



**Developing innovative new medicines for
brain injury and neurodegeneration**

Larry Glass, CEO

(lglass@neurenpharma.com)

Neuren Pharmaceuticals

- Targeting brain injury and neurodegeneration
- Two NCEs in Phase II under US INDs
- Trial costs covered by grants from US Army and NHMRC—\$23 million total
- Subsidiary (Perseis Therapeutics) developing mAbs for breast, other cancers
- Operations in US, New Zealand and Australia
- 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

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

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

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

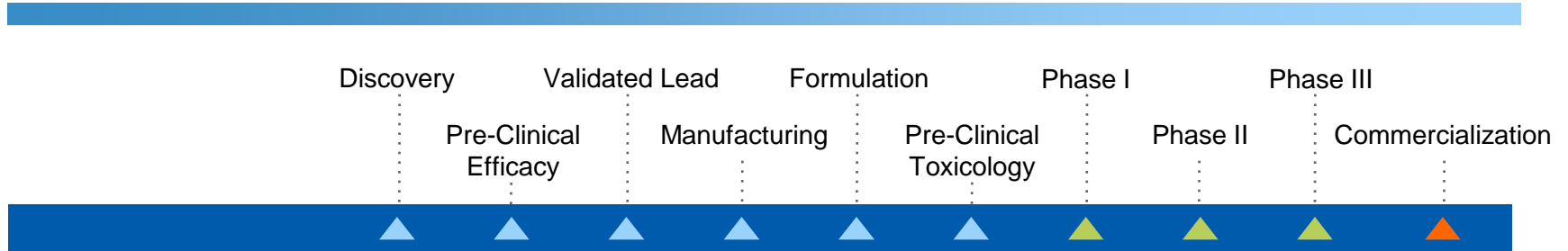
Mike Bickerdike, PhD, Director, Preclinical R&D

20+ years of research, drug discovery and non-clinical development in the neurosciences; former research project leader and department director at Vernalis Research (UK)

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



Motiva™
(nefiracetam)

Apathy and depression (post-stroke, Parkinson's, Alzheimer's)

NNZ-2566 (IV)

Traumatic brain injury (moderate to severe)

NNZ-2566 (oral)

Mild traumatic brain injury/Rett Syndrome

NNZ-2591 (DKP)

Parkinson's / peripheral neuropathy

Perseis Therapeutics
(Oncology Subsidiary)

Breast/other cancers

NNZ-2566 product overview

- Synthetic analogue of IGF-1(1-3), a naturally occurring neuropeptide
- Phase II for moderate to severe traumatic brain injury (Fast Track)
- Direct costs covered by grants from US Army
- 18 Level I and II trauma centers in the US and AU (11 active)
- 3 cohorts; Cohort 1 completed; Cohort 2 more than 2/3 enrolled
- IND for exception from informed consent (EFIC) approved by FDA
- Forecast completion of enrolment: Q4 2012
- Regulatory strategy: single Phase II, single pivotal trial under SPA
- Oral formulation in development for mild TBI, Rett Syndrome

NNZ-2566 clinical profile

Mechanism of action

- Inhibits upregulation of inflammatory cytokines (IL-6, IL-1 β , TNF- α , E-Selectin, IFN- δ)
- Normalizes pro-apoptotic Bax and anti-apoptotic Bcl-2 expression
- Inhibits microglial activation
- Attenuates post-injury seizures (convulsive and non-convulsive)
- Relevant to multiple acute and chronic conditions

Excitotoxicity

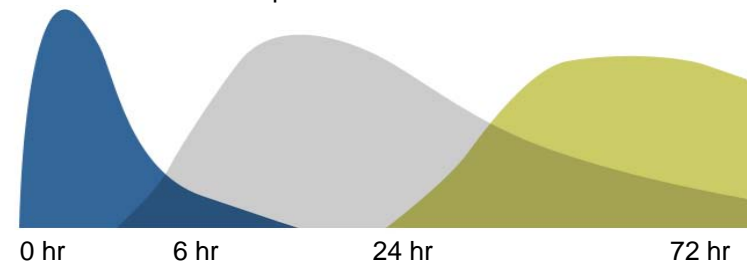
- NMDA activation
- Ca influx

Inflammation

- Pro-inflammatory cytokine elevation
- Free radical production

Neuronal Death

- Necrosis
- Apoptosis



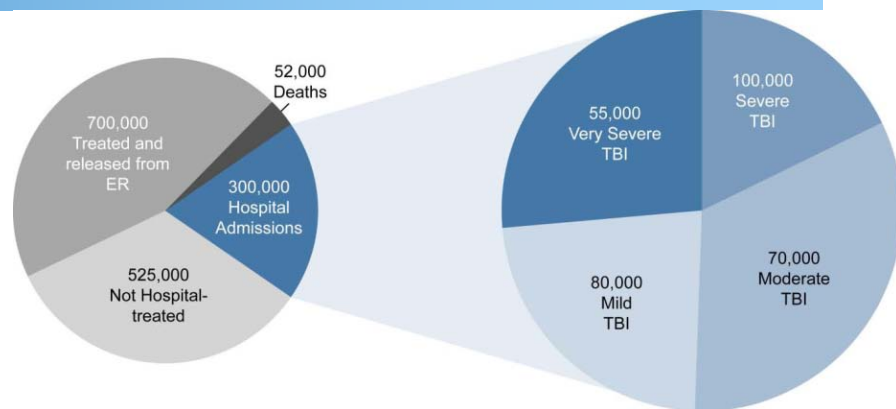
Clinical trial design (moderate to severe TBI)

- Double-blind, placebo controlled, rising dose
- 260 acute, non-penetrating TBI patients
- Randomized 2:1 drug to placebo
- Administration of drug within 8 hours of injury (6 hours under EFIC protocol)
- Bolus dose followed by 72 hours of continuous infusion
- Endpoints
 - Safety
 - Pharmacokinetics
 - Functional outcomes: **Glasgow Outcome Scale-Extended (GOS-E)**; **ADL (Mayo-Portland Adaptability Index)**; neurocognitive function; mood
 - Biological outcomes: **seizures** detected by continuous EEG; serum biomarkers of neuronal, glial and axonal cell damage; intracranial pressure

TBI commercialization strategy

Market

- 1.5 million brain injuries per year in the US alone
- No approved therapies
- At \$12,000 for IV and \$3,000 for oral:
 - **Moderate – severe = ~\$2 billion**
 - **Mild = ~\$2 billion**
 - **~\$500 million peak sales forecast (15% penetration)**

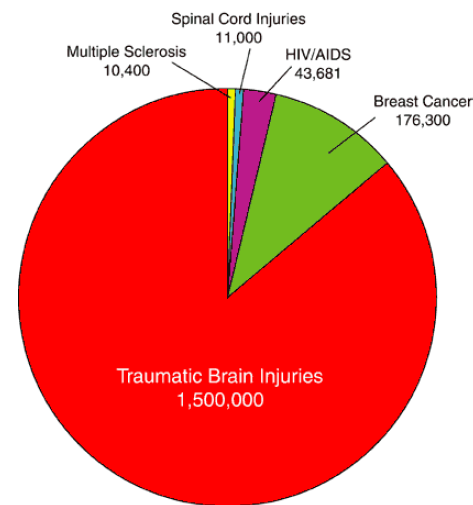


Competitive advantages

- Only product that addresses the full range of TBI from mild to severe
- Only competitive product at Phase II or beyond is progesterone
- Two approvable outcomes: neurological function *and* prevention of seizures
- Broad MOA of a naturally occurring neuropeptide
- Excellent safety profile; no known or expected drug interactions
- KOLs already involved and committed

Partnership status

- US Army supporting development; will be a major client (no residual rights)
- Partnership discussions underway



Comparison of Annual Incidence

NNZ-2566 franchise

Intravenous administration

Moderate to severe TBI

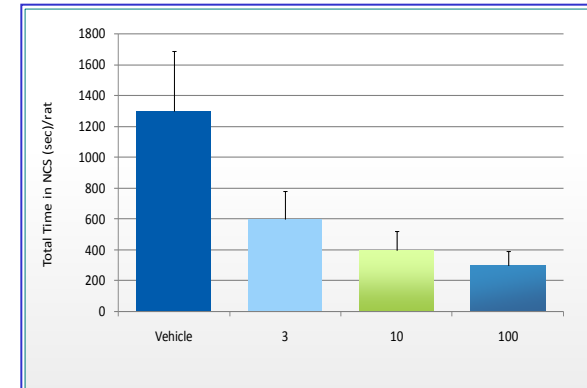
Penetrating brain injury

Stroke

Cardiac arrest

Perinatal asphyxia

Non-convulsive seizures in other CNS injuries/conditions



Oral administration

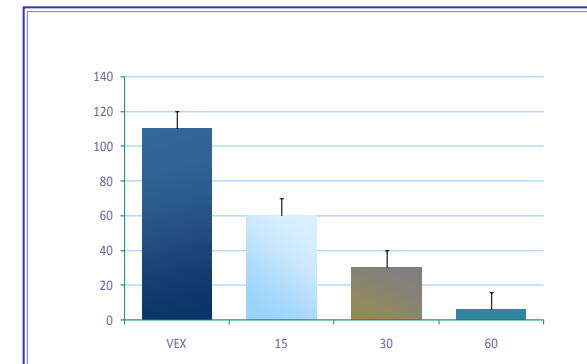
Mild TBI/concussion

Rett Syndrome/autism spectrum disorders

Post-stroke recovery

Prophylaxis following transient ischaemic attack

Chemotherapy-induced neuropathy



Rett Syndrome and autism spectrum disorders (ASDs)

ASDs are disorders of connections between brain neurons

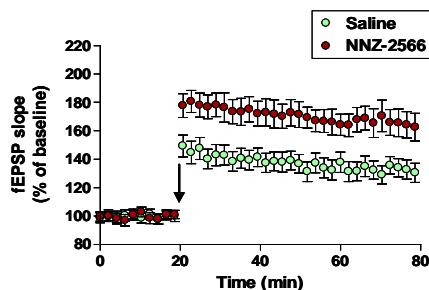
- Rett Syndrome has too few, Fragile X Syndrome has too many.
- Autism has ~ 70% patients with too many and ~ 30% patients with too few
- No approved drugs

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, social contact and motor functions, epilepsy
- Large market potential – US = **\$430 million**; global = **\$1.2 billion**



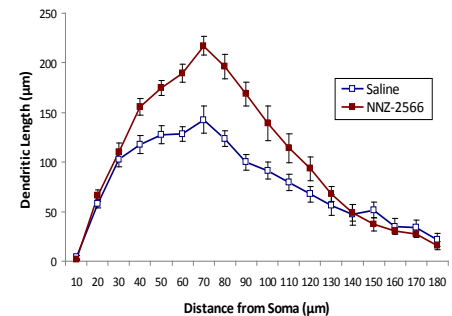
NNZ-2566 rescues brain function in MECP2 model



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.

Key milestones through 2012

Milestone	Forecast
Exception from Informed Consent approved by FDA	Q3 2011 ✓
Complete pharmacokinetic and toxicology studies for oral NNZ-2566	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:	1.14 billion
Market Cap:	\$20 million
Current Share Price:	\$0.018
Cash:	~A\$9 million
Employees:	9