Countdown to Phase 3 Results

Phase 3 Trial Concludes

From MST estimates, the Phase 3 Lavender Trial of Neuren's (NEU.AX) drug candidate, trofinetide, has been completed. Acadia Pharmaceuticals (NASDAQ:ACAD), NEU's licensing partner, has confirmed the results are on track for end CY21. We expect positive Phase 3 results to trigger a stock re-rating. Trofinetide in Rett Syndrome represents ~35% of our $463m valuation on a risk adjusted probability of 60%. Positive trial results would see ~US$108m added to our valuation. This compares to its current market capitalisation of A$212m.

Licensing revenue to flow: FDA approval and US market entry, will trigger milestone payments of ~A$111m over CY22 and CY23 , with double-digit royalties on net sales to follow. The FDA has awarded Fast Track status which offers an expedited review and hence potentially an earlier market entry if the relevant criteria are met.

NEU retains the rights to ex-North American (ex-NAM) markets. We expect NEU to confirm partner/s to commercialise trofinetide in these markets following news of a positive Lavender Trial outcome.

Market opportunity: There are no approved treatments for Rett Syndrome. We derive our estimate of a ~US$4bn total potential market from ~26,000[1] Rett patients in the developed markets and the average annual cost of an orphan drug of ~US$150K[2].

NNZ-2591 Phase 2 Trial on Hold

The Phase 2 trial program of NEU's other drug candidate, NNZ-2591, is on hold while NEU responds to FDA queries regarding its Investigational New Drug (IND) applications to commence trials in Angelman and Phelan-McDermid syndromes. NEU also submitted its IND for Pitt-Hopkins Syndrome on 1 October. Given the similarity of the conditions and assuming a similar trial format, we believe there are queries regarding this application as well.

Financials, Valuation, Risks, Sensitivities

End-Q3FY21 cash was $33.6m. NEU will fund NNZ-2591 Phase 2 trials to CY22 readout and, on positive results, seek a partner to continue ongoing development. Under these assumptions, no further capital will be needed to develop trofinetide and NNZ-2591 in its planned targets. MST's valuation of $3.58(dil) ps is derived from a risk adjusted DCF and carries the usual risks of drug development, as outlined on p2.

Further NEU research reports are available at mstaccess.com.au

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 3 trial results in Rett Syndrome are expected by end of CY21 with NNZ-2591 to enter Phase 2 trials in CY22.

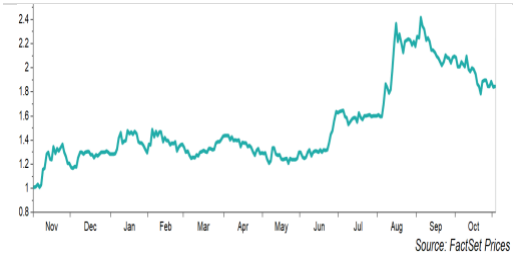
Board and management are well credentialled with in-depth experience in drug development and commercialisation.

Company data	
Stock	ASX: NEU
Primary Exchange	ASX
Price	A$1.67
Market Cap	A212m
Valuation	A$463m
Valuation ps	A$3.58 (dil)
Net cash (30/09/21)	A$33.6m
Shares on issue	126m
Options/Rights	3m

Next steps

- Q4CY21 Top line Phase 3 results for trofinetide in Rett Syndrome
- Q4CY21 Resubmission of INDs for Phase 2 trials of NNZ-2591
- H1CY22 Commence Phase 2 trials of NNZ-2591

Share price performance (12 months)



Source: FactSet Prices

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[1] Based on incidence of 1/12,500 in the US, EU and Japan population < 60 years old.

[2] 2020 Deloitte Global Life Sciences Outlook.

Phase 3 Trial Results Trigger Stock Re-Rating over Next Weeks?

NEU is trialling its two drug candidates, trofinetide and NNZ-2591, in a range of neurodevelopmental conditions. Trofinetide has completed its first Phase 3 trial with results pending. NNZ-2591 is on the cusp of commencing three Phase 2 trials. From a valuation perspective, NEU's current market capitalisation of $212m compares to MST's risk-adjusted DCF valuation of A$463m and, by peer comparison , OPT.AX and PAR.AX, $437m and $565m respectively. MST expects a material reassessment of the stock on positive Phase 3 trial results.

Phase 3 Trial Results by End-CY21

ACAD has confirmed the topline results of the Phase 3 trial are on track by end-CY21. The ACAD agreement includes ~A$111m payments over CY22/23 on FDA approval and market entry with 10%+ sales royalties to follow. MST expects NEU to confirm partner/s for ex-NAM rights following a positive trial read out.

NEU to Respond to NNZ-2591 FDA Queries

NEU has submitted Investigational New Drug (IND) applications to commence Phase 2 trials in Pitt-Hopkins, Phelan-McDermid and Angelman syndromes. The FDA has requested additional clinical assessments to be included in the Angelman trial. Given the similarity of the syndromes, we assume NEU will need to amend all three trials. MST sees the key risk as delay to the start of the trials as it re-designs the trial protocols.

Strong Cash Position to Leverage Further NNZ-2591 Potential

Cash of $33.6m at 30 September 2021 is expected to fund the planned Phase 2 NNZ-2591 program. We expect positive results to attract a licensing partner, with the agreement to include an upfront payment and funding of ongoing development. NEU is also targeting Prader-Willi Syndrome. It has reported strong preclinical data and is expected to transition straight to a Phase 2 trial.

Potential Value Drivers in CY21/22

Q4CY21: 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 with milestone payments
CY22: ACAD to announce plans for development of trofinetide in Fragile X Syndrome
CY22: Phase 2 trials of NNZ-2591 in three conditions
CY22: Licensing agreement for NNZ-2591 post positive Phase 2 trials

Valuation and Key Risks

We value NEU at $463m ($3.58 per share, fully diluted) 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. Positive Phase 3 results in Rett Syndrome would add some US$108m to the valuation. We assume ex-NAM rights for trofinetide are licensed on positive Phase 3 results and NNZ-2591 licensed on positive Phase 2 data in CY22. Our valuation includes a $36.25m payment at end-CY21. The payment assumes a ex-NAM licensing deal for trofinetide in Rett Syndrome. There is a risk that such an agreement is not reached until FY22.

Our valuation is subject to the usual upside/downside risks and assumptions regarding clinical trial timing, market approval and entry, pricing, market penetration and sales royalties/licensing payments. The COVID pandemic has resulted in clinical trial delays with some trials being abandoned.

Exhibit 1 – NEU financial summary

Neuren Pharmaceuticals
Year ending 31 December

STATEMENT OF COMPREHENSIVE INCOME	UNITS	2018A	2019A	2020A	2021E	2022E	2023E
Revenue							
Revenue from Licensing Agreements	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	120	558	1,805
Interest Paid	A$000						
Net Interest Received	A$000	218	389	192	120	558	1,805
Profit (loss) before income tax	A$000	3,073	-10,816	-6,386	20,292	78,730	72,727
Income tax expense	A$000						
Profit after income tax	A$000	3,073	-10,816	-6,386	20,292	78,730	72,727
Total Comprehensive Profit (loss) attributable	A$000	3,073	-10,816	-6,386	20,292	78,730	72,727
Marginal tax rate							
Profit after tax	A$000	3,073	-10,816	-6,386	20,292	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	67,780	146,510	219,237
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	68,302	147,032	219,759
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	68,312	147,042	219,769
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	67,753	146,483	219,210
Minority Interest	A$000						
Net assets attributable	A$000	24,669	13,847	27,461	67,753	146,483	219,210
Equity	A$000	126,426	126,426	146,426	166,426	166,426	166,426
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	-90,170	-11,440	61,287
Total Equity	A$000	24,669	13,847	27,461	67,753	146,483	219,210

STATEMENT OF CASH FLOWS	UNITS	2018A	2019A	2020E	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 Incenitve	A$000	446	450	500	1,000	500	500
Interest Received	A$000	165	413	192	120	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	20,292	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	20,000		
Payments of Share Issue Expenses	A$000	-16					
Net Cash Provided from Financing Activites	A$000	11,714	1,860	20,000			
Net Increase/Decrease in cash	A$000	18,122	-9,871	13,644	40,292	78,730	72,727
Cash equivalents at beginning of year	A$000	4,706	23,576	13,844	27,488	67,780	146,510
Cash and cash equivalents at end of year	A$000	23,576	13,844	27,488	67,780	146,510	219,237

Source: Company reports, MST estimates.

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