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Neuren (NEU) – ASX Announcement **29 October 2020**

Quarterly report and cash flow statement for Q3 2020

Highlights:

- **Enrolment continuing in the LAVENDER Phase 3 trial of trofinetide in Rett syndrome – results expected in H2 2021**
- **On track for commencement of NNZ-2591 Phase 2 trials:**
  - **Initiated manufacturing campaign to supply Phase 2 trials**
  - **Completed non-clinical toxicity studies to support clinical trials**
  - **Completed first stage of Phase 1 clinical trial**
  - **IND applications to FDA planned for H1 2021**
  - **Submitted 3 Orphan designation applications for Europe – decisions due in January**
- **Rett syndrome program discussed with 4 national regulatory agencies in Europe**
- **$26.5 million cash at 30 September 2020:**
  - **US partner ACADIA is fully funding the trofinetide Phase 3 program**
  - **Neuren is funded to achieve Phase 2 data for NNZ-2591 in 3 indications**
- **Discussions ongoing with potential partners for markets in Asia**
- **Evaluating potential additional indications for NNZ-2591**

Melbourne, Australia: Neuren Pharmaceuticals (ASX: NEU) today filed its quarterly activity and cash flow Report for Q3 2020.

Neuren CEO Jon Pilcher commented: "During the quarter, ACADIA confirmed that results of the trofinetide Phase 3 trial in Rett syndrome are expected in H2 2021 and Neuren achieved all the planned milestones to remain on track to start NNZ-2591 Phase 2 trials in 2021. We are also exploring commercial options for our products in Asian markets, including China, as well as commencing evaluation of more potential indications for NNZ-2591. Neuren has five treatments in clinical development for serious conditions in children with no approved medicines. The lead program is in a single Phase 3 trial, fully funded by a partner from which we may receive milestone payments of up to US$455 million, double digit percentage royalties on all sales and one third of the sale value of a Priority Review Voucher. Few ASX biotech companies can match the potential upside from Neuren's funded pipeline over the next two years."

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Commentary on Q3 events and outlook

During the quarter, Neuren's US partner ACADIA continued to enrol new subjects into the LAVENDER Phase 3 trial of trofinetide in Rett syndrome. ACADIA confirmed that results from the trial are expected in the second half of 2021.

Neuren discussed the Rett syndrome program at virtual meetings with four national regulatory agencies, receiving valuable advice in advance of the European Medicines Agency (EMA)'s centralized procedure.

Neuren achieved a number of milestones towards the commencement of Phase 2 clinical trials for NNZ-2591 in patients with each of Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome. The gateway to commencing the trials is approval by the US Food and Drug Administration (FDA) of Investigational New Drug (IND) applications. Neuren's target remains submission of the IND applications in the first half of 2021, incorporating data from manufacturing, non-clinical studies and the Phase 1 clinical trial.

The drug substance manufacturing campaign to supply the Phase 2 trials was initiated following excellent progress made in the optimisation and scale up of the manufacturing process. The first stage of the ongoing Phase 1 clinical trial in Australia has been completed. The trial is assessing safety, tolerability and pharmacokinetics in healthy volunteers to inform the safety and efficacy assessment in patients for the Phase 2 trials. The remaining non-clinical toxicity studies required to support the clinical trials have also been completed. Final reports from two non-clinical studies are pending for inclusion in the application for ethics approval to start the final stage of the Phase 1 trial. Results from the trial are expected to be available in January. In parallel, the Neuren team is actively engaging with the patient communities and key physicians across all three disorders in preparation for the Phase 2 trials.

Neuren also submitted Orphan designation applications for each of Phelan-McDermid, Angelman and Pitt Hopkins syndromes in Europe. Neuren previously received US Orphan Drug designation for all three syndromes from the FDA. Under the EMA Orphan procedure timetable, decisions are expected in January 2021.

Cash reserves at 30 September 2020 were $26.5 million, funding Neuren through to achieving Phase 2 data for NNZ-2591 in three indications, while ACADIA fully funds the trofinetide Phase 3 program. ACADIA's 10-Q financial report for the quarter ended 30 June 2020 disclosed trofinetide costs in those three months for external service providers alone of US$11.5 million.

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In addition, Neuren is exploring options with potential partners for markets in Asia. The market and regulatory environment for rare disease treatments in China is developing rapidly and there are already well-established orphan drug programs in Japan, South Korea and Taiwan. To date this potential for Neuren's products in Asia has not been factored into any valuations of the business.

Finally, potential additional indications for NNZ-2591 are under evaluation. While Phase 2 trials in the three lead indications remain the primary focus, Neuren believes that NNZ-2591 may have broader application in neurological conditions. Further updates will be provided in due course.

Commentary on Q3 cash flows

Net cash of $1.3 million was used in operating activities, including R&D Tax Incentive received of $0.5 million and R&D payments of $1.3 million, mainly comprising the NNZ-2591 non-clinical studies and Phase 1 trial. Approximately $19 million net of costs was received in July from the placement of 14.3 million shares at $1.40. The carrying value in AUD of USD cash held to eliminate exchange rate risk for USD expenditure fell by $0.3 million due to the weakening of the USD against the AUD, having previously increased by $0.4 million in H1 2020.

Listing Rule 4.7C.3

In item 6 of the Appendix 4C cash flow report for the quarter, payments to Related Parties of approximately $75,000 comprised non-executive directors' fees.

About Neuren

Neuren has two new drug therapies in clinical development for five serious neurological disorders that emerge in early childhood, none of which have any approved medicines.

The lead drug compound, trofinetide, is currently in a Phase 3 clinical trial for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. Because of the urgent unmet need, the programs have each been granted Fast Track designation by the US Food and Drug Administration (FDA) and "orphan drug" designation in both the United States and the European Union, a designation that provides incentives to encourage therapies for rare and serious diseases.

Neuren has granted an exclusive licence to ACADIA Pharmaceuticals Inc. for the development and commercialisation of trofinetide in North America, while retaining all rights outside North America.

Neuren is advancing the development of its second drug candidate NNZ-2591 for Phelan-McDermid, Angelman and Pitt Hopkins syndromes, each of which has received orphan drug designation in the

United States. Neuren has commenced a Phase 1 clinical trial of NNZ-2591 and plans to initiate Phase 2 trials in all three disorders in 2021.

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ASX Listing Rules information
This announcement was authorized to be given to the ASX by the board of directors of Neuren Pharmaceuticals Limited, Suite 201, 697 Burke Road, Camberwell, VIC 3124

Forward-looking Statements

This 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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Appendix 4C

Quarterly cash flow report for entities subject to Listing Rule 4.7B

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Name of entity

Neuren Pharmaceuticals Limited

ABN	**Quarter ended ("current quarter")**
72 111 496 130	30 September 2020

Consolidated statement of cash flows		**Current quarter $A'000**	**Year to date (9 months) $A'000**
1.	**Cash flows from operating activities**		
1.1	Receipts from customers	-	-
1.2	Payments for		
	(a) research and development	(1,341)	(5,462)
	(b) product manufacturing and operating costs	-	-
	(c) advertising and marketing	-	-
	(d) leased assets	-	-
	(e) staff costs	(352)	(1,109)
	(f) administration and corporate costs	(169)	(593)
1.3	Dividends received (see note 3)	-	-
1.4	Interest received	22	128
1.5	Interest and other costs of finance paid	-	-
1.6	Income taxes paid	-	-
1.7	Government grants and tax incentives	529	579
1.8	Other (provide details if material)	-	-
1.9	**Net cash from / (used in) operating activities**	**(1,311)**	**(6,457)**

2.	**Cash flows from investing activities**		
2.1	Payments to acquire or for:		
	(a) entities	-	-
	(b) businesses	-	-
	(c) property, plant and equipment	-	(6)
	(d) investments	-	-
	(e) intellectual property	-	-
	(f) other non-current assets	-	-

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Consolidated statement of cash flows		Current quarter $A'000	Year to date (9 months) $A'000
2.2	Proceeds from disposal of:		
	(a) entities	-	-
	(b) businesses	-	-
	(c) property, plant and equipment	-	-
	(d) investments	-	-
	(e) intellectual property	-	-
	(f) other non-current assets	-	-
2.3	Cash flows from loans to other entities	-	-
2.4	Dividends received (see note 3)	-	-
2.5	Other (provide details if material)	-	-
2.6	**Net cash from / (used in) investing activities**	**-**	**(6)**

3.	**Cash flows from financing activities**		
3.1	Proceeds from issues of equity securities (excluding convertible debt securities)	20,072	20,216
3.2	Proceeds from issue of convertible debt securities	-	-
3.3	Proceeds from exercise of options	-	-
3.4	Transaction costs related to issues of equity securities or convertible debt securities	(1,075)	(1,075)
3.5	Proceeds from borrowings	-	-
3.6	Repayment of borrowings	-	-
3.7	Transaction costs related to loans and borrowings	-	-
3.8	Dividends paid	-	-
3.9	Other (provide details if material)	-	-
3.10	**Net cash from / (used in) financing activities**	**18,997**	**19,141**

4.	**Net increase / (decrease) in cash and cash equivalents for the period**		
4.1	Cash and cash equivalents at beginning of period	9,172	13,844
4.2	Net cash from / (used in) operating activities (item 1.9 above)	(1,311)	(6,457)
4.3	Net cash from / (used in) investing activities (item 2.6 above)	-	(6)

Consolidated statement of cash flows		Current quarter $A'000	Year to date (9 months) $A'000
4.4	Net cash from / (used in) financing activities (item 3.10 above)	18,997	19,141
4.5	Effect of movement in exchange rates on cash held	(326)	10
4.6	**Cash and cash equivalents at end of period**	**26,532**	**26,532**

5.	Reconciliation of cash and cash equivalents at the end of the quarter (as shown in the consolidated statement of cash flows) to the related items in the accounts	Current quarter $A'000	Previous quarter $A'000
5.1	Bank balances	701	734
5.2	Call deposits	25,831	8,438
5.3	Bank overdrafts	-	-
5.4	Other (provide details)	-	-
5.5	**Cash and cash equivalents at end of quarter (should equal item 4.6 above)**	**26,532**	**9,172**

6.	Payments to related parties of the entity and their associates	Current quarter $A'000
6.1	Aggregate amount of payments to related parties and their associates included in item 1	75
6.2	Aggregate amount of payments to related parties and their associates included in item 2	-

Note: if any amounts are shown in items 6.1 or 6.2, your quarterly activity report must include a description of, and an explanation for, such payments.

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7.	**Financing facilities** *Note: the term "facility' includes all forms of financing arrangements available to the entity.* *Add notes as necessary for an understanding of the sources of finance available to the entity.*	**Total facility amount at quarter end $A'000**	**Amount drawn at quarter end $A'000**
7.1	Loan facilities	-	-
7.2	Credit standby arrangements	-	-
7.3	Other (please specify)	-	-
7.4	**Total financing facilities**	-	-

7.5	**Unused financing facilities available at quarter end**	-

7.6 Include in the box below a description of each facility above, including the lender, interest rate, maturity date and whether it is secured or unsecured. If any additional financing facilities have been entered into or are proposed to be entered into after quarter end, include a note providing details of those facilities as well.

8.	**Estimated cash available for future operating activities**	**$A'000**
8.1	Net cash from / (used in) operating activities (item 1.9)	(1,311)
8.2	Cash and cash equivalents at quarter end (item 4.6)	26,532
8.3	Unused finance facilities available at quarter end (item 7.5)	-
8.4	Total available funding (item 8.2 + item 8.3)	26,532
8.5	**Estimated quarters of funding available (item 8.4 divided by item 8.1)**	20

Note: if the entity has reported positive net operating cash flows in item 1.9, answer item 8.5 as "N/A". Otherwise, a figure for the estimated quarters of funding available must be included in item 8.5.

8.6 If item 8.5 is less than 2 quarters, please provide answers to the following questions:

8.6.1 Does the entity expect that it will continue to have the current level of net operating cash flows for the time being and, if not, why not?

Answer:

8.6.2 Has the entity taken any steps, or does it propose to take any steps, to raise further cash to fund its operations and, if so, what are those steps and how likely does it believe that they will be successful?

Answer:

8.6.3 Does the entity expect to be able to continue its operations and to meet its business objectives and, if so, on what basis?

Answer:

Note: where item 8.5 is less than 2 quarters, all of questions 8.6.1, 8.6.2 and 8.6.3 above must be answered.

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Compliance statement

1 This statement has been prepared in accordance with accounting standards and policies which comply with Listing Rule 19.11A.

2 This statement gives a true and fair view of the matters disclosed.

Date: 29 October 2020

Authorised by: The Board
(Name of body or officer authorising release – see note 4)

Notes

1. This quarterly cash flow report and the accompanying activity report provide a basis for informing the market about the entity's activities for the past quarter, how they have been financed and the effect this has had on its cash position. An entity that wishes to disclose additional information over and above the minimum required under the Listing Rules is encouraged to do so.

2. If this quarterly cash flow report has been prepared in accordance with Australian Accounting Standards, the definitions in, and provisions of, *AASB 107: Statement of Cash Flows* apply to this report. If this quarterly cash flow report has been prepared in accordance with other accounting standards agreed by ASX pursuant to Listing Rule 19.11A, the corresponding equivalent standard applies to this report.

3. Dividends received may be classified either as cash flows from operating activities or cash flows from investing activities, depending on the accounting policy of the entity.

4. If this report has been authorised for release to the market by your board of directors, you can insert here: "By the board". If it has been authorised for release to the market by a committee of your board of directors, you can insert here: "By the [*name of board committee – eg Audit and Risk Committee*]". If it has been authorised for release to the market by a disclosure committee, you can insert here: "By the Disclosure Committee".

5. If this report has been authorised for release to the market by your board of directors and you wish to hold yourself out as complying with recommendation 4.2 of the ASX Corporate Governance Council's *Corporate Governance Principles and Recommendations*, the board should have received a declaration from its CEO and CFO that, in their opinion, the financial records of the entity have been properly maintained, that this report complies with the appropriate accounting standards and gives a true and fair view of the cash flows of the entity, and that their opinion has been formed on the basis of a sound system of risk management and internal control which is operating effectively.

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