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Bioshares 2026

6 August 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.

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Neuren, trofinetide and ercanetide were born at the University of Auckland

Professor Sir Peter Gluckman

- Neuren's first Chief Scientific Officer
- Pioneering work on neuroprotective effects of growth factors, including IGF-1 and related peptides

Distinguished Professor Dame Margaret Brimble

- Created improved synthetic analogues of IGF-1 related peptides, with protease resistance to increase half-life and increased lipophilicity to enhance blood/brain barrier transit

Dr Jian Guan

- Leading research on the science of IGF-1 and related peptides and potential roles in neurological conditions

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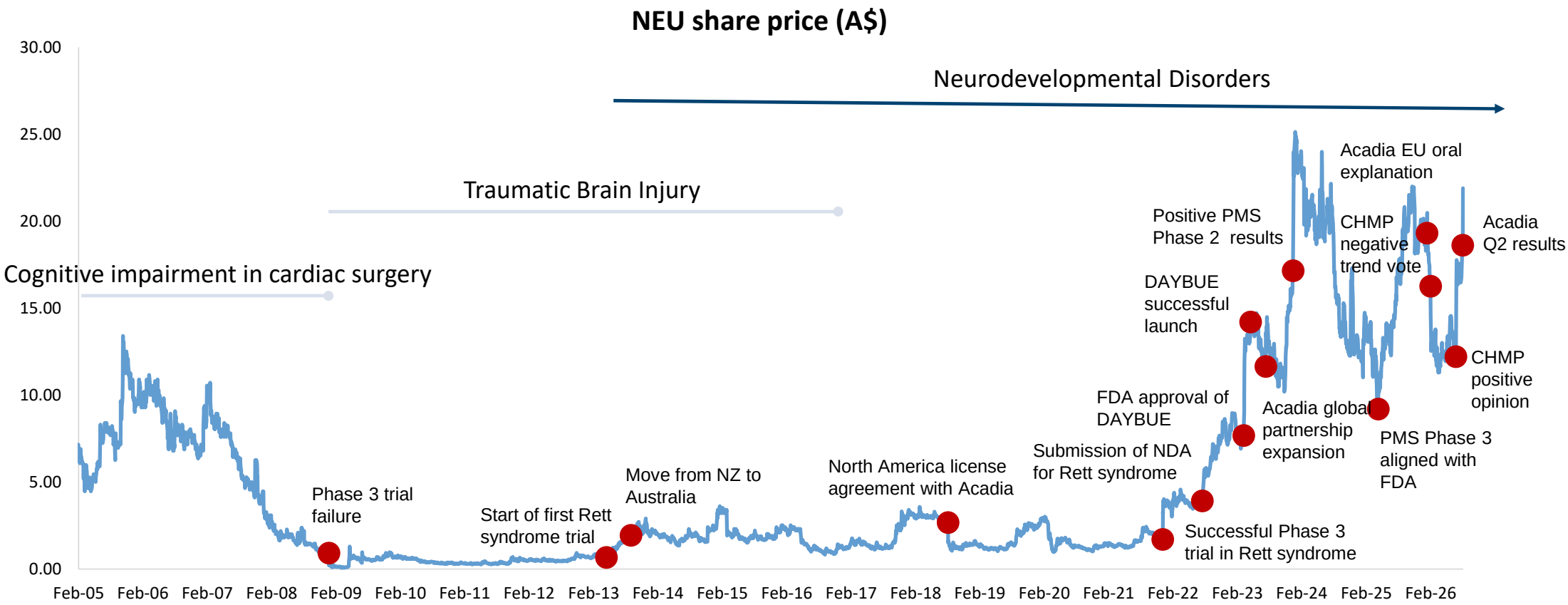
21 years since the IPO – a lesson in determination and flexibility



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Large potential upside for shareholders is enabled by financial strength

A$293
million cash as at 31 Mar 2026

Long-term income growth from DAYBUE® (trofinetide)

A$543m 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 syndrome licensed to Acadia, with same economics to Neuren as trofinetide. Neuren retains rights to all other indications

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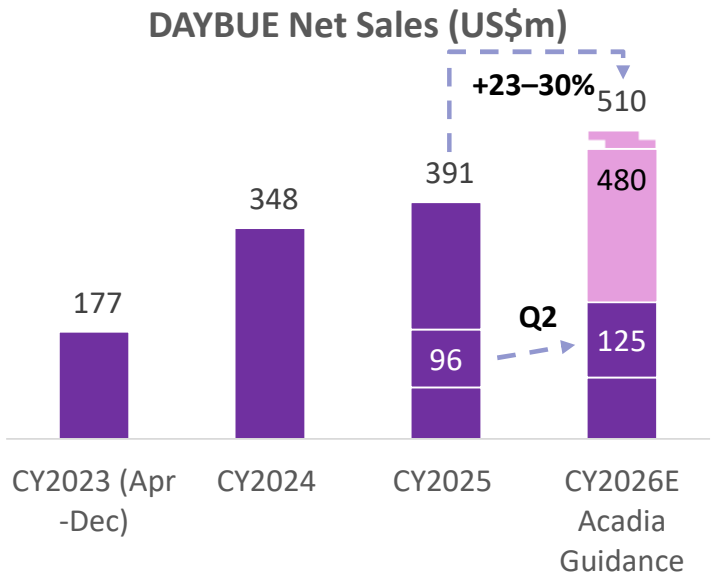
Refer to www.acadia.com for DAYBUE prescribing information and important safety information. DAYBUE is not approved for sale in Australia
NNZ-2591 is an investigational medicine and is currently not approved for sale in any country

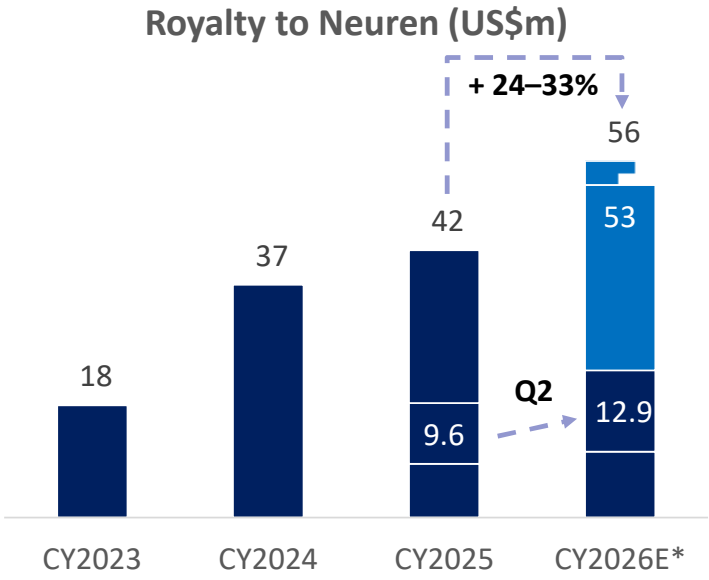
Strong DAYBUE sales momentum

Q2 sales growth almost entirely volume-driven, as strong STIX adoption exceeding expectations

Launch in Germany anticipated in early Q4 2026 pending EC approval decision

Record quarterly sales in Q2 and anticipated EU commercial launch in Q4 led to upgraded 2026 guidance by Acadia





Refer to www.acadia.com for DAYBUE prescribing information and important safety information. DAYBUE is not approved for sale in Australia

* Full year estimates based on Acadia full year 2026 DAYBUE Net Sales Guidance of US$480-510m

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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

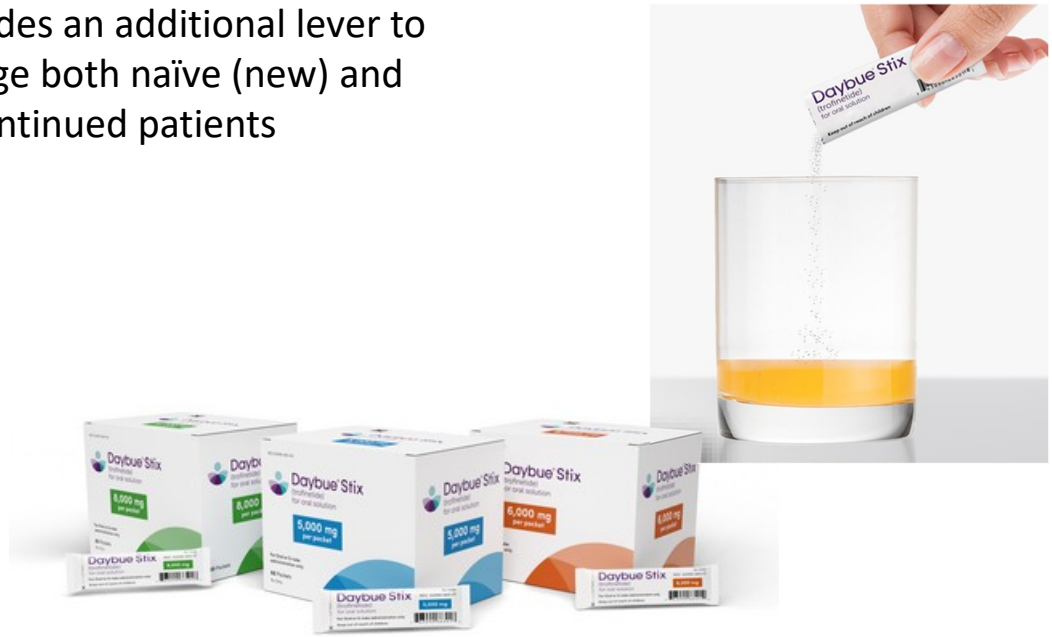
Highly portable

Reduced sugar content

Dye & preservative-free

Incremental demand potential

Provides an additional lever to engage both naïve (new) and discontinued patients



Refer to www.acadia.com for DAYBUE prescribing information and important safety information. DAYBUE is not approved for sale in Australia

Source: Acadia Q4 and Full Year 2025 Earnings Call presentation

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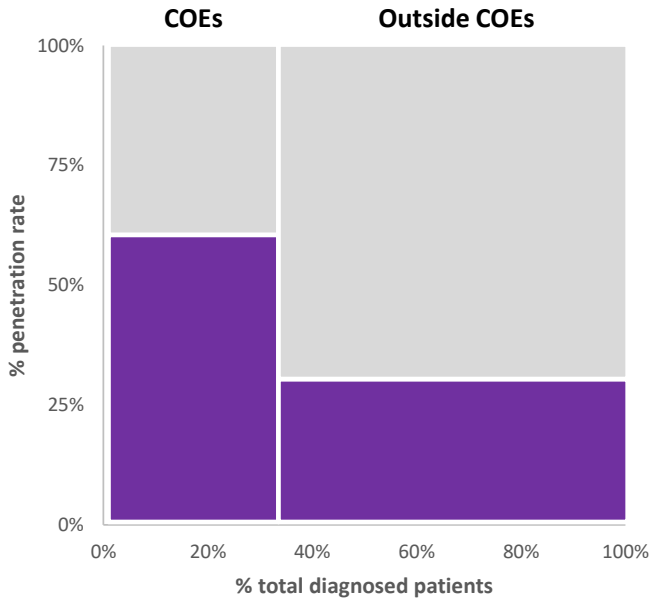
Significant upside in the US

Delphi expert consensus panel recently recommended DAYBUE as part of the standard of care for eligible patients with Rett syndrome[1]

US Penetration Rate[2]



DAYBUE STIX accelerating engagement with new and returning patients

- Limited launched in Q1 2026 focusing on COEs
- Broadly available from early Q2 2026
- 40% of all US DAYBUE patients now receiving STIX
- 45% of STIX demand in Q2 were from new or returning patients

[1] Prange EO, Beisang A, Pehlivan D, et al. Expert Consensus on Real-World Use of Trofinetide for Rett Syndrome Using a Modified Delphi Method. Ann Child Neurol. 2026; 4:38-51.
[2] Acadia Q1 2026 Earnings Call

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Long term growth opportunity through global expansion

			
	Approved Mar 2023	**Positive CHMP opinion Jun 2026**	**Phase 3 results Sep–Nov 2026**
	6,000 - 9,000 Rett patients[1]	9,000 - 12,000 Rett patients[1]	1,000 Rett patients[1]
Future Development Milestones[2]		**US$35m** following 1st commercial sale in Europe	**US$15m** following 1st commercial sale in Japan
Future Sales Milestones[2]	**Net Sales in a CY** / **US$m** ≥US$500m / **50** ≥US$750m / **100** ≥US$1bn / **150**	Up to **US$170m** on achievement of escalating annual net sales thresholds	Up to **US$110m** on achievement of escalating annual net sales thresholds
Tiered Royalty Rates (% of net sales)[3]	**Annual Net Sales** / **Rates** ≤US$250m / **10%** >US$250m, ≤US$500m / **12%** >US$500m, ≤US$750m / **14%** >US$750m / **15%**	**Mid-teens to low-20s %** of net sales	**Mid-teens to low-20s %** of net sales

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

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 (ercanetide) 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[2]
- ✓ 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

NNZ-2591 is an investigational medicine and is currently not approved for sale in any country

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

[2] NEU-2591-PMS-001: An Open-Label Study of the Safety, Tolerability, and Pharmacokinetics of Oral NNZ-2591 in Phelan- McDermid Syndrome - 13 weeks treatment of patients age 3-12 years at 4 US sites

Koala - the first ever Phase 3 trial in PMS

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

Same age range (3-12) and same length of treatment (13 weeks) as Phase 2

Target dosing equivalent to dose tested in Phase 2[1]

~20 trial sites, mostly in US

Program fully funded from existing cash

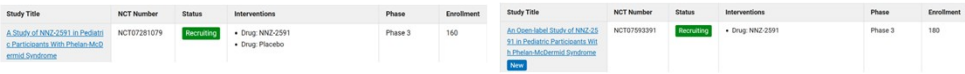
Koala

Up to 4 weeks

Double-blind, 13 weeks

Open label, 12 months

ClinicalTrials.gov



Screening

160

80 **NNZ-2591**

80 **Placebo**

NNZ-2591

Randomised 1:1 giving estimated 95% power for each co-primary endpoint

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[1] 12.5 mg/kg per day in Phase 3 vs 12 mg/kg in Phase 2, and titration period two weeks in Phase 3 vs six weeks in Phase 2

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.

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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)** [3] (P = 0.0001[2]) [3]

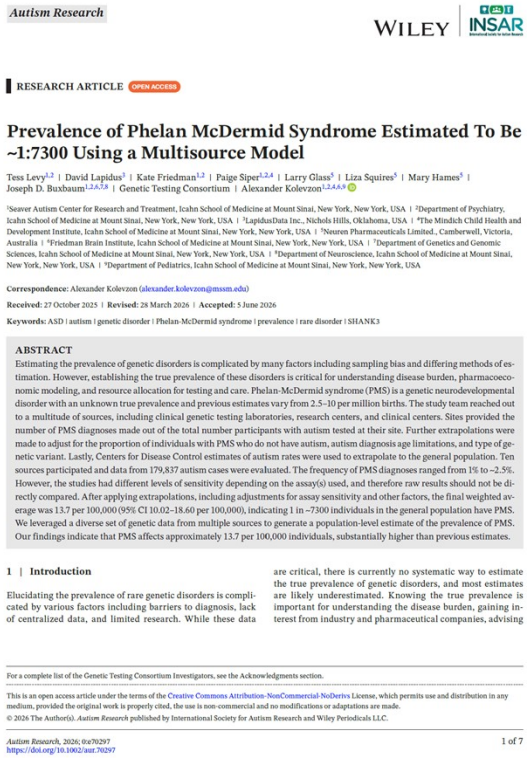
Key Secondary Endpoint in Koala	Phase 2 Results[1]
Caregiver Impression of Change (CIC) score	**15/18 subjects showed improvement** **Mean score: 2.7** **(P = 0.0003[2])**

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

[1] NEU-2591-PMS-001: An Open-Label Study of the Safety, Tolerability, and Pharmacokinetics of Oral NNZ-2591 in Phelan-McDermid Syndrome - 13 weeks treatment of patients age 3-12 years at 4 US sites
[2] Wilcoxon signed rank test - p-values are nominal without type 1 error control
[3] Based on post hoc analysis of overall VABS-3 secondary endpoint

Neuren's leadership in science and community for PMS

Recent published study supported by Neuren estimating the prevalence of PMS to be 1 in 7,300 people



Autism Research

WILEY | INSAR

RESEARCH ARTICLE OPEN ACCESS

Prevalence of Phelan McDermid Syndrome Estimated To Be ~1:7300 Using a Multisource Model

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Keywords: ASD | autism | genetic disorder | Phelan-McDermid syndrome | prevalence | rare disorder | SHANK3

ABSTRACT

Estimating the prevalence of genetic disorders is complicated by many factors including sampling bias and differing methods of estimation. However, establishing the true prevalence of these disorders is critical for understanding disease burden, pharmacoeconomic modeling, and resource allocation for testing and care. Phelan-McDermid syndrome (PMS) is a genetic neurodevelopmental disorder with an unknown true prevalence and previous estimates vary from 2.5–10 per million births. The study team reached out to a multitude of sources, including clinical genetic testing laboratories, research centers, and clinical centers. Sites provided the number of PMS diagnoses made out of the total number participants with autism tested at their site. Further extrapolations were made to adjust for the proportion of individuals with PMS who do not have autism, autism diagnosis age limitations, and type of genetic variant. Lastly, Centers for Disease Control estimates of autism rates were used to extrapolate to the general population. Ten sources participated and data from 179,837 autism cases were evaluated. The frequency of PMS diagnoses ranged from 1% to ~2.5%. However, the studies had different levels of sensitivity depending on the assay(s) used, and therefore raw results should not be directly compared. After applying extrapolations, including adjustments for assay sensitivity and other factors, the final weighted average was 13.7 per 100,000 (95% CI 10.02–18.60 per 100,000), indicating 1 in ~7300 individuals in the general population have PMS. We leveraged a diverse set of genetic data from multiple sources to generate a population-level estimate of the prevalence of PMS. Our findings indicate that PMS affects approximately 13.7 per 100,000 individuals, substantially higher than previous estimates.

1 | Introduction

Elucidating the prevalence of rare genetic disorders is complicated by various factors including barriers to diagnosis, lack of centralized data, and limited research. While these data are critical, there is currently no systematic way to estimate the true prevalence of genetic disorders, and most estimates are likely underestimated. Knowing the true prevalence is important for understanding the disease burden, gaining interest from industry and pharmaceutical companies, advising

For a complete list of the Genetic Testing Consortium Investigators, see the Acknowledgments section.

Honoured to support and participate in the biannual PMSF Family Conference as the Presenting Sponsor

pmsf Family CONFERENCE
Presented by neuren pharmaceuticals

Koala Phase 3 study continues to gain momentum

Koala

- 13 trial sites in US/Canada activated + 4 scheduled Aug
- 100+ patients referred to sites or await site activation
- First completers of 13-weeks study enrolled in OLE
- First patient from Phase 2 study enrolled in OLE

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CONTACT

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Some key factors in Neuren's (eventual) success

- Determination, flexibility, honesty
- Found a sharp focus on a large, real and urgent unmet need
- Close partnership with
- Patient and evolving s

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Some perspectives on the move from NZ to Australia – then and now

- **The landscape in 2013:**
  - Neuren was at a low ebb and embarking on a new direction in neurodevelopmental disorders that needed support
  - Major new shareholder in Australia
  - Interaction with investors was face-to-face
  - US was the focus for development
  - Businesses operated with team all together in an office!
  - New management team would not have moved to NZ (despite its attractions)
- **Would it be necessary today – maybe not?**
  - US is still the focus
  - Times have changed - Neuren now operates virtually – we recruit the best people wherever they are
  - Interaction with investors is often via zoom (although face-to-face is still important)