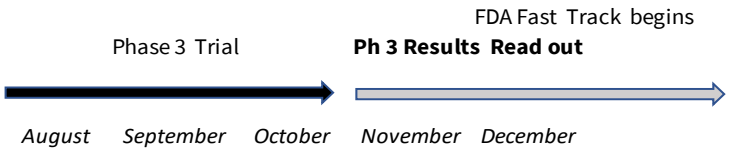
The countdown starts

Acadia Pharmaceuticals (NASDAQ:ACAD), NEU's US partner for its drug trofinetide, has announced completion of enrolment of its Phase 3 trial in Rett Syndrome. The announcement clears potential timing risk for the trial and starts the countdown to the results.



We assume completion of the trial by end October and trial readout by the CY end given the 12 week treatment period. The trial has FDA Fast 9athways to approval.

MST's valuation assumes a probability of approval of 60%. It is based on the successful Phase 2 trial which showed statistically significant results in high dose patients. In terms of market opportunity, there are no approved treatments for Rett syndrome. A potential market of ~US$4bn can be derived from ~26,000[1] Rett patients in the developed markets and the annual cost of an orphan drug of ~US$150K.[2]

Strong newsflow from key milestones

Over H2CY21 NEU plans to submit its Investigational New Drug (IND) application and commence Phase 2 trials of NNZ-2591 in three other neurodevelopmental conditions. CY21 & 22 are set to be transformative years. The key potential milestones include:

- 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 Phase II results for NNZ-2591 in its three conditions

Financials, valuation, risks, sensitivities

NEU is planning to fund NNZ-2591 Phase 2 trials to CY22 readout from its existing cash reserves (A$18.2m cash at end Q2FY21). NEU will then seek to license NNZ-2591 to fund its ongoing development. Under these assumptions, no further capital will be required to develop trofinetide and NNZ-2591 to achieve its planned targets. MST estimates licensing revenues of US$100m over CY21-23 arising from news of FDA approval of trofinetide (from ACAD), and licensing agreements for trofinetide's ex-NAM rights and NNZ-2591 on positive Phase 2 results. MST's risk adjusted DCF valuation of $3.93ps compares to a share price of $1.85. 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
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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. 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 credentialled with in-depth experience in drug development and commercialisation.

Company data	
Stock	ASX: NEU
Primary Exchange	ASX
Price	A$1.79
Market cap	A$211m
Valuation (per share)	A$3.93 diluted
Net cash (30/06/21)	A$18.2m
Shares on issue	114.6m
Options/Rights	3m

MST Access Live

Video Link - Interview with Jon Pilcher, CEO
(10 August 2021)

Next steps

- H2CY21 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)

[1] Based on incidence of 1/12,500 in the US, EU and Japan < 60 yrs old population, [2] 2020 Deloitte Global Life Sciences Outlook

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$18.25 (30.06.21) 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$220m. MST valuation of A$463m is calculated on an industry-based risk adjusted DCF. Given the current trading levels of ~A$209m, we expect a material re assessment of the stock on positive Phase 3 trial results. By peer, other drugs in Phase 3 include OPT.AX of A$439m and PAR.AX of $493m.
- ACAD has licensed the NAM rights of trofinetide in both Rett and Fragile X syndromes. The agreement sees milestone payments of up to 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

- The successful Phase 1 trial presented 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 first to transition straight to Phase 2 trial. It showed 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 market approval & entry, pricing, market penetration, sales royalties/ licensing payments and potential competitor therapies.

Exhibit 1 – MST Forecast Financial Summary

Neuren Pharmaceuticals

STATEMENT OF COMPREHENSIVE INCOME	UNITS	2018A	2019A	2020A	2021E	2022E	2023E
Revenue							
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
Expenses							
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	%						
Profit after tax	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
Current Assets							
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					
Total current assets	A$000	26,639	14,396	28,010	48,182	126,912	199,639
Non-Current Assets							
Property, plant and equipment	A$000	2	10	10	10	10	10
Intangible Assets	A$000	1					
Total non-current assets	A$000	3	10	10	10	10	10
Total Assets	A$000	26,639	14,406	28,020	48,192	126,922	199,649
Current Liabilities							
Trade and other payables	A$000	1,973	559	559	559	559	559
Total current liabilities	A$000	1,973	559	559	559	559	559
Non-Current Liabilities							
-							
Total Liabilities	A$000	1,973	559	559	559	559	559
Net Assets	A$000	24,669	12,519	27,461	47,633	126,363	199,090
Minority Interst	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
Total Equity	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 Incensitve	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
Net Cash Flow from Operating Activities	A$000	6408	-11719	-6,356	20,172	78,730	72,727
Cash Flows from Investing Activities	A$000						
Net Cash Flow from Investing Activities	A$000		-12				
Cash Flows from Financing Activities							
Proceeds from Issue of Shares	A$000	11,730	1,860	20,000			
Payments of Shares Issue Expenses	A$000	-16					
Net Cash Provided from Financing Acitivites	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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