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Investor Presentation

8 January 2015

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.

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Company Snapshot

- Stock code ASX: NEU – market cap approximately A$250 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
  - Rett syndrome results successfully demonstrated clinical benefit
  - Fragile X syndrome, Concussion, Traumatic Brain Injury will report results in H2 2015
  - Cash reserves A$21 million
- Key strategic relationships
  - US Army Medical Research & Materiel Command
  - International Rett Syndrome Foundation
  - Fragile X Research Alliance
  - Fragile X Drug Validation Initiative

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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

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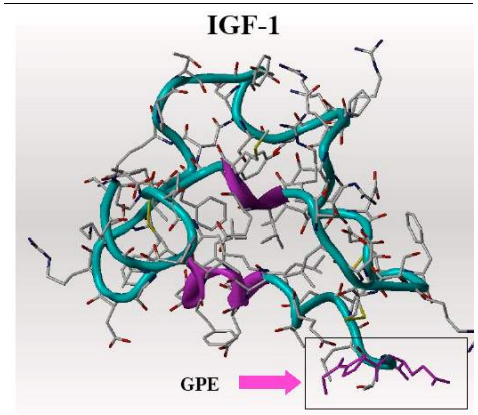
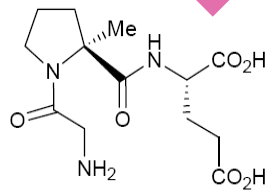
Scientific Foundation

- **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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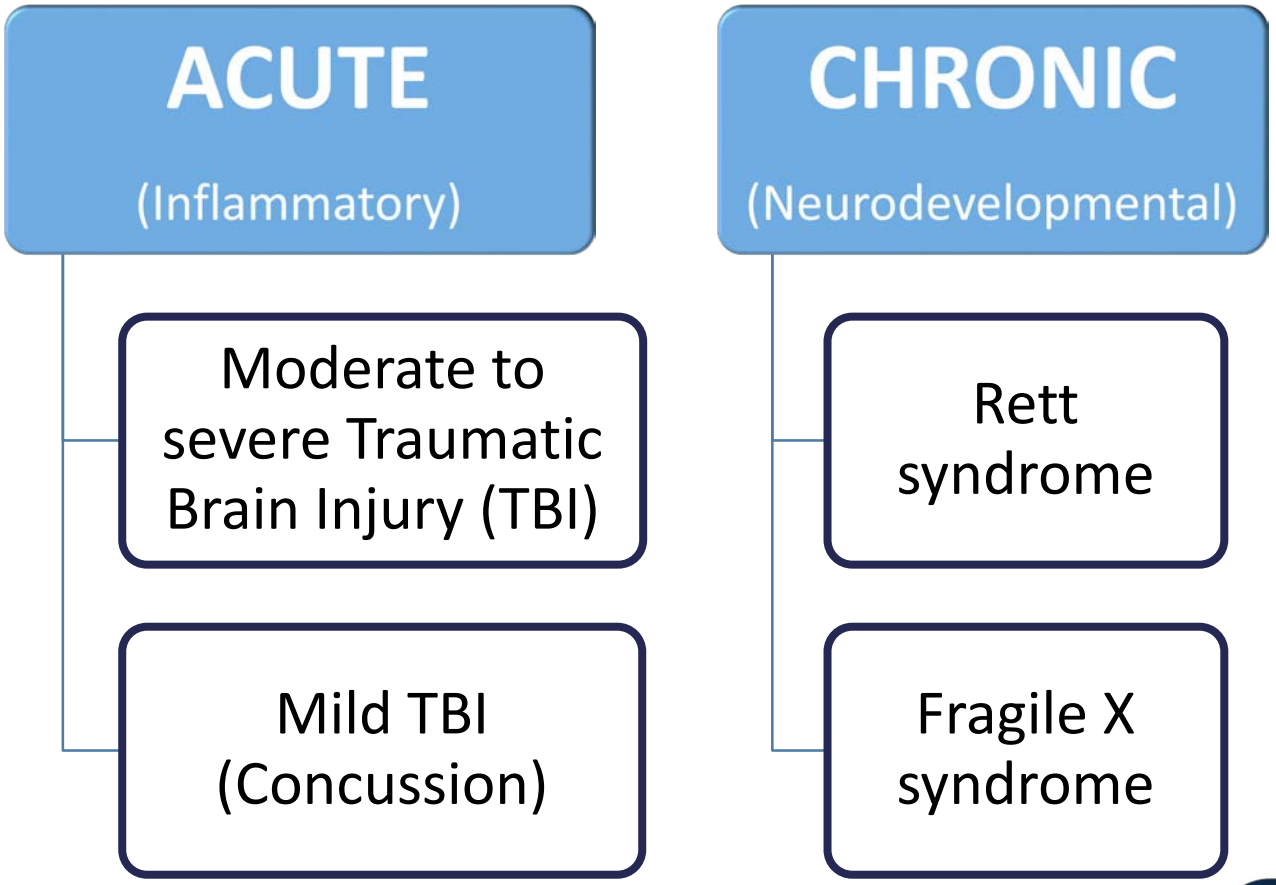
NNZ -2566 Clinical Strategy



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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
  - Muscle rigidity
  - Seizures
  - Older individuals tend to have more severe symptoms

- Profound disability and financial burden for >50,000 patients and families globally
- No approved treatments available and very few in development
- NNZ-2566 in Rett syndrome
  - Successful Phase 2 trial in adults and adolescents
  - Fast Track designation granted by the FDA, applications submitted for Orphan Drug and Breakthrough Therapy

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Phase 2 trial outline

- Double-blind, placebo-controlled dose escalation Phase 2 trial at 3 US hospitals of two dose levels of NNZ-2566:
  - 35 mg per kg of body weight twice daily
  - 70 mg per kg of body weight twice daily
- 56 subjects aged 16 to 45 years randomized, with 53 subjects completing the trial
  - Cohort 0 (9 patients): 35 mg/kg vs. placebo, 14 days of treatment, post-treatment follow-up at Day 28
  - Cohort 1 (18 subjects): 35 mg/kg vs. placebo, 28 days of treatment, post-treatment follow up at Day 40
  - Cohort 2 (29 patients): 70 mg/kg vs. placebo, 28 days of treatment, post-treatment follow up at Day 40
- Primary endpoint- safety and tolerability of NNZ-2566 compared with placebo
- 6 core efficacy outcome measures – group and individual analyses
- Other exploratory efficacy measures

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Results highlights

- Achieved primary endpoint - both dose levels of NNZ-2566 were well tolerated after 28 days of treatment and no safety concerns were identified
- Higher dose (70mg/kg twice daily) exceeded the pre-specified criteria for improvement in core efficacy measures compared with placebo
- The clinical benefit in the trial encompassed core symptoms of Rett syndrome and was observed in both clinician and caregiver assessments
- Efficacy results after only 28 days treatment in a profoundly ill population exceeded Neuren's expectations
- Meeting with FDA expected in H1 2015 to discuss further development
- Applications for both Orphan Drug and Breakthrough Therapy designation submitted to FDA in December 2014

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Results - safety

- Adverse events recording, laboratory measurements and physical observations
- Dose-dependent and time-dependent patterns were not observed in the adverse events reported during the trial
- No consistent dose-dependent trends in observations or laboratory measurements were detected
- Four serious adverse events were reported
  - all were deemed unrelated to treatment
  - two occurred after the treatment period had completed
  - one subject discontinued treatment following two serious adverse events, although the events were deemed unrelated to treatment

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Results – core efficacy measures

- 6 core efficacy measures in 4 efficacy domains, including clinician assessments and caregiver assessments specific to Rett syndrome signs and symptoms
- The pre-specified criteria for evidence of clinical benefit in the analysis of the mean responses for treatment groups required improvement in at least 2 core outcome measures from 2 different efficacy domains, with no clinically significant worsening in all other core endpoints.
- Higher dose (70mg/kg) compared with placebo exceeded this requirement at day 26:
  - 3 measures from 3 different efficacy domains achieved the target - Motor-Behavior Assessment Change Index, Clinical Global Impression of Improvement and Caregiver Top 3 Concerns
  - No clinically significant worsening in the remaining 3 core endpoints
- 70mg/kg dose also achieved the requirement in the subject-level efficacy analysis:
  - Changes in all 6 core outcome measures for each subject were combined in an efficacy score
  - Mean efficacy scores were then compared with placebo

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Results – core efficacy measures (continued)

- Lower dose (35mg/kg) showed improvement in core efficacy measures, but magnitude less than the pre-specified target
- Both doses showed trends of increasing effect with duration of treatment
- Clinical benefit encompassed core symptoms of Rett syndrome and was observed in both clinician and caregiver assessments
- The probability of observing this degree of clinical benefit both in the group-level and subject-level analyses and no clinically significant worsening in any endpoint purely by chance (the “false-positive” rate) was determined by permutation testing as 2.3% (p=0.023)

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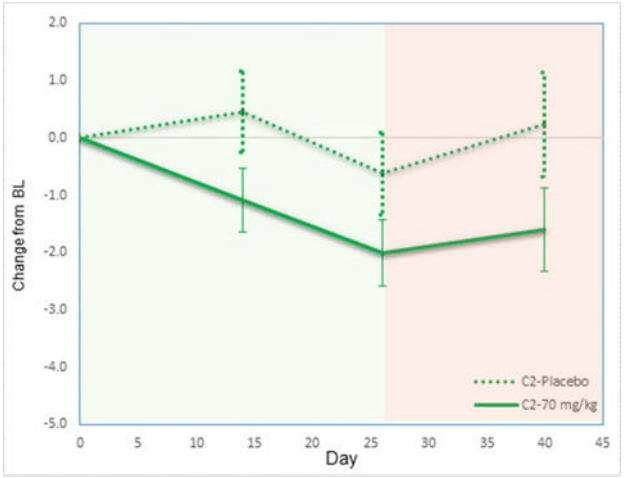
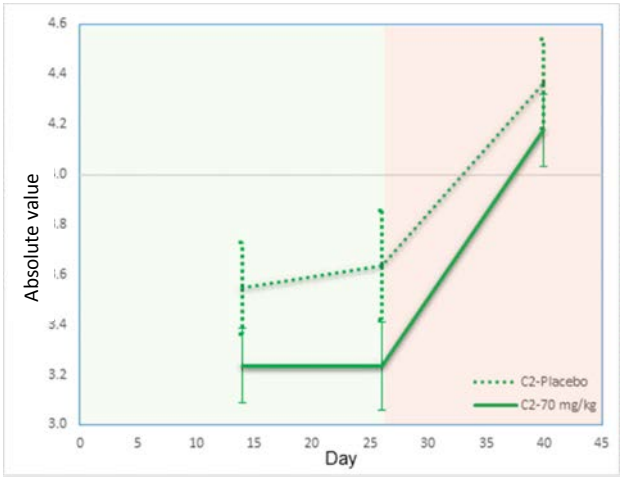
Results – core efficacy measures that met target

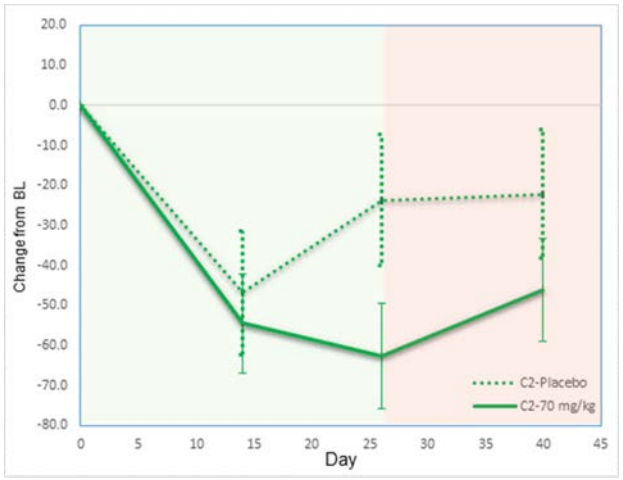
Motor Behavior Assessment Change Index



Clinical Global Impression of Improvement



Caregiver Top 3 Concerns



- Solid line is 70mg/kg, dotted line is placebo
- The two different shaded areas indicate the treatment period and the period post-cessation of treatment
- A negative value on the y-axis indicates benefit

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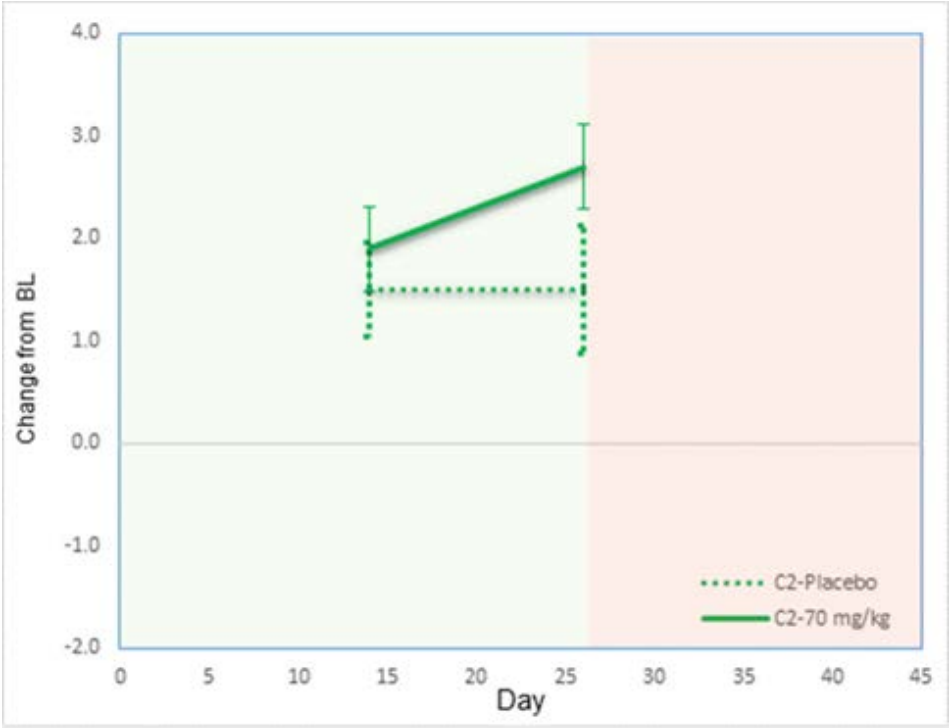
Results – core efficacy measures that met target

Mean subject-level efficacy score



- Solid line is 70mg/kg, dotted line is placebo
- The two different shaded areas indicate the treatment period and the period post-cessation of treatment
- A positive value on the y-axis indicates benefit

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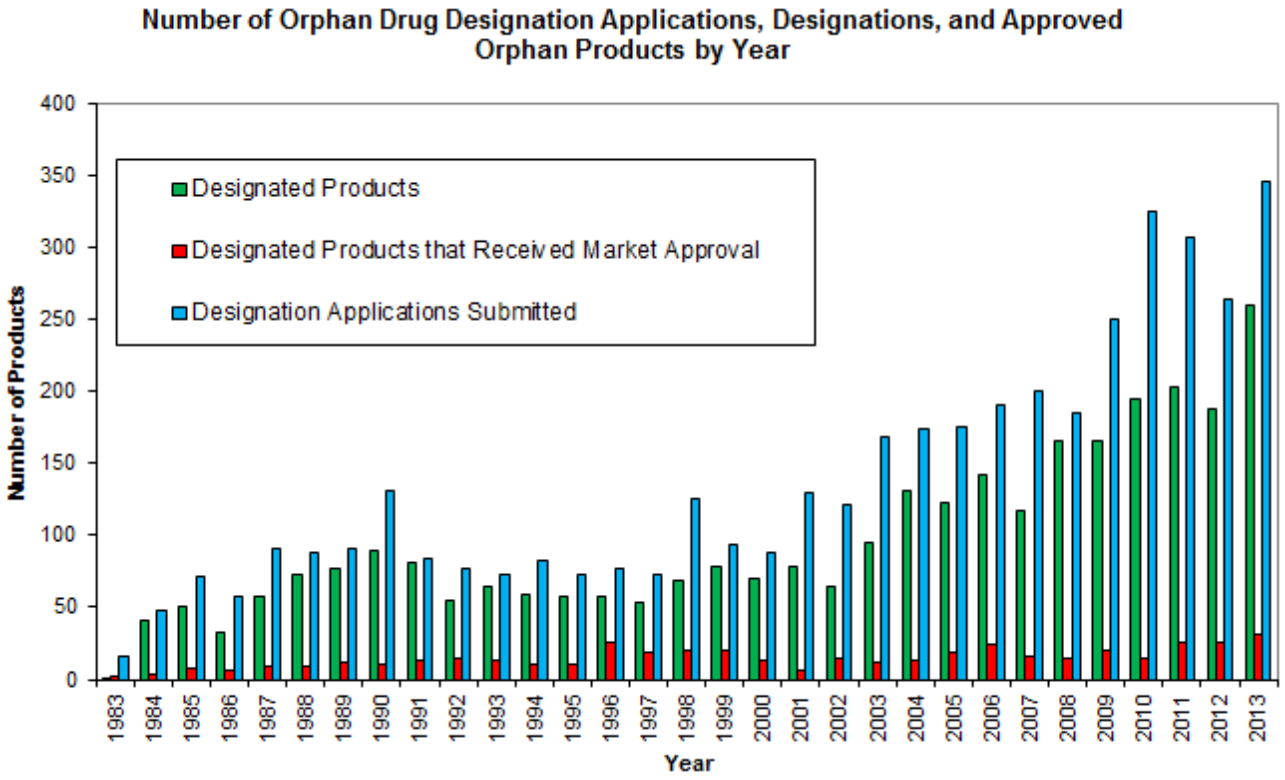
“Orphan drug” designation

- 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 has applied for Rett syndrome
- Pharma companies increasingly pursuing orphan drugs



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“Breakthrough Therapy” designation

- Intended to streamline drug development and review of innovative new medicines that address unmet medical needs for serious diseases or conditions
- The criteria for breakthrough therapy require preliminary clinical evidence indicating that the drug may demonstrate a substantial improvement over existing therapies on at least one clinically significant endpoint
- Commitment that FDA will work closely with the sponsor and give intensive guidance on an efficient drug development program
- Rolling review of marketing application
- FDA will respond to Breakthrough Therapy applications within 60 days after submission

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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 14-45 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
  - Top-line results expected in H2 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 US trauma centres – 196 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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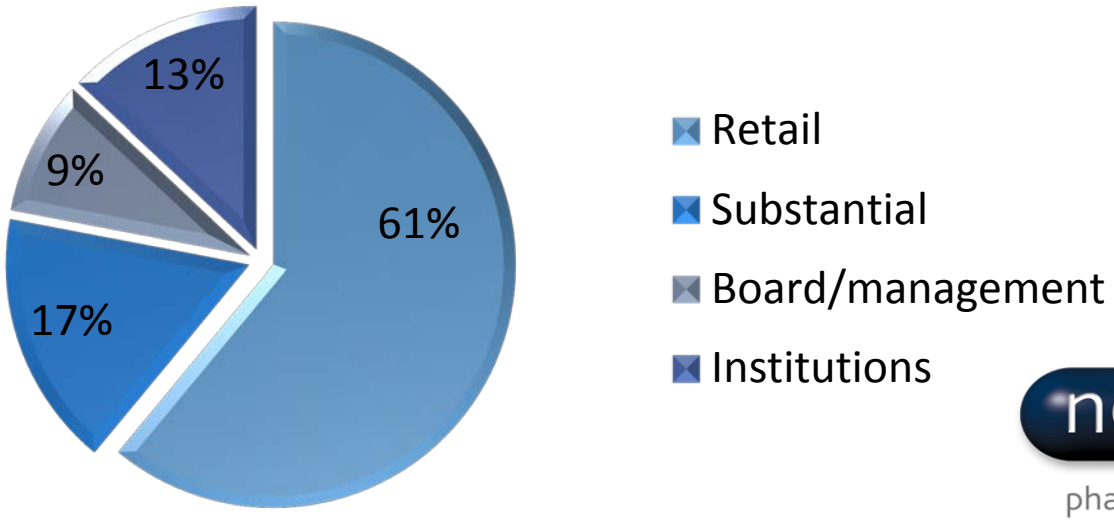
Shareholdings and Financial Position

- Fully funded through to completion of Phase 2 trials in 4 different indications
- A$21m cash reserves at 31 December 2014

Shares outstanding:	1.63 billion
Options outstanding:	116 million (1.3 cents to 3.8 cents per share)
Closing price 8 January 2015	17 cents
52 week range:	6.4 cents – 17.5 cents



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Neuren expected milestones

Outcome of Rett syndrome Orphan Drug and Breakthrough Therapy applications	H1-2015
Rett Syndrome FDA meeting	H1-2015
Top-line results for Fragile X Phase 2	H2-2015
Top-line results for *INTREPID*	H2-2015
Top-line results for Concussion Phase 2	H2-2015

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