Neuren Pharmaceuticals

Annual General Meeting

May 2011

Forward Looking Statement

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.

Neuren is…

- A biopharmaceutical company—not pure biotech, not pure small molecules
- With scientific origins in one of the leading research institutions in the world
- Always science driven, knowing that great science is where it all begins
- Also knowing that great science isn't enough for a company
- Committed to drug development practices at the highest global standards
- Turning science into products—not turning science into more science
- Working with the best of the best in science, manufacturing, medicine, regulatory affairs, clinical development
- Skeptical of the catechism of "classical" drug development, knowing that there's a better way and that sometimes the better way means changing the rules
- Fully capable of effectively managing complex programmes with outside experts without losing control of quality, cost or vision
- The ideal partner—world class science and product development expertise, cost-effective, driven by results not ego, able to deliver quality results at the same level as *any* partner

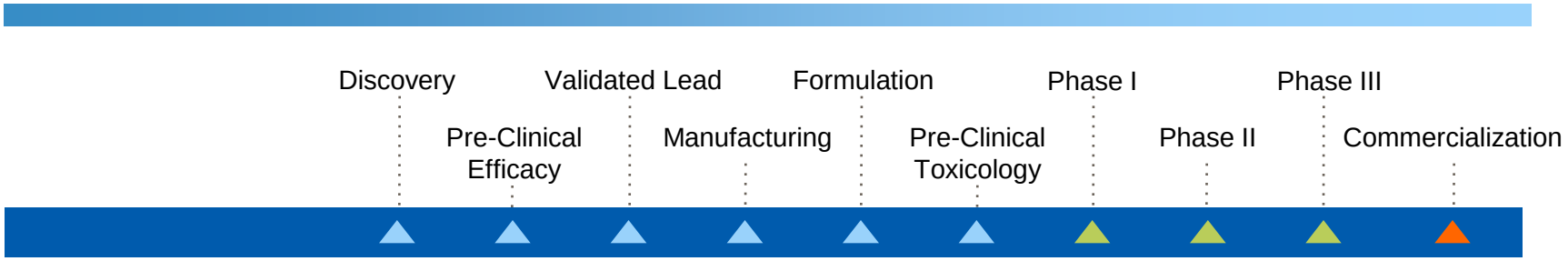
Neuren's people are…

- A small group of incredibly dedicated professionals
- Average of 20+ years of global experience in drug development, clinical trials, regulatory affairs, business development, corporate finance
- All key staff have been with Neuren for >5 years
- A team in every sense of the word
- Every senior member of the team has leadership experience
- The same team that delivered on a complex, international pivotal trial on schedule, under budget, with quality comparable to that of any company
- The same team that brought NNZ-2566 from discovery to Phase II in 7 years for under $10m—less than half the average time and cost for pharma
- A team that has delivered and managed >$200m in partnerships, licenses, collaborations and alliances
- A team that understands that drug development has a beginning, a middle and an end and is much more than a paycheck
- A team that believes what we do is for patients, families and shareholders
- A team that understands what it takes to deliver shareholder value

2010: Significant progress on all programmes

- Initiated the *INTREPID-2566* Phase 2 trial of NNZ-2566
- Completed manufacturing process development and validation
- Completed the Phase I safety/PK trial in female volunteers
- Initiated the oral formulation development program for NNZ-2566
- Additional US Army funding of approximately US$4.3m
- Initiated reproductive toxicology and other required safety studies
- Research collaboration with the Rett Syndrome Research Trust
- Initiated the Phase 2 trial of Motiva® to treat post-stroke apathy
- NZ$250,000 grant to Perseis to expand the discovery program
- Key patents issued; progress on important patent applications

Product Pipeline



Motiva™ (nefiracetam)	Apathy and depression (post-stroke, Parkinson's, Alzheimer's)
NNZ-2566 (IV)	Traumatic brain injury (moderate to severe)
NNZ-2566 (oral)	Mild TBI/stroke recovery/Rett Syndrome
NNZ-2591 (DKP)	Parkinson's / peripheral neuropathy
Perseis Therapeutics (Oncology Subsidiary)	Breast/other cancers

Two dynamic clinical programs

NNZ-2566

- Phase II *INTREPID*$^{-2566}$ trial initiated in Q2 2010 for moderate to severe traumatic brain injury
- Fast Track designation
- Oral formulation development initiated Q2 2010 for mild TBI and other indications
- Partnership with US Army since 2004
- US$22.8 million in total funding from the US Army
- Applications in other indications (e.g., Rett Syndrome) being explored

Motiva®

- Treatment for psychiatric and behavioral effects of stroke, TBI and chronic CNS disorders
- 7 studies in post-stroke patients (1 US/Canada; 6 Japan)—all with positive efficacy
- Safety confirmed in >1700 patients
- Phase IIb trial initiated in Q2 2010
- Funded by NHMRC grant to the University of Western Australia (Sergio Starkstein, MD, PhD)

NNZ-2566

Innovation in the Treatment of Brain Injury and Neurodegeneration

NNZ-2566: Therapeutic and Regulatory Strategy

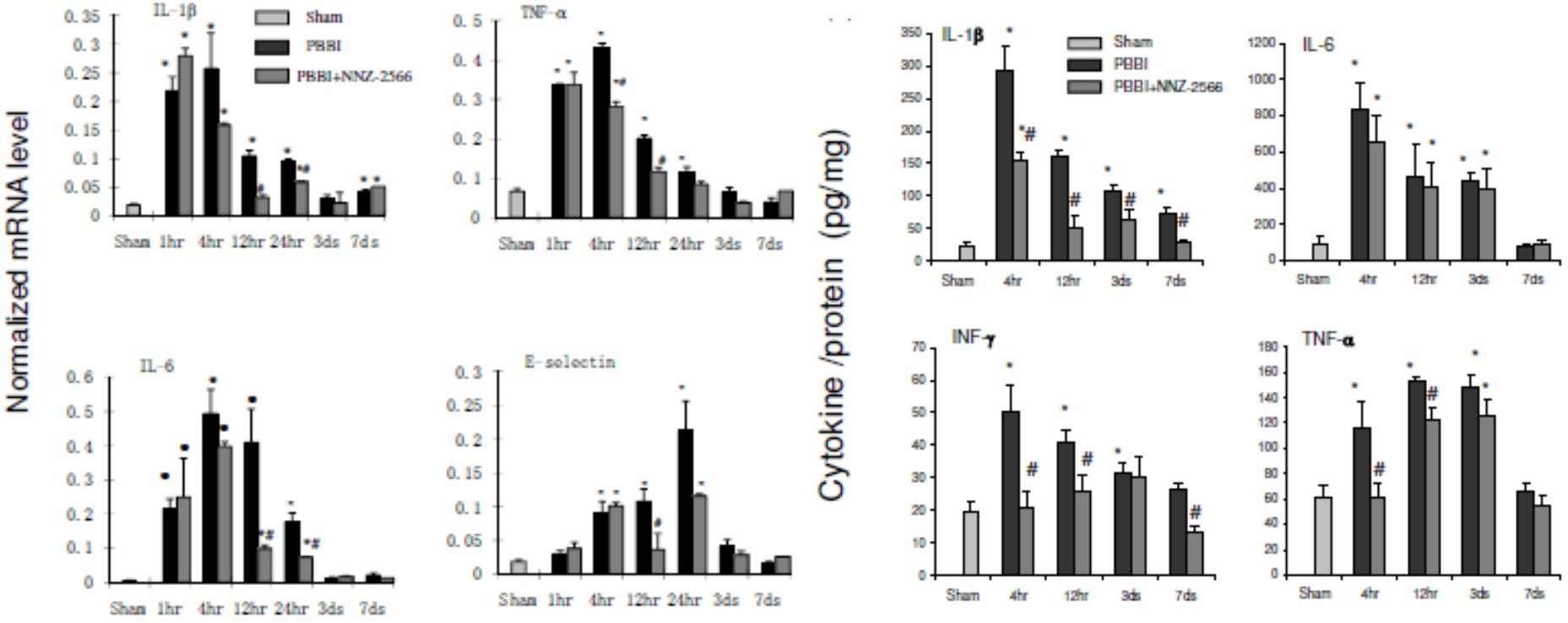
Therapeutic strategy: rescue brain cells from the effects of acute and chronic neurodegeneration

- Synthetic analogue of naturally occurring neuroprotective peptide—the brain's response to injury
- Prevents secondary brain injury—damage to cells adjacent to the primary injury
- Prevents convulsive and non-convulsive seizures which also cause secondary brain injury
- Inhibits activation of inflammatory response to brain injury and chronic CNS disorders
- Blocking the inflammatory response inhibits cell death (apoptosis and necrosis)
- Blocking the inflammatory response normalizes brain cell function

Regulatory strategy for TBI

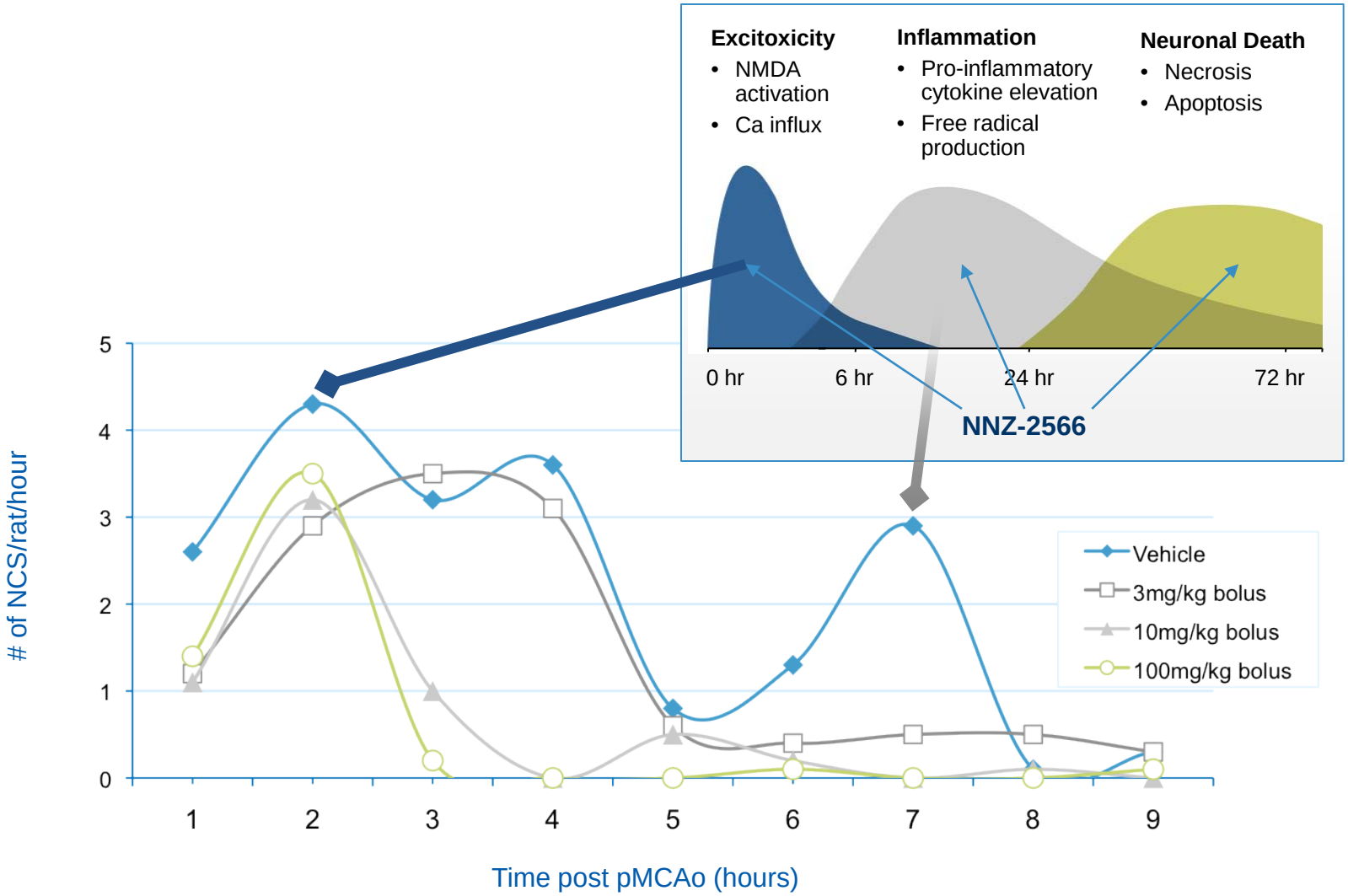
- Any one validated endpoint plus a functional measure is potentially approvable
- Prevention of post-injury seizures is approvable alone
- Planning for single Phase II to single pivotal Phase III—possible with strong efficacy data
- Phase II designed and powered to deliver definitive results across multiple, approvable endpoints

NNZ-2566 targets multiple inflammatory processes



Gene expression **Protein expression**

NNZ-2566 prevents post-injury seizures



NNZ-2566: Risk profile supports optimism

Pharmacokinetics

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

Miscellaneous

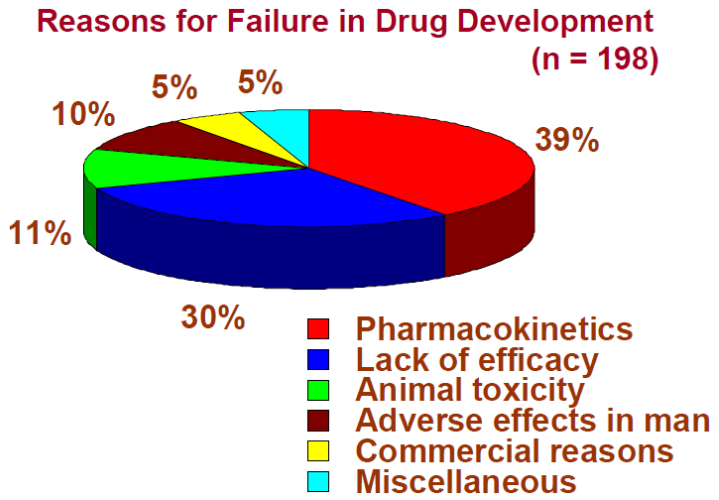
- Manufacturing—fully validated; suitable for Phase III; simple oral formulation ✓
- Regulatory—Fast Track; good relationship with FDA ✓
- Intellectual property—key patents issued ✓
- Staff and CRO resources—in place and working well ✓

Animal toxicity

- Safe and well-tolerated with good safety margin ✓
- Reproductive toxicolog—underway but no data yet ?

Adverse effects in patients

- 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 ?



Commercial reasons

- 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

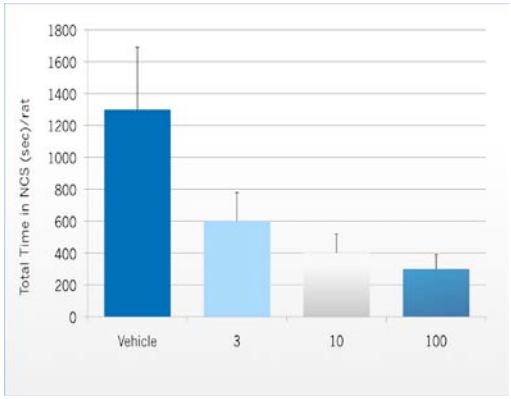
- Mechanism of action—directly relevant to TBI pathology ✓
- Mode of action—multiple modes of action address complex, post-injury cascade; dose-response replicated in diverse brain injury models ✓
- Clinical trial design—endpoints directly translated from preclinical findings; powered to detect approvable benefit ✓

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

NNZ-2566—a growing franchise

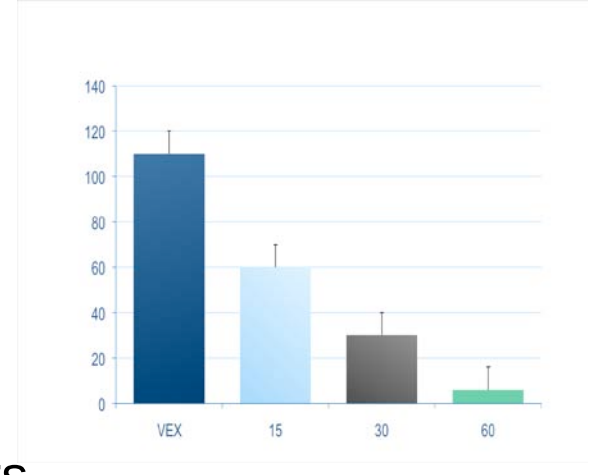
Intravenous administration

- Stroke
- Cardiac arrest
- Perinatal asphyxia
- Penetrating brain injury
- Non-convulsive seizures in other CNS injuries/conditions



Oral administration

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



Rett Syndrome and autism spectrum disorders (ASDs)

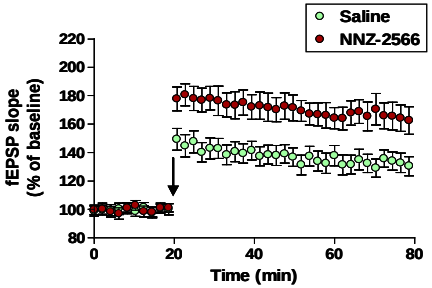
ASDs are disorders of connections between brain cells (neurons)

- Rett Syndrome has too few connections
- Fragile X Syndrome has too many connections
- Autism has ~ 70% patients with too many and ~ 30% patients with too few

Rett Syndrome

- Largest single cause of intellectual disability and autism in females; genetic disorder caused by a mutation in the *MECP2* gene
- Normal infant development followed by loss of language, loss of social contact, loss of motor functions, epilepsy

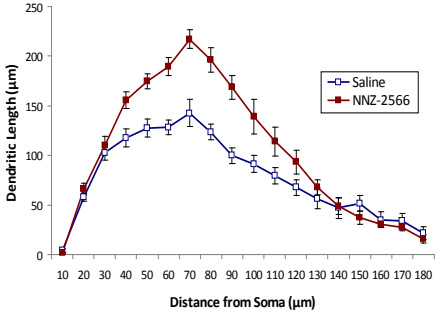
NNZ-2566 rescues brain function in MECP2 model



NNZ-2566 (20 mg/kg i.p. 1/day) increases hippocampal LTP in the mecp2 mouse model of Rett syndrome (Jaenisch mecp2y/- knockout).

Improves neuronal plasticity

Overcomes neuronal structural deficit



NNZ-2566 increases mean dendrite length in the CA1 region of the hippocampus in mecp2 mutant mice.

Motiva®(nefiracetam)

A Safe Drug for a Difficult to Treat Indication with Unmet Need

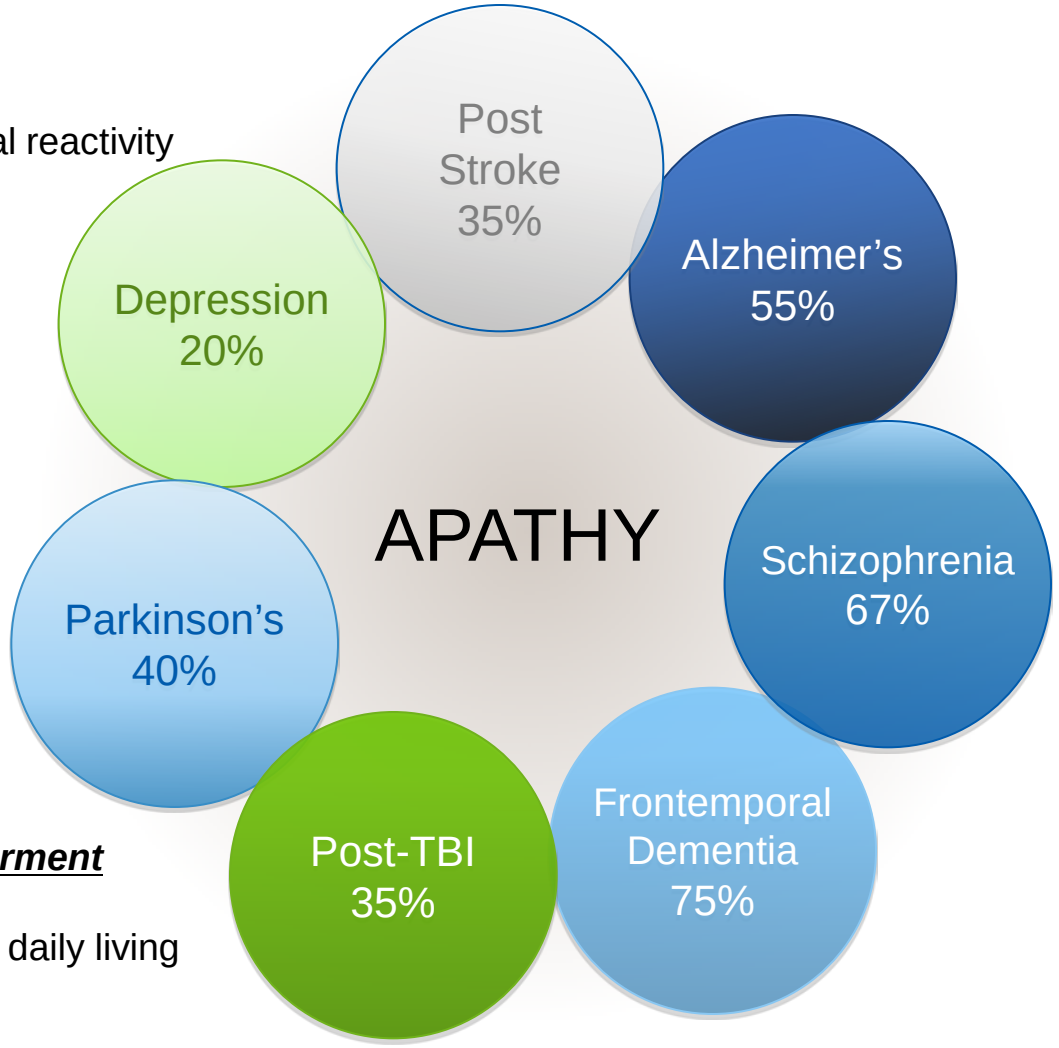
Apathy Syndrome: Apathy is *not* depression

Symptoms of apathy
Loss of motivation and initiation
Lack of persistence
Emotional indifference or diminished emotional reactivity
Decreased social engagement

Symptoms of depression
Dysphoria, sadness
Hopelessness
Guilt, self-criticism
Suicidal ideation
Sleep problems
Loss of appetite

Overlapping symptoms
Lack of interest in events or activities
Lack of energy
Psychomotor slowing
Fatigue
Decreased insight

Apathy causes significant functional impairment
Inability to participate in treatment
Reduced ability to perform normal activities of daily living
Significant burden on caregivers, family
Poor clinical outcomes



Motiva: Efficacy data in stroke patients (Japan)

3 Phase IIa open-label studies

- 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

- 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

- 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)

Motiva: Efficacy data in stroke patients (US/Canada)

Phase IIb (US IND)

- Sponsored by Daiichi
- Led by Robert Robinson (U of Iowa), Sergio Starkstein (U of Western AU), Catherine Clarence-Smith (Prestwick Pharmaceuticals)
- Randomised, double-blind, placebo-controlled, 2 dose trial
- 159 patients; 600 mg/day, 900 mg/day or placebo for 12 weeks
- Primary endpoint: Hamilton Depression Scale (HAM-D)
- Secondary endpoints: Apathy Scale, Symbol Digit Modality Test (SDMT), Burden Inventory (BI), other ADL and psychiatric tests

Study results

- HAM-D: not statistically significant overall
  1. Statistically significant benefit in most severely depressed quintile
  2. 70% placebo response rate makes interpretation difficult
- 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. Repeated measures ANOVA for time by treatment group (p=0.05)
  3. Repeated measures ANOVA for time by 900 mg/day vs. placebo (p=0.01)
  4. Dose-dependent effect on remission (75% reduction in apathy score) (p=0.031)
- Side effects no different than placebo

Perseis Therapeutics Ltd.

Targeting Trefoil Factors to Treat Breast and Other Cancers

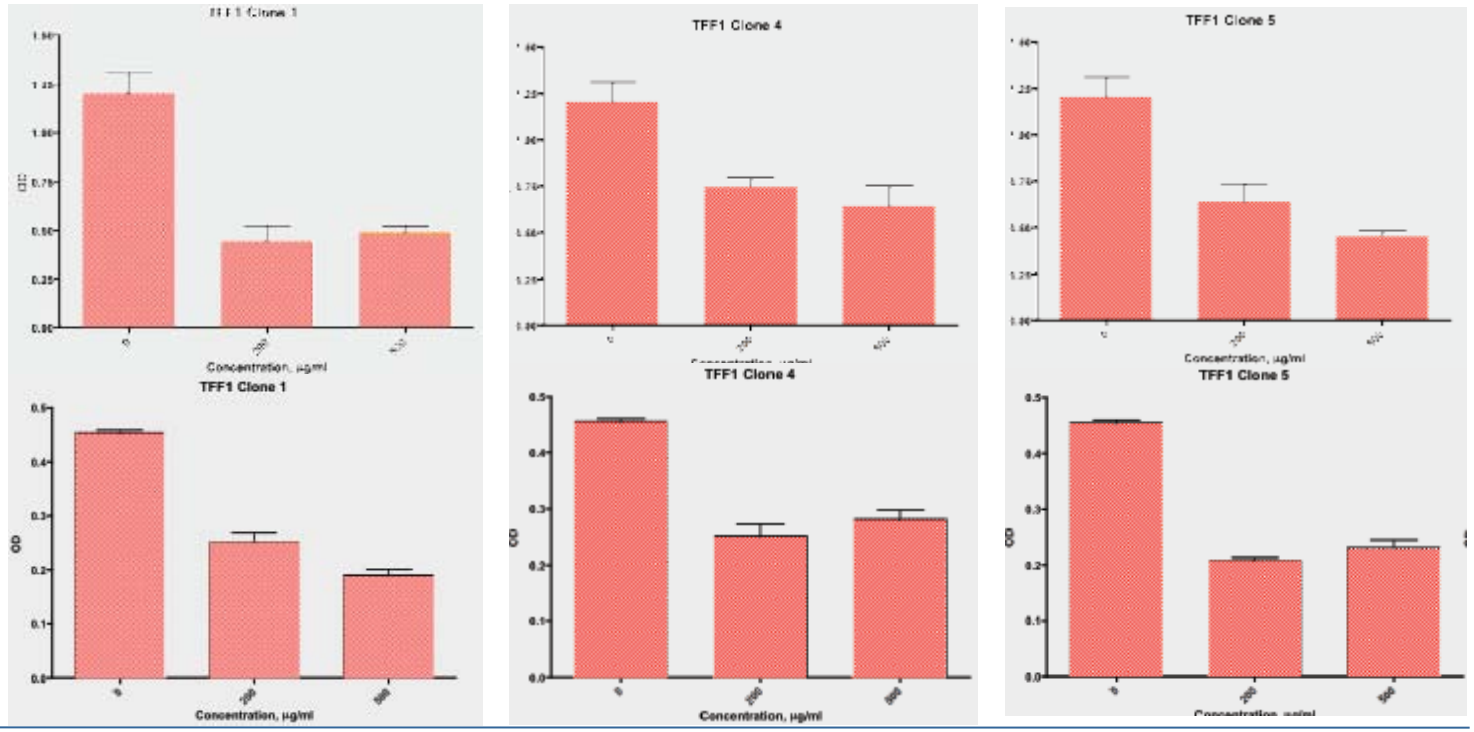
Perseis Therapeutics: Profile

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 major milestone expected in Q3 2011—in vivo efficacy of selected antibodies
- Commercialisation strategy: partnership with in vivo proof of concept
- Recently selected lead antibodies are from the University of Queensland (UCSF library)

Gastric cancer

Breast cancer



Recent publications

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."

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."

Recent commentary

- Statement by the Director of Defense Medical R&D Program before the US Congress (September 2010)

“Currently, the only promising therapy for TBI in large clinical trials is progesterone and NNZ 2566.”

- Thomson Reuters. *The Ones to Watch* (April – June 2010)

- *NeuroInvestment* (December 2010)

“Neuren's synthetic analog of this active fragment, NNZ-2566, is in Phase IIb testing in TBI, and they have recently announced success in producing an oral version. Given the preliminary indication of structural improvement, Neuren may prioritize Rett Syndrome, given that they are receiving some financial support from the Rett Syndrome Trust (for testing NNZ-2566 in the MeCP2 model), but this has potential applicability to Fragile X and autism in general. The mechanisms by which Glypromate and NNZ-2566 are believed to exert neuroprotectant effects include their impact on inflammatory cytokines and upon microglial activation, with the latter having been postulated (via microglial hyper release of glutamate) as a neuroimmune factor in the etiology of autism.”

Where Are We Now?

Key programmes fully funded through significant milestones

NNZ-2566

- Cohort 1 completed; Cohort 2 underway; 18 sites lined up; new IND for EFIC
- Oral development on track for Phase I in Q4 2011, Phase II in mild TBI in Q2 2012
- Development for Rett Syndrome/autism spectrum disorders initiated
- Additional indications being explored

Motiva

- Efficacy data in hand from 7 completed studies
- Two clinical sites actively recruiting in Phase II trial; third site selected
- Protocol amended to include apathy persisting beyond 3 months
- Protocol amendment to include patients with apathy and depression being evaluated

Perseis

- Three lead antibodies from UCSF fully human library being scaled up for in vivo testing
- Results from breast cancer model expected Q3 2011; gastric cancer study under discussion

Actively focused on partnering opportunities for all programmes

Further Information

Larry Glass, CEO
+1 (301) 941 1830
lglass@neurenpharma.com

New Zealand

Level 2, 57 Wellington Street
Freemans Bay 1011, Auckland
New Zealand

+64 (9) 3700 200
1 800 259 181 (Australia Freephone)

USA

3 Bethesda Metro Center, Suite 700
Bethesda, Maryland 20814 USA