Neuren reports 2015 full-year financial results

Business progress since 1 January 2015:

- **US Food and Drug Administration (FDA) granted Orphan Drug designation for trofinetide in Rett syndrome**
- **European Medicines Agency (EMA) granted Orphan Drug designation for trofinetide in both Rett syndrome and Fragile X syndrome**
- **Rettsyndrome.org committed funding of up to US$1 million towards the cost of Neuren's pediatric Phase 2 trial**
- **New patent granted in the US covering the use of trofinetide to treat Rett syndrome**
- **Enrolment of subjects completed in the Phase 2 trial of trofinetide in moderate to severe traumatic brain injury; top-line results expected in April 2016**
- **New capital of $6.3 million raised in share placement**
- **New patent granted in Europe covering the composition of NNZ-2591**
- **Top-line results from the Fragile X syndrome Phase 2 trial established proof of concept and provided a strong rationale to move forward with developing trofinetide for Fragile X syndrome**
- **Significant investments made in trofinetide manufacturing processes**
- **Leading US healthcare investment bank Leerink Partners appointed to advise Neuren's board**

Financials:

- **Cash reserves at 31 December 2015 – $16.6 million (31 December 2014: $20.8 million)**
- **Loss after tax – $13.4 million (2014: $8.3 million)**
- **Net cash used in operating activities – $12.7 million (2014: $7.1 million)**

Melbourne, Australia, 25 February 2016: Neuren Pharmaceuticals (ASX: NEU) today reported its financial results for the year to 31 December 2015 and highlighted the business progress made since 1 January 2015.

Neuren Executive Chairman Richard Treagus commented "Neuren's progress in 2015 has further underscored the value of trofinetide as a potential treatment for neurodevelopmental disorders. We are advancing development for both Rett syndrome and Fragile X syndrome with promising Phase 2 trial data, Orphan Drug designation in the US and EU, and the strong support of the Rett and Fragile X clinical experts and patient communities. In the coming months we look forward to assessing the role of trofinetide in traumatic brain injury with our partners in the US Army."

Summary of business progress

In February 2015, the US Food and Drug Administration (FDA) granted Orphan Drug designation to Neuren's drug trofinetide for treatment of Rett syndrome. Orphan Drug designation qualifies the sponsor of the drug for 7 years of marketing exclusivity following marketing authorisation. The FDA had previously granted Orphan Drug designation to Neuren for trofinetide in Fragile X syndrome.

In July and August 2015, the European Commission adopted the decision by the European Medicines Agency (EMA) to grant Orphan Designation to trofinetide for both Rett syndrome and Fragile X syndrome in the European Union (EU). Orphan designation in the EU enables sponsors to benefit from 10 years of market exclusivity once the medicine is on the market. During that exclusivity period, the EMA and the EU Member States shall not accept another application for a marketing authorisation, for the same therapeutic indication, in respect of a similar medicinal product.

In October 2015, the International Rett Syndrome Foundation (Rettsyndrome.org) made a financial commitment of up to US$1 million, to continue its support of Neuren's clinical trials of trofinetide for the treatment of Rett syndrome. Rettsyndrome.org previously supported Neuren's Phase 2 clinical trial in adults and adolescents, which successfully demonstrated clinical improvement from treatment with trofinetide. The new commitment will provide funding towards the cost of Neuren's pediatric clinical trial in 2016, in children below the age of 16 with Rett syndrome.

In October 2015, following examination, the US Patent and Trademark Office confirmed the issue of a new patent concerning the use of trofinetide to treat Rett syndrome. The patent is expected to expire in January 2032.

In October 2015, Neuren completed enrolment of the 260 subjects required in the Phase 2 trial of trofinetide in moderate to severe traumatic brain injury (the "INTREPID^{2566}" trial). The trial involved treatment in hospital for 72 hours and follow-up assessments for 3 months after randomization. Top-line results are expected to be available in April 2016.

In November 2015, Neuren raised new capital of $6.3 million in a share placement to further strengthen the cash reserves from which Neuren will fund the development of trofinetide for Rett syndrome through into 2017.

In November 2015, the European Patent Office confirmed the grant of a new patent covering the composition of Neuren's drug candidate NNZ-2591. The new patent will expire in August 2024, with the potential to extend protection for up to 5 years through Supplementary Protection Certificates. Similar patents have previously been issued in the United States and Japan.

In December 2015, Neuren announced top-line results from its Phase 2 clinical trial in Fragile X syndrome, which successfully established proof of concept and provided a strong rationale for Neuren to move forward with developing trofinetide for Fragile X syndrome. In this initial small trial with a relatively short treatment period, trofinetide was very well tolerated, with the high dose demonstrating a consistent pattern of clinical improvement, observed in both clinician and caregiver assessments. Improvements were seen across core symptoms of Fragile X syndrome, including higher sensory tolerance, reduced anxiety, better self-regulation and more social engagement.

During the year, Neuren made significant investments in the trofinetide manufacturing processes in anticipation of pivotal clinical trials, New Drug Applications and commercial supply. This includes

optimisation and scale up of the drug substance synthesis, development of the commercial finished product presentation and manufacture of drug substance for chronic toxicity studies. These investments will benefit all indications for trofinetide.

In January 2016, Neuren engaged Leerink Partners, a leading US investment banking firm specializing in healthcare, as its sole corporate adviser to assist the board in evaluating all future options to ensure that trofinetide is developed and commercialized as quickly as possible for the benefit of all stakeholders. A number of international pharmaceutical companies have expressed interest in the trofinetide development programs following the release of the clinical trial results.

Summary of consolidated financial results for the year to 31 December 2015

	2015	2014
	$m	$m
Grant income	1.7	2.9
Interest income	0.3	0.6
Foreign exchange gain	1.1	0.9
Total revenue	**3.1**	**4.4**
Research & Development	(14.1)	(10.0)
Corporate & Administration	(1.9)	(1.7)
Share based payments amortisation	(1.2)	(0.9)
Impairment loss	-	(0.1)
Loss before tax	**(14.1)**	**(8.3)**
R&D Tax Incentive	0.7	-
Loss after tax	**(13.4)**	**(8.3)**
Operating cash outflow	(12.7)	(6.4)
New share capital	7.5	2.2
Effect of exchange rates on cash balances	1.0	0.7
Cash at 31 December	**16.6**	**20.8**

The consolidated loss after tax for the year ended 31 December 2015 was $13.4 million. The loss increased by $5.1 million, mainly due to the following:

- An increase of $4.1 million in research and development costs, with higher costs for completion of the Fragile X syndrome clinical trial, drug supply for trials and manufacturing scale-up, partly offset by the completion of the Rett syndrome clinical trial at the end of 2014;
- A decrease of $1.3 million in grant revenue from the US government as the funding reached the maximum in May 2015; and
- An increase of $0.3 million in the non-cash share based payments expense; offset by:
- Research and development tax credits refunded of $0.7 million (2014: nil).

Cash reserves at 31 December 2015 were $16.6 million (2014: $20.8 million). Operating cash outflow increased from $6.4 million to $12.7 million, mainly due to the higher development costs and lower grant receipts, partly offset by the R&D tax credits refunded. Financing provided cash of $7.5 million (2014: $2.2 million), due to the share placement proceeds of $6.3 million and options exercise proceeds of $1.2 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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