Neuren (NEU) - ASX Announcement **27 February 2019**

Fundamentals strong as Neuren advances neuroscience drug therapy pipeline

Business and financial highlights:

- **Cash reserves at 31 December 2018 of $23.6 million (31 December 2017: $4.7 million)**
- **Cash inflow from operations of $6.4 million (2017: outflow $5.6 million)**
- **Fundamentally strong position following the ACADIA partnership for trofinetide in North America**
  - **ACADIA committed to invest circa. US$55 million (A$77 million) in Rett syndrome Phase 3 and providing expert development and commercialization capabilities for trofinetide in the US**
  - **Neuren retains 100% of the rights to trofinetide outside North America, with free and full access to utilise the US regulatory package for registration in other territories**
  - **Received $13.5 million non-dilutive funding, enabling Neuren to advance development of NNZ-2591 for neurodevelopmental disorders - positive results in Phelan-McDermid syndrome model recently announced**
  - **Strong participation for Neuren in the commercial value of trofinetide in North America through double digit percentage royalties, milestone payments of up to US$455 million (A$640 million) and one third of the market value of any Rare Pediatric Disease Priority Review Voucher**
- **Torreya appointed to advise on rest-of-world partnerships and potential corporate transactions**
- **Profit after tax of $3.1 million (2017: $3.3 million)**
- **$10.3 million received from Lanstead to 31 December 2018, with 6 settlements still remaining.**

Melbourne, Australia, 27 February 2019: Neuren Pharmaceuticals (ASX: NEU) today reported its financial results for 2018. During the year Neuren transitioned into a fundamentally stronger position due to the licence agreement with ACADIA Pharmaceuticals for trofinetide in North America, which was executed in August 2018. Cash reserves at 31 December 2018 were $23.6 million, compared with $4.7 million at the start of the year. Cash flow of $6.4 million was generated from operations, compared with an outflow of $5.6 million in 2017.

The partnership with ACADIA has provided five key financial benefits to Neuren:

- ACADIA is investing circa. US$55 million (A$77 million at the current exchange rate) into the Rett syndrome Phase 3 program. This would otherwise have been a minimum capital raise requirement for Neuren in order to continue development for Rett syndrome.
- Receipt of the first payment from ACADIA of $13.5 million provided non-dilutive funding necessary for Neuren to retain ownership and advance the development of NNZ-2591 as a therapy for neurodevelopmental disorders, which is now a major focus for the Company. These disorders

include Phelan-McDermid syndrome (PMS), for which Neuren recently announced positive effects in a pre-clinical model. The program of standard non-clinical safety studies required before filing an Investigational New Drug application (IND) with the FDA and commencing clinical trials are in progress.

- ACADIA provides expert execution capabilities in the US for Phase 3 and commercialization of trofinetide. The Neuren and ACADIA teams are collaborating very effectively on the development of trofinetide for North America, including the ongoing preparations for the Rett syndrome Phase 3 trial, due to commence in the second half of 2019.
- Neuren secured strong participation in the future value of trofinetide in the US through double digit percentage royalties on all sales, plus payments of up to US$455 million ($640 million at the current exchange rate) on achievement of development and annual sales milestones, as well as one third of the market value of any Rare Pediatric Disease Priority Review Voucher, if awarded by the US Food and Drug Administration upon approval of a New Drug Application for trofinetide.
- Neuren has retained 100% of the rights to trofinetide outside North America, with free and full access to utilise the US regulatory package for registration in other territories. Anticipating discussions with interested parties, Neuren recently appointed Torreya, a global investment bank specialising in life sciences, as its corporate advisor. Torreya is working closely with Neuren management and will assist the board in considering all potential corporate transactions including individual products, territories, or Neuren's entire business.

Neuren anticipates that a redacted version of the licence agreement with ACADIA will be filed with the US Securities and Exchange Commission as a material contract exhibit to ACADIA's Annual Report on Form 10-K, which will be available to view via the SEC Filings section of ACADIA's website

Financials summary:

	2018	**2017**
	$m	**$m**
Revenue from ACADIA agreement	13.5	-
R&D Tax Incentive	0.4	0.6
Interest income	0.3	0.1
Foreign exchange gain	1.0	-
Gain in fair value of Lanstead settlements	-	9.5
Total income	**15.2**	**10.2**
Research & Development	(6.1)	(5.1)
Corporate & Administration	(2.1)	(1.6)
Foreign exchange loss	-	(0.2)
Loss in fair value of Lanstead settlements	(3.9)	-
Profit after tax	**3.1**	**3.3**
Cash flow from operations	6.4	(5.6)
Cash flow from financing	11.7	5.3
Effect of exchange rates on cash balances	0.7	(0.1)
Cash at 31 December	**23.6**	**4.7**
Profit / (loss) after tax excluding change in fair vaue of Lanstead settlements	**7.0**	**(6.2)**

In July 2017 Neuren entered into a funding arrangement with Lanstead Capital which provided new capital of $10 million as part of a raising at $1.24 per share. Neuren received $1.5 million from Lanstead at the close of the capital raise, with the remaining $8.5 million to be received in a series of 18 settlements, each of which is calculated by reference to the volume weighted average price at which Neuren's shares are traded in the previous 20 days. This means that the total amount received by Neuren could be more or less than $10 million, depending upon the share price. At 31 December 2018, Neuren had already received $10.3 million from Lanstead with 6 settlements still to be received in the first half of 2019.

About Neuren

Neuren Pharmaceuticals Limited (Neuren) is a biopharmaceutical company developing new therapies for neurodevelopmental and neurodegenerative disorders and brain injury. Neuren has completed Phase 2 development of its lead drug candidate trofinetide for Rett syndrome and has completed a Phase 2 clinical trial in Fragile X syndrome. The programs in Rett syndrome and Fragile X syndrome have each been granted Fast Track designation by the US Food and Drug Administration and Orphan Drug designation in both the United States and the European Union. Neuren has granted an exclusive license to ACADIA Pharmaceuticals Inc. for the development and commercialization of trofinetide in North America, whilst retaining all rights to trofinetide outside North America. Neuren is advancing the development of its second drug candidate NNZ-2591 for neurodevelopmental disorders.

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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.