



# INVESTOR PRESENTATION

4 September 2014

# Forward Looking Statements

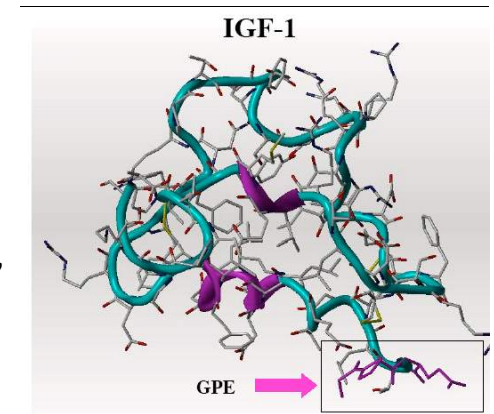
This presentation contains forward looking statements that involve risks and uncertainties. Although we believe that the expectations reflected in the forward looking statements are reasonable at this time, Neuren can give no assurance that these expectations will prove to be correct. Actual results 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.

# Company Snapshot

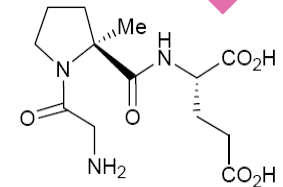
- Stock code ASX: NEU – market cap approximately A\$150 million
- Developing treatments for chronic and acute neurological conditions
  - Large markets with no therapies currently available
  - Potential for abbreviated regulatory pathways and orphan drug designation
- Fully funded through to completion of Phase 2 trials in 4 different indications
  - Trials in progress – Rett Syndrome, Fragile X Syndrome, Concussion, Traumatic Brain Injury
  - Trials will report results from Q4 2014
  - Cash reserves A\$22 million
- Key strategic relationships
  - US Army Medical Research & Materiel Command
  - International Rett Syndrome Foundation
  - Fragile X Research Alliance
  - Fragile X Drug Validation Initiative

# Scientific Foundation

- ❑ **IGF-1** is a naturally occurring growth factor in the brain
- ❑ Glypromate (**GPE**) separates from IGF-1 in the brain
- ❑ IGF-1 and GPE maintain and restore equilibrium in the brain
- ❑ **NNZ-2566** is a synthetic analogue of GPE with a longer half-life, better stability and suitability as an oral medication
- ❑ **NNZ-2566** influences the processes in impaired development and injury of the brain
  - Inflammation
  - Microglial function
  - Synaptic plasticity (inter-neuronal communication)
- ❑ **NNZ-2591** is in the same class of peptides, with higher bioavailability and potential for a solid oral dosage form
- ❑ **NNZ-2566** and **NNZ-2591** each potentially treats a wide range of neurological conditions



**NNZ-2566**



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# Intellectual Property

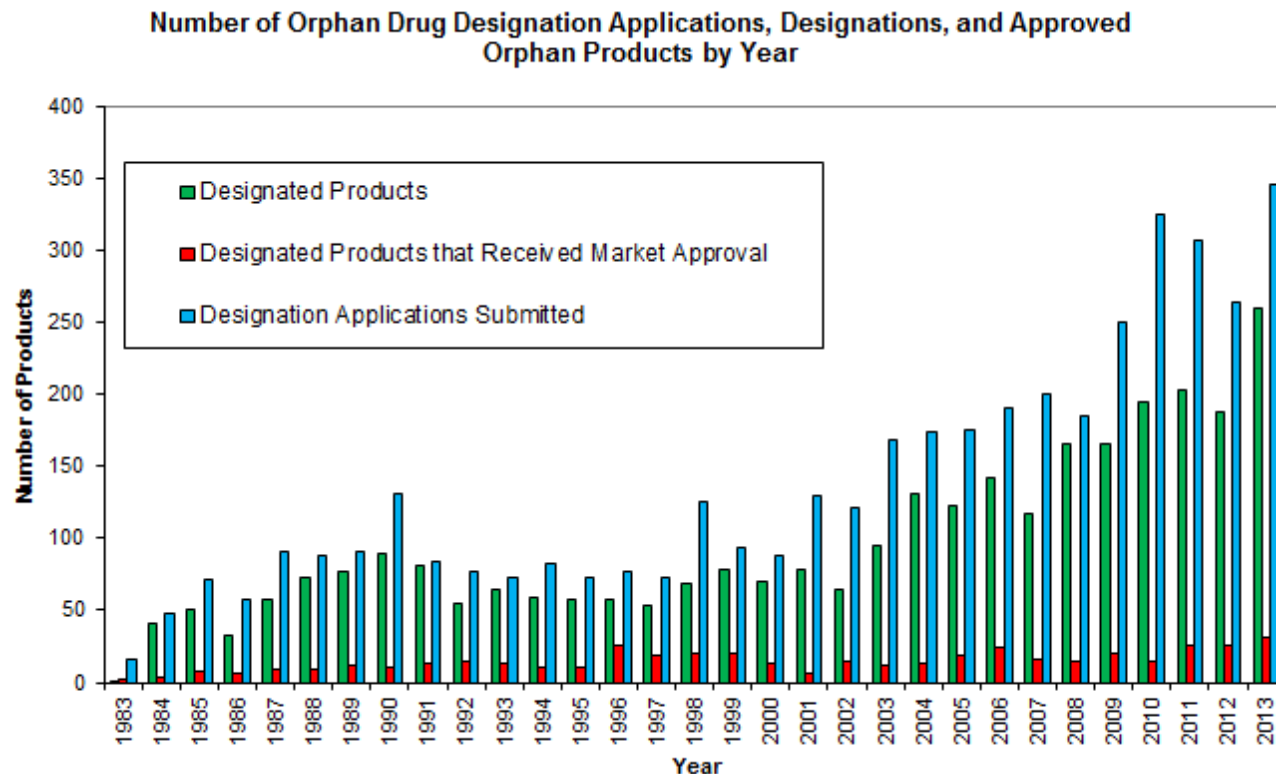
- ▣ Broad patent estate with no royalties payable
- ▣ **NNZ-2566** and other GPE analogues
  - 8 issued US patents covering composition, oral formulation and methods of use
  - Remaining patent life between 8 and 16 years
  - Further patent applications pending
  - Additional market exclusivity may be available via Orphan and Pediatric Drug designations
- ▣ **NNZ-2591** and other bicyclic analogues
  - 4 issued US patents covering composition, formulation and methods of use
  - Remaining patent life between 12 and 16 years
  - Further patent applications pending

# Strategy

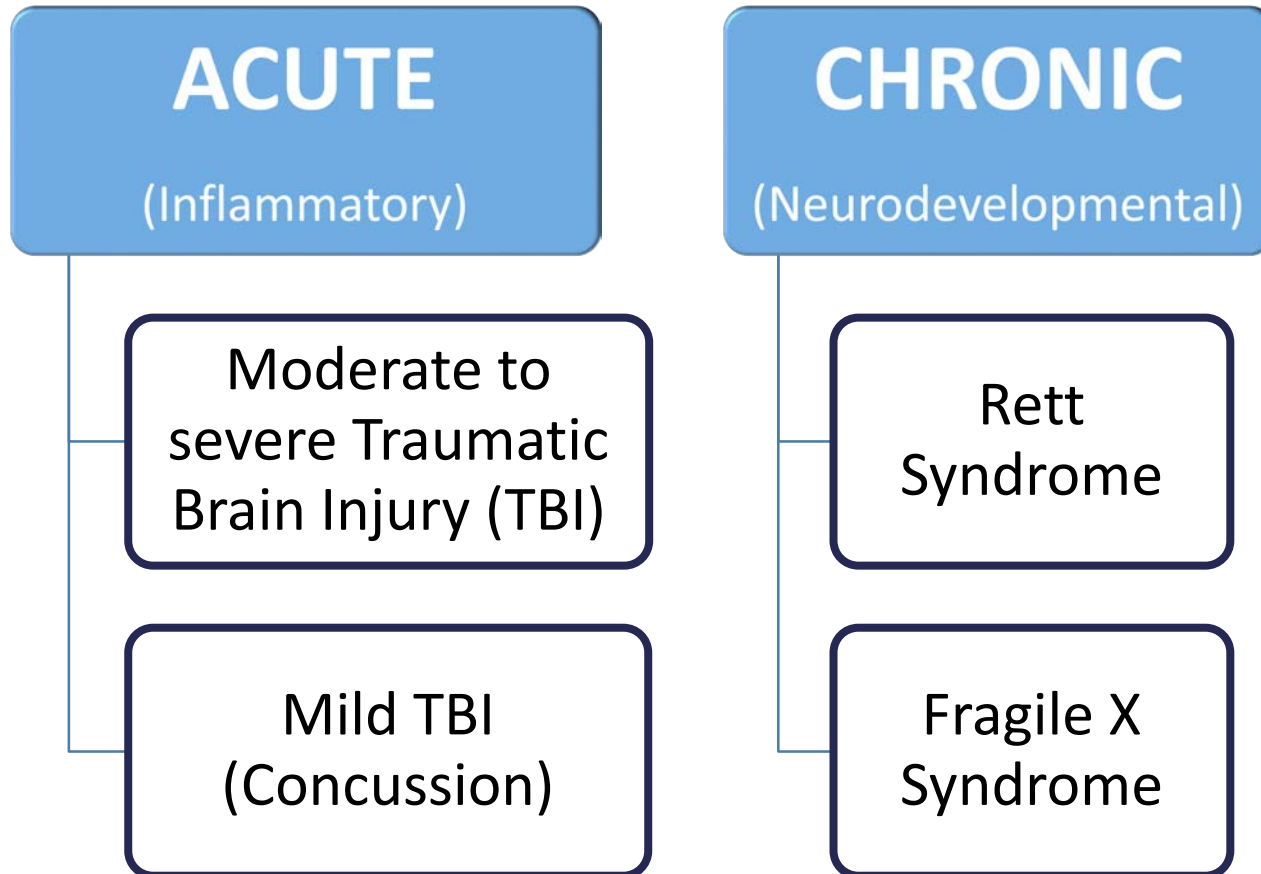
- Demonstrate the therapeutic benefit of **NNZ-2566** in human subjects in both acute and chronic conditions
- Potential to establish a “**gateway**” to autism and other neurodevelopmental disorders
- Criteria for selecting therapeutic targets
  - Significant unmet need and commercial opportunity with no approved drugs
  - Regulatory advantages – eligible for *Fast Track*, *Orphan Drug*, *Breakthrough Therapy*
  - Strong support from advocacy groups and other stakeholders
- Realising value
  - Generate clinical data with NNZ-2566 in Phase 2 clinical trials
  - Advance pre-clinical development of NNZ-2591
  - Optimise manufacturing process for commercial product supply
  - Maintain dialogue with potential partners

# “Orphan drug” designation

- FDA may grant “orphan drug” designation to a drug to treat a rare condition – provides 7 years of marketing exclusivity following approval, as well as other incentives
- Neuren received orphan drug for Fragile X Syndrome and will apply for Rett Syndrome
- Pharma companies increasingly pursuing orphan drugs



# NNZ -2566 Clinical Strategy



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# NNZ-2566 in Rett Syndrome

- Mutation in a gene on the X chromosome - 1 / 10,000 females (20,000 USA)
- Most physically disabling of the autism spectrum disorders - symptoms include:
  - Intellectual disability, loss of speech and motor control
  - Compulsive hand movements
  - Disorders of breathing and cardiovascular function
  - Extreme anxiety
  - Seizures
- Profound disability and financial burden for >50,000 patients and families globally
- Phase 2 trial in females aged 16-40 with Rett Syndrome
  - Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 28 days
  - 3 trial sites in the United States
  - Enrolment completed, top-line results expected in Q4 2014
- “Fast Track” designation granted by the FDA



# NNZ-2566 in Fragile X Syndrome

- Mutation on the X chromosome affecting both males and females - 1 / 4,000 males and 1 / 6,000 females (58,000 USA)
- The most common inherited cause of intellectual disabilities and the most common known cause of autism - symptoms include:

- Intellectual disabilities
- Anxiety and unstable mood
- Seizures (approximately 1 in 4)
- Attention deficit, hyperactivity and autistic behaviour



- Phase 2 trial in males aged 16-40 with Fragile X Syndrome
  - Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 28 days
  - Approximately 60 subjects targeted to complete the trial – 10 trial sites in the United States
  - Top-line results expected in Q2 2015
- “Fast Track” and “Orphan Drug” designation granted by the FDA

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# NNZ-2566 in Traumatic Brain Injury (TBI)

- > 1.5 million head injuries annually in the US alone; >75% are mild (Concussion)
- Leading cause of death and disability, especially in young and elderly
- Serious health and economic effects of Concussion in sporting codes
- Partnership funding of ~US\$25 million by US Army
- Phase 2 trial (“INTREPID”) in moderate to severe TBI
  - Safety and efficacy of treatment with intravenous NNZ-2566 for 72 hours
  - 260 subjects to be enrolled in up to 22 US trauma centres – 170 enrolled to date
  - “Fast Track” designation granted by the FDA
- Phase 2 trial in mild TBI (Concussion)
  - Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 7 days
  - 132 subjects with mild TBI to be enrolled at US military training facility
- Top-line results expected from both trials in H2 2015

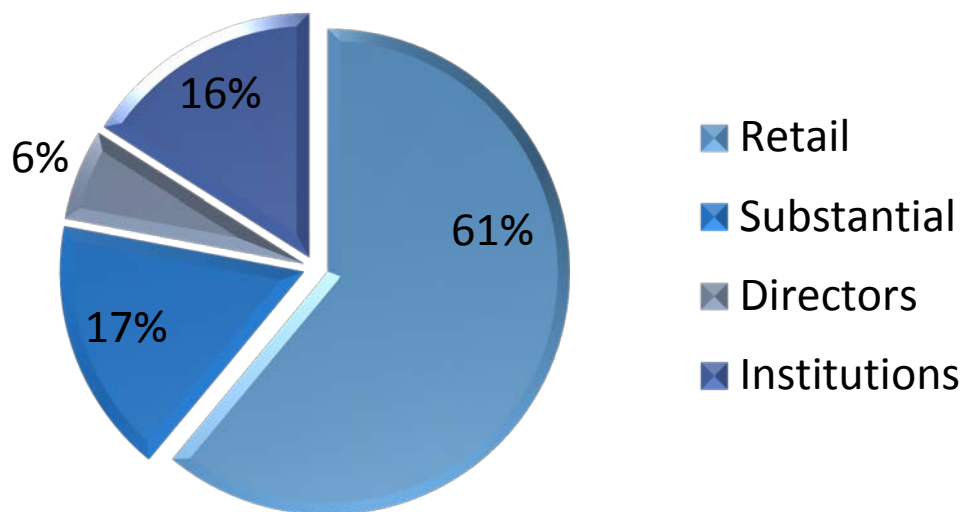
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# Shareholdings and Financial Position

- Fully funded through to completion of Phase 2 trials in 4 different indications
- A\$22m cash reserves at 30 June 2014

Shares outstanding:	1.59 billion
Options outstanding:	147 million (1.3 cents to 3.8 cents per share)
Closing price 28 August 2014	10.0 cents
52 week range:	6.4 cents – 14.5 cents



# Investment Summary

- **Patented drug analogues** of naturally occurring brain growth factors
- Potentially applicable to both **acute** and **chronic** neurological conditions – large markets with no therapies currently available
- **Compelling pre-clinical efficacy data** in TBI, Fragile X and Rett Syndrome models
- Abbreviated regulatory pathways - with possible **Orphan Drug** designation
- Experienced product development and commercial management team
- Phase 2 clinical trials in 4 indications will report results from Q4 2014
- Clinical data will provide the basis for FDA and partnering discussions

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# Expected Milestones

Top-line results for Rett Phase 2	Q4-2014
Complete enrollment in Fragile X Phase 2	H2-2014
Complete enrollment in <i>INTREPID</i>	H1-2015
Top-line results for Fragile X Phase 2	Q2-2015
Top-line results for <i>INTREPID</i>	H2-2015
Top-line results for Concussion Phase 2	H2-2015