Neuren Pharmaceuticals

July 2011
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.

[Motiva® is a registered trademark of Neuren Pharmaceuticals]
Neuren Pharmaceuticals

- Focused on acute and chronic indications in neurology and psychiatry
- Targeting very large markets with unmet need and little or no competition
- Two molecules in Phase II clinical trials under US INDs
- Trial costs covered by grants from US Army and NHMRC—$23 million total
- Perseis subsidiary developing antibodies for breast, other cancers (partnership with New Zealand Breast Cancer Research Trust)
- Experienced management team (all with >5 years at Neuren)

Larry Glass, Chief Executive Officer
30+ years of life sciences experience in management and business development; former CEO of CRO supporting major pharmaceutical and biotechnology companies and US government agencies including NIH, CDC and the US Army

Maggie Scott, RN, CCRP, Director, Clinical Operations
25+ years of management experience in global clinical trials and regulatory affairs; former manager of Greenlane Clinical Research organization; led 3 clinical development programs resulting in NDAs

Mike Bickerdike, PhD, Director, Preclinical R&D
20+ years of research, drug discovery and non-clinical development in the neurosciences; former research project leader and department director at Vernalis Research (UK)

Rob Turnbull, Chief Financial Officer
20+ years experience in corporate finance; former PricewaterhouseCoopers accountant in Auckland, Toronto and London specializing in financial reporting by foreign registrants in the U.S. and securities regulation

Douglas Wilson, MB, ChB, PhD (Director and CMO)
40+ years in academic medicine and the pharmaceutical industry in the US and EU; former CMO of Boehringer Ingelheim responsible for all clinical development and FDA interactions

James Bonnar, Director, QA and Regulatory Affairs
20+ years of experience in quality assurance and regulatory affairs for drug development and manufacturing in NZ, China, the US and the UK
Product Pipeline

<table>
<thead>
<tr>
<th>Discovery</th>
<th>Validated Lead</th>
<th>Formulation</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
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<tbody>
<tr>
<td>Pre-Clinical Efficacy</td>
<td>Manufacturing</td>
<td>Pre-Clinical Toxicology</td>
<td>Phase I</td>
<td>Phase II</td>
<td>Commercialization</td>
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**Motiva® (nefiracetam)**
Apathy and Depression (Post-Stroke, Parkinson’s, Brain Injury)

**NNZ-2566 (IV)**
Traumatic Brain Injury (moderate to severe)

**NNZ-2566 (oral)**
Mild TBI/Rett Syndrome/Stroke Recovery

**NNZ-2591 (DKP)**
Parkinson’s/Neuropathy/Cognition

**Perseis Therapeutics (Oncology Subsidiary)**
Breast/Other Cancers
NNZ-2566

Innovation in the Treatment of Brain Injury and Neurodegeneration

NNZ-2566, a glypromate analog, attenuates brain ischemia-induced nonconvulsive seizures in rats
(Xi-Chun M Lu, Yuanzheng Si, Anthony J Williams, Jed A Hartings, Divina Gryder, Frank C Tortella
(Journal of Cerebral Blood Flow & Metabolism 2009; 1 –9)

“Results indicate that NNZ-2566 possesses a unique therapeutic potential as a safe prophylactic agent that synergistically provides neuroprotection and reduces injury-induced seizures.”
NNZ-2566: Applicability across multiple indications

**Intravenous administration**
- TBI (moderate to severe)*
- Stroke
- Cardiac arrest
- Perinatal asphyxia
- Penetrating brain injury
- Non-convulsive seizures in other CNS injuries/conditions

**Oral administration**
- Mild TBI*
- Rett Syndrome/other autism spectrum disorders*
- Post-stroke recovery
- Prophylaxis following transient ischemic attack
- Chemotherapy-induced neuropathy

* Development initiated
Annual TBI incidence and patient disposition (US)

Oral therapy

- 700,000 Treated and released from ER
- 300,000 Hospital Admissions
- 525,000 Not Hospital-treated
- 52,000 Deaths

Intravenous therapy

- 55,000 Very Severe TBI
- 80,000 Mild TBI
- 70,000 Moderate TBI
- 100,000 Severe TBI

Oral therapy? Intravenous therapy
Dose-dependent prevention of seizures

- Excitotoxicity
  - NMDA activation
  - Ca influx
- Inflammation
  - Pro-inflammatory cytokine elevation
  - Free radical production
- Neuronal Death
  - Necrosis
  - Apoptosis

# of NCS/rat/hour vs. Time post pMCAo (hours)

- Vehicle
- 3mg/kg bolus
- 10mg/kg bolus
- 100mg/kg bolus

NNZ-2566
Therapeutic strategy—enhancing the brain’s response to injury

• Synthetic analogue of naturally occurring peptide
• Inhibits inflammatory cascade following brain injury
• Prevents secondary brain injury—damage to cells adjacent to the primary injury
• Prevents convulsive and non-convulsive seizures

Regulatory strategy—two opportunities for success

• Any validated endpoint plus a functional measure is approvable
• Prevention of post-injury seizures is approvable as a single endpoint
• No established standard for size of effect; must be “clinically meaningful”
• Fast Track designation facilitates communication with FDA
• Planning for single Phase II progressing to single pivotal Phase III
• Phase II designed and powered to deliver definitive results
Why drugs fail

NNZ-2566: TBI profile significantly de-risked

Pharmacokinetics (39%)
- Blood-brain barrier penetration ✓
- Linear pharmacokinetics (PK) ✓
- Comparable PK in healthy volunteers and patients ✓
- Oral bioavailability ✓

Animal toxicity (11%)
- Safe and well-tolerated with good safety margin ✓
- Reproductive toxicology—underway but no data yet?

Adverse effects in patients (10%)
- Drug appeared to be safe, well-tolerated in Cohort 1 ✓
- Safety at higher dose—to be determined ?
- Cardiovascular safety—low risk but no data yet ?

Miscellaneous (5%)
- Manufacturing—fully validated; suitable for Phase III; simple oral formulation ✓
- Regulatory—Fast Track; good relationship with FDA ✓
- Intellectual property—key patents issued ✓
- Staff and CRO resources—in place and working well ✓

Commercial reasons (5%)
- Market competition—none now, limited in the future ✓
- Reimbursement not expected to be an issue ✓
- Strong partnering opportunities ✓
- Financing—shareholders plus US Army ✓

Lack of efficacy (30%)
- Mechanism of action—directly relevant to TBI pathology ✓
- Preclinical efficacy—MOA addresses complex, post-injury cascade; dose-response in diverse brain injury models ✓
- Clinical trial design—endpoints directly translated from preclinical findings; powered to detect approvable benefit ✓
NNZ-2566: TBI commercialization strategy

Market potential
• 1.5 million brain injuries per year in the US alone
• No approved therapies
• Assuming $12,000 for IV and $3,000 for oral as price point, total potential market value is:
  • Moderate – severe = ~$2 billion
  • Mild = ~$2 billion
• ~$500 million peak annual sales forecast (15% market penetration; hospitalized and emergency department patients only)

Competitive advantages
• Only product to address the full range of TBI from mild to severe
• Only competitive product currently in Phase II or beyond is progesterone
• Two approvable outcomes: neurological function and prevention of seizures
• Mechanism reflects broad pharmacologic actions of a naturally occurring product
• Excellent safety profile: few serious adverse events and no known or expected drug interactions
• Key opinion leaders (KOLs) already involved and committed

Partnership opportunities
• US Army financing development; will be a major client (no residual rights)
• Partnership opportunities in multiple fields and indications for oral and intravenous
“In conclusion, apathy has received increasing attention because of its effect on emotion, behavior, and cognitive function. The current study is the first randomized double-blind treatment trial to be conducted among a large group of stroke patients with coexistent apathy and depression, and our results suggest that nefiracetam may be an effective treatment for this clinically important condition.”
Apathy Syndrome: Apathy is *not* depression

Apathy involves loss of interest and emotion and is often characterized by a flattening affect. Though distinct, apathy is often present across many disorders.
Efficacy data in stroke patients—7 clinical trials

3 Phase IIa open-label studies (Japan)

- 165 patients; dosing up to 16 weeks
- Endpoint: Global Improvement Rating (GIR)
- GIR results: Min = 12.5%; Max = 58.8% (450 mg/day x 16 weeks)

1 Phase IIb randomised, placebo controlled study (Japan)

- 321 patients; 3 doses for 8 weeks; 150, 300, 450 mg/day
- GIR results: 24.5%, 28.4%, 41.7% (dose dependent)

2 Phase III randomised, placebo controlled studies (Japan)

- Study 1: 268 patients; 450 mg/day or placebo x 8 weeks
  1. GIR results for all patients: drug vs placebo = 32.3% vs 10.1% (p<0.001);
  2. GIR results for patients <3 months post-stroke: drug vs placebo = 68.4% vs 0.0% (P<0.001)
- Study 2: 267 patients; 450 mg/day nefiracetam or 90 mg/day idebenone x 8 weeks
  1. GIR results: nefiracetam vs idebenone = 37.6% vs 26.9% (p=0.068)

1 Phase IIb randomised, placebo controlled study (US/Canada)

- 159 patients; 600 mg/day, 900 mg/day or placebo for 12 weeks
- Hamilton Depression Scale: Not significant except in most severely depressed pts.
- 51% of patients met diagnostic criteria for apathy
- Statistically significant time- and dose-dependent effects on the Apathy Scale
  1. Repeated measures ANOVA for time (p=0.001)
  2. Dose-dependent effect on remission (75% reduction in apathy score) (p=0.031)
Market potential for apathy in selected indications

Stroke: $1,248,000,000¹

Traumatic brain injury: $840,000,000

Parkinson’s disease: $1,056,000,000

Peak annual sales forecast² (10% market penetration): ~$280 million

¹ Assumes reimbursement at $2,400 per year
² For stroke, traumatic brain injury and Parkinson’s disease
Perseis Therapeutics Ltd.

Targeting Trefoil Factors to Treat Breast and Other Cancers
Developing antibodies for the treatment of breast and other cancers

• Founded in 2009 by Neuren Pharmaceuticals and the NZ Breast Cancer Research Trust
• Targeting Trefoil Factors, which play a significant role in the growth and spread of solid tumors
• Next milestone—*in vivo* efficacy of selected antibodies in breast and gastric cancer models
• Commercialisation strategy: partnership with *in vivo* proof of concept
• Recently selected lead antibodies are from the University of Queensland (UCSF library)

![Graphs showing concentration vs. TFF1 Clone](image)
Corporate Highlights
## Key milestones through 2012

<table>
<thead>
<tr>
<th>Milestone</th>
<th>Forecast</th>
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<tbody>
<tr>
<td>Exception from Informed Consent approved by FDA</td>
<td>Q3 2011</td>
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<tr>
<td>Results of in vivo assessment of cancer antibodies (Perseis)</td>
<td>Q4 2011</td>
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<tr>
<td>File IND for oral NNZ-2566 in mild TBI</td>
<td>Q1 2012</td>
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<tr>
<td>Complete Phase I trial of oral NNZ-2566</td>
<td>Q1 2012</td>
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<tr>
<td>Initiate Phase IIa trial of oral NNZ-2566 in mild TBI</td>
<td>Q2 2012</td>
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<tr>
<td>File IND for oral NNZ-2566 in Rett Syndrome</td>
<td>Q3 2012</td>
</tr>
<tr>
<td>Initiate Phase IIa trial of oral NNZ-2566 in Rett Syndrome</td>
<td>Q4 2012</td>
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<tr>
<td>Complete enrollment of Phase II trial in moderate – severe TBI</td>
<td>Q4 2012</td>
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## Financial Snapshot

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<tbody>
<tr>
<td>ASX ticker:</td>
<td>NEU</td>
</tr>
<tr>
<td>Outstanding Shares:</td>
<td>911 million</td>
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<td>Market Cap:</td>
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<tr>
<td>Current Share Price:</td>
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<tr>
<td>Cash:</td>
<td>~A$7 million (after rights issue allotment)</td>
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<td>Employees:</td>
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Investment Summary

Well positioned in the field of neurological injury and disease

Growing pipeline with two dynamic clinical programs

• Two drugs in Phase II clinical trials for CNS indications
• Oral form of NNZ-2566 approaching clinical trials
• Leveraging NNZ-2566 to pursue additional indications
• Opportunities for Motiva® in additional indications

Funded beyond 2012 by equity and grants; no debt

Pipeline backed by strong patent portfolio

Preclinical candidates advancing in neurology and cancer
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