

Developing new therapies for debilitating neurodevelopmental disorders that emerge in early childhood and are characterised by impaired connections and signalling between brain cells



World's **1st and only** approved therapy for **Rett Syndrome¹**

Clinical development in **5 more** neurodevelopmental disorders, all with **Orphan Drug** designation, with no existing approved therapies².

Neuren owns all intellectual property, with **no royalties payable to 3rd parties**

Compound	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Registration	Commercial rights	
Trofinetide	Rett NA	[Progress bar from Preclinical to Phase 3]					Daybue [™] (trofinetide)	ACADIA
	Rett RoW	[Progress bar from Preclinical to Phase 3]						neuren
	Fragile X	[Progress bar from Preclinical to Phase 2]						NA: ACADIA RoW: neuren
NNZ-2591	Phelan-McDermid	[Progress bar from Preclinical to Phase 2]						neuren
	Angelman	[Progress bar from Preclinical to Phase 2]						
	Pitt Hopkins	[Progress bar from Preclinical to Phase 2]						
	Prader-Willi	[Progress bar from Preclinical to Phase 1]						

Highlights

- ❖ DAYBUE[™] (trofinetide) approved by US FDA as the 1st and only treatment for Rett syndrome
- ❖ Neuren's potential revenue for Rett syndrome in the US alone of US\$73m³ plus up to US\$350m sales milestones plus 10-15% royalties
- ❖ Advancing partnering discussions for trofinetide outside North America
- ❖ Accelerating Phase 2 development of NNZ-2591 in 4 indications, with potential markets 5x Rett syndrome
- ❖ NNZ-2591 novel mechanism of action has many more potential applications
- ❖ Deep expertise in clinical development of orphan neurodevelopmental indications

Corporate

ASX Listing Code	NEU
Market Cap (13 Mar 2023)	A\$1,172m
Cash Balance (31 Dec 2022)	A\$40m
Headquarters	Melbourne, Australia

¹ Currently approved in US only

² Except growth hormone to treat some aspects of Prader-Willi

³ Assuming the product is launched in the US, US\$33m is received as one third share of the value of a Rare Pediatric Disease Priority Review Voucher