4C Q1FY21 in line, focus on Q4CY21 Phase 3 results

NEU's ASX Q1FY21 quarterly report was inline with our expectations. A key focus was the Phase 3 trial of trofinetide in Rett Syndrome as it approaches full enrolment and on track to announce results in Q4CY21. Trofinetide in Rett Syndrome represents ~35% of our $463m valuation on a risk adjusted probability of 60%. Positive trial results would see a 60% increase in our Rett valuation and a likely re-rating of the stock.

We look at the potential success and commercialisation parameters.

- Likelihood of Approval – To gain approval, the Phase 3 clinical trial must demonstrate safety and efficacy. The Phase 2 trial showed a clear benefit and no serious side effects. It gives confidence to the Phase 3 trial as the trials share the same endpoints - change in the baseline scores for the Rett Syndrome Behaviour Questionnaire (RSBQ) and Clinical Global Impression-Improvement (CGI-I). The Phase 2 trial reported statistically significant results for the high dose patients with RSBQ of p =0.042 and CGI-I of p= 0.029.(Statistically significant = ≤0.05). The larger group of 180 high dose patients in the Phase 3 trial versus 51 in in Phase 2 should amplify the trends seen in the Phase 2, giving greater confidence of success in the Phase 3.
- Market Opportunity - The Phase 3 trial includes patients from 5 -20 yrs of age, thereby including adult patients. The Rett patient population in developed markets is estimated at ~26,000[1]. As an orphan drug, trofinetide is likely to attract higher prices. In 2019, in the US, the average annual cost of a new orphan drug was US$150,854 versus US$33,654 for a new non-orphan drug. An average cost of US$150K, presents a 'theoretical' market value of ~US$4bn. From a competition perspective there are no approved treatments for Rett syndrome, leaving an open market.
- Orphan drug benefits –Trofinetide's FDA and EMA orphan drug status offers extended market exclusivity of up to 7.5 yrs in the US and 12 yrs in the EU. The extended market protection will be of interest to potential partners for the ex North American(NAM) rights.

Financials

By MST estimates, NEU is funded to the NNZ-2591 CY22 Phase 2 trial readout, with A$22.6m cash at end Q1FY21. Over CY21-23 milestone and licensing revenues of US$100m are assumed from; a positive trofinetide trial with subsequent FDA approval, an ex-NAM licensing agreement and a licensing agreement on positive NNZ-2591 Phase 2 results. Under these assumptions, we estimate further capital to develop the current disease indications for trofinetide and NNZ-2591 is not expected.

Valuation, Risks, Sensitivities

MST values NEU at $463m, $3.93 per share on a risk adjusted DCF. Key assumptions include positive Phase 3 trofinetide results and licensing of its exNAM rights as well as positive data from NNZ-2591 Phase 2 trial in CY22. The valuation is subject to other risks of drug development as noted in the following valuation summary.

For further NEU Research Reports please visit: www.access.com.au

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

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

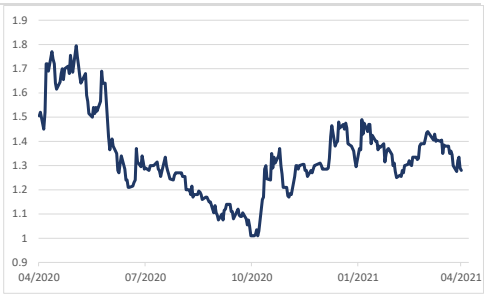
Board and management are well credentialled with in-depth experience in drug development and commercialisation.

Company data	
Stock	ASX: NEU
Price	A$1.30
Market cap	A$152m
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 Ttrofinetide 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 is seeking a partner for ex-NAM rights. NNZ-2591 reported a successful Phase 1 trial in February 2021. Neuren plans to submit an Investigational New Drug (IND) 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 forecast, cash of A$22.6m 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 of US$15m and funding of ongoing development expenditure.

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 valuation (See NEU report Nov 5 2020). MST valuation is calculated on an industry-based risk adjusted DCF. Positive Phase 3 trial results would add ~US$108m. However, given the current trading levels of ~A$150m, we would expect a material re assessment of the stock.
- ACAD has licensed the NAM rights of trofinetide in both Rett and Fragile X syndrome. FDA approval and market launch are expected to trigger milestone payments of up US$455m and sales royalties of 10%+.
- NEU retains the rights of trofinetide for ex NAM markets. The MST model assumes licensing deal/s are confirmed. An upfront payment of US$15m is estimated in late CY21 on positive topline data.

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 compelling efficacy reported 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 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 trofinetideex NAM
- CY22 FDA approval of trofinetide in Rett syndrome and market entry
- CY22 ACAD 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 trofinetide's exNAM 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 risk 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
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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