

Neuren reports 2014 half-year financial results

Business progress since 1 January 2014:

- **NNZ-2566 product development**
 - Rett Syndrome Phase 2 trial enrolment completed – results expected in Q4 2014
 - Fragile X Syndrome Phase 2 trial commenced – results expected in Q2 2015
 - Ground-breaking Phase 2 trial in Concussion awaiting US Army administrative approval to commence enrolment – results expected in H2 2015
 - Intrepid Phase 2 trial enrolment has accelerated – results expected in H2 2015
 - US Army grant increased by US\$3 million and extended to 31 December 2015
- **NNZ-2591 product development**
 - Encouraging results in pre-clinical model of multiple sclerosis, adding to results in a broad range of neurological conditions
 - IND enabling activities initiated, commencing with manufacturing optimisation
 - New US patent granted, claiming improvement in impaired cognitive function
- **Other**
 - Leadership team strengthened further in CMC aspects of pharmaceutical development
 - Development and funding of Perseis Therapeutics Limited's anti-cancer intellectual property discontinued
- **Financials**
 - Cash reserves at 30 June 2014 – \$22.5 million (31 December 2013: \$24.4million)
 - Loss after tax – \$4.7 million (2013: \$3.1 million)
 - Net cash used in operating activities – \$2.6 million (2013: \$2.4 million)
 - Reporting currency changed to Australian dollars from 1 January 2014

Melbourne, Australia, 28 August 2014: Neuren Pharmaceuticals (ASX: NEU) today reported its financial results for the half-year to 30 June 2014 and highlighted the significant business progress made since 1 January 2014.

Neuren Executive Chairman Richard Treagus commented “Neuren now has Phase 2 clinical trials in progress in four different conditions with serious unmet need. Results from the Rett Syndrome trial will be released before the end of the year. With cash reserves of \$22.5 million and further expertise added to the management team, we are in a strong position to execute our strategy.”

Having taken steps in 2013 to reorganise the business, refine the corporate strategy and secure the necessary funding, Neuren's focus in 2014 has turned to the execution of four Phase 2 clinical trials and further manufacturing process development for NNZ-2566 and NNZ-2591.

In June 2014 Neuren completed enrolment of subjects in its Phase 2 clinical trial of NNZ-2566 in **Rett Syndrome**. Top-line results from the trial are expected to be released in the 4th quarter of 2014. The double-blind, placebo-controlled trial is the first commercial multi-site clinical trial in Rett Syndrome, for which currently there is no approved therapy available. As well as the primary endpoint of safety and tolerability, a number of different measures will be analysed for signs of clinical efficacy. Neuren's Rett Syndrome program has received Fast Track designation from the US Food and Drug Administration (FDA), which is designed to expedite the development and review of important new medicines. Neuren also intends to seek orphan drug designation from the FDA after completing the current clinical trial. Orphan drug designation is a special status that the FDA may grant to a drug to treat a rare disease or condition. Orphan drug designation qualifies the sponsor of the drug for various incentives, including seven years of marketing exclusivity following approval.

Neuren commenced a Phase 2 clinical trial of NNZ-2566 in **Fragile X Syndrome** at the Rush University Medical Center in Chicago in January 2014. Five further sites have been added since May and four more are preparing to start. A number of recent initiatives to raise awareness of the trial and ease the logistical burden on families are accelerating the rate of enrolment. The additional sites will reinforce this momentum. 17 subjects have been enrolled to date and a total of 60 subjects are targeted to complete the trial. Top-line results are expected to be announced in the second quarter of 2015. The FDA has granted orphan drug designation and Fast Track designation to NNZ-2566 for treatment of Fragile X Syndrome.

Neuren is awaiting US Army administrative sign-off to commence enrolment in a Phase 2 clinical trial of NNZ-2566 in **Concussion** (also referred to as mild traumatic brain injury) with the 82nd Airborne Division at Fort Bragg in North Carolina. The trial is a continuation of the collaboration between Neuren and the US Army on the development of potential therapies for traumatic brain injury. Approximately 132 subjects with mild traumatic brain injury will be enrolled, with top-line results from the trial expected to be available in the second half of 2015. There is currently no approved drug therapy available for acute concussion and Neuren's trial is a world-first commercially sponsored clinical trial of a potential new medicine in this therapeutic area.

In July 2014, Neuren announced that the grant supporting its collaboration with the US Army on the development of potential therapies for traumatic brain injury (TBI) had been increased by approximately US\$3 million and extended to 31 December 2015. The grant provides funding towards Neuren's costs associated with the Phase 2 clinical trial of NNZ-2566 in **moderate to severe TBI** (the "INTREPID" trial) and the Phase 2 clinical trial of NNZ-2566 in mild TBI (Concussion). In the INTREPID trial, 169 subjects have been enrolled to date, with the rate of enrolment accelerating significantly in recent months. Three additional sites have joined the trial this week. Neuren expects to be able to complete enrolment of 260 subjects in the first half of 2015 and announce top-line results from the trial in the second half of 2015.

Neuren recently announced that it had initiated activities that will support filing of an Investigational New Drug application (IND) with the FDA and subsequent clinical development of **NNZ-2591**. Encouraging results in a pre-clinical model of multiple sclerosis have now been added to positive

results in preclinical models of cognitive impairment, Fragile X syndrome, traumatic brain injury, stroke, Parkinson's disease and peripheral neuropathy. NNZ-2591 has a number of pharmacological attributes that make it an attractive candidate for further development, including excellent oral bioavailability, likely suitability for development of a solid oral dosage form and potential for improved stability. In June 2014, the US Patent and Trademark Office issued a new patent covering NNZ-2591, claiming improvement in impaired cognitive performance. The patent is expected to expire in 2031.

Neuren's leadership team has been strengthened further by the addition of Dr Clive Blower as Vice-President: Product Development and Technical Affairs, supporting the strategy to optimise the technical attributes, manufacturing process and commercial product supply of NNZ-2566 and NNZ-2591. Clive was previously directly responsible for the Chemistry Manufacturing and Controls (CMC) aspects of the development of Axiron® (marketed by Eli Lilly) through to submission of a marketing application to the FDA, as well as the establishment of commercial product supply.

Neuren confirmed today that it has discontinued all activities and funding of the anti-cancer programs conducted by its subsidiary Perseis Therapeutics. This has no impact on Neuren, other than a small non-cash impairment charge of approximately \$31,000, and it enables full management focus on NNZ-2566 and NNZ-2591.

Summary of consolidated financial results for the half-year to 30 June 2014

	Jun 2014	Jun 2013
	\$m	\$m
Grant income	1.4	2.7
Interest income	0.3	0.1
Total revenue	1.7	2.8
Research & Development	(4.4)	(3.9)
Corporate & Administration	(1.0)	(1.1)
Share based payments amortisation	(0.4)	(0.5)
Foreign exchange loss	(0.5)	(0.0)
Depreciation & amortisation	(0.1)	(0.2)
Restructuring costs	0.0	(0.2)
Loss before and after tax	(4.7)	(3.1)
Operating cash outflow	(2.6)	(2.4)
New share capital	1.1	0.5
Effect of exchange rates on cash balances	(0.4)	0.2
Cash at 30 June	22.5	3.5
Cash at 31 December		24.4

The Company's functional currency and the Group's reporting currency were changed from New Zealand dollars to Australian dollars from 1 January 2014. This change followed the transfer of the Company's corporate office from Auckland, New Zealand to Melbourne, Australia. The prior period comparative numbers have been restated in Australian dollars in order to provide meaningful comparable information.

The Group's loss after tax attributable to equity holders of the Company for the six months ended 30 June 2014 was \$4.7 million (six months ended 30 June 2013: \$3.1 million). The increased loss was mainly due to the following:

- Research and development costs increased from \$3.9 million to \$4.4 million, reflecting the addition of costs for the Rett Syndrome and Fragile X Syndrome clinical trials, offset by lower manufacturing costs for the Intrepid clinical trial;
- Grant revenue from the US Army decreased from \$2.7 million to \$1.4 million, reflecting the lower Intrepid clinical trial manufacturing costs;
- Foreign exchange losses increased by \$0.5 million, due mainly to the revaluation of US dollar cash deposits and outstanding forward contracts to purchase US dollars, as the Australian dollar strengthened against the US dollar; offset by
- Interest income increased by \$0.3 million, due to interest on Australian dollar cash deposits following the capital raising completed in October 2013.

Cash reserves at 30 June 2014 were \$22.5 million (31 December 2013: \$24.3 million). Net cash used in operating activities was \$2.6 million, offset by \$1.1 million proceeds received from the exercise of share options. The strengthening of the Australian dollar against the US dollar during the period resulted in a loss of \$0.4 million on the revaluation of US dollar cash reserves.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a publicly listed biopharmaceutical company focusing on the development of new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions such as Rett Syndrome and Fragile X Syndrome as well as acute neurological injuries. Neuren presently has a clinical stage molecule, NNZ-2566 in four Phase 2 clinical trials as well as NNZ-2591 in pre-clinical development.

Forward-looking Statements

This ASX-announcement contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks and important factors that may cause the actual results, performance or achievements of Neuren to be materially different from the statements in this announcement.

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