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

Discov	very Va	alidated	Lead	Formula	ition	Phase I		Phase II	I
	Pre-Clinica Efficacy	al	Manufact	uring	Pre-Clinica Toxicology		Phase II	Cor	mmercialization
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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

Annual TBI incidence and patient disposition (US)





Dose-dependent prevention of seizures





NNZ-2566 TBI: Therapeutic and Regulatory Strategy

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



H. Kubinyi. Drug Research: Myths, Hype and Reality, Nature Reviews Drug Discovery (2003).



NNZ-2566: TBI profile significantly de-risked

Pharmacokinetics, animal toxicity, adverse effects in patients, commercial and miscellaneous issues account for 60% of drug failure.

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, postinjury 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 = \sim \$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



Motiva®(nefiracetam)

A Novel Compound with Broad Neurobehavioral Potential

Double-Blind Treatment of Apathy in Patients with Poststroke Depression Using Nefiracetam (Robert G. Robinson, M.D., Ricardo E. Jorge, M.D., Kathleen Clarence-Smith, M.D., Ph.D., Sergio Starkstein, M.D.) (*The Journal of Neuropsychiatry and Clinical Neurosciences* 2009; 21:144–151)

"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

neuren

pharmaceuticals

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 GIR results for all patients: drug vs placebo = 32.3% vs 10.1% (p<0.001); 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 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 Repeated measures ANOVA for time (p=0.001)

2. Dose-dependent effect on remission (75% reduction in apathy score) (p=0.031)

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

¹Assumes 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

Perseis Therapeutics: Profile

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)





Corporate Highlights

Milestone	Forecast
Exception from Informed Consent approved by FDA	Q3 2011
Results of in vivo assessment of cancer antibodies (Perseis)	Q4 2011
File IND for oral NNZ-2566 in mild TBI	Q1 2012
Complete Phase I trial of oral NNZ-2566	Q1 2012
Initiate Phase IIa trial of oral NNZ-2566 in mild TBI	Q2 2012
File IND for oral NNZ-2566 in Rett Syndrome	Q3 2012
Initiate Phase IIa trial of oral NNZ-2566 in Rett Syndrome	Q4 2012
Complete enrollment of Phase II trial in moderate – severe TBI	Q4 2012



Financial Snapshot

ASX ticker:	NEU
Outstanding Shares:	911 million
Market Cap:	\$14.6 million
Current Share Price:	\$0.016
Cash:	~A\$7 million (after rights issue allotment)
Employees:	9



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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