Improving the Lives of People with Neurodevelopmental Disabilities

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

Daybue™ (trofinetide)

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 therapies[2].

no royalties payable to 3rd parties

Indication	Compound	Geography	Preclinical	Phase 1	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				Phase 2 top-line results released		
Pitt Hopkins	NNZ-2591	World				Phase 2 top-line results in Q2 2024		neuren
Angelman	NNZ-2591	World				Phase 2 top-line results in Q3 2024		
Prader-Willi	NNZ-2591	World						

Highlights

- DAYBUE™ (trofinetide) approved by US FDA as the 1st and only treatment for Rett syndrome
- Successful DAYBUE US launch, with Q2 2023 net sales of US$23m and Q3 2023 net sales of US$67m and Q4 2023 net sales guidance of US$80-88m
- Total economics to Neuren from global trofinetide partnership with Acadia up to US$1bn plus 10 to low 20s% royalties
- Accelerating 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 (20 Dec 2023)	A$2.9bn
Cash Balance (30 Sep 2023)	A$230m
Headquarters	Melbourne, Australia

[1] *Currently approved in US only*
[2] *Except growth hormone to treat some aspects of Prader-Willi*