

## Positive FDA discussions support Phase 2 timetable

NEU has reported positive meetings with the FDA to discuss its planned IND<sup>1</sup> submissions for Phase 2 clinical trials of its drug, NNZ-2591. NEU will incorporate the feedback and lodge its IND applications in three targeted conditions: Phelan Mc Dermid, Angelman and Pitt Hopkins syndromes. The IND submission triggers a 30 day review period for the FDA to raise any queries. In the absence of queries, NEU aims to commence the Phase 2 trials in H2CY21, with top-line results planned for H2CY22.

Each trial will include 10 to 20 paediatric patients for treatment over 13 weeks. The trials will take place in Australia (Angelman) and the US (Phelan-McDermid and Pitt Hopkin). The primary objective is to confirm safety and tolerability of dosing. It will also assess efficacy through the Clinical Global Impression-Improvement (CGI-I) scale and other measures.

### CY21/22 Transformative years

The news sees NEU on track to deliver its next planned milestone for CY21 - submission of the IND for Phase 2 trials for NNZ-2591. CY21 & 22 are set to be transformative years for NEU with a list of potential major milestones including:

- CY21 Positive Phase 3 results of trofinetide in Rett Syndrome
- CY21/22 ex-NAM licensing agreements/ payments for trofinetide
- CY22 FDA approval of trofinetide in Rett syndrome
- CY22 ACAD's program for trofinetide in Fragile X Syndrome
- CY22 Phase II results for NNZ-2591 in its three conditions
- CY22 NNZ-2591 licensing agreement on positive Phase 2 results

### Financials

By MST estimates, with A\$22.6m cash at end Q1FY21, NEU is funded to the CY22 Phase 2 trials readout of NNZ-2591. NEU will seek to license NNZ-2591's ongoing development. Under these assumptions, no further capital will be required to develop trofinetide and NNZ-2591 in its announced targets. In addition, MST estimates licensing revenues of US\$100m over CY21-23 on news of positive Phase 3 trofinetide trial results and FDA approval, a licensing agreement for trofinetide's ex-NAM rights and a licensing agreement on positive NNZ-2591 Phase 2 results.

### Valuation, Risks, Sensitivities

MST's risk adjusted DCF valuation of \$3.93ps compares to a share price of \$1.25. It is based on a probability of 60% for approval of trofinetide and 25% for NNZ-2591. The valuation is subject to the usual risks of drug development as noted in the following valuation summary.

For further NEU Research Reports please visit: [www.mstaccess.com.au](http://www.mstaccess.com.au)

<sup>1</sup> Investigational New Drug



Neuren Pharmaceuticals is an ASX listed biotechnology company developing drugs for debilitating neurodevelopmental disorders. Trofinetide and NNZ-2591 are targeting six disorders for which there are no approved therapies. Trofinetide Phase III trial results in Rett Syndrome are expected in late CY21 with NNZ-2591 to enter Phase 2 trials in H2CY21.

Board and management are well credentialed with in-depth experience in drug development and commercialisation.

#### Company data

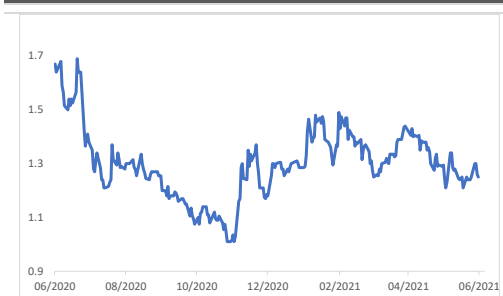
|                       |                 |
|-----------------------|-----------------|
| Stock                 | ASX: NEU        |
| Primary Exchange      | ASX             |
| Price                 | A\$1.25         |
| Market cap            | A\$147m         |
| Valuation (per share) | A\$3.93 diluted |

|                     |          |
|---------------------|----------|
| Net cash (31/03/21) | A\$22.6m |
| Shares on issue     | 114.6m   |
| Options/Rights      | 3m       |

#### Next steps

- H1CY21 Submit IND for Phase 2 trials of NNZ-2591
- H2CY21 Commence Phase 2 trial
- Late CY21 Top line Phase III results trofinetide in Rett Syndrome

#### Share Price Performance (12 months)



## Company Snapshot

NEU is trialling its two drugs, trofinetide and NNZ 2591 in a range of neurodevelopmental conditions. Trofinetide, its most advanced program, is in late-stage Phase 3 trial in Rett Syndrome with results expected in Q4CY21. NEU has licensed the North American (NAM) rights to Acadia Pharmaceuticals (NASDAQ:ACAD). It aims to confirm a partner for ex-NAM rights on positive Phase 3 trial result read out.

NEU announced a successful Phase 1 trial of NNZ-2591 in February 2021. It plans to submit Investigational New Drug (IND) applications to the US FDA to commence Phase 2 trials in three syndromes, Pitt Hopkins, McDermid Phelan and Angelman. It has also reported strong preclinical data in Prader-Willi Syndrome. By MST forecasts, cash of A\$22.6m (31.03.21) will fund the upcoming NNZ-2591 Phase 2 trials. We expect positive results to attract a licensing partner with the agreement to include an upfront payment and funding of the ongoing development.

### Investment thesis

Positive Phase 3 trial and FDA approval to trigger stock re-rating

- In MST's view, there is a disconnect between the stage of development and market potential of NEU's assets and its current market capitalisation of A\$147m. (See NEU report Nov 5 2020). MST valuation of A\$463m is calculated on an industry-based risk adjusted DCF. Given the current trading levels of ~A\$150m, we expect a material re assessment of the stock on positive Phase 3 trial results.
- ACAD has licensed the NAM rights of trofinetide in both Rett and Fragile X syndromes. The agreement sees milestone payments of up US\$455m and sales royalties of 10%+ on FDA approval and market launch in the two conditions.
- NEU retains the rights of trofinetide for ex NAM markets. The MST model assumes licensing deal/s are confirmed on positive Phase 3 results in Rett Syndrome.

#### NNZ-2591

- Successful Phase 1 trial presents NEU with a platform to effectively take new indication targets for NNZ-2591 directly from pre-clinical studies to Phase 2 trial. Prader-Willi syndrome will be the next of these additional indications following strong efficacy data in a mouse model of the syndrome.
- NEU has also developed a proprietary process for large scale manufacturing with very high purity and yield which are expected to give NNZ-2591 a commercial advantage.

### CY21/22 Potential Value Drivers

Significant value-adding events should emerge over the next two years

- CY21 Positive Phase 3 results of trofinetide in Rett Syndrome
- CY21/22 Licensing agreements/upfront payments for trofinetide ex NAM
- CY22 FDA approval of trofinetide in Rett syndrome and market entry
- CY22 ACAD to announce plans for development of trofinetide in Fragile X Syndrome
- CY22 Release of Phase II results for NNZ-2591 in three conditions
- CY22 Licensing agreement for NNZ-2591 post positive Phase 2 trials

### Valuation and key risks, sensitivities

MST values NEU at \$463m, \$3.93 per share on a risk adjusted DCF. Key assumptions include probability of approval of 60% for trofinetide in Rett Syndrome and 25% for NNZ-2591 in its targeted conditions. It assumes ex NAM rights are licensed on positive Phase 3 results and NNZ-2591 licensed on positive Phase 2 data in CY22. MST's valuation is subject to the usual risks of drug development. They include clinical trial timing, market approval & entry, pricing, market penetration, sales royalties/ licensing payments. The COVID pandemic has resulted in clinical trial delays with abandonment of some trials. The failure to secure licensing agreements may see NEU assume the regulatory approval and commercialisation roles which is likely to see an extension of the forecast timelines, additional costs and changes to the revenue forecasts.

## Exhibit 1 – MST Forecast Financial Summary

**Neuren Pharmaceuticals**

| STATEMENT OF COMPREHENSIVE INCOME              | UNITS  | 2018A  | 2019A   | 2020A  | 2021E   | 2022E  | 2023E  |
|--|--------|--------|---------|--------|---------|--------|--------|
| <b>Revenue</b>                                 |        |        |         |        |         |        |        |
| Revenue from License                           | A\$000 | 13,544 |         |        | 36,250  | 79,750 | 72,500 |
| Australian R&D tax incentive                   | A\$000 | 446    | 495     | 500    | 1,000   | 500    | 500    |
| Gross Profit                                   | A\$000 | 13,098 | 300     | 500    | 37,250  | 80,250 | 73,000 |
| <b>Expenses</b>                                |        |        |         |        |         |        |        |
| R&D  | A\$000 | -6,101 | -9,858  | -5,000 | -15,000 |        |        |
| Administration                                 | A\$000 | -2,074 | -1,713  | -2,000 | -2,000  | -2,000 | -2,000 |
| Other  | A\$000 | -3,921 | -261    |        |         |        |        |
| Amortisation of intangibles                    | A\$000 | -72    | -72     | -72    | -72     | -72    | -72    |
| Depreciation                                   | A\$000 | -6     | -6      | -6     | -6      | -6     | -6     |
| Operating profit (loss)                        | A\$000 | 1,002  | -12,686 | -6,578 | 20,172  | 78,172 | 70,922 |
| Interest received                              | A\$000 | 218    | 389     | 192    |         | 558    | 1,805  |
| Interest Paid                                  | A\$000 |        |         |        |         |        |        |
| Net Interest Received                          | A\$000 | 218    | 389     | 192    |         | 558    | 1,805  |
| Profit (loss) before income tax                | A\$000 | 3,073  | -10,816 | -6,386 | 20,172  | 78,730 | 72,727 |
| Income tax expense                             | A\$000 |        |         |        |         |        |        |
| Profit after income tax                        | A\$000 | 3,073  | -10,816 | -6,386 | 20,172  | 78,730 | 72,727 |
| Total comprehensive profit (loss) attributable | A\$000 | 3,073  | -10,816 | -6,386 | 20,172  | 78,730 | 72,727 |
| Marginal tax rate                              | %      |        |         |        |         |        |        |
| <b>Profit after tax</b>                        | A\$000 | 3,073  | -10,816 | -6,386 | 20,172  | 78,730 | 72,727 |

| STATEMENT OF FINANCIAL POSITION  | UNITS  | 2018A   | 2019A    | 2020A    | 2021E   | 2022E   | 2023E   |
|--|--------|---------|----------|----------|---------|---------|---------|
| <b>Current Assets</b>  |        |         |          |          |         |         |         |
| Trade and other receivables  | A\$000 | 942     | 522      | 522      | 522     | 522     | 522     |
| Cash and cash equivalents  | A\$000 | 23,576  | 13,844   | 27,488   | 47,660  | 126,390 | 199,117 |
| Other (Financial assets measured at fair value through profit or loss) | A\$000 | 2,121   |          |          |         |         |         |
| <b>Total current assets</b>  | A\$000 | 26,639  | 14,396   | 28,010   | 48,182  | 126,912 | 199,639 |
| <b>Non-Current Assets</b>  |        |         |          |          |         |         |         |
| Property, plant and equipment  | A\$000 | 2       | 10       | 10       | 10      | 10      | 10      |
| Intangible Assets  | A\$000 | 1       |          |          |         |         |         |
| <b>Total non-current assets</b>  | A\$000 | 3       | 10       | 10       | 10      | 10      | 10      |
| <b>Total Assets</b>  | A\$000 | 26,639  | 14,406   | 28,020   | 48,192  | 126,922 | 199,649 |
| <b>Current Liabilities</b>   |        |         |          |          |         |         |         |
| Trade and other payables   | A\$000 | 1,973   | 559      | 559      | 559     | 559     | 559     |
| <b>Total current liabilities</b>                                       | A\$000 | 1,973   | 559      | 559      | 559     | 559     | 559     |
| <b>Non-Current Liabilities</b>   |        |         |          |          |         |         |         |
| -  |        |         |          |          |         |         |         |
| <b>Total Liabilities</b>   | A\$000 | 1,973   | 559      | 559      | 559     | 559     | 559     |
| <b>Net Assets</b>  | A\$000 | 24,669  | 12,519   | 27,461   | 47,633  | 126,363 | 199,090 |
| Minority Interest  | A\$000 |         |          |          |         |         |         |
| Net assets attributable  | A\$000 | 24,669  | 13,847   | 27,461   | 47,633  | 126,363 | 199,090 |
| Equity   | A\$000 | 126,426 | 126,426  | 146,426  | 146,426 | 146,426 | 146,426 |
| Other Reserves   | A\$000 | -8,497  | -8,503   | -8,503   | -8,503  | -8,503  | -8,503  |
| Accumulated Deficit  | A\$000 | -93,260 | -104,076 | -110,462 | -90,290 | -11,560 | 61,167  |
| <b>Total Equity</b>  | A\$000 | 24,669  | 13,847   | 27,461   | 47,633  | 126,363 | 199,090 |

| STATEMENT OF CASH FLOWS                            | UNITS  | 2018A  | 2019A   | 2020A  | 2021E   | 2022E   | 2023E   |
|--|--------|--------|---------|--------|---------|---------|---------|
| Receipts from license agreement                    | A\$000 | 13,544 |         |        | 36,250  | 79,750  | 72,500  |
| Tax paid   |        |        |         |        |         |         |         |
| Receipts from Australian R&D Tax Incentive         | A\$000 | 446    | 450     | 500    | 1,000   | 500     | 500     |
| Interest Received                                  | A\$000 | 165    | 413     | 192    |         | 558     | 1,805   |
| GST Refunded                                       | A\$000 | 95     | 102     |        |         |         |         |
| Payments for Employees and Directors               | A\$000 | -1,909 | -1,742  | -2,000 | -2,000  | -2,000  | -2,000  |
| R&D and Other Payments                             | A\$000 | -6,118 | -10,942 | -5,048 | -15,078 | -78     | -78     |
| <b>Net Cash Flow from Operating Activities</b>     | A\$000 | 6408   | -11719  | -6,356 | 20,172  | 78,730  | 72,727  |
| <b>Cash Flows from Investing Activities</b>        | A\$000 |        |         |        |         |         |         |
| <b>Net Cash Flow from Investing Activities</b>     | A\$000 |        | -12     |        |         |         |         |
| <b>Cash Flows from Financing Activities</b>        |        |        |         |        |         |         |         |
| Proceeds from Issue of Shares                      | A\$000 | 11,730 | 1,860   | 20,000 |         |         |         |
| Payments of Shares Issue Expenses                  | A\$000 | -16    |         |        |         |         |         |
| <b>Net Cash Provided from Financing Activities</b> | A\$000 | 11,714 | 1,860   | 20,000 |         |         |         |
| Net Increase/Decrease in cash                      | A\$000 | 18,122 | -9,871  | 13,644 | 20,172  | 78,730  | 72,727  |
| Cash equivalents at beginning of year              | A\$000 | 4,706  | 23,576  | 13,844 | 27,488  | 47,660  | 126,390 |
| Cash and cash equivalents at end of year           | A\$000 | 23,576 | 13,844  | 27,488 | 47,660  | 126,390 | 199,117 |

Source: Company Reports, MST Assumptions

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