

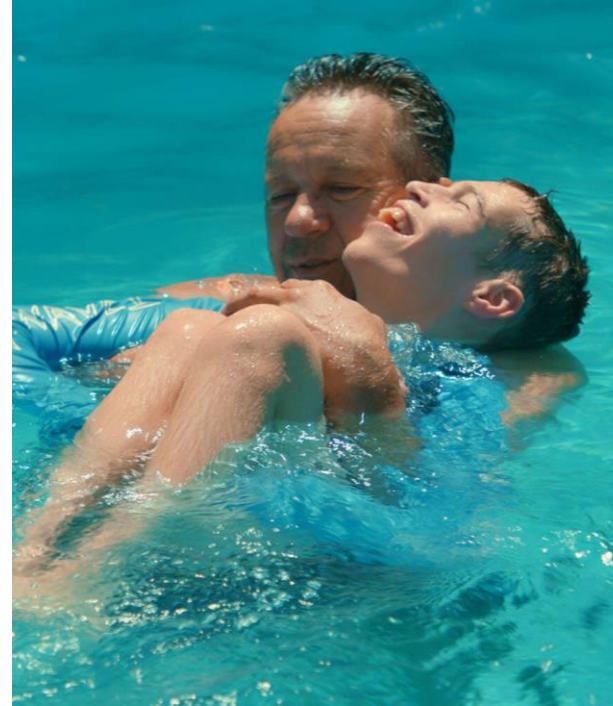
neuren

pharmaceuticals

Bell Potter Emerging Leaders Conference

12 Sep 2023

IMPROVING THE LIVES OF PEOPLE WITH
NEURODEVELOPMENTAL DISABILITIES

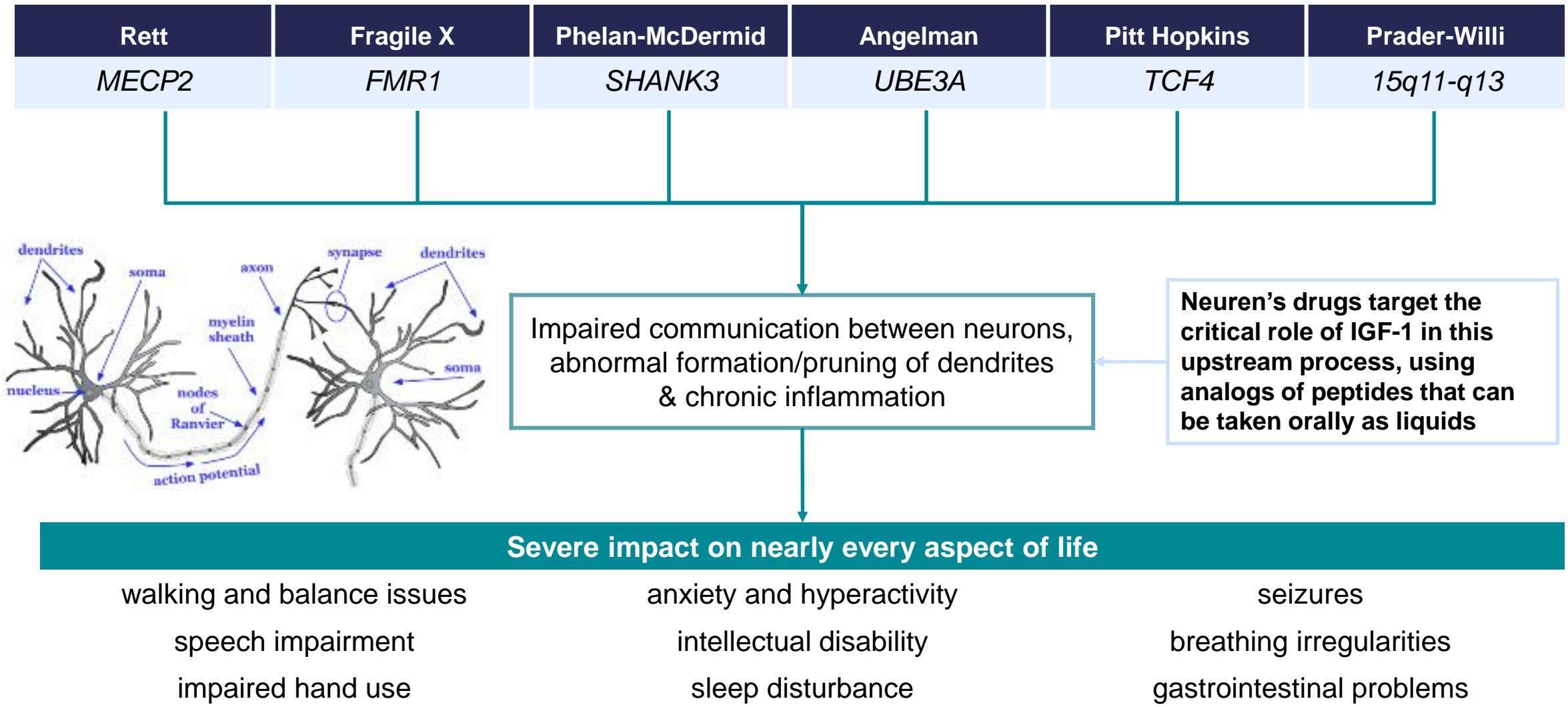


Forward looking statements




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.



Seeking a ground-breaking impact on neurodevelopmental disorders



Commercial and late-stage pipeline

Indication	Compound	Geography	Preclinical	Phase 1	Phase 2	Phase 3	Registration	Commercial rights	
Rett	Trofinetide	US	[Progress bar: Preclinical to Phase 3]					[Progress bar: Phase 3 to Registration]	
		RoW	[Progress bar: Preclinical to Phase 1]						
Fragile X	NNZ-2591	World	[Progress bar: Preclinical to Phase 1]						
	Trofinetide	World	[Progress bar: Preclinical to Phase 1]						
Phelan-McDermid	NNZ-2591	World	[Progress bar: Preclinical to Phase 1]						
		World	[Progress bar: Preclinical to Phase 1]						
Pitt Hopkins	NNZ-2591	World	[Progress bar: Preclinical to Phase 1]						
Angelman	NNZ-2591	World	[Progress bar: Preclinical to Phase 1]						
Prader-Willi	NNZ-2591	World	[Progress bar: Preclinical to Phase 1]						

Three key drivers transforming near term value

1

Realise Neuren's share of **trofinetide value in the US** through Acadia's successful commercialization of



2

Realise Neuren's share of **trofinetide ex-US** value through expanded global partnership with Acadia

3

Confirm efficacy of **NNZ-2591** in Phase 2 trials for four valuable indications, with global rights retained by Neuren

First top-line results in **Dec 2023** for **Phelan-McDermid syndrome**

Economics to Neuren from trofinetide partnership with Acadia

	US	Europe	Japan	Other	Total
Potential Rett patients	6,000 - 9,000 ¹	9,000 - 14,000 ¹	2,000 - 3,000 ¹	~30,000 ²	
Currently identified Rett patients	4,500 ¹	~4,000 ²	~1,000 ²	~2,000 ²	
Average net price per patient p.a.	US\$375,000 ³				
Payments already received	US\$60mUS\$100m.....			US\$160m
Future payments before royalties	US\$438m⁴	US\$215m	US\$129m	US\$83m	US\$865m
Tiered royalties % of net sales⁵	10-15%	Mid-teen to low 20s %			

¹ Acadia estimates

² Neuren estimates based on prevalence studies and patient organisations

³ Acadia estimate, includes assumptions for average weight of expected patient population, compliance rates to therapy and mandatory government discounts; the list price will be US\$21.10 per mL

⁴ Including 1/3 share of Rare Pediatric Disease Priority Review Voucher assuming market value of US\$100m

⁵ Royalty rates payable on the portion of annual net sales that fall within the applicable range

Successful DAYBUE US launch – update from Acadia Q2 earnings call

Strong and broad based demand

- **400+** prescribers have written subscriptions
- Enrollment forms from **all sectors**
- As of 2 Aug 2023, **7 out of 10** written prescriptions from 2Q had converted to paid
- Patient mix is consistent with the **broad label**

Accelerating payer coverage

- **1/3** of covered lives already covered by formal plans, and coverage is accelerating
- **2/3** through medical exception or letter of medical necessity
- **Payer mix consistent** with expectations (60% Medicaid, 25% commercial plans, remainder Medicare & other)
- **Re-authorizations consistent** with expectation and other rare disease products

Positive caregivers testimonials

“She is more alert, will move her head back and forth following a conversation between two people, she laughs appropriately during conversations.”

“She is more alert and focused and was able to sit and play cards. At a therapy session today, she was able to complete several exercises.”

“One of the noticeable changes was more purposeful hand use. She is able to point at and touch her tablet and even use a spoon.”

“Mom reported that hand wringing had decreased and that her daughter reached for food at dinner.”

“I want to share the consistent and up-to date communication we have received from our FAM and Acadia Connect... We are so grateful for the Acadia team, the communication and engagement.”

5x larger opportunity for NNZ-2591

Disorder	Gene mutation	Published prevalence estimates	Potential patients		
			US ¹	Europe ¹	Asia ^{1, 2}
Phelan-McDermid	<i>SHANK3</i>	1/8,000 to 1/15,000 males and females	22,000	28,000	81,000
Angelman	<i>UBE3A</i>	1/12,000 to 1/24,000 males and females	14,000	18,000	52,000
Pitt Hopkins	<i>TCF4</i>	1/34,000 to 1/41,000 males and females	7,000	9,000	25,000
Prader-Willi	<i>15q11-q13</i>	1/10,000 to 1/30,000 males and females	13,000	16,000	47,000
			56,000	71,000	205,000

- Current opportunity for NNZ-2591 is more than 5 times the Rett Syndrome opportunity³
- There are many other neurodevelopmental disorders potentially relevant for NNZ-2591 mechanism of action

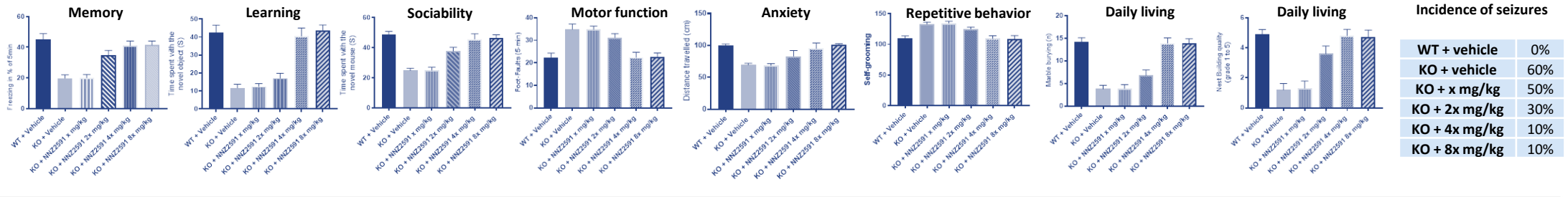
¹ Estimates derived by applying the mid-point of the prevalence estimate range to the populations under 60 years

² Asia comprises Japan, Korea, Taiwan, Israel and urban populations of China and Russia

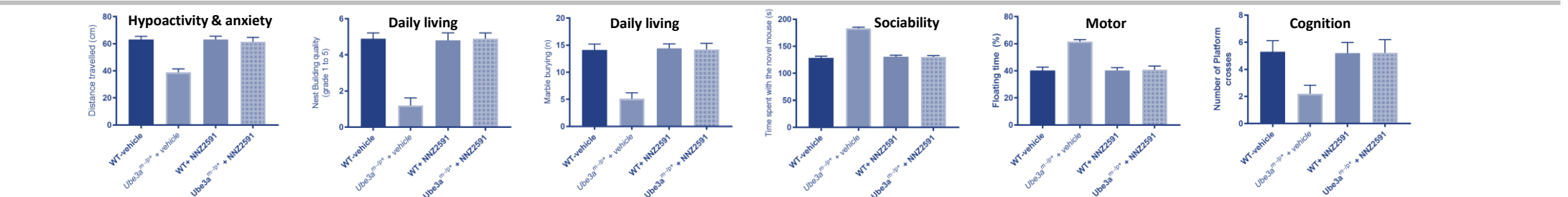
³ Based on number of potential patients globally

Clear and consistent efficacy in animal models

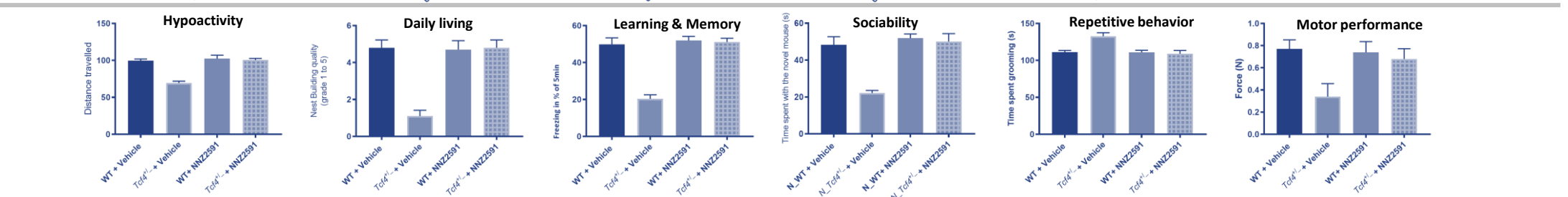
Phelan-McDermid



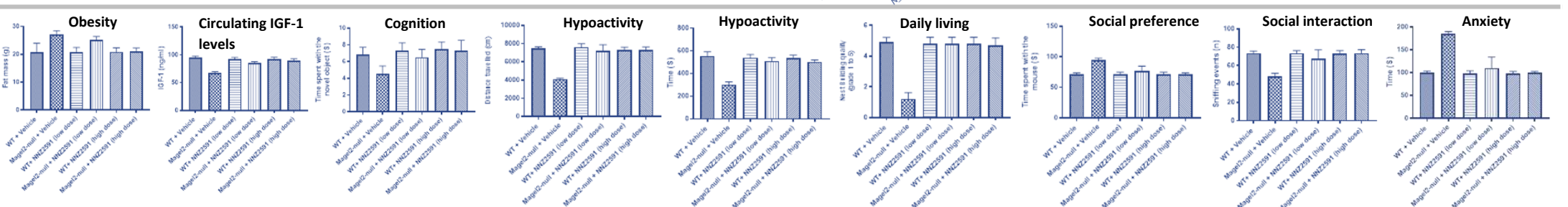
Angelman



Pitt Hopkins



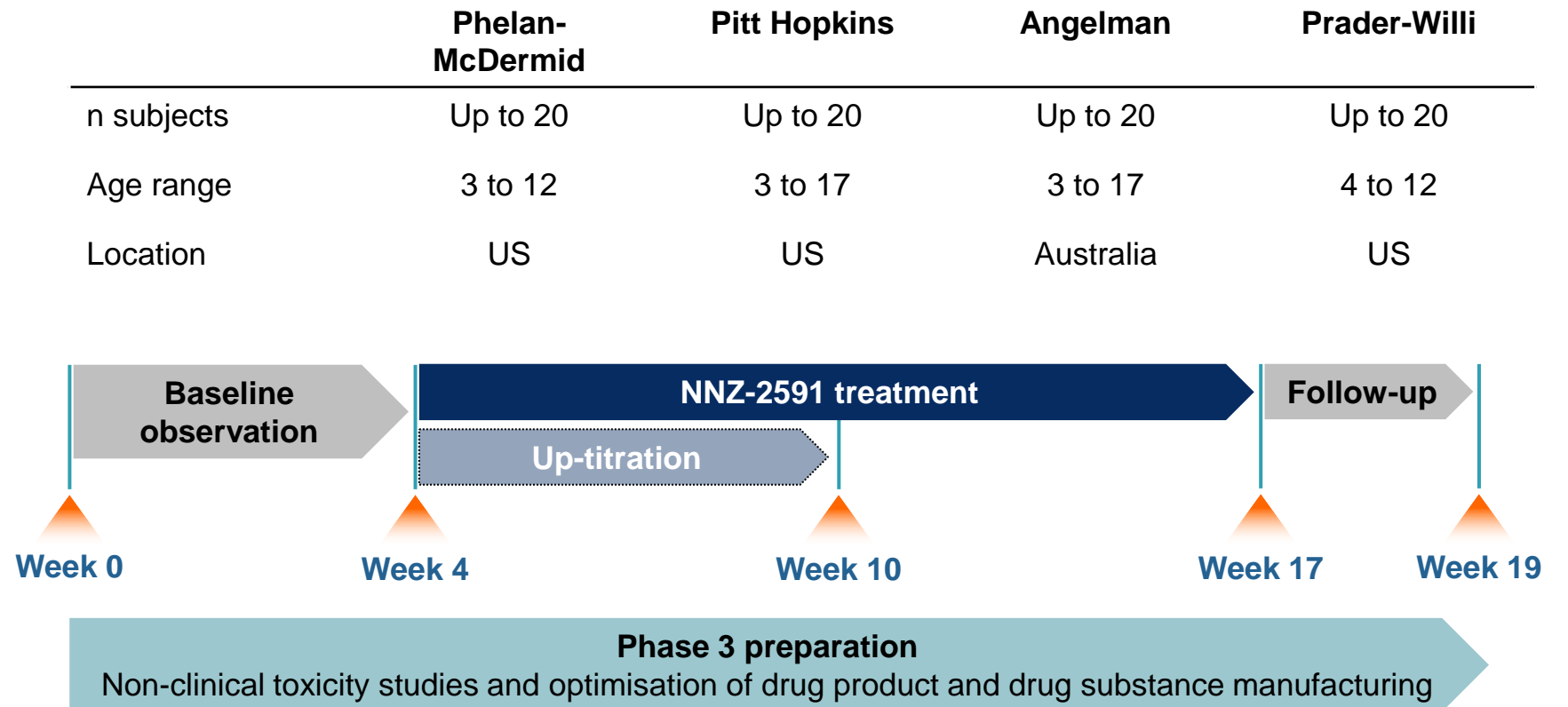
Prader-Willi



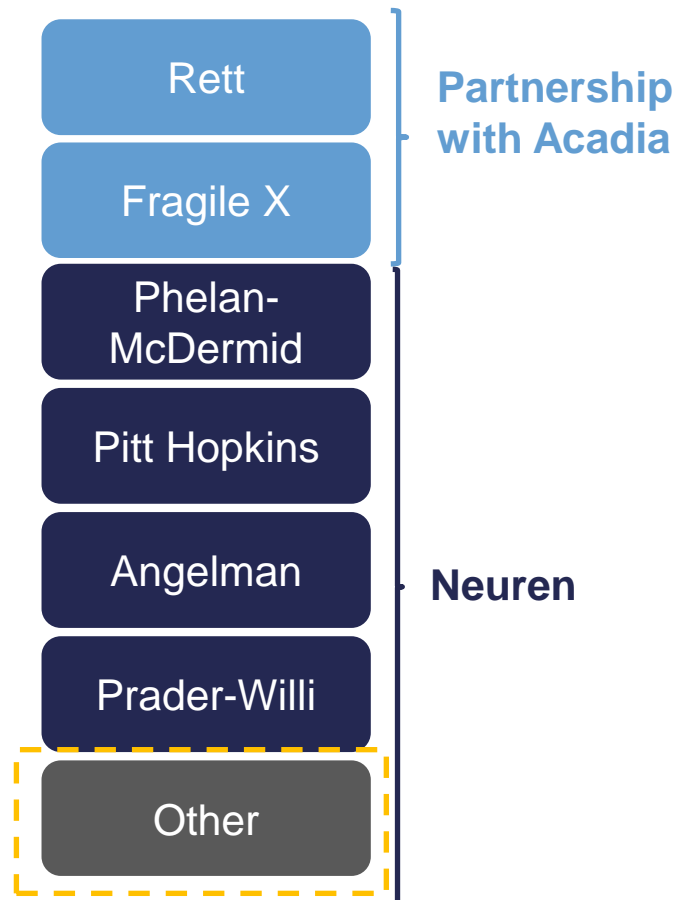
Key features of first Phase 2 trials

Overall aim - expedite data that informs the design of subsequent registration trials and prepare for Phase 3 in parallel

- Prioritising speed to data
- Maximising opportunity to demonstrate effects
- Confirm safety and PK in pediatric patients
- Assess treatment impact across multiple efficacy measures to select primary endpoint for registration trial
- First top-line results in Dec 2023 for Phelan-McDermid syndrome



Value of NNZ-2591 further enhanced by Acadia partnership expansion



- Exclusive worldwide licence to Acadia for Rett and Fragile X syndromes only - which couldn't be developed by Neuren independently
- Neuren retains worldwide rights to NNZ-2591 in all other indications
- Potential future payments to Neuren for NNZ-2591 in Rett and Fragile X syndromes identical to the payments for trofinetide inside and outside North America

Highlights

1

DAYBUE™ (trofinetide) approved by US FDA as the 1st and only treatment for Rett syndrome, launched by partner Acadia in April 2023

2

Total economics to Neuren from global trofinetide partnership with Acadia up to US\$1bn¹ plus 10 to low 20s % royalties

3

Successful DAYBUE US launch, with Q2 2023 net sales of US\$23m and Q3 2023 net sales guidance of US\$45-55m

4

Accelerating Phase 2 development of NNZ-2591 in 4 indications, with potential markets 5x Rett syndrome

5

NNZ-2591 novel mechanism of action has many more potential applications, with Rett and Fragile X licensed to Acadia

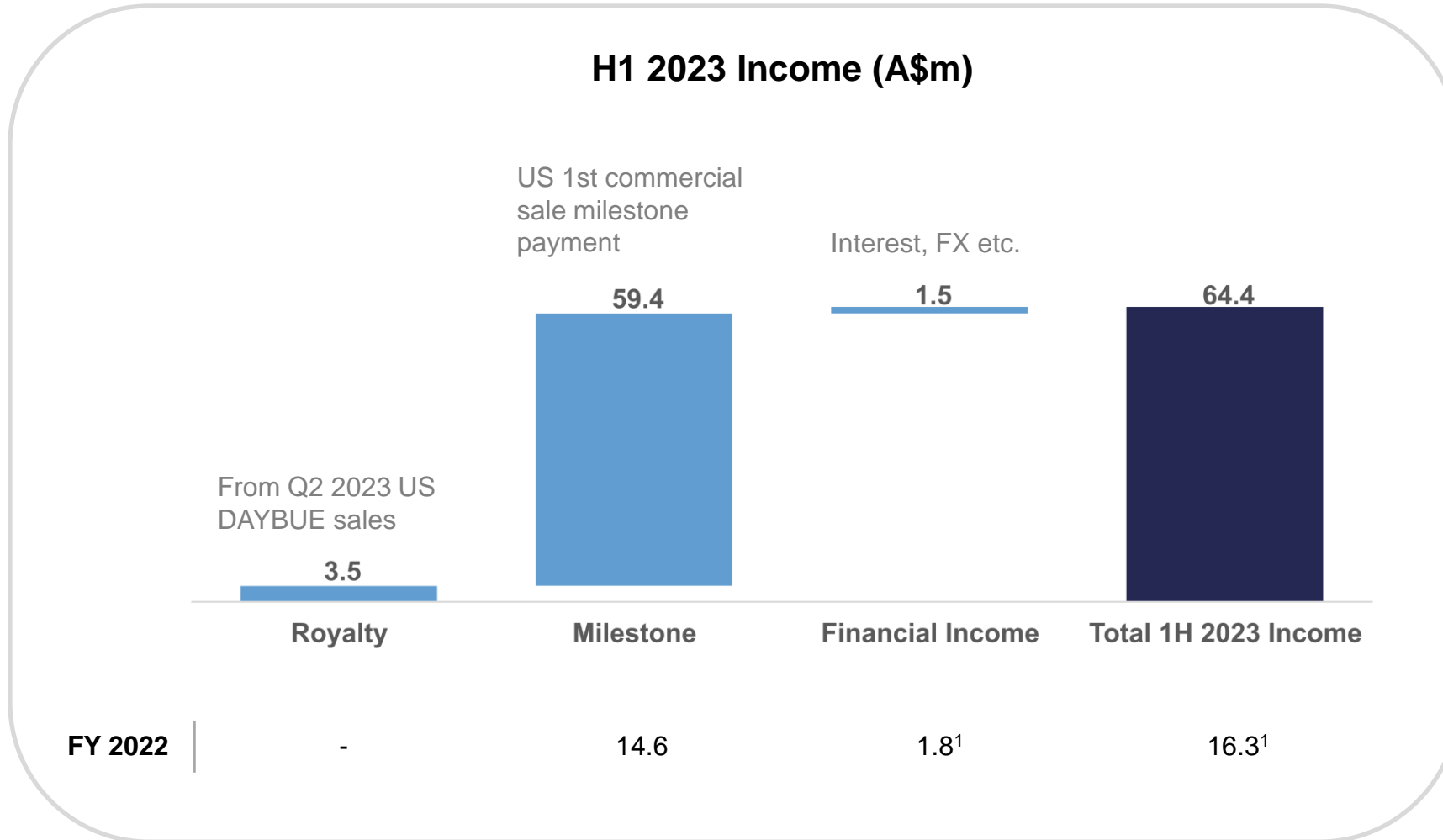
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A\$224m pro forma cash at 30 June 2023² – well positioned to maximize the benefits of all value creating opportunities

¹ Including payments already received and future payments

² Including US\$100 million up-front payment received in July 2023

Transformation underway



H1 2023 Net Profit After Tax

A\$47.8m

¹ Reported income less loss on financial derivative

Transforming catalysts in 2023

Commercial



**Trofinetide
NA**

**Trofinetide
RoW**

NNZ-2591

Development

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- ✓ DAYBUE for Rett syndrome approved by FDA
- ✓ Priority Review Voucher awarded to Acadia
- ✓ First US commercial sale - US\$40m milestone payment
- ✓ First royalty on net sales
 - Ongoing quarterly royalties
 - Priority Review Voucher value one third share – estimated as US\$33m
- ✓ Global trofinetide partnership with Acadia
- ✓ Receive US\$100m upfront payment from Acadia
- ✓ Initiate Prader-Willi syndrome Phase 2 trial
- ✓ Complete enrolment in Phelan-McDermid syndrome Phase 2 trials
 - Complete enrolment in Pitt Hopkins and Angelman syndrome Phase 2 trials
 - Top-line results for Phelan-McDermid syndrome in Dec 2023

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