First step to FDA approval met

FDA Acceptance

NEU's North American partner, Acadia Pharmaceuticals' (NASDAQ: ACAD), has announced that the FDA has accepted its New Drug Application (NDA) for approval of trofinetide in Rett Syndrome. The acceptance confirms that ACAD's submission included sufficient data to determine the drug's safety and efficacy, benefit/risk profile, that the drug label (package insert) is appropriate and meets adequate manufacturing standards. Under its agreement with ACAD, the NDA acceptance triggers a US$10m payment to NEU.

Further revenues to flow?

The FDA has awarded the NDA Priority Review status which will see a decision regarding approval in six-months (12 Mar 2023) rather than the standard 10 months. Under its agreement with ACAD, the first commercial sale triggers a S$40m milestone payment to NEU. NEU is also to receive one-third of the market value of a Rare Paediatric Disease Priority Review Voucher (PRV) if granted by the FDA. PRVs may be awarded by the FDA on approval of a drug for rare paediatric diseases. PRVs are currently valued at ~US$100m, implying ~US$33m to NEU.

CY23 Watershed Year on Track

As previously flagged, CY23 promises to be a watershed year for NEU. FDA approval and market entry will trigger further milestone payments and commencement of sale royalties. In MST's view, recent R&D disappointments in ACAD's R&D portfolio are likely to see the company focus more resources to the commercialisation of trofinetide. NEU is pursuing licensing agreements of trofinetide in ex-NAM markets and plans to announce the Phase 2 results of its other drug, NNZ-2591, over CY23. All potentially offer significant milestones and licensing revenues and valuation increments.

Valuation, Risks, Sensitivities

Our revised valuation of $8.72 per share (previously $6.84 ps) is based on a 12-month forward risk-adjusted DCF. The uplift in valuation reflects the impact of FDA acceptance with a 90% probability of approval (previously 80%) and a faster ramp up of US sales. MST notes the regulatory requirements and commercialisation approach in ex-NAM markets are yet to be confirmed. ACAD's development of trofinetide in Fragile X syndrome is also to be confirmed. As NNZ-2591 commences its Phase 2 trials, efficacy and safety data are yet to be confirmed. MST valuation assumptions may not be realised. The valuation is subject to the usual biotech upside/downside risks as outlined in the Valuation section on page 4.

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. NEU's US partner for trofinetide, Acadia Pharmaceuticals (NASDAQ: ACAD), reported positive Phase 3 trial results in Rett Syndrome in Q4CY21. It has also reported acceptance of its IND for FDA approval with a decision expected March 12 2023. NNZ-2591's Phase 2 trial program has commenced with results expected over CY23. Board and management are well credentialled.

Company data	
Stock	ASX: NEU
Primary Exchange	ASX
Price	A$6.59
Market cap	A$845m
Valuation (per share)	A$8.72 (prev. $6.84)
Net cash June CY22	A$31.1m
Shares on issue	126m
Options/Rights	3m

Potential Milestones

- H2CY22 – Commence Prader-Willi Phase 2 trial
- CY22/23 – ex-NAM Rett Syndrome licensing deals
- H1CY23 - FDA approval trofinetide in Rett Syndrome
- CY23 – Results of the four Phase 2 NNZ-2591 trials

Share Price Performance (12 months)

Exhibit 1 – MST Financial Summary

Neuren Pharmaceuticals Limited — NEU-AU

Year end 31 December

MARKET DATA

Share Price	**A$**	**6.59**
52 week high / low	A$	1.62 - 7.18
Valuation (12 month forward)	**A$**	**8.72**
Market capitalisation	**A$m**	**845**
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)	(4.6)	80.7	57.2
EPS Underlying (undiluted)	**¢**	**(8.6)**	**(6.6)**	**(4.6)**	**80.7**	**57.2**
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**	**775.0**	**849.9**	**849.9**	**849.9**	**849.9**
Net debt /(cash)	$m	(24.2)	(36.8)	(32.2)	(136.2)	(210.0)
Enterprise value	**$m**	**750.8**	**813.1**	**817.7**	**713.7**	**639.9**
EV/Sales	x	919.0	226.2	84.0	4.3	5.5
EV/EBITDA	**x**	**(80.4)**	**(103.8)**	**(176.8)**	**4.8**	**6.2**
EV/EBIT	x	(80.4)	(103.8)	(176.8)	4.8	6.2
Net debt / Enterprise Value	x	(0.0)	(0.0)	(0.0)	(0.2)	(0.3)
Gearing (net debt / EBITDA)	**x**	**2.6**	**4.7**	**7.0**	**(0.9)**	**(2.1)**
Operating cash flow per share	$	(0.1)	(0.1)	(0.0)	0.8	0.6
Price to operating cash flow	**x**	**(95.9)**	**(85.2)**	**(178.8)**	**8.2**	**11.5**
Free cash flow	**$m**	**(8.1)**	**(10.0)**	**(4.8)**	**104.0**	**73.8**
Free cash flow per share	$	(0.07)	(0.08)	(0.04)	0.81	0.57
Price to free cash flow	**x**	**(95.9)**	**(85.2)**	**(178.5)**	**8.2**	**11.5**
Free cash flow yield	**%**	**-1.0%**	**-1.2%**	**-0.6%**	**12.2%**	**8.7%**
Book value / share	$	0.21	0.30	0.25	1.06	1.63
Price to book (NAV)	**x**	**32.0**	**21.7**	**26.4**	**6.2**	**4.0**
NTA / share	$	0.21	0.30	0.25	1.06	1.63
Price to NTA	**x**	**32.0**	**21.7**	**26.4**	**6.2**	**4.0**
EBITDA margin	%	n/m	n/m	n/m	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	n/m	151.2	33.9

PROFIT AND LOSS (A$)		FY20	FY21	FY22E	FY23E	FY24E
Revenue & Other Income	$m	0.8	3.6	9.7	166.1	116.5
Expenses	$m	(10.2)	(11.4)	(14.4)	(18.5)	(14.1)
EBITDA	**$m**	**(9.3)**	**(7.8)**	**(4.6)**	**147.6**	**102.4**
Depreciation & amortisation	$m	-	-	-	-	-
EBIT	$m	(9.3)	(7.8)	(4.6)	147.6	102.4
Net interest	$m	0.1	0.0	0.4	1.0	3.0
Pretax Profit	$m	(9.2)	(7.8)	(4.3)	148.6	105.4
Tax expense	$m	-	-	(0.5)	(44.6)	(31.6)
Minorities	$m	-	-	-	-	-
Underlying NPAT	**$m**	**(9.2)**	**(7.8)**	**(4.7)**	**104.0**	**73.8**

BALANCE SHEET (A$)		FY20	FY21	FY22E	FY23E	FY24E
Cash	$m	24.2	36.8	32.2	136.2	210.0
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**	**33.0**	**145.2**	**215.7**
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**	**32.2**	**136.2**	**210.0**

CASH FLOW (A$)		FY20	FY21	FY22E	FY23E	FY24E
Receipts from customers	$m	-	-	6.7	107.0	110.7
Payments to suppliers and employees	$m	(1.4)	(2.7)	(2.9)	(5.1)	(5.2)
R&D	$m	(7.8)	(9.8)	(11.4)	(13.3)	(8.9)
Govt Grants, Rebates & Milestones	$m	0.9	2.5	3.0	59.1	5.8
Interest	$m	0.2	0.1	0.3	1.0	3.0
Tax	$m	-	-	(0.5)	(44.6)	(31.6)
Operating cash flow	**$m**	**(8.1)**	**(10.0)**	**(4.8)**	**104.0**	**73.8**
Capex	$m	(0.0)	(0.0)	(0.0)	-	-
Acquisitions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Investing cash flow	**$m**	**(0.0)**	**(0.0)**	**(0.0)**	**-**	**-**
Borrowings	$m	-	-	-	-	-
Equity	$m	19.1	22.2	(0.0)	-	-
Dividend	$m	-	-	-	-	-
Financing cash flow	**$m**	**19.1**	**22.2**	**(0.0)**	**-**	**-**
Change in Cash / FX	$m	11.1	12.2	(4.8)	104.0	73.8
Year end cash	**$m**	**24.2**	**36.8**	**32.2**	**136.2**	**210.0**

FDA accepts NDA and awards priority review for approval

NEU's North American partner, Acadia Pharmaceuticals' (NASDAQ: ACAD) has announced that the FDA has:

- accepted its New Drug Application (NDA) for approval of trofinetide in Rett Syndrome. Under its agreement with ACAD, NEU is to receive US$10m on meeting this milestone.

- granted a priority review of the application. The designation allows for a decision for approval within 6 months rather than the standard 10 months. ACAD has announced that the FDA has assigned a Prescription Drug User Fee Act (PDUFA) date of 12 March 2023. FDA approval and market launch are expected to trigger a second milestone payment from ACAD, with the agreement allowing for a payment of US$40m on of the first commercial sale with further sales-based payments to follow.

Further advantages to flow:

- Fast Track Status and Orphan Drug Designation (ODD) have been awarded for the treatment of Rett syndrome in the U.S. ODD offers extended market exclusivity of 7.5 years in paediatric diseases in the US and in the EU, 12 years.

- Rare Paediatric Disease (RPD) designation has also been granted by the FDA. Upon FDA approval of a product with RPD designation, the FDA may award a Priority Review Voucher (PRV), which can be used to obtain priority review for a subsequent NDA application or be sold for use in other drug applications. The current PRV value is ~US$100m. Under its agreement with ACAD, if the FDA awards a PRV, NEU is eligible to receive one third of the value, currently~US$33m.

FDA approval to bring significant revenue streams for NEU

ACAD has the rights to develop and commercialise trofinetide in the North American markets for both Rett and Fragile X syndrome. The US$455m agreement potentially allows for US$105m of development milestones with US$350m of sales-based milestones and double digit, escalating tiered percentage royalties on net sales. To date, ACAD's focus has been on Rett Syndrome as the more advanced program. There has not been any announcement regarding the on-going development in Fragile X.

Potential news flow over 2022/23

- Milestone payment of US$40m is payable on the first commercial sale
- US$33m payment to NEU as its one third entitlement of an estimated US$100m sale of PRV if awarded
- Sales to trigger double digit royalties on net sales
- Rest of World licensing news flow - NEU retains the rights to the data for use in commercialisation of trofinetide in ex-NAM markets. In MST's opinion, the NDA acceptance by the FDA and RPD designation will strengthen potential licensing negotiations.

Valuation

Our revised valuation of $8.72 per share (previously $6.84ps) is based on a 12-month forward risk-adjusted DCF. The increase in valuation reflects:

- A higher probability of approval. The FDA's acceptance of the NDA brings a higher probability of approval, as the trial moves further along the development timeline and reduces risk. MST assigned an 80% probability of approval on the announcement of the successful Phase 3 trial. The average success rate for approval of drugs for neurological diseases once regulatory submission of the NDA has been approved is ~87%. MST has increased its probability of approval from 80% to 90%.

- MST also notes that ACAD has announced a number of setbacks in its in-house R&D portfolio. Its marketed drug, pimavanserin, was approved for the treatment of hallucinations and delusions associated with Parkinson's disease psychosis in 2016. ACAD has been seeking approval for its use in hallucinations and delusions associated with dementia-related psychosis (DRP). ACAD recently announced that it received its second Complete Response Letter from the FDA, stating the FDA cannot approve the NDA in its current form.

In addition, ACAD has announced that it is discontinuing the development of its drug, ACP-044, in acute and chronic pain following its Phase 2 trial in treatment of pain following bunionectomy. ACAD acquired the drug through its US$52.5m acquisition of CerSci Therapeutics in 2020. Data for another ACAD drug candidate, ACP-319, also did not support advancement to Phase 2 trials in a number of central nervous diseases

In MST's view, the R&D pipeline disappointments place a greater focus on trofinetide and is likely to see the assignment of additional ACAD resources to drive its marketing on approval. MST's revised model assumes the additional sales and marketing resources will see a faster ramp up of sales than previously assumed.

Risks and Sensitivities

The ACAD valuation is subject to the usual upside/downside risks and sensitivities around the assumptions relating to clinical trial patient recruitment, timing, costs, regulatory approval, market entry, pricing, market penetration and sales royalties/licensing payments.

Trofinetide in Rest of World/Fragile X

NEU plans to market trofinetide in EU and rest of world. Requirements regarding data to support European Medicines Agency (EMA) approval of trofinetide in Rett Syndrome and other jurisdictions are yet to be established. Further data may be required and bring costs and timing uncertainty.

The commercial arrangements of the ex-NAM markets are yet to be confirmed. Key assumptions include the licensing of trofinetide's ex-NAM rights on/or prior to FDA approval. NEU may choose to market the drugs in certain markets, resulting in different revenue cost models.

ACAD is yet to confirm the ongoing development program for trofinetide in Fragile X syndrome, presenting upside/downside risk to MST's valuation assumptions.

NNZ-2591

MST assumes that NNZ-2591 is licensed on a positive Phase 2 data in CY23. As yet, there are no efficacy data to support its effect in the clinical setting, presenting probability of approval and sales forecast challenges. In MST's view, approval of trofinetide presents upside risk to patient enrolment in NNZ-2591's clinical trial program. We believe that the widespread news of the success of the Rett Syndrome Phase 3 trials amongst the patient and medical communities is likely to drive interest.

The failure to secure licensing agreements may see NEU assume the regulatory approval and commercialisation roles. A change in commercialisation strategy may alter MST's valuation assumptions with possible extension of the forecast timelines, additional costs and upside/downside changes to the revenue and profit forecasts.

The COVID pandemic presents as another risk. It has resulted in clinical trial delays with the abandonment of some trials, noting that trofinetide's Phase 3 trial in Rett syndrome was not delayed despite significant COVID outbreaks during the trial.

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