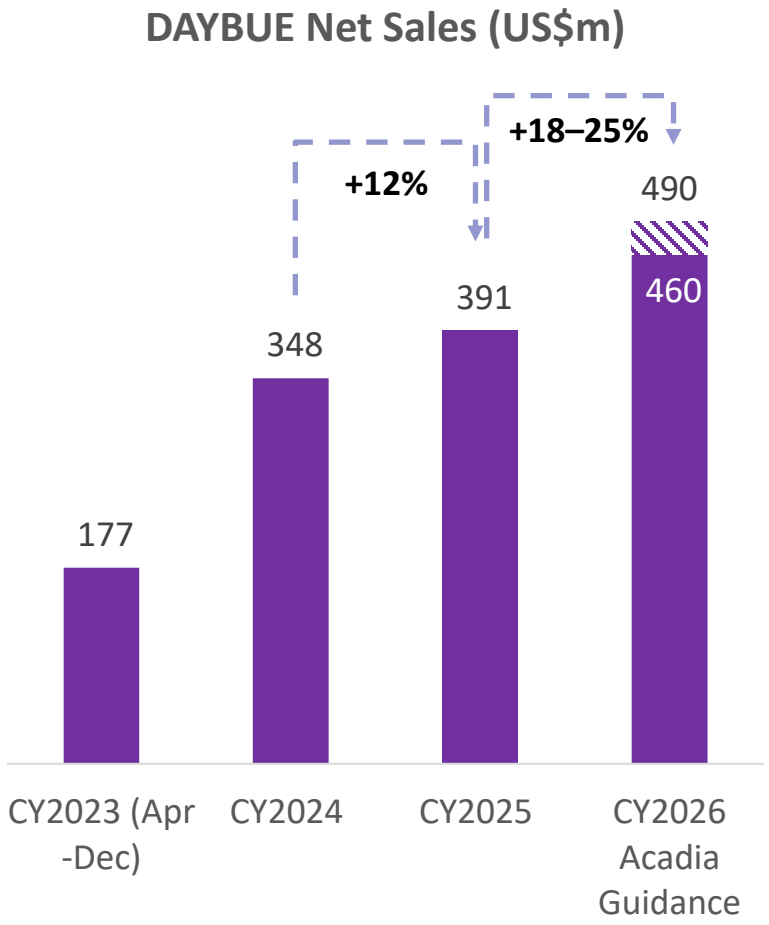
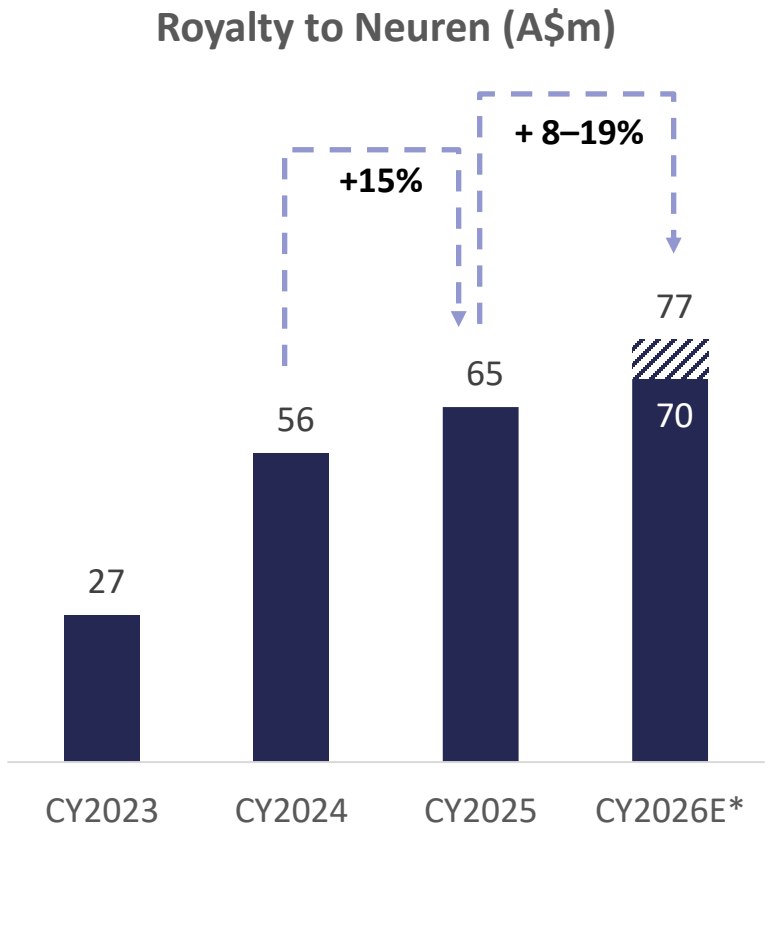
Growing income from US plus international named patient programs

1,070 patients on active treatment[1]

DAYBUE STIX launch during H1 2026





[1] Acadia Q4 and Full Year 2025 Earnings Call presentation

* 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

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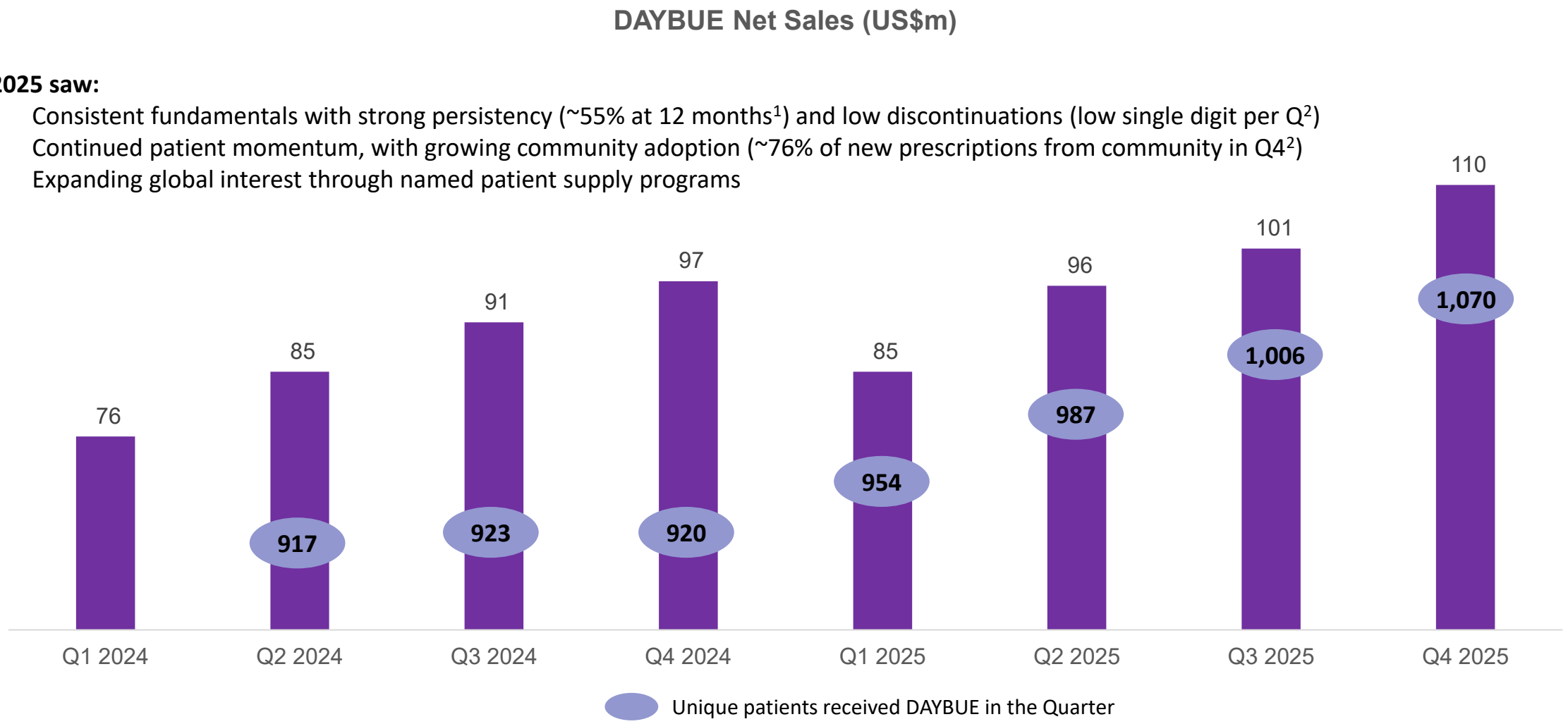
Consistent growth trajectory driven by volume

2025 saw:

- Consistent fundamentals with strong persistency (~55% at 12 months[1]) and low discontinuations (low single digit per Q[2])
- Continued patient momentum, with growing community adoption (~76% of new prescriptions from community in Q4[2])
- Expanding global interest through named patient supply programs



[1] Acadia J.P. Morgan Healthcare Conference presentation 13 Jan 2026
[2] Acadia Q4 and Full Year 2025 Earnings Call

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DAYBUE STIX: a new formulation based on Rett community feedback

Powder for oral solution approved by US FDA in December 2025

Differentiated features

Mixes easily into beverages

Customizable volume

No refrigeration required

Highly portable

Reduced sugar content

Dye & preservative-free

Early launch progress

Strong early interest from HCP's and Care Givers

Initial supply in channel, first patients shipped

Broader commercial launch targeted for early Q2 2026

Incremental demand potential

Provides an additional lever to engage both naïve and discontinued patients

Will be available across both COE and Community settings

Source: Acadia Q4 and Full Year 2025 Earnings Call presentation

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Significant upside in the US

1

Expand number of diagnosed patients

- Currently **6,000** up from 4,500 in 2023
- Theoretical prevalence 6,000 – 9,000

2

Expand % of patients starting therapy

- ~**2,000** patients treated since launch[1]
- DAYBUE STIX enables engagement with **~400** additional naïve and discontinued patients

3

Maintain or improve persistency

- Persistency on therapy after 12 months increased to ~**55%**[1]

Illustrative potential active patient numbers conservatively assuming 50% long-term persistency

% starting therapy	Number of diagnosed patients			
	6,000	7,000	8,000	9,000
40	1,200	1,400	1,600	1,800
50	1,500	1,750	2,000	2,250
60	1,800	2,100	2,400	2,700
70	2,100	2,450	2,800	3,150

Illustrative potential active patient numbers table comprises Neuren calculations

[1] Acadia J.P. Morgan Healthcare Conference presentation 13 Jan 2026

[2] Acadia Q4 and Full Year 2025 Earnings Call

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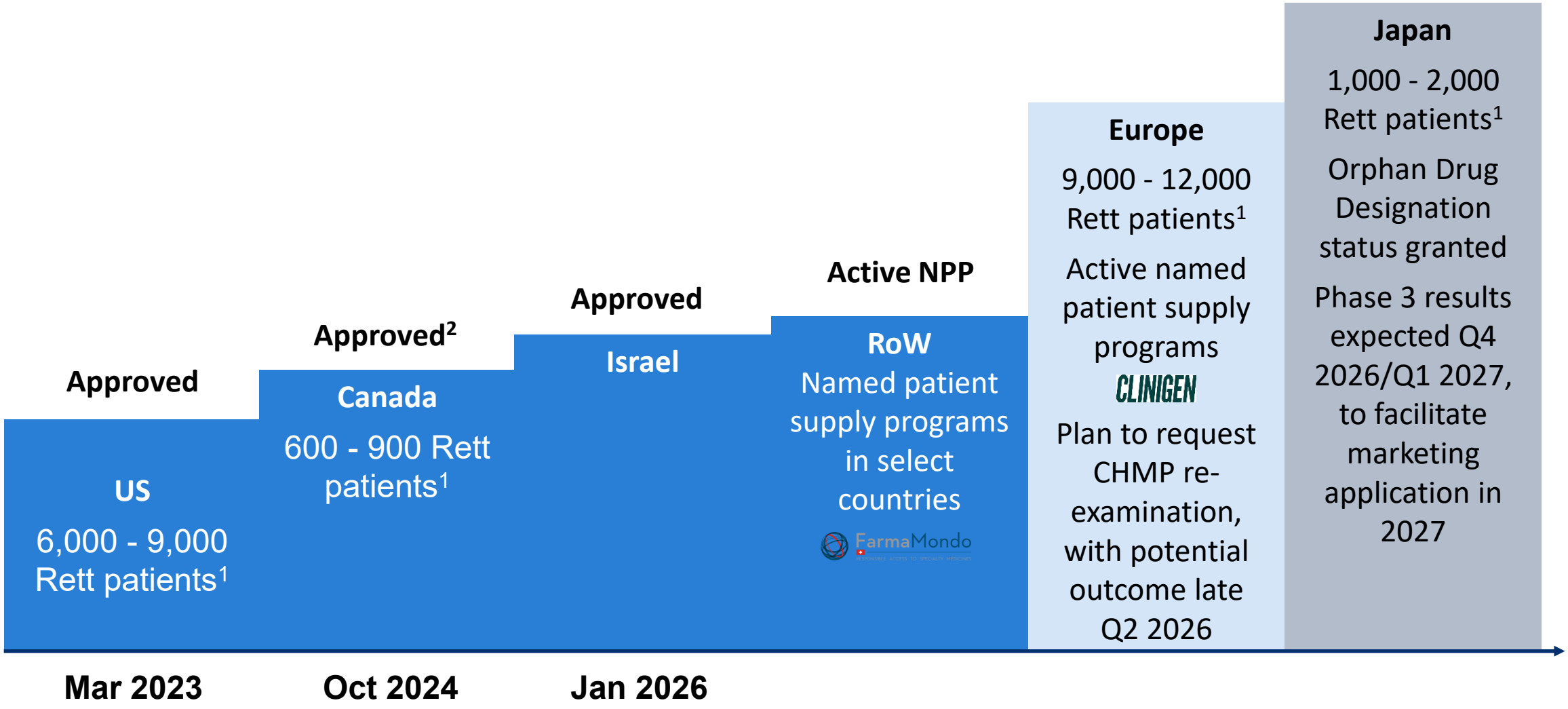
Long term growth opportunity for trofinetide through global expansion



[1] Acadia estimates
[2] Reimbursement currently not recommended by CDA-AMC

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NNZ-2591

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Neuren is leading the development of a first treatment for Phelan-McDermid syndrome (PMS)

The Voice of the Patient…..[1]

"PMS has an overwhelming unmet medical need.
There are no FDA approved treatments for PMS despite its severely debilitating manifestations. Parents and caregivers are open to trying almost anything to try to relieve their child's suffering; most have tried an incredibly high number of treatments and approaches for symptom management, with very little success."

"PMS has severe quality of life impacts on those living with the disease, as well as on parents and siblings.
Most activities of daily life, including ***communicating needs or wants, self-care (bathing, dressing, toileting) and socializing with peers/siblings*** *are affected. Most individuals living with PMS rely on their parents and caregivers for all their daily needs, and many require 24-hour care."*

Developmental delay/intellectual impairment (lack of safety awareness) and communication issues *are the most troublesome concerns.*
Improved cognitive functioning and improved communication *are the most desired outcomes.*

cureSHANK

pmsf

Koala

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NNZ-2591 development program

- ✓ Orphan Drug designation (US and EU)
- ✓ Rare Pediatric Disease designation (US)
- ✓ Meaningful improvements rated by clinicians and caregivers in open-label Phase 2 trial
- ✓ Alignment with FDA on single Phase 3 trial design and endpoints to support a New Drug Application
- ✓ Fast Track designation (US)
- ✓ Koala Phase 3 trial recruiting

[1] Excerpts from Voice of the Patient Report of Externally-Led Patient-Focused Drug Development Meeting Nov 2022

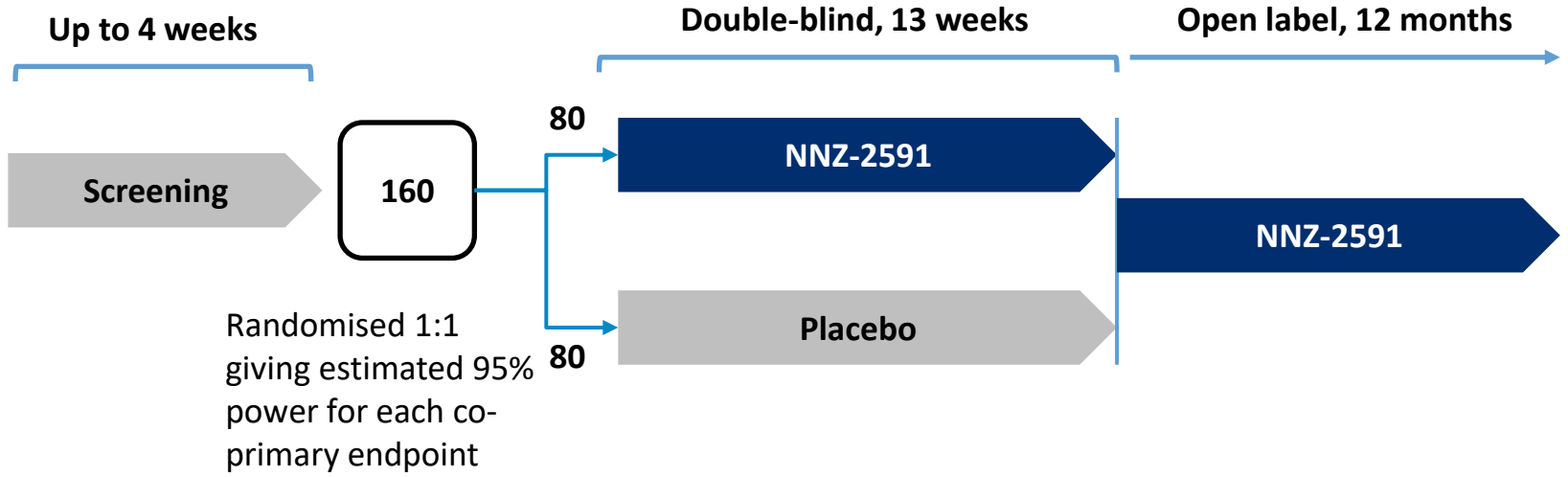
Dosing commenced in Koala - the first ever Phase 3 trial in PMS

Alignment with FDA on single Phase 3 trial design and endpoints to support a NDA

As of 27 Feb 2026

First 2 patients dosed
+
>70 patient referrals

Koala



Key Phase 3 endpoints robustly positive in Phase 2 trial

RESEARCH ARTICLE OPEN ACCESS

NNZ-2591 in Children and Adolescents With Phelan-McDermid Syndrome

Single-Group, Open-Label, Phase 2 Trial Results

Ann M. Neumeyer,[1] Siddharth Srivastava,[2] J. Lloyd Holder, Jr.,[3] Mark A. Milad,[4] Liza Squires,[5] Nancy Elizabeth Jones,[5] Larry Glass,[5] and Elizabeth Berry-Kravis[6]

Neurol Genet 2026;12:e200338. doi:10.1212/NXG.0000000000200338

Abstract

Background and Objectives

Phelan-McDermid syndrome (PMS) is a rare genetic neurodevelopmental disorder with no currently approved treatments. NNZ-2591, a synthetic analog of the insulin-like growth factor 1 metabolite cyclic glycine-proline, was evaluated in children and adolescents with PMS in a phase 2, multisite, open-label clinical trial.

Methods

Participants aged 3–12 years at screening received twice-daily oral NNZ-2591 for 13 weeks; doses were uptitrated from 4 mg/kg to 12 mg/kg over 6 weeks (NCT05025241). Safety and pharmacokinetic profiles were primary end points; 14 efficacy assessments were secondary end points, which included global and symptom-specific PMS assessments, quality of life, communication, behavior, adaptive behavior/self-care, gastrointestinal health, and sleep assessments. Wilcoxon signed-rank tests evaluated change from or observed change relative to baseline vs the null median, with $p < 0.05$ indicating significance.

Results

Eighteen participants received NNZ-2591 (mean [SD] age 8.6 years, mean [SD] weight: 30.4 [10.8] kg). NNZ-2591 was well tolerated; most treatment-emergent adverse events were mild to moderate. Significant improvements from baseline were observed in 10 of 14 efficacy assessments at week 13, including global and symptom-specific PMS assessments, quality of life, behavior, gastrointestinal symptoms, and sleep. At week 13, the PMS-specific Clinical Global Impression (CGI) of Improvement mean (SD) score was 2.4 (0.9) and the median (range) score was 2.0 (1.0, 4.0) ($p < 0.0001$), with 16 of 18 participants showing improvement; the PMS-specific Caregiver Impression of Change mean (SD) score was 2.7 (1.0) and the median (range) score was 3.0 (1.0, 5.0) ($p = 0.0003$), with 15 of 18 participants showing improvement. PMS-specific assessment subdomains of communication, cognition/learning, and socialization showed consistent improvements. A 24-hour steady-state area under the curve ($AUC_{24,ss}$) was estimated for each participant using a one-compartment, linear, population pharmacokinetic model where clearance and volume of distribution parameters were scaled by body weight. Participants with an NNZ-2591 $AUC_{24,ss} > 300$ μg · h/mL experienced improvements in the PMS-specific CGI of Improvement scores.

Discussion

For children and adolescents with PMS, NNZ-2591 appeared generally safe, with clinicians and caregivers reporting meaningful improvements in important symptoms of PMS. The benefit-risk and pharmacokinetic profiles support continued evaluation of NNZ-2591 for PMS.

Trial Registration Information

ClinicalTrials.gov; NCT05025241. Submitted August 24, 2021. First participant enrolled on August 8, 2022.

[1]Lurie Center for Autism, Massachusetts General Hospital, Lexington, MA; [2]Department of Neurology, Rosamund Stone Zander Translational Neuroscience Center, Boston Children's Hospital, Boston, MA; [3]Department of Pediatrics, Baylor College of Medicine, Houston, TX; [4]Milad Pharmaceutical Consulting, Plymouth, MI; [5]Neuren Pharmaceuticals, Camberwell, Australia; [6]Department of Pediatrics, Rush University Medical Center, Chicago, IL.

Co-primary Endpoints in Koala	Phase 2 Results[1]
Phelan-McDermid Syndrome Assessment of Change (PMSA-C), *previously referred to as CGI-I in Phase 2*	**16/18 subjects showed improvement** **Mean score: 2.4** ($P < 0.0001$[2])
Receptive Communication sub-domain of the Vineland Adaptive Behavior Scales, 3rd Edition (VABS-3 Receptive-Raw Score)	**16/18 subjects showed improvement** **Mean improvement: 7.5 (from baseline of 29.0)** ($P = 0.0001$[2])

Key Secondary Endpoint in Koala	Phase 2 Results[1]
Caregiver Impression of Change (CIC) score	**15/18 subjects showed improvement** **Mean score: 2.7** ($P = 0.0003$[2])

NNZ-2591 was safe and well tolerated in Phase 2, with no clinically meaningful changes in safety parameters during treatment

[1] 13 weeks treatment of patients age 3-12 years in open label trial at 4 US sites
[2] Wilcoxon signed rank test

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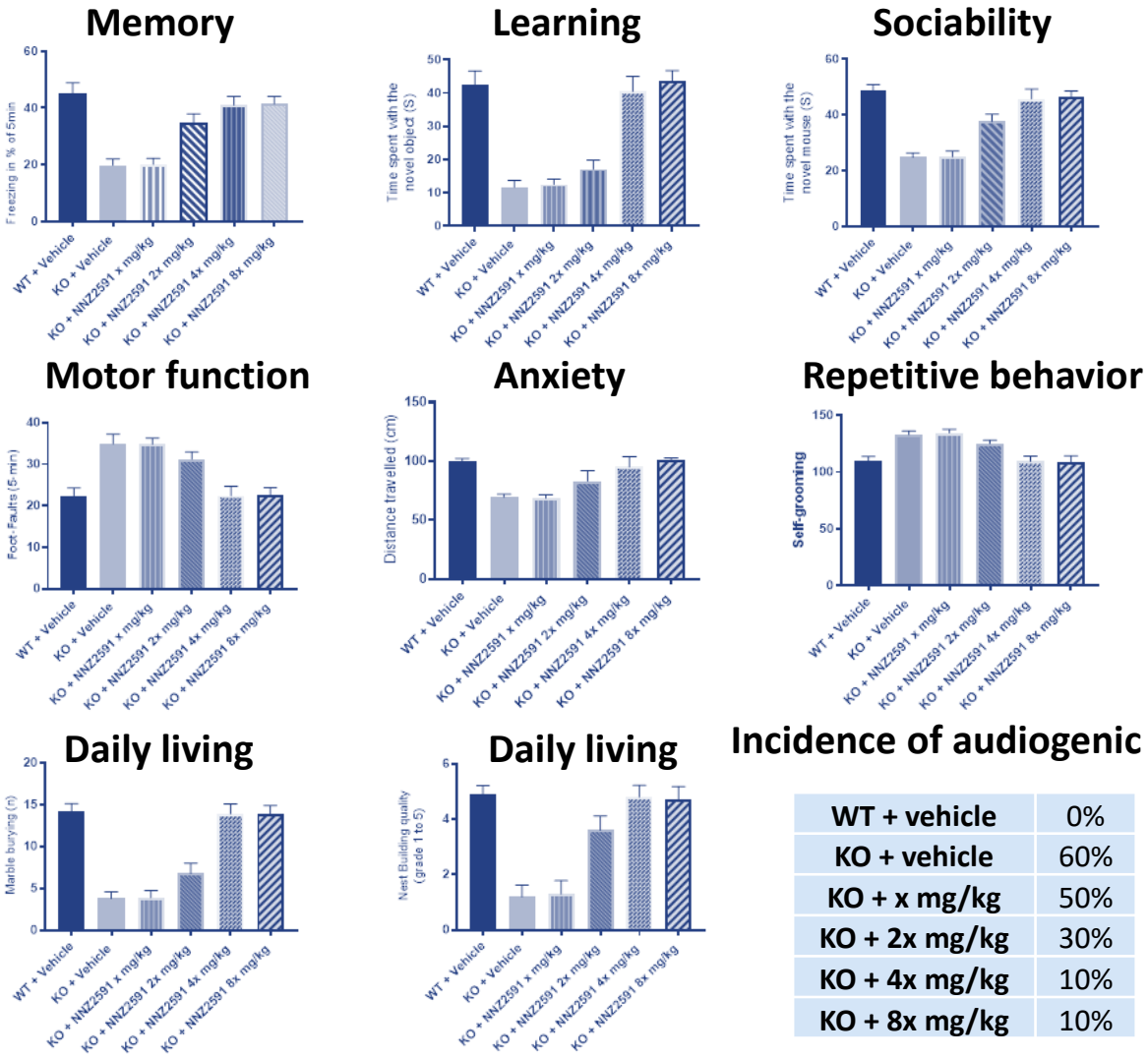
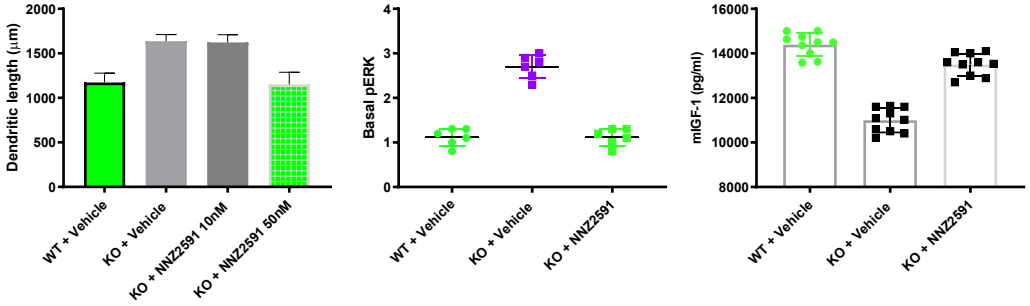
Supported by clear efficacy and dose response in *shank3* model of PMS

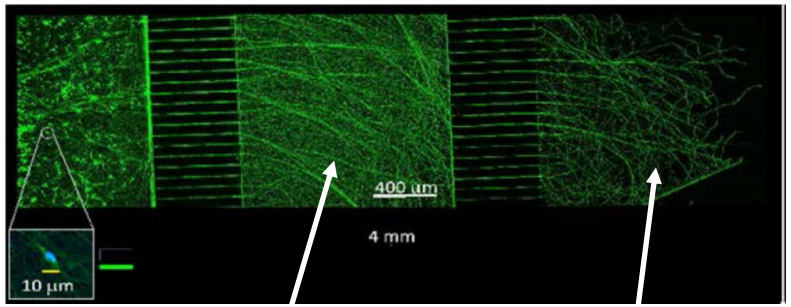


Incidence of audiogenic seizures

WT + vehicle	0%
KO + vehicle	60%
KO + x mg/kg	50%
KO + 2x mg/kg	30%
KO + 4x mg/kg	10%
KO + 8x mg/kg	10%

In biochemical testing, NNZ-2591 was shown to normalize the abnormal length of dendritic spines that form the synapse, the excess activated ERK protein (pERK) and the depressed level of IGF-1 in *shank3* knockout mice





Abnormal dendrites in *shank3* knockout mice cells in culture

Normalization after treatment with NNZ-2591

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Leading the development of a first treatment for Pitt Hopkins syndrome (PTHS)

Patients stories[1]

"She was tested earlier for Angelman and Rett Syndrome, but they were of course negative. I had a strange feeling that something was wrong with her already when she was a newborn…I started to see different doctors with her, but they just told me nothing was wrong, until we met a Neurologist who told us that she had Cerebral Palsy and that she would not able to walk, ever…She doesn't talk but when she was about one year old she was saying a few words that never ever came back..."

"Caleb is currently 10 months old and he does not sit or roll yet and is not really interested in toys. He is currently in an early intervention program and is going through physical therapy, and sees a vision teacher and special education teacher…It has not been an easy journey thus far. I still do not how and where I get all my strength from. I know things will only get harder as he gets older but I am ready to accept the challenge and take each day as it comes."

PITT HOPKINS RESEARCH FOUNDATION

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NNZ-2591 development program

- ✓ Orphan Drug designation (US and EU)
- ✓ Fast Track designation (US)
- ✓ Rare Pediatric Disease designation (US)
- ✓ Consistent efficacy observed in TCF4 model of PTHS
- ✓ Meaningful improvements rated by clinicians and caregivers in open-label Phase 2 trial

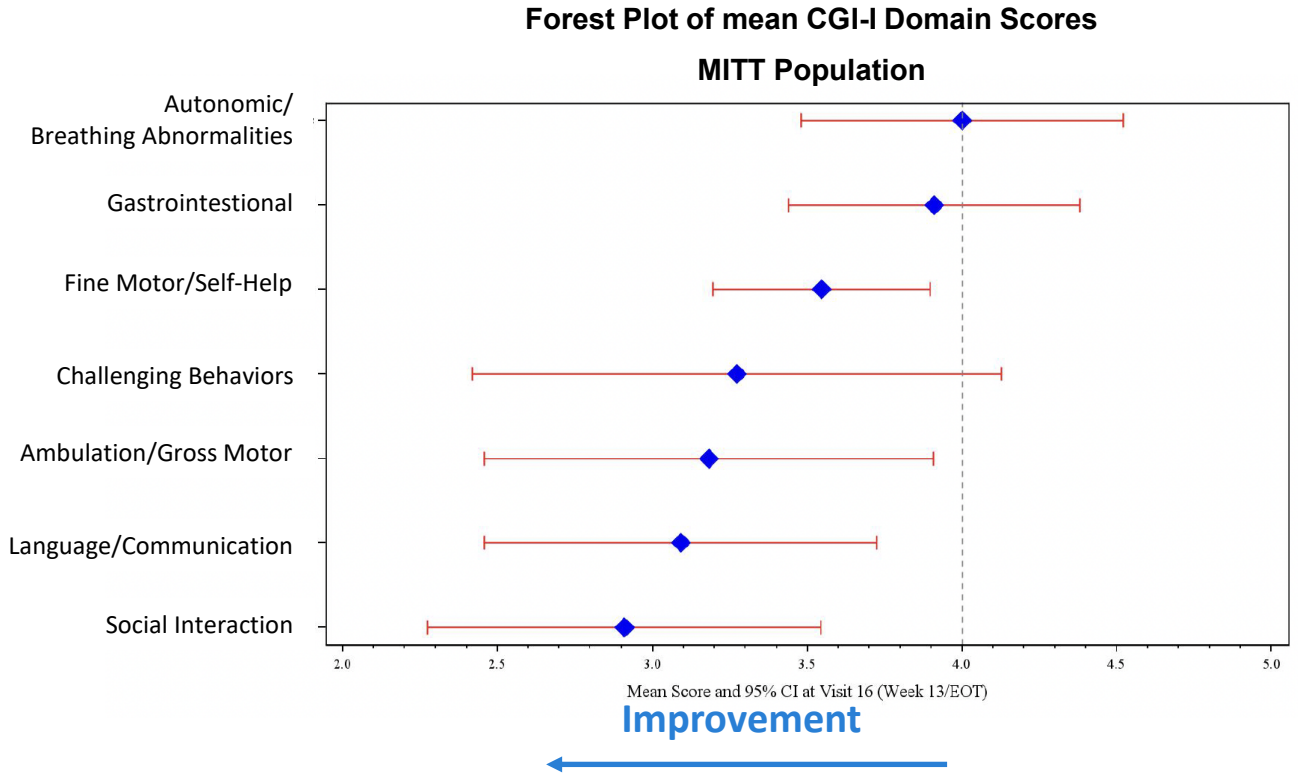
[1] Pitt Hopkins Research Foundation

Meaningful improvements observed in Phase 2 clinical trial

- 13 weeks treatment of patients age 3-12 years in open label trial at 5 US sites
- Mean **CGI-I** of **2.6** with 9 out of 11 children showing improvement ($p = 0.0039$[1])
- NNZ-2591 was safe and well tolerated, with no clinically meaningful changes in safety parameters during treatment

Improvements were seen in clinically important aspects of Pitt Hopkins syndrome, including:

- communication
- social interaction
- cognition; and
- motor abilities



[1] Wilcoxon signed rank test

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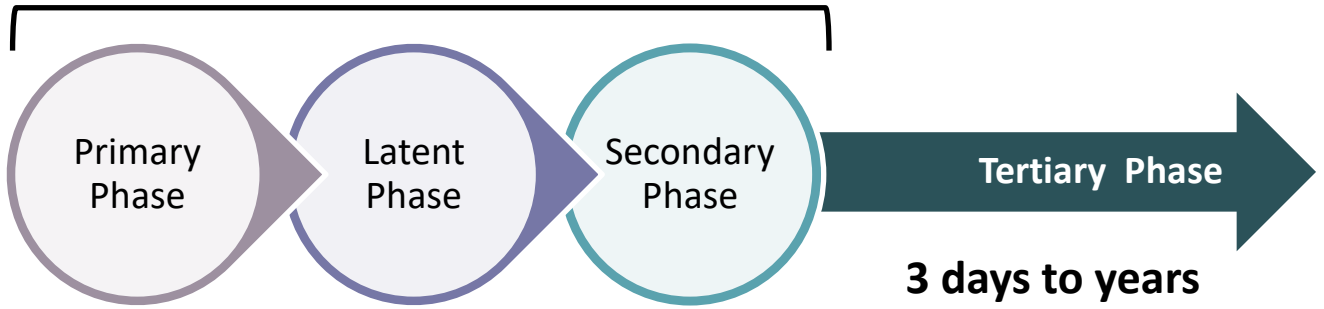
Hypoxic-Ischemic Encephalopathy (HIE)

Causes of HIE
Situations where the global oxygenation of the blood flow to the brain is impacted in utero, during birth, or shortly after, that can cause fetal distress, e.g.:

- Placental issues
- Uterine rupture
- Fetal maternal hemorrhage
- Maternal infection
- Shoulder dystocia
- Cord compression and cord issues
- Sudden unexpected postnatal collapse

Standard of care is therapeutic hypothermia (TH), which reduces mortality and morbidity



40-45% of children who survive HIE have significant neurodevelopmental impairment at 2 yrs of age

Even among children not diagnosed with neurodevelopmental impairment at 2, many manifest **cognitive, behavioural** and other **functional difficulties** as they reach school age and beyond

NNZ-2591

- **IGF-1** promotes cell survival, modulates inflammation, and regulates synaptic transmission
- **IGF-1** levels are reduced in infants with HIE, correlating with HIE severity and outcome
- Supporting data from a range of in-vitro and in-vivo models

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NNZ-2591 in HIE – targeting a new paradigm of treatment

HIE program retains all the advantages of the other NNZ-2591 programs:

- Orphan Drug
- Pediatric
- Urgent unmet need
- Limited competition
- Leverages the non-clinical and manufacturing platform that has been built

Clinical & Regulatory

- Concentration of clinical sites at large hospitals available
- Formal partnership with patient advocacy group

HOPE for HIE
awareness • education • support

Commercial

- No approved drug therapy
- Critical unmet need to **improve long-term outcomes** with a neuroprotective treatment post TH
- **Repeating pool of patients** ~6,000 p.a. in the US[1]
- Addressable in ICUs - a **new in-hospital channel** for Neuren
- Eligible for **Orphan and Rare Pediatric Disease** designations

[1] Neuren estimates based on various published literature

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FDA feedback identified clear path for NNZ-2591 in HIE and PTHS

HIE

FDA

- Generally accepted Neuren's IND-opening proposal and the doses to be evaluated
- Guidance on the inclusion/exclusion criteria and safety monitoring
- Requested additional juvenile animal data

Generate additional **Juvenile animal data**

IND application submission

1 month PK, tolerability & safety study in neonates/infants with HIE

Potential Phase 2/3 study, subject to FDA discussion on endpoints, study population, and safety monitoring

PTHS

FDA

- 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

Assess **alternative** trial designs and endpoint analysis methodologies

Further FDA interaction

Potential Phase 2/3 study

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Substantial market opportunities in PMS, PTHS and HIE

Disorder	Published prevalence estimates	Potential patients		
		US	Europe	Japan
PMS	1/8,000 to 1/15,000 males and females[1] ~1% of autism patients have SHANK3 mutations	19,000 - 36,000[4]	21,000 - 41,000[4]	5,000 - 9,000[4]
PTHS	1/34,000 to 1/41,000 males and females[2]	7,000 - 8,000[4]	8,000 - 9,000[4]	1,000 - 2,000[4]
HIE	2-3 / 1,000 births in high income countries; 10-30 / 1,000 births in low and mid income countries[3]	*Addressable patients*[5]		
		~6,000 p.a.	~7,400 p.a	~1,140 p.a.

[1] Phelan McDermid Syndrome Foundation (PMSF) (www.pmsf.org)
[2] Pitt Hopkins Research Foundation (PHRF) (pitthopkins.org)
[3] Hope for HIE (Hope for HIE - Hypoxic Ischemic Encephalopathy)
[4] Estimates based on United Nations population data 2024, derived by applying the estimated prevalence range to the populations under 60 years
[5] Neuren estimates based on various published literature and company publications

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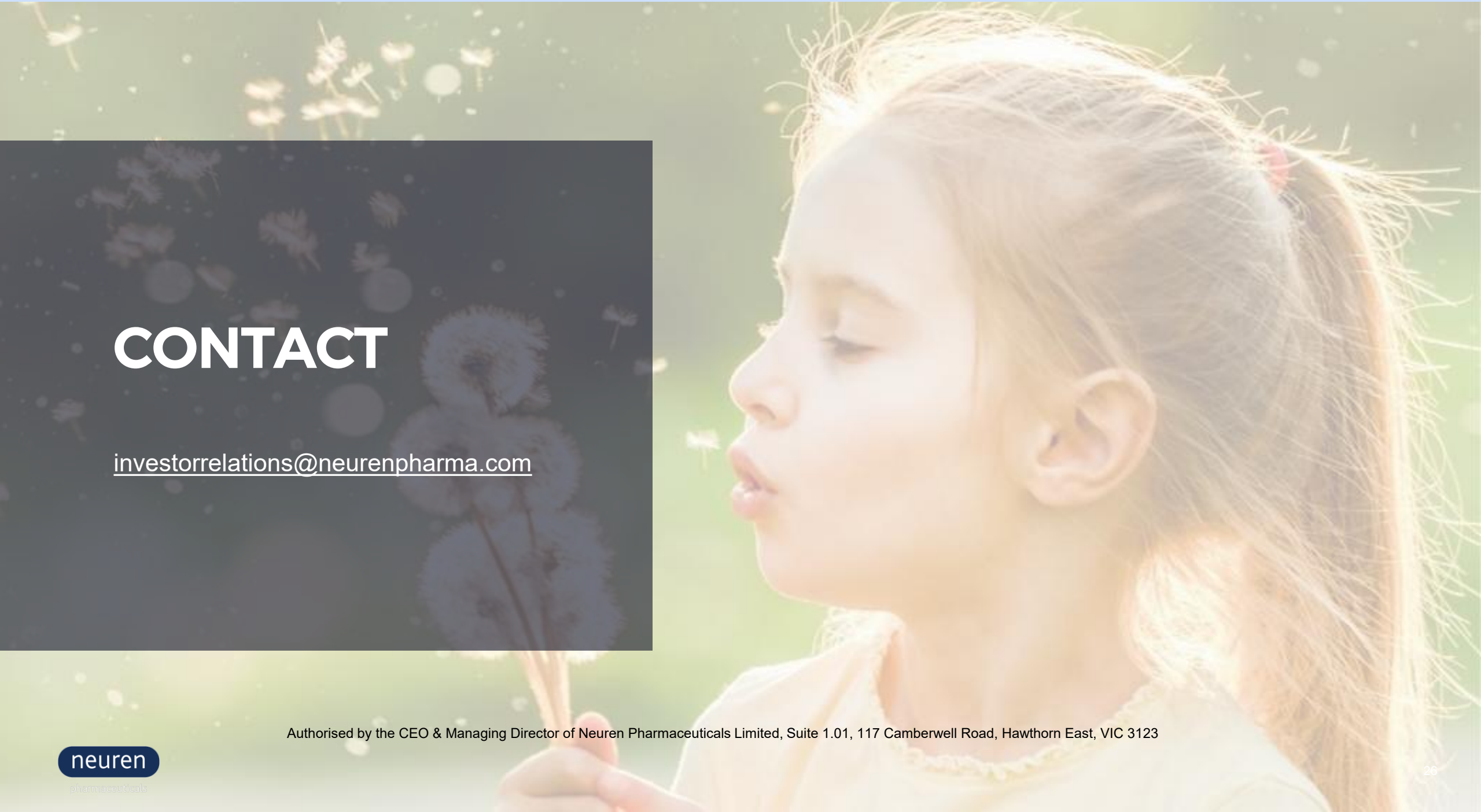

Authorised by the CEO & Managing Director of Neuren Pharmaceuticals Limited, Suite 1.01, 117 Camberwell Road, Hawthorn East, VIC 3123