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INVESTOR PRESENTATION

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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\$140 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

- Three trials underway, one trial in preparation
- Trials will report results from H2 2014
- Cash reserves A\$24 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
 - Produced following brain injury and stress
 - One of the brain's self-repair mechanisms
- Glypromate (GPE) is considered the active part of the molecule
- □ NNZ-2566 is a synthetic analogue of Glypromate
- NNZ-2566 influences the processes underlying acute and chronic CNS disorders
 - Inflammation
 - Microglial function
 - Synaptic plasticity (inter-neuronal communication)
 - Abnormal electrical activity (e.g., seizures)
- NNZ-2566 potentially treats a wide range of neurological conditions
- □ NNZ-2591 is in the same class of peptides







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
- 6 pending applications
- Remaining patent life between 8 and 16 years
- Additional market exclusivity may be available via Orphan and Pediatric Drug designations

NNZ-2591 and other bicyclic analogues

- 3 issued patents covering composition, formulation and methods of use
- 3 pending applications
- Remaining patent life between 12 and 16 years



Strategy

- Demonstrate the therapeutic benefit of NNZ-2566 in human subjects in both <u>acute</u> and <u>chronic</u> 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



Number of Orphan Drug Designation Applications, Designations, and Approved Orphan Products by Year

NNZ -2566 Clinical Strategy



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 commenced April 2013

- Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 28 days
- Three trial sites in the United States
- Approximately 54 subjects expected to be enrolled
- 47 subjects enrolled to date; 33 have completed the entire study
- "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 commenced Jan 2014
 - Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 28 days
 - At least 60 subjects required to complete the trial six trial sites in the United States
- "Fast Track" and "Orphan Drug" designation granted by the FDA





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NNZ-2566 in Traumatic Brain Injury

- > 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
- Partnership funding of ~US\$23 million by US Army
- \$4+ billion estimated global market potential
- 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 18 US trauma centres 140 enrolled to date
 - "Fast Track" designation granted by the FDA
- Phase 2 trial in Concussion to commence in H1 2014
 - 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



Shareholdings and Financial Position

Fully funded through to completion of Phase 2 trials in 4 different indications

• A\$24m cash reserves at 28 February 2014

16%

• A\$3.7m expected from options through 2016

Shares outstanding: Options outstanding: Closing price 28 Feb 2014 52 week range:

6%

17%

1.54 billion
173 million (1.3 cents to 3.8 cents per share)
9.0 cents
3 cents – 14.5 cents
3 cents – 14.5 cents
61%
Retail
Substantial
Directors
Institutions

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 clinical and commercial management team
- Phase 2 clinical trials in 4 indications will report results from mid-2014
- Clinical data will provide the basis for FDA and partnering discussions



Expected Milestones

Complete enrollment in Rett Phase 2	1H-2014
Initiate Concussion Phase 2	1H-2014
Top-line results for Rett Phase 2	2H-2014
Complete enrollment in Fragile X Phase 2	2H-2014
Complete enrollment in INTREPID	2H-2014
Top-line results for Fragile X Phase 2	1H-2015
Top-line results for INTREPID	1H-2015
Top-line results for Concussion Phase 2	2H-2015

