

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 Syndrome**¹

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

no royalties payable to 3rd parties

Indication	Compound	Geography	Preclinical	Phase 2	Phase 3	Registration	Commercial rights
Rett	Trofinetide	US	Daybue™ (trofinetide)				
		RoW					
	NNZ-2591	World	ACADIA				
Fragile X	Trofinetide	World					
	NNZ-2591	World					
Phelan-McDermid	NNZ-2591	World	Positive Phase 2 results				
Pitt Hopkins	NNZ-2591	World	Positive Phase 2 results				
Angelman	NNZ-2591	World	Phase 2 top-line results in Q3 2024				
Prader-Willi	NNZ-2591	World					
Undisclosed Indications	NNZ-2591	World					

Highlights

- ❖ DAYBUE™ (trofinetide) approved by US FDA as the 1st and only treatment for Rett syndrome
- ❖ Successful DAYBUE US launch, with CY2023 net sales of US\$177m and CY2024 net sales guidance of US\$370-420m
- ❖ Total economics to Neuren from global trofinetide partnership with Acadia up to US\$1bn plus 10 to low 20s% royalties
- ❖ Accelerating Phase 2 development of NNZ-2591 in multiple indications, with positive results for Phelan-McDermid syndrome and Pitt Hopkins 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 (15 Jul 2024)	A\$2.6bn
Cash Balance (31 Mar 2024)	A\$243m
Headquarters	Melbourne, Australia

¹ Currently approved in US only

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