

## Neuren reports 2014 full-year financial results

### Business progress since 1 January 2014:

- Trofinetide proposed as International Nonproprietary Name (INN) for NNZ-2566
- Results from Phase 2 clinical trial in Rett syndrome successfully demonstrated clinical benefit from treatment with trofinetide
- Application submitted to US Food and Drug Administration (FDA) for Breakthrough Therapy designation for trofinetide in Rett syndrome
- Orphan Drug designation granted by FDA for trofinetide in Rett syndrome
- Orphan Drug applications to the European Medicines Agency underway for both Rett syndrome and Fragile X syndrome
- Fragile X syndrome Phase 2 trial commenced
- Enrolment accelerated in moderate to severe traumatic brain injury Phase 2 trial (INTREPID)
- Concussion phase 2 trial commenced
- Grant award supporting Neuren's brain injury collaboration with the US Army increased by approximately US\$3 million and extended to 31 December 2015
- Neuren leadership team strengthened in technical and manufacturing aspects of pharmaceutical development

### Financials:

- Cash reserves at 31 December 2014 – \$20.8 million (31 December 2013: \$24.4 million)
- Loss after tax – \$8.3 million (2013: \$10.4 million)
- Net cash used in operating activities – \$6.4 million (2013: \$7.1 million)
- Reporting currency changed to Australian dollars from 1 January 2014

**Melbourne, Australia, 25 February 2015:** Neuren Pharmaceuticals (ASX: NEU) today reported its financial results for the year to 31 December 2014 and highlighted the business progress made since 1 January 2014.

Neuren Executive Chairman Richard Treagus commented "We have made substantial progress in Neuren's development and commercialisation strategy during the last year. Neuren now has Orphan Drug designation in both Rett syndrome and Fragile X syndrome and the successful results from the Rett syndrome clinical trial, the first Phase 2 trial of trofinetide, exceeded our expectations. 2015 will see further critical milestones as we agree with the FDA the remaining development in Rett syndrome and complete the Phase 2 trials in Fragile X syndrome, traumatic brain injury and concussion."

In January 2015, the World Health Organization's latest publication of proposed International Nonproprietary Names for Pharmaceutical Substances (INN), trofinetide was included as the proposed INN for glycyl-2-methyl-L-prolyl-L-glutamic acid, which was originally designated by Neuren as NNZ-2566. The proposal is subject to a four-month period for comment before trofinetide can be confirmed as the INN.

In November 2014, Neuren announced top-line results from its Phase 2 clinical trial in Rett syndrome, which successfully demonstrated clinical benefit from treatment with trofinetide. The benefit observed in the trial encompassed many of the core symptoms of Rett syndrome and was observed in both clinician and caregiver assessments. An application for Breakthrough Therapy was submitted to the US Food and Drug Administration (FDA) at the end of December 2014. Neuren expects to meet with the FDA in the first half of 2015 to discuss the remaining requirements for the development of trofinetide in Rett syndrome.

In February 2015, the FDA granted Orphan Drug designation to trofinetide for treatment of Rett syndrome. Orphan Drug designation is a special status that the FDA may grant a drug intended to treat a rare disease or condition. Orphan Drug designation qualifies the sponsor of the drug for 7 years of marketing exclusivity following marketing authorisation. The FDA previously granted Orphan Drug designation to Neuren for trofinetide in Fragile X syndrome in October 2013.

Neuren has commenced the process of Orphan Drug applications to the European Medicines Agency for trofinetide in both Rett syndrome and Fragile X syndrome. Orphan Drug designation in the European Union qualifies the sponsor of the drug for 10 years of marketing exclusivity following marketing authorisation.

Neuren commenced a Phase 2 clinical trial of trofinetide in Fragile X syndrome in the United States in January 2014. The number of trial sites will increase significantly during 2015 to accelerate enrolment. Top-line results are expected in the second half of 2015.

In September 2014, Neuren commenced a Phase 2 clinical trial of trofinetide in Concussion (also referred to as mild traumatic brain injury) with the US Army's 82nd Airborne Division. The trial is a continuation of the collaboration between Neuren and the US Army on the development of potential therapies for traumatic brain injury (TBI). The trial will be expanded in 2015 to include civilian trial sites. Top-line results are expected in the second half of 2015.

In July 2014, Neuren announced that the grant supporting its collaboration with the US Army 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 trials of trofinetide in moderate to severe TBI (the "INTREPID" trial) and mild TBI (Concussion). In the INTREPID trial, the rate of enrolment accelerated significantly during 2014 as additional sites joined the trial and trials competing for subjects completed enrolment. Neuren expects top-line results from the trial in the second half of 2015.

Neuren's leadership team was strengthened further in August 2014 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 trofinetide as it progresses towards the final stages of development.

### Summary of consolidated financial results for the year to 31 December 2014

	<b>2014</b>	<b>2013</b>
	<b>\$m</b>	<b>\$m</b>
Grant income	2.9	4.8
Interest income	0.6	0.2
Foreign exchange gain	0.9	-
<b>Total revenue</b>	<b>4.4</b>	<b>5.0</b>
Research & Development	(10.0)	(8.6)
Corporate & Administration	(1.6)	(1.7)
Share based payments amortisation	(0.9)	(0.6)
Foreign exchange loss	-	(1.4)
Depreciation & amortisation	(0.1)	(0.4)
Impairment loss	(0.1)	(2.7)
<b>Loss before and after tax</b>	<b>(8.3)</b>	<b>(10.4)</b>
Operating cash outflow	(6.4)	(7.1)
New share capital	2.2	26.2
Effect of exchange rates on cash balances	0.7	0.2
<b>Cash at 31 December</b>	<b>20.8</b>	<b>24.4</b>

Neuren's reporting currency was changed from New Zealand dollars to Australian dollars from 1 January 2014. This change followed the transfer of the Company's business 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 consolidated loss after tax was \$8.3 million (2013: \$10.4 million). The loss decreased by \$2.1 million, mainly due to the following:

- An increase of \$1.4 million in research and development costs, with higher costs for the Rett syndrome and Fragile X syndrome clinical trials partly offset by lower costs for the Intrepid clinical trial; and
- A decrease of \$1.9 million in grant revenue from the US government, reflecting the lower costs for the Intrepid trial; offset by:
- An increase of \$0.4 million in interest income due to higher cash balances following the share placement in October 2013;
- Foreign exchange gains of \$0.9 million (2013: loss of \$1.4 million), mainly due to an increase in value of US dollar denominated cash balances, following the strengthening of the US dollar against the Australian dollar; and
- A non-cash impairment loss in 2013 of \$2.7 million following a review of the carrying value of the acquired intellectual property related to Motiva™.

Cash reserves at 31 December 2014 were \$20.8 million (2013: \$24.4 million). Net cash used in operating activities decreased from \$7.1 million to \$6.4 million, due to a reduction in payments for staff and directors. The exercise of share options provided net cash from financing activities of \$2.2 million. In 2013, net cash provided from financing activities was \$26.2 million, comprising \$3.6 million from the exercise of share options and \$23.5 million from a share placement and share purchase plan, less issue expenses of \$0.9 million.

## **About Neuren**

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. The novel drugs target chronic conditions as well as acute neurological injuries. Neuren presently has a clinical stage molecule, trofinetide, in 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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