FDA filing starts the clock to revenues

Acadia Pharmaceuticals (NASDAQ:ACAD), Neuren Pharmaceuticals' (NEU.AX) licensing partner for trofinetide in the North American (NAM) markets, has announced that it has filed a New Drug Application (NDA) for approval of trofinetide in Rett Syndrome patients two years and older.

The news starts the clock towards a number of important milestone payments for NEU. The Phase 3 trial has been awarded FDA Fast Track, Orphan Drug status and Rare Paediatric Disease Voucher designation. Under NEU's agreement with ACAD the potential milestones include;

- FY22 – US$10m on acceptance of the NDA by the FDA;
- FY23 – US$40m on the first commercial sale
- FY23 – ~US$33m of the sale of the Rare Paediatric Disease Voucher.

The FDA approval decision is expected within ~ six months of acceptance. On market entry, NEU is entitled to double-digit percentage royalties on net sales and sales milestones of up to US$350m for both Rett and Fragile X. ACAD also has the North American rights of trofinetide in Fragile X syndrome. NEU has completed a Phase 2 trial. The development program for Fragile X has not been announced.

NEU retains the rights to trofinetide to ex-NAM markets. NEU is expected to license the rights over FY22/23. MST assumes a US$40m upfront payment. News of FDA approval is likely to present a stronger negotiating position for NEU, presenting upside risk.

Financials, Valuation, Risks, Sensitivities

Cash of $34.1m at Q1FY22 is expected to be sufficient to fund Phase 2 trials of NEU's other drug, NNZ-2591 to readout in CY23. MST valuation of $6.84ps assumes of 30% probability of approval of NNZ-2591. The valuation is subject to the upside/downside risks and sensitivities of drug development including clinical trial patient recruitment, timing and costs, regulatory approval and market entry, pricing, market penetration and sales royalties/licensing payments. Further development of trofinetide in Fragile X syndrome is yet to be confirmed. The valuation summary on page 3 provides more detail.

For further NEU Research Reports please visit: www.mstaccess.com.au
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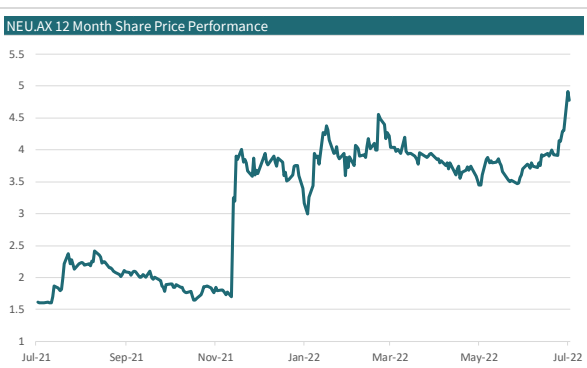
Neuren Pharmaceuticals is an ASX listed biotechnology company developing drugs for debilitating neurodevelopmental disorders. Trofinetide and NNZ-2591 are targeting six disorders for which there are no approved therapies. Positive Phase III trial results in Rett Syndrome were reported in Q4CY21. The FDA decision for approval is expected in early CY23. Trofinetide is also targeting Fragile X syndrome. NEU is to commence four Phase 2 trials of NNZ-2591 over H2CY22. The results are planned for CY23. Board and management are well credentialled with in-depth experience in drug development and commercialisation.

Company data	
Stock	ASX: NEU
Primary Exchange	ASX
Price	A$5.06
Market cap	A$652m
Valuation (per share)	A$6.84 (unchanged)
Net cash (30.03.22)	A$34.1m
Shares on issue	126m
Options/Rights	3m

Potential Milestones

- H2CY22 – Commence other NNZ-2591 Phase 2 trials
- CY22/23 – ex-NAM Rett Syndrome licensing deals
- H1CY23 - FDA approval trofinetide in Rett Syndrome
- CY23 - Phase 2 NNZ-2591 trials results

Share Price Performance (12 months)



Financial Summary

Exhibit 1 – MST Financial Summary

Neuren Pharmaceuticals Limited — **NEU-AU**

Year end 31 December

MARKET DATA

Share Price	**A$**	**5.06**
52 week high / low	A$	5.06 - 1.58
Valuation (12 month forward)	**A$**	**6.84**
Market capitalisation	**A$m**	**652**
Shares on issue	m	126
Options	m	3
Other equity	m	-
Potential shares on issue (diluted)		129

12 month performance

INVESTMENT FUNDAMENTALS		FY20	FY21	FY22E	FY23E	FY24E
EPS Reported (undiluted)	¢	(8.6)	(6.6)	1.3	77.1	54.7
EPS Underlying (undiluted)	**¢**	**(8.6)**	**(6.6)**	**1.3**	**77.1**	**54.7**
Underlying EPS growth	%	n/m	n/m	n/m	n/m	n/m
P/E Reported (undiluted)	**x**	**n/m**	**n/m**	**n/m**	**n/m**	**n/m**
P/E at Valuation	x	n/m	n/m	n/m	n/m	n/m
Dividend	**¢**	**-**	**-**	**-**	**-**	**-**
Payout ratio	%	0%	0%	0%	0%	0%
Yield	**%**	**-**	**-**	**-**	**-**	**-**

KEY RATIOS (A$)		FY20	FY21	FY22E	FY23E	FY24E
Forecast year end shares	m	118	129	129	129	129
Market cap (Y/E / Spot)	**$m**	**595.1**	**652.6**	**652.6**	**652.6**	**652.6**
Net debt /(cash)	$m	(24.2)	(36.8)	(38.5)	(137.9)	(208.4)
Enterprise value	**$m**	**570.9**	**615.8**	**614.1**	**514.7**	**444.1**
EV/Sales	x	698.8	171.3	32.3	3.1	3.8
EV/EBITDA	**x**	**(61.1)**	**(78.6)**	**106.8**	**3.5**	**4.3**
EV/EBIT	x	(61.1)	(78.6)	123.1	3.7	4.5
Net debt / Enterprise Value	x	(0.0)	(0.1)	(0.1)	(0.3)	(0.5)
Gearing (net debt / EBITDA)	**x**	**2.6**	**4.7**	**(6.7)**	**(0.9)**	**(2.0)**
Operating cash flow per share	$	(0.1)	(0.1)	0.0	0.8	0.6
Price to operating cash flow	**x**	**(73.7)**	**(65.5)**	**267.4**	**6.2**	**8.7**
Free cash flow	**$m**	**(8.1)**	**(10.0)**	**1.7**	**99.4**	**70.5**
Free cash flow per share	$	(0.07)	(0.08)	0.01	0.77	0.55
Price to free cash flow	**x**	**(73.6)**	**(65.4)**	**388.4**	**6.6**	**9.2**
Free cash flow yield	**%**	**-1.4%**	**-1.5%**	**0.3%**	**15.2%**	**10.8%**
Book value / share	$	0.21	0.30	0.30	1.07	1.62
Price to book (NAV)	**x**	**24.6**	**16.6**	**17.0**	**4.7**	**3.1**
NTA / share	$	0.21	0.30	0.30	1.07	1.62
Price to NTA	**x**	**24.6**	**16.6**	**17.0**	**4.7**	**3.1**
EBITDA margin	%	n/m	n/m	30%	89%	88%
ROE (Average Equity)	%	n/m	n/m	n/m	n/m	n/m
ROA (EBIT)	%	n/m	n/m	n/m	n/m	n/m
Interest cover (EBIT / net interest)	x	n/m	n/m	6.7	129.7	32.1

PROFIT AND LOSS (A$)		FY20	FY21	FY22E	FY23E	FY24E
Revenue & Other Income	$m	0.8	3.6	19.0	166.1	116.5
Costs	$m	(10.2)	(11.4)	(13.3)	(18.5)	(14.1)
EBITDA	**$m**	**(9.3)**	**(7.8)**	**5.8**	**147.6**	**102.4**
Depreciation & amortisation	$m	-	-	(0.8)	(6.6)	(4.7)
EBIT	$m	(9.3)	(7.8)	5.0	141.0	97.7
Net interest	$m	0.1	0.0	0.7	1.1	3.0
Pretax Profit	$m	(9.2)	(7.8)	5.7	142.0	100.8
Tax expense	$m	-	-	(0.7)	(42.6)	(30.2)
Minorities	$m	-	-	-	-	-
Underlying NPAT	**$m**	**(9.2)**	**(7.8)**	**5.0**	**99.4**	**70.5**

BALANCE SHEET (A$)		FY20	FY21	FY22E	FY23E	FY24E
Cash	$m	24.2	36.8	38.5	137.9	208.4
Receivables	$m	0.8	3.3	0.8	9.0	5.7
Inventory	$m	-	-	-	-	-
PPE	$m	0.0	0.0	0.0	0.0	0.0
Intangibles	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Total Assets	**$m**	**25.0**	**40.0**	**39.2**	**146.9**	**214.2**
Payables	$m	0.8	0.8	0.8	9.0	5.7
Borrowings	$m	-	-	-	-	-
Leases	$m	-	-	-	-	-
Provisions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Total Liabilities	**$m**	**0.8**	**0.8**	**0.8**	**9.0**	**5.7**
Shareholder's Equity	**$m**	**24.2**	**39.2**	**38.5**	**137.9**	**208.4**

CASH FLOW (A$)		FY20	FY21	FY22E	FY23E	FY24E
Receipts from customers	$m	-	-	13.3	106.9	110.7
Payments to suppliers and employees	$m	(1.4)	(2.7)	(3.3)	(5.1)	(5.2)
R&D	$m	(7.8)	(9.8)	(13.3)	(13.3)	(8.9)
Govt Grants, Rebates & Milestones	$m	0.9	2.5	5.7	59.1	5.8
Interest	$m	0.2	0.1	0.7	1.1	3.0
Tax	$m	-	-	(0.7)	(42.6)	(30.2)
Operating cash flow	**$m**	**(8.1)**	**(10.0)**	**2.4**	**106.1**	**75.2**
Capex	$m	(0.0)	(0.0)	(0.8)	(6.6)	(4.7)
Acquisitions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Investing cash flow	**$m**	**(0.0)**	**(0.0)**	**(0.8)**	**(6.6)**	**(4.7)**
Borrowings	$m	-	-	-	-	-
Equity	$m	19.1	22.2	-	-	-
Dividend	$m	-	-	-	-	-
Financing cash flow	**$m**	**19.1**	**22.2**	**-**	**-**	**-**
Change in Cash / FX	$m	11.1	12.2	1.7	99.4	70.5
Year end cash	**$m**	**24.2**	**36.8**	**38.5**	**137.9**	**208.4**

Source: Company Reports, MST Assumptions

Valuation, Key Risks and Sensitivities

Our valuation of $6.84 per share is based on a 12-month forward risk-adjusted DCF. MST's valuation is subject to the usual upside/downside risks and sensitivities of drug development, including clinical trial patient recruitment, timing and costs, regulatory approval and market entry, pricing, market penetration and sales royalties/licensing payments.

Key assumptions include that trofinetide's ex-NAM rights are licensed on/prior to FDA approval and NNZ-2591 is licensed on a positive Phase 2 data in CY23. The COVID pandemic has resulted in clinical trial delays with the abandonment of some trials. We note that trofinetide's Phase 3 trial in Rett syndrome has not been delayed despite significant COVID outbreaks during the trial. In our view, enrolment in NNZ-2591's clinical trial program will be enhanced by the widespread news of the success of the Rett Syndrome Phase 3 trials amongst the patient and medical communities. The ACAD agreement includes the rights for use of trofinetide in Fragile X. ACAD is yet to confirm further development plans for the additional indication

Requirements regarding data to support European Medicines Agency approval of trofinetide in Rett Syndrome and other jurisdictions are yet to be established. The failure of NEU to secure licensing agreements of trofinetide in Rett Syndrome in the ex-NAM markets, may see an extension of the forecast timelines, additional costs and changes to the revenue forecasts. NNZ-2591 is commencing in its Phase 2 trials. As yet there is no efficacy data in patients. The Phase 2 trials will present first data. These uncertainties bring upside/downside risk to MST forecasts.

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