

neuren

pharmaceuticals

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



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

Clinical development in **multiple** 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	<div></div>					<div>Daybue Trofinetide</div>
		RoW	<div></div>					
	NNZ-2591	World	<div></div>					<div>ACADIA</div>
Fragile X	Trofinetide	World	<div></div>					
	NNZ-2591	World	<div></div>					
Phelan-McDermid	NNZ-2591	World	<div></div> Positive Phase 2 results					<div>neuren</div>
Pitt Hopkins	NNZ-2591	World	<div></div> Positive Phase 2 results					
Angelman	NNZ-2591	World	<div></div> Positive Phase 2 results					
Prader-Willi	NNZ-2591	World	<div></div>					
Undisclosed Indications	NNZ-2591	World	<div></div>					

Highlights

- ❖ DAYBUE™ (trofinetide) approved by US FDA and Health Canada 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\$340-350m
- ❖ 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, Pitt Hopkins syndrome and Angelman 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 (25 Nov 2024)	A\$1.6bn
Cash Balance (30 Sep 2024)	A\$210m
Headquarters	Melbourne, Australia

¹ Currently approved in US and Canada only

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