



# Investor Update

7 August 2018

# 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.

# Introduction

“Neuren is in a far stronger position following the partnership with ACADIA Pharmaceuticals announced today. It makes a very significant and immediate difference in our ability to complete the development of trofinetide for patients in North America and around the world. Importantly, Neuren now has its lead program fully funded, we have access to ACADIA's broad range of capabilities in the US and we have secured significant participation in the potential value of trofinetide in North America, as well as retaining the future value of both trofinetide outside North America and NNZ-2591.”

Richard Treagus, Executive Chairman



# Strong position following ACADIA partnership

- ▣ Capabilities and funding secured to get trofinetide to market
  - Exclusive license to ACADIA for North America, Neuren retains all rights outside North America
  - Neuren to receive up to US\$465 million plus royalties plus share of value of Rare Pediatric Disease Voucher if awarded
  - ACADIA provides the strong capabilities and resources required to execute and fund Rett syndrome Phase 3 trial, commencing in H2 2019 following completion of manufacturing activities
  - All data and regulatory documents may be freely used by Neuren outside North America
- ▣ Neuren now in a position to advance NNZ-2591
  - Demonstrated efficacy in models of Parkinson's disease, stroke, traumatic brain injury, peripheral neuropathy, Fragile X syndrome, memory impairment and multiple sclerosis
  - Neuren now has funds to accelerate development

# Key features of ACADIA partnership

- ACADIA granted exclusive license for North America, Neuren retains all rights outside North America
- Neuren receives:
  - Milestone payments up to US\$465 million
  - Escalating tiered double-digit percentage royalties on net sales of trofinetide in North America
  - One third of the value of any Rare Pediatric Disease Priority Review Voucher awarded by the FDA
- Milestone payments comprise:
  - US\$10 million within 60 days
  - Up to US\$105 million on achievement of development milestones for Rett syndrome and Fragile X syndrome
  - Up to US\$350 million on achievement of thresholds of annual net sales of trofinetide in North America
- Neuren has free access to all data and regulatory information for use outside North America

# Key features of ACADIA partnership

- ACADIA provides the strong capabilities and resources that are necessary to fund and execute Phase 3 development in the US
  - ACADIA team has proven record developing and commercializing CNS disorders in the US that have no approved therapies and high unmet need
  - ACADIA will fund all development costs for trofinetide in North America, including ~US\$55 million for Rett syndrome
  - Neuren will complete certain in-progress preparatory activities, funded by existing resources including ACADIA's exclusivity investment of US\$4 million
  - Neuren has equal membership of Joint Steering Committee to direct the development of trofinetide in all indications
- ACADIA has right of first negotiation with Neuren for rights outside North America
- Neuren has an obligation not to develop a competing product in indications for which ACADIA develops and commercializes trofinetide

# Advancing NNZ-2591

- Demonstrated efficacy in pre-clinical models of Parkinson's disease, stroke, traumatic brain injury, peripheral neuropathy, Fragile X syndrome, memory impairment and multiple sclerosis
- Funds now available to accelerate CMC and pre-clinical development required prior to clinical trials
- Issued composition of matter patents in US, Japan and all EPO countries except Turkey
  - Expire 2024, potential to extend to 2029
- Method of treatment patents and applications for NNZ-2591
  - Issued US patent for autism spectrum disorders and neurodevelopmental disorders and pending applications in Europe and Japan – expire 2034
  - 3 issued US patents for Parkinson's, peripheral neuropathy and cognitive impairment – expire 2024

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# Neuren's near-term priorities

- Actively support ACADIA in development of trofinetide in North America
  - US Phase 3 program in Rett syndrome
  - Fragile X syndrome development plan
- Advance Neuren's strategy to develop and commercialise trofinetide outside North America
  - Engage with regulatory authorities to confirm any additional requirements beyond US data for registration in Rett syndrome
- Accelerate development of NNZ-2591
  - Advance CMC and toxicology studies to enable clinical trials
  - Confirm indication strategy to maximise value worldwide