Neuren (NEU) - ASX Announcement **24 August 2017**

Neuren reports 2017 half-year results

Business progress since 1 January 2017:

- **Significant clinical benefit demonstrated in Rett syndrome Phase 2 pediatric clinical trial:**
  - **The highest dose of trofinetide achieved statistically significant clinical benefit compared with placebo for each of three syndrome-specific efficacy measures.**
  - **The improvement and separation between trofinetide and placebo continued to increase through to the time that treatment ceased, suggesting that further benefit may be achieved with longer treatment duration.**
  - **Evidence of biological activity across multiple symptom areas, indicating the potential for disease modification rather than simply addressing isolated symptoms.**
  - **Trofinetide was well tolerated and had a good safety profile in these younger subjects, with no dose-limiting effects observed.**
- **Two new patents to 2032 granted in the United States and Europe, concerning the use of trofinetide to treat Fragile X syndrome and autism spectrum disorders respectively.**
- **End of Phase 2 Type B Meeting with the FDA granted for October 2017, to discuss the remaining development for trofinetide to treat Rett syndrome, including the Phase 3 trial design.**
- **Placement of new shares completed in July 2017, raising $11.5 million at 6.2 cents per share, to enable initiation of key preparatory activities for a Rett syndrome Phase 3 trial.**

Financials:

- **Loss after tax – $3.6 million (2016: $7.4 million)**
- **Net cash used in operating activities – $3.6 million (2016: $8.4 million)**
- **Cash reserves at 30 June 2017 – $1.3 million (31 December 2016: $5.1 million)**
- **Cash proceeds of $3 million received in July 2017 from share placement**

Melbourne, Australia, 24 August 2017: Neuren Pharmaceuticals (ASX: NEU) today reported financial results for the half-year to 30 June 2017 and highlighted business progress since 1 January 2017.

Neuren Executive Chairman Richard Treagus commented "The results of our Rett syndrome Phase 2 trial were deeply encouraging and greatly increased the confidence of Neuren and the leading clinical experts with whom we are working to develop trofinetide for this serious condition, which currently has no approved treatments. Since then we have gained additional patent protection, removed funding uncertainty enabling pre-Phase 3 preparations to continue and we have been granted an End of Phase 2 Meeting with the FDA in October 2017. We are looking forward to discussing with the FDA our Phase 3 plans, after which we will carefully consider commercialisation options."

In March 2017, Neuren reported top-line results for its double-blind, randomized, placebo controlled Phase 2 clinical trial in 82 girls with Rett syndrome, aged 5 to 15. The highest dose of trofinetide achieved statistically significant clinical benefit compared with placebo for each of three syndrome-specific efficacy measures, the Rett Syndrome Behaviour Questionnaire (p=0.042), the Clinical Global Impression of Improvement (p=0.029) and the Rett Syndrome Domain Specific Concerns (p=0.025). These measures included assessments of both clinicians and caregivers. Improvements from baseline were considered by the leading physicians to be clinically meaningful, particularly in a short duration trial. The improvement and the separation between trofinetide and placebo continued to increase through to the time that treatment ceased after 6 weeks, suggesting that further benefit may be achieved with longer treatment duration. The results provided evidence of biological activity across multiple symptom areas, indicating the potential for disease modification rather than simply addressing isolated symptoms. In addition, trofinetide was well tolerated and had a good safety profile in these younger subjects, with no dose-limiting effects observed.

In May 2017, Neuren announced the grant of two new patents in the United States (9,708,366) and Europe (2667715) concerning the use of trofinetide to treat autism spectrum disorders. Each patent will expire in January 2032. The US patent covers the use of trofinetide to treat Fragile X syndrome. A similar patent covering its use in Rett syndrome was issued to Neuren in December 2015. The European patent concerns the use of trofinetide to treat autism spectrum disorders, which include Rett syndrome and Fragile X syndrome. The new patents supplement issued composition of matter patents, which expire in 2022 with the potential to extend to 2027, and orphan drug designation for both Rett syndrome and Fragile X syndrome, which provides a market exclusivity period following marketing authorization of 7 years in the United States and 10 years in the European Union.

In July 2017, the FDA Division of Neurology Products granted Neuren's request for an End-of-Phase 2 meeting to discuss the remaining development plan for trofinetide in Rett syndrome. The FDA scheduled a face-to-face Type B meeting in October 2017, at which Neuren and its clinical experts will discuss with the FDA proposals for the remaining development, including the Phase 3 trial design.

In July 2017, Neuren completed a placement of new shares to enable initiation of key non-clinical and manufacturing activities on the critical path for commencing a Phase 3 trial in Rett syndrome. The placement raised $11.5 million at 6.2 cents per share, comprising $10 million from Lanstead Capital and $1.5 million from Rettsyndrome.org and Neuren's directors and management. Neuren received cash of $3 million in July 2017, comprising the investment from Rettsyndrome.org and Neuren's directors and management and $1.5 million from Lanstead. The remaining $8.5 million was invested in a Sharing Agreement with Lanstead, which enables Neuren to secure much of the potential upside from anticipated near term news flow. The Sharing Agreement provides that Neuren's economic interest will be determined and payable in 18 monthly settlements commencing in September 2017. Each settlement will be determined by comparing the volume weighted average price at which Neuren's shares are traded in the 20 days preceding each settlement (VWAP) with a price of 8.86 cents per share (Benchmark Price). If the VWAP exceeds the Benchmark Price, Neuren will receive more than 100 per cent of the monthly settlement due on a pro rata basis, with no upper limit. Should the VWAP be below the Benchmark Price, Neuren will receive less than 100 per cent of the monthly settlement on a pro rata basis. If the VWAP for each settlement is equal to the Benchmark Price, Neuren will receive $8.5 million in total. If the VWAP for each settlement is higher than the Benchmark Price, Neuren will receive proportionately more than $8.5 million, with no upper

limit. If the VWAP for each settlement is lower than the Benchmark Price, Neuren will receive proportionately less than $8.5 million.

Summary of consolidated financial results for the half-year to 30 June 2017

	HY 2017	HY 2016
	$m	$m
Grant income	-	0.2
Interest income	-	0.1
Total revenue	**-**	**0.3**
Research & Development	(2.1)	(6.7)
Corporate & Administration	(1.1)	(1.1)
Share based payments amortisation	(0.3)	(0.5)
Foreign exchange loss	(0.1)	(0.3)
Loss before tax	**(3.6)**	**(8.3)**
R&D Tax Incentive	-	0.9
Loss after tax	**(3.6)**	**(7.4)**
Operating cash outflow	**(3.6)**	**(8.4)**
Cash at 30 June 2017 and 31 December 2016	**1.3**	**5.1**

The loss after tax reduced to $3.5 million from $7.4 million, mainly due to the following:

- A decrease of $4.6 million in research and development costs, resulting from completion of the Rett syndrome pediatric clinical trial in March 2017, and completion of expenditure on the Fragile X syndrome clinical trial in the prior period, together with lower expenditure on manufacturing scale-up and toxicity studies;
- A decrease of $0.2 million in the non-cash share based payments expense as instruments reached the end of required vesting periods of service;
- A decrease of $0.3 million in interest and grant income due to lower cash balances and the completion in 2016 of the grant funding from rettsyndrome.org; and
- Income tax benefit of $0.9 million in the six months to 30 June 2016, being receipt of the R&D Tax Incentive in respect of 2015.

About trofinetide

Trofinetide is a synthetic analogue of a naturally occurring neurotrophic peptide derived from IGF-1, a growth factor produced by brain cells. In animal models, trofinetide exhibits a wide range of important effects including inhibiting neuroinflammation, normalizing the role of microglia, correcting deficits in synaptic function and regulating oxidative stress response. Trofinetide is being developed both in intravenous and oral formulations for a range of acute and chronic conditions. The most advanced program is for Rett syndrome, supported by rettsyndrome.org. Both the Rett syndrome and Fragile X syndrome programs have been granted Fast Track designation by the US Food and Drug Administration (FDA) and have orphan drug designation in both the United States and the European Union. Following marketing authorization, orphan drug designation provides a market exclusivity period of 7 years in the United States and 10 years in the European Union.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for brain injury, neurodevelopmental and neurodegenerative disorders. 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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