



Chairman's Address – Dr. Richard Treagus

Annual Shareholders' Meeting

Of

Neuren Pharmaceuticals Limited,

30 April 2014

Good morning Ladies and Gentlemen. Welcome to our Annual Shareholders' Meeting in Melbourne. It is my pleasure to present Neuren's achievements for the financial year ended 31 December 2013, and also provide an update on the overall position of the company and the progress of our key clinical development programs.

I am required to remind you that today's presentation contains some forward-looking statements that are subject to risks which could cause the outcomes to be different from our anticipated outcomes.

At our Shareholders' Meeting last year I took the opportunity to outline a strategy that would increase our prospects for success, and at the same time announced a range of structural changes to our business that we considered to be necessary. I am pleased to report that since last year we have made substantial progress in many of these areas and the business is in a very much stronger position.

Neuren's corporate office was relocated from Auckland to Melbourne, the number of directors was reduced, resulting in a more efficient and engaged Board, and the management team was both re-organised and strengthened.

In October of last year Neuren completed a successful capital raising of A\$23 million at a price of 11.5 cents per share. We received strong support from our existing shareholders as well as from a number of new institutional investors. We now have approximately 16% of our issued shares held by institutional shareholders in Australia, New Zealand, Hong Kong and London.

Neuren has benefited from a greater level of research coverage, a stronger company profile, improved turnover in its shares, a reduction in the number of outstanding share options, and more recently, promotion into the S&P/ASX All Ordinaries index.

During 2013 Neuren's share price appreciated significantly as we progressed and made the changes that I have outlined. We are of the view that the more recent fall in the share price reflects the price weakness observed in tech stocks in general, as well as biotechnology stocks in particular, and that it holds little direct correlation to Neuren's fortunes. We remain on-schedule and on-budget with respect to our key clinical programs. And, most importantly, Neuren has the necessary funding as well as the pre-requisite management skills in place that will allow us to complete all four key clinical trials.

In my report to you this morning I wish to discuss three areas in a little more detail:

1. Neuren's strategy
2. Financial position
3. Key clinical trial programs for NNZ-2566

Neuren's Strategy

Neuren's strategy is designed to maximise the commercial value of our two lead drug molecules NNZ-2566 and NNZ-2591. There are a number of key elements that underpin this approach:

Firstly, we have a well-defined clinical trial program for NNZ-2566 that is aimed at eliciting the first signals of meaningful clinical benefit in human subjects suffering conditions for which there are few or no effective drug therapy options currently available. We believe that to the extent we are successful in the clinic, it will place Neuren in a strong position with respect to the US Food and Drug Administration (FDA) and potential strategic partners alike.

Secondly, we continue to pursue favourable regulatory pathways and we were naturally delighted to have been granted orphan drug status by the FDA for NNZ-2566 in the indication of Fragile X Syndrome towards the end of last year. Orphan drugs represent an attractive commercial opportunity given the 7 years of marketing exclusivity conferred on them as well as regulatory advantages and potential for accelerated development. The absolute number of designated orphan drugs reached an all-time high in 2013 and we anticipate that interest in orphan drugs from the large pharmaceutical companies will remain strong. We intend to seek orphan drug status for NNZ-2566 in Rett Syndrome, subject to the outcomes of the current clinical trial. We also believe that some of our NNZ-2566 development programs may be potential candidates for Breakthrough Therapy designation, which would provide more intensive FDA guidance on an efficient development program.

Thirdly, we continue to add to the number of patents that protect the Neuren drug technology. During 2013 the US Patent and Trademark Office issued three new patents covering NNZ-2566, NNZ-2591 and their related analogues. Furthermore, Neuren made a formal application to the World Health Organisation for a unique International Non-proprietary Name (INN) for NNZ-2566. If granted, it is intended that this INN will replace the current NNZ-2566 designation.

Fourthly, we are working closely with a number of parties to refine the physical and technical attributes of NNZ-2566, as well as optimise the manufacturing process and projected unit costs for commercial supply of drug product. I am pleased to report that we have already identified significant opportunity in this area.

Whilst the focus of our investment and execution is quite rightly on NNZ-2566, we are also seeking to advance the commercial potential of Neuren's lead cyclic dipeptide, NNZ-2591, which is our second drug candidate, providing both additional opportunity as well as risk mitigation. Our strategy with NNZ-2591 is to enhance our understanding of its unique mechanism of action, its potency and efficacy relative to NNZ-2566 and to seek further evidence of the commercial potential of NNZ-2591 in select neurological conditions.

Under a Cooperative R & D Agreement with the Walter Reed Army Institute of Research, we are evaluating the pharmacology and preclinical efficacy of NNZ-2591 in the same Traumatic Brain Injury model that was used to elucidate the effects of NNZ-2566. In addition to studying cellular and molecular effects, the US Army is characterizing the impact of the drug on the restoration of synaptic function, which was a key finding in the study of NNZ-2591 in the Fragile X Syndrome model. Initial results from the ongoing studies indicate that NNZ-2591 has a statistically significant effect on biomarkers of synaptic signalling as well as on the re-growth of the connections that form synapses. Additionally, we have observed statistically significant reduction in brain injury biomarkers and inhibition of inflammatory cytokines. These results reinforce those of previous studies and suggest that NNZ-2591 offers promise both for acute treatment of brain injury and for supporting recovery. Beneficial effects on synaptic plasticity could also potentially prove to be an important element of long-term treatment for many chronic neurological diseases and conditions.

Building on this primary research, Neuren has recently commenced testing of NNZ-2591 in a mouse model of multiple sclerosis (MS) known as the Experimental Autoimmune Encephalitis (EAE) model, which is the most widely accepted model of MS. We are testing NNZ-2591 in a model of relapsing – remitting MS, which represents approximately 85% of cases. MS is an autoimmune disease in which the patient's antibodies attack the myelin sheath surrounding nerve fibres, interfering with transmission of nerve signals. MS is also characterized by high levels of inflammatory cytokines and abnormal functioning of microglia, which are phenomena that NNZ-2591 appears to

address. There are between 2 and 2.5 million cases of MS worldwide and, while there are approved medicines available, there remains considerable unmet need for a safe drug that is more effective in treating the disease, possibly in combination with current therapies, and is potentially able to treat ongoing cognitive impairment as well. MS is a priority target for a number of big pharma companies. Results of this ongoing study are expected in the second half of 2014.

Financial Position

Neuren's consolidated loss after tax for the year ended 31 December 2013 was NZ\$12.3 million, or NZ\$9.1 million before a non-cash impairment charge of NZ\$3.2 million that resulted from our decision not to invest further in the development of Motiva (nefiracetam).

Further ensuring that we retain our focus on the development of NNZ-2566 and NNZ-2591, Neuren and its joint venture partner Breast Cancer Research Trust are currently exploring strategic options for the anti-cancer programs conducted by Neuren's subsidiary, Perseis Therapeutics. We expect to provide an outcome from this review by mid-2014.

In order to better reflect Neuren's business environment and financial risks, our reporting currency was changed from New Zealand dollars to Australian dollars effective 1 January 2014. Neuren's cash reserves at 31 March 2014 were A\$23.4 million, which is sufficient to fund the four phase 2 clinical trials of NNZ-2566 through to completion.

There are presently 1.54 billion shares on issue and 172 million share options remain outstanding, at an average weighted exercise price of approximately 2 cents per share. 124 million of these share options are held by Neuren's leadership team and Neuren's largest shareholder.

NNZ-2566 Clinical Program Update

Rett Syndrome

Rett Syndrome is a neurological disorder that occurs almost exclusively in females following apparently normal development for the first six months of life. Typically, between 6 to 18 months of age, patients experience a period of rapid decline with loss of purposeful hand use and spoken communication. Many patients have recurrent seizures. They experience a variety of motor problems including increased muscle tone (spasticity) and abnormal movements. These individuals are never able to provide fully for their own needs, with most requiring life-long medical care and 24 hour a day supportive care as they grow older. In addition to direct costs for medical and related services, costs for institutional and special education services as well as the financial and emotional impact on families are very large. Rett Syndrome is caused by mutations on the X chromosome on a gene called *MECP2*. Rett Syndrome strikes all racial and ethnic groups and occurs worldwide in approximately 1 in every 10,000 live female births. Currently, there are no medicines approved or in late stage development for the treatment of Rett Syndrome.

In April 2013, Neuren initiated a Phase 2 double-blind, placebo-controlled clinical trial of NNZ-2566 in Rett Syndrome. Two dose levels of orally administered NNZ-2566 are being tested in female subjects aged 16 to 45 years.

As at 28 April 49 subjects have been enrolled at three study sites and 36 subjects have completed the entire study. To date there have been three meetings of the independent Drug Safety Monitoring Committee (DSMC) and no safety concerns have been identified.

The study duration for each subject is ten weeks, including the screening and follow-up periods. We are aiming to enrol approximately 54 subjects and expect the last subject to be enrolled by June 2014, enabling top-line results to be made available in Q4 2014. As well as the primary endpoint of safety and

tolerability, a number of different measures will be analysed for signs of clinical efficacy.

Fragile X Syndrome

Fragile X Syndrome is the most common inherited cause of intellectual disability and the most common known cause of autism. It affects 1 out of 4000 males and 1 out of 6-8000 females. Fragile X Syndrome is due to a gene mutation on the X chromosome that impacts the FMRP protein, which is responsible for regulating the synapses of nerve cells. Clinically, Fragile X Syndrome is characterized by intellectual handicap, hyperactivity and attentional problems, autistic symptoms, anxiety, emotional lability and epilepsy. Generally, males are more severely affected than females. Currently, there are no medicines approved for the treatment of Fragile X Syndrome.

Neuren's Phase 2 double-blind, placebo-controlled clinical trial of NNZ-2566 in males aged 16 to 40 years commenced in January 2014. The trial is designed to assess the safety and efficacy of two dose levels of oral NNZ-2566 administered for 28 days. The trial is intended to enrol at least 60 subjects at 6 sites across the US.

We aim to complete enrolment by the end of 2014, with top-line results available in the first half of 2015.

INTREPID²⁵⁶⁶ (moderate to severe Traumatic Brain Injury "TBI")

As at 28 April 2014, 145 subjects had been enrolled in the INTREPID Phase 2 clinical trial using the intravenous dosage form of NNZ-2566.

Two factors are currently assisting to accelerate the rate of subject enrolment. Firstly, we are increasing the number of US trauma centres participating in the trial and secondly, two large studies that were directly competing with INTREPID for subjects recently completed enrolment. We remain committed to completing enrolment by the end of 2014 and providing top-line results in the first half of 2015.

The primary challenge of clinical trials in TBI is the wide variability in the patient population with respect to demographics, type and location of injury, and injury severity. These variables can impact clinical outcomes and the classification schemes available to control for their effects are not as robust as we would like. This is the principal reason that the sample size of the INTREPID trial is large by Phase 2 standards and larger than our other trials. The INTREPID clinical protocol nevertheless includes three biomarkers of brain injury that are collected during the first 72 hours following injury, beginning prior to administration of the study drug or placebo. Biomarkers can serve both as a means for more effectively stratifying subjects and as a measure of drug effect. We have recently analysed biomarker data (without un-blinding the data) for the first 70 subjects in the trial to determine their correlation with mortality and functional outcome. The results of this analysis, which were presented at a recent Traumatic Brain Injury Conference, indicate that the biomarkers provide a sensitive, specific and statistically significant indicator of clinical outcome. These findings appear to justify our belief that these biomarkers will serve as a central component when we complete the INTREPID trial and analyse the un-blinded trial data for signs of clinical efficacy.

Concussion (mild Traumatic Brain Injury)

Each year, approximately 1.7 million people in the US alone sustain a traumatic brain injury (TBI). Of these, 75% are classified as mild-TBI or concussion. Increasingly it is recognised that concussion can result in long-term or permanent impairments and disabilities including Chronic Traumatic Encephalopathy. This growing awareness combined with a lack of effective drug interventions means that there is a significant unmet medical need and a large global market.

Neuren is at an advanced stage of preparation to commence its Phase 2 clinical trial of the oral dosage form of NNZ-2566 in concussion at Womack Army Medical Centre, Fort Bragg, North Carolina. This will be a world first given that there is no other commercial sponsor-led drug clinical trial in concussion.

The study is placebo-controlled, double-blind and designed to assess the safety and efficacy of two dose levels of oral NNZ-2566, administered for 7 days. Using a range of measures, the trial will assess the extent and the rate of neurological recovery in the immediate post-injury period. It is scheduled to commence by the end of June this year, complete enrolment of approximately 132 subjects in the first half of 2015 and report top-line results in the second half of 2015.

Concluding remarks

In conclusion, the company is well placed to deliver on a number of important milestones during 2014 and 2015. I re-iterate our belief that we have a unique opportunity to make a real difference in patients' lives and at the same time realise the full potential and value of Neuren's underlying assets.

I wish to thank the Neuren team for their concerted efforts, my fellow Board members for their invaluable guidance, the patients, parents and clinicians that make our clinical trials possible, and our shareholders for the support and faith that they have placed in Neuren.

Thank you.

Richard Treagus

In order to keep up to date with news about Neuren, we encourage you to register for "e-mail alerts" on our new website at www.neurenpharma.com



ANNUAL SHAREHOLDERS' MEETING

30 April 2014

Richard Treagus, Executive Chairman
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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.

2013 Achievements

▣ Strategy

- Therapeutic focus of NNZ-2566 and NNZ-2591 expanded from acute brain injury to chronic neurological conditions
- \$23 million new capital raised to fund four Phase 2 trials through to completion
- Leadership team reorganized and strengthened; corporate office moved from New Zealand to Melbourne, Australia

▣ NNZ-2566 in Rett Syndrome

- Phase 2 trial commenced in the US in April 2013 – on track to report results in H2 2014
- Fast Track designation received from the US Food and Drug Administration (FDA)

▣ NNZ-2566 in Fragile X Syndrome

- Fast Track and Orphan Drug designation received from the FDA
- NNZ-2591 shown to normalize Fragile X characteristics in a validated pre-clinical model

▣ Intellectual Property

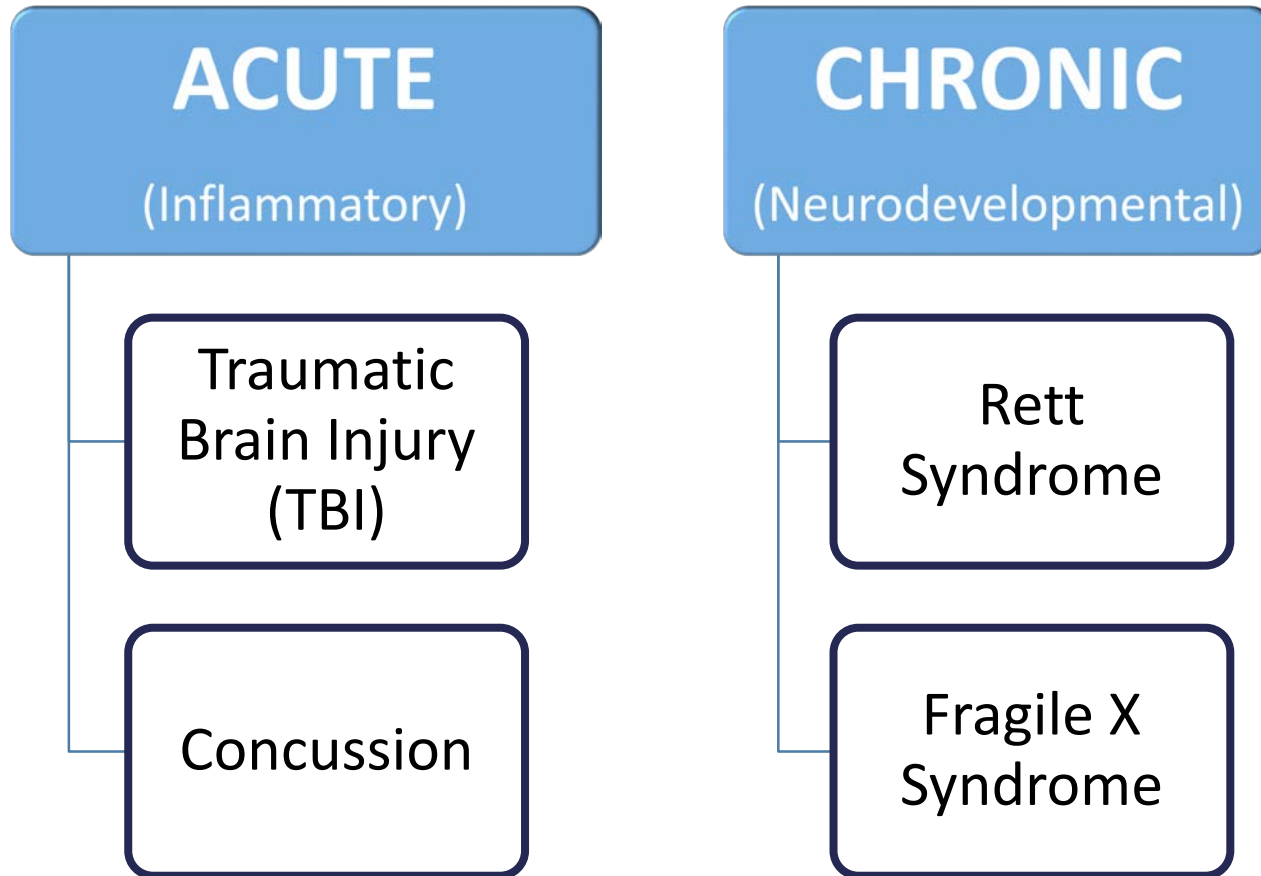
- 3 new patents issued by US Patent and Trademark Office



Strategy

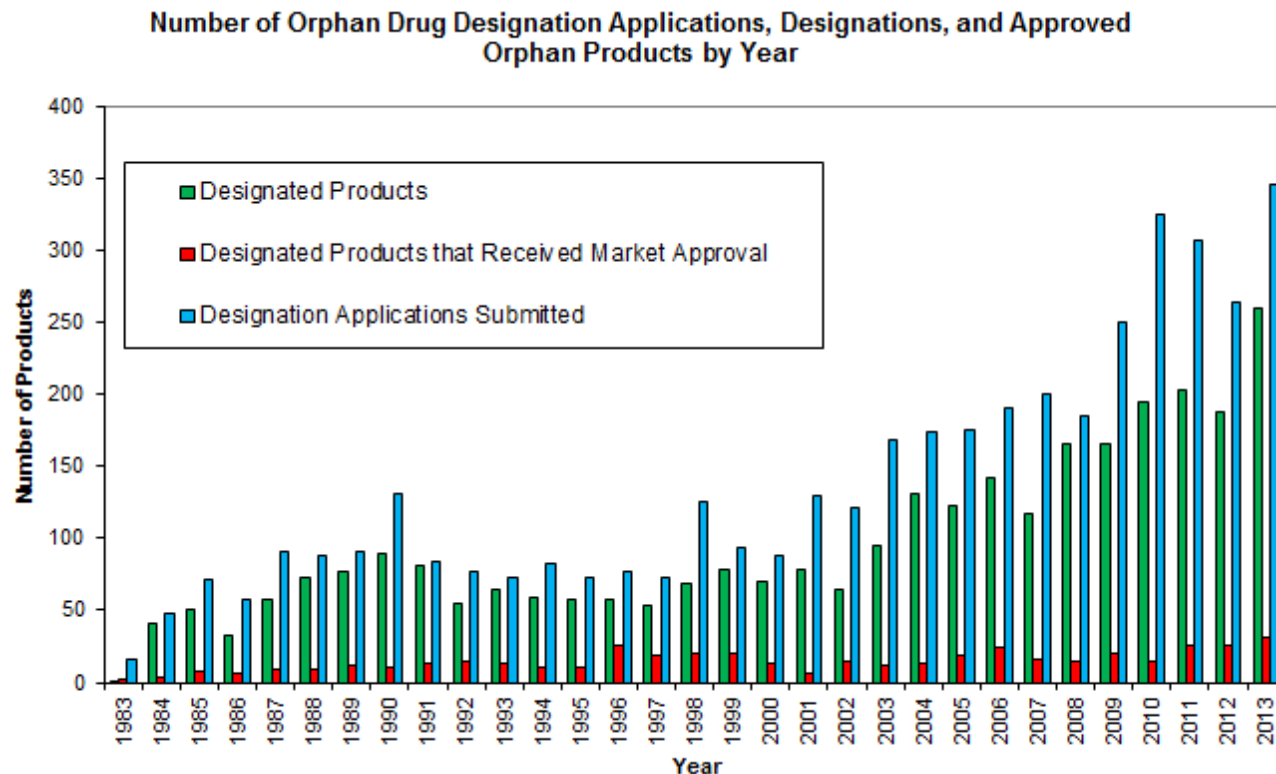
- Demonstrate the therapeutic benefit of **NNZ-2566** in human subjects in both **acute** and **chronic** conditions
- Potential to establish a “**gateway**” to autism and other neurodevelopmental disorders
- Criteria for selecting therapeutic targets
 - Significant unmet need and commercial opportunity 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

NNZ -2566 Clinical Strategy



“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 will apply for Rett Syndrome
- Pharma companies increasingly pursuing orphan drugs



Advancing commercial potential of NNZ-2591

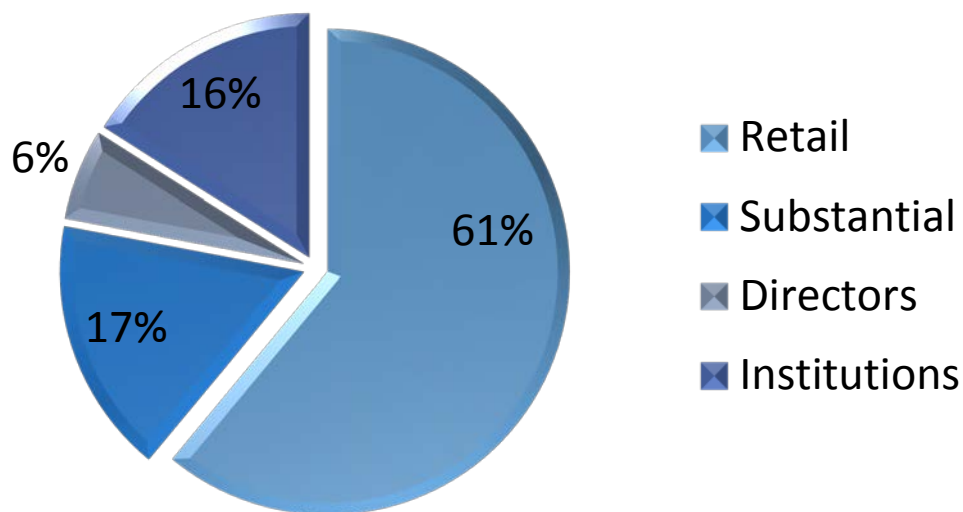
- Enhancing understanding of unique mechanism of action, potency compared with NNZ-2566 and commercial potential in neurological conditions
- Cooperative R&D agreement with US Army
 - Evaluation of pharmacology and preclinical efficacy in Traumatic Brain Injury model
 - Early results indicate increase in biomarkers of synaptic signalling, re-growth of the connections that form synapses, reduction in brain injury biomarkers and inhibition of inflammatory cytokines
- Pre-clinical testing in multiple sclerosis
 - Testing in Experimental Autoimmune Encephalitis (EAE) mouse model has commenced
 - Targeting inflammatory cytokines and abnormal functioning of microglia
 - Results expected in H2 2014

Shareholdings and Financial Position

Fully funded through to completion of Phase 2 trials in 4 different indications

- A\$23.4m cash reserves at 31 March 2014
- A\$3.7m expected from options through 2016

Shares outstanding:	1.54 billion
Options outstanding:	172 million (1.3 cents to 3.8 cents per share)
Closing price 28 April 2014	9.1 cents
52 week range:	3 cents – 14.5 cents



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
 - Extreme anxiety
 - Seizures
- Profound disability and financial burden for >50,000 patients and families globally
- Phase 2 trial in females aged 16-40 with Rett Syndrome commenced April 2013
 - Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 28 days
 - Three trial sites in the United States
 - Approximately 54 subjects expected to be enrolled
 - 49 subjects enrolled to date; 36 have completed the entire study
- “Fast Track” designation granted by the FDA



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 16-40 with Fragile X Syndrome commenced Jan 2014
 - Safety and efficacy of treatment with two dose levels of oral NNZ-2566 for 28 days
 - At least 60 subjects required to complete the trial - six trial sites in the United States
- “Fast Track” and “Orphan Drug” designation granted by the FDA

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NNZ-2566 in Traumatic Brain Injury

- ▣ > 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
- ▣ Partnership funding of ~US\$23 million by US Army
- ▣ Phase 2 trial (“Intrepid”) in moderate to severe TBI
 - “Fast Track” designation granted by the FDA
 - Safety and efficacy of treatment with intravenous NNZ-2566 for 72 hours
 - 260 subjects to be enrolled in 18 US trauma centres – 145 enrolled to date at 11 centres
 - Analysis of effect on brain injury biomarkers will provide important indicator of clinical outcome
- ▣ Phase 2 trial in Concussion to commence in H1 2014
 - 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 facility
 - A range of measures to assess the extent and rate of neurological recovery

Expected Milestones

Complete enrollment in Rett Phase 2	June 2014
Initiate Concussion Phase 2	H1-2014
Top-line results for Rett Phase 2	Q4-2014
Complete enrollment in Fragile X Phase 2	H2-2014
Complete enrollment in <i>INTREPID</i>	H2-2014
Top-line results for Fragile X Phase 2	H1-2015
Top-line results for <i>INTREPID</i>	H1-2015
Top-line results for Concussion Phase 2	H2-2015