All Systems Go

NEU has announced that the US Food and Drug administration (FDA) has 'opened' the Investigational New Drug (IND) for NEU's third Phase 2 clinical trial of NNZ-2591. NEU has resolved the queries that were raised by the FDA late last year regarding the trials' designs. The Phase 2 trials include Angelman, Pitt-Hopkins and Phelan-McDermid Syndromes. NEU also plans to submit an IND for Prader-Willi in H1CY22.

NEU has reported strong supporting data of NNZ-2591's efficacy in preclinical mouse model studies. Its Phase 1 trial in healthy subjects to demonstrate safety, reported no Serious Adverse Events (SAE).

Potential Strong CY22/23 Trofinetide Revenues

NEU's other drug, trofinetide reported strongly positive Phase 3 trial results in Rett Syndrome in late CY21. Through its North American (NAM) licensing agreement with Acadia Pharmaceuticals (NASDAQ:ACAD), revenues of ~A$115m are expected over CY22/23 on filing of a New Drug Application (NDA) and US market entry, with double digit sales royalties to follow. NEU is expected to confirm the licensing rights for ex-NAM Rett markets over CY22. In MST's view, the strength of Phase 3 trial results will provide significant support to NEU's negotiations.

Targeted Diseases Offer Advantages

From a valuation perspective, NEU's targeted conditions carry a number of advantages;

- All six syndromes are seriously debilitating, life-long conditions, presenting a strong long-term clinical need.
- From a competitive perspective, there are no approved treatments.
- As rare diseases, drug pricing is generally attractive with the average annual cost of an orphan drug of ~US$150K.
- Both trofinetide and NNZ-2591 have been awarded orphan drug designation by the FDA and European Medical Agency (EMA) regulatory bodies, bringing extended patent life for both drugs.
- NNZ-2591's announced targeted syndromes offer five times the treatment population of Rett Syndrome.

Financials, Valuation, Risks, Sensitivities

Cash at FY21 end was $36.8m. The cash is planned to fund NNZ-2591 Phase 2 trials to CY23 readout. MST's valuation assumes NEU will license NNZ-2591's ongoing development. Under this option, no further capital will be required to develop either drug in its planned targets. MST's risk adjusted DCF valuation of $6. 21ps(dil). The valuation is subject to the upside/downside risks and sensitivities 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. NEU's trofinetide US partner, Acadia Pharmaceuticals (NASDAQ:ACAD) reported positive Phase III trial results in Rett Syndrome in Q4CY21. NNZ-2591 is to commence four Phase 2 trials in H1CY22.

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$4.00
Market cap	A$504m
Valuation (per share)	A$6.21 diluted
Net cash (31/12/21)	A$36.8m
Shares on issue	126m
Options/Rights	3m

Potential Milestones

- Apr 22 - ACAD to present Phase 3 trial results at American Academy Neurology Mtg.
- H1CY22 - Commence Phase 2 trials NNZ-2591 in four syndromes
- mid CY22 –US NDA submission for trofinetide
- CY22 – ex-NAM commercial partnerships
- Q1CY23 - FDA approval trofinetide
- H1CY23 – Results for four NNZ-2591 Phase 2

Share Price Performance (12 months)

Source: FactSet Prices

Exhibit 1 – MST Forecast Financial Summary

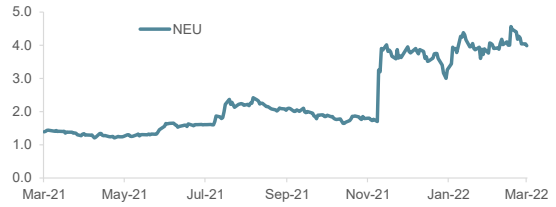
Neuren Pharmaceuticals Limited — NEU-AU

Year end 31 December

MARKET DATA

Share Price	**A$**	**4.00**
52 week high / low	A$	4.68 - 1.20
Valuation (12 month forward)	**A$**	**6.21**
Market capitalisation	**A$m**	**504**
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)	53.3	6.5	39.8
EPS Underlying (undiluted)	**¢**	**(8.6)**	**(6.6)**	**53.3**	**6.5**	**39.8**
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**	**470.4**	**515.9**	**515.9**	**515.9**	**515.9**
Net debt /(cash)	$m	(24.2)	(36.8)	(105.6)	(114.0)	(165.3)
Enterprise value	**$m**	**446.2**	**479.1**	**410.3**	**401.8**	**350.5**
EV/Sales	x	546.2	133.3	2.5	8.2	3.0
EV/EBITDA	**x**	**(47.8)**	**(61.1)**	**4.0**	**33.8**	**4.7**
EV/EBIT	x	(47.8)	(61.1)	4.2	40.6	5.0
Net debt / Enterprise Value	x	(0.1)	(0.1)	(0.3)	(0.3)	(0.5)
Gearing (net debt / EBITDA)	**x**	**2.6**	**4.7**	**(1.0)**	**(9.6)**	**(2.2)**
Operating cash flow per share	$	(0.1)	(0.1)	0.6	0.1	0.4
Price to operating cash flow	**x**	**(58.2)**	**(51.7)**	**6.9**	**49.6**	**9.2**
Free cash flow	**$m**	**(8.1)**	**(10.0)**	**68.8**	**8.4**	**51.3**
Free cash flow per share	$	(0.07)	(0.08)	0.53	0.07	0.40
Price to free cash flow	**x**	**(58.2)**	**(51.7)**	**7.5**	**61.1**	**10.1**
Free cash flow yield	**%**	**-1.7%**	**-1.9%**	**13.3%**	**1.6%**	**9.9%**
Book value / share	$	0.21	0.30	0.82	0.88	1.28
Price to book (NAV)	**x**	**19.4**	**13.1**	**4.9**	**4.5**	**3.1**
NTA / share	$	0.21	0.30	0.82	0.88	1.28
Price to NTA	**x**	**19.4**	**13.1**	**4.9**	**4.5**	**3.1**
EBITDA margin	%	n/m	n/m	64%	24%	65%
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	90.1	4.6	27.9

PROFIT AND LOSS (A$)		FY20	FY21	FY22E	FY23E	FY24E
Total Revenue & Other Income	$m	0.8	3.6	162.6	49.2	115.1
COGS	$m	-	-	(33.3)	(10.1)	(26.2)
Gross margin	**$m**	**0.8**	**3.6**	**129.2**	**39.0**	**88.9**
Corporate costs	$m	(10.2)	(11.4)	(25.5)	(27.2)	(13.5)
EBITDA	**$m**	**(9.3)**	**(7.8)**	**103.7**	**11.9**	**75.4**
Depreciation & amortisation	$m	-	-	(6.5)	(2.0)	(4.6)
EBIT	$m	(9.3)	(7.8)	97.2	9.9	70.8
Net interest	$m	0.1	0.0	1.1	2.2	2.5
Pretax Profit	$m	(9.2)	(7.8)	98.3	12.1	73.3
Tax expense	$m	-	-	(29.5)	(3.6)	(22.0)
Minorities	$m	-	-	-	-	-
Underlying NPAT	**$m**	**(9.2)**	**(7.8)**	**68.8**	**8.4**	**51.3**

BALANCE SHEET (A$)		FY20	FY21	FY22E	FY23E	FY24E
Cash	$m	24.2	36.8	105.6	114.0	165.3
Receivables	$m	0.8	3.3	6.7	2.0	4.7
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**	**112.3**	**116.0**	**170.0**
Payables	$m	0.8	0.8	6.7	2.0	4.7
Borrowings	$m	-	-	-	-	-
Leases	$m	-	-	-	-	-
Provisions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Total Liabilities	**$m**	**0.8**	**0.8**	**6.7**	**2.0**	**4.7**
Shareholder's Equity	**$m**	**24.2**	**39.2**	**105.6**	**114.0**	**165.3**

CASH FLOW (A$)		FY20	FY21	FY22E	FY23E	FY24E
Receipts from customers	$m	-	-	133.3	40.5	104.9
Payments to suppliers and employees	$m	(1.4)	(2.7)	(39.0)	(13.9)	(31.3)
R&D	$m	(7.8)	(9.8)	(19.9)	(23.4)	(8.4)
Govt Grants, Rebates & Milestones	$m	0.9	2.5	29.2	8.6	10.2
Interest	$m	0.2	0.1	1.1	2.2	2.5
Tax	$m	-	-	(29.5)	(3.6)	(22.0)
Operating cash flow	**$m**	**(8.1)**	**(10.0)**	**75.3**	**10.4**	**55.9**
Capex	$m	(0.0)	(0.0)	(6.5)	(2.0)	(4.6)
Acquisitions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Investing cash flow	**$m**	**(0.0)**	**(0.0)**	**(6.5)**	**(2.0)**	**(4.6)**
Borrowings	$m	-	-	-	-	-
Equity	$m	19.1	22.2	-	-	-
Dividend	$m	-	-	-	-	-
Financing cash flow	**$m**	**19.1**	**22.2**	**-**	**-**	**-**
Change in Cash / FX	$m	11.1	12.2	68.8	8.4	51.3
Year end cash	**$m**	**24.2**	**36.8**	**105.6**	**114.0**	**165.3**

Source: Company Reports, MST Assumptions

NNZ 2591 Phase 2 Trial Program to commence

Neurodevelopmental Syndrome	Genetic Mutation	Clinical Trial		Trial Endpoints	Ethics Approval
Angelmann	UBE3A	Phase 2	up to 20 3-17 yr olds in 13 week trial	Safety, tolerability, pharmacokinetics and efficacy	Ethics Aproval √
Pitt-Hopkins	TCF4	Phase 2	up to 20 3-17 yr olds in 13 week trial	Safety, tolerability, pharmacokinetics and efficacy	Subject to Ethics Approval
Phelan-MeDermid	Shank3	Phase 2	up to 20 3-12 yr olds in 13 week trial	Safety, tolerability, pharmacokinetics and efficacy	Subject to Ethics Approval
Prader-Willi	15q11-q13	IND to commence Phase 2 to be submitted			

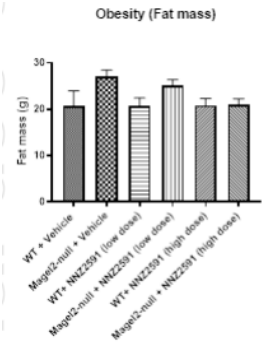
In late 2021, NEU submitted INDs to the FDA to commence Phase 2 trials of NNZ-2591 in Angelman, Pitt Hopkins and Phelan-McDermid syndromes. The FDA raised queries regarding the designs of the Phase 2 trials. Following re-submission of the INDs, NEU has announced that the regulatory agency has 'opened' the INDs, allowing the trials to proceed. The trial program will provide the first opportunity to demonstrate efficacy and safety in the patient populations. The trials are planned to commence in H1CY22 with results anticipated in H1CY23. NEU also plans to submit an IND for a Phase 2 trial in a fourth disorder, Prader-Willi syndrome.

The trial cohorts comprise children aged 3-17 years (3-12yrs in Phelan-McDermid) with the aim of limiting the heterogeneity of the syndrome presentations. A commonality of symptoms enhances the probability of capturing any change over the trial period. The age group in Phelan-McDermid Syndrome trial has been furthered narrowed to account for greater variability in behaviour of these children in their teen years. Subsequent trials are expected to examine NNZ-2591 in adult populations. NEU will also carry out foundational work to prepare for Phase 3 development of NNZ-2591 across the multiple indications.

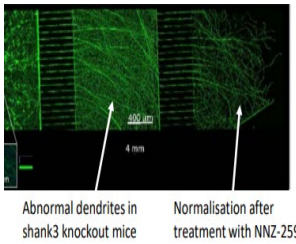
Proof to date

Preclinical Studies

Studies in mouse models of all four syndromes have demonstrated strong effect of NNZ-2591 across a broad range of syndrome related symptoms, including behaviour, learning and memory, sociability, anxiety, motor function, seizure reduction/elimination. In Prader-Willi, where excessive eating is a feature, NNZ-2591 normalised the fat mass, as well as insulin and IGF-1 levels. In a Phelan-McDermid model, NNZ-2591 showed restoration of the nerve cells' dendrites, important in the transmission of the neural signals from nerve cell to nerve cell.



Preclinical Studies of NNZ-2591 in Prader-Willi Syndrome, show the disease model mice (Magel2) with high dose NNZ-2591 reduces the fat mass to the level of the control mice (WT).



In a Phelan-McDermid model, NNZ-2591 demonstrated showed normalisation of the dendrites.

Phase 1 Trial

NEU conducted a seven-day Phase 1 trial of NNZ-2591 in healthy volunteers with twice daily dosing at the levels to be used in Phase 2 trials. There were no significant adverse events (SAE), with drowsiness the most common adverse effect reported.

Support from Trofinetide's Success

NNZ-2591 is a novel synthetic analog or replica of a key neural peptide, cyclic glycine-proline (cGP). Trofinetide is an analog of glycine-proline-glutamate (GPE), another key neural peptide. Both peptides are intimately involved in the regulation of the growth hormone, IGF-1. IGF-1 plays many roles within the body, which include foetal development and growth over childhood and adolescence. It also has a neural protective role, regulating nerve transmission and the development and maintenance of the synapses. All the targeted syndromes arise from genetic mutations and result in significant effects on the function of the body's nervous system. Given the relationship between GPE and cGP and IGF-1, in MST's view, the success of trofinetide adds support to NNZ-2591's approach.

Potential Milestones

Apr CY22 - ACAD to present trofinetide's Lavender trial results at American Academy Neurology Meeting

H1CY22 - Commence Phase 2 Angelman, Pitt-Hopkins, Phelan-McDermid and Prader-Willi syndromes

mid CY22 - Submission of New drug Application (NDA) for approval of trofinetide in Rett syndrome

CY22 - Commercial partnerships for trofinetide in ex North America regions

Q1CY23 - FDA approval trofinetide in Rett Syndrome

H1CY23 - Announcement of results of Phase 2 NNZ-2591 trials

Valuation, Key Risks and Sensitivities

We value NEU at $6.21 per share(dil) on a 12-month forward risk-adjusted DCF basis. MST's valuation is subject to the usual upside/downside risks and sensitivities of drug development, including clinical trial success and timing, market approval and entry, pricing, market penetration and sales royalties/licensing payments. The COVID pandemic has resulted in clinical trial delays with abandonment of some trials. We note that trofinetide's Phase 3 trial in Rett syndrome did not experience any delay despite significant COVID outbreaks in the US during the trial.

A key assumption is that trofinetide's exNAM rights are licensed. Assumptions have been made regarding the likely terms of the agreements. Requirements regarding data to support EMA approval and other jurisdictions are yet to be established. Both bring up/downside risk to MST forecasts. The ACAD agreement includes the rights for use of trofinetide in Fragile X Syndrome. ACAD is yet to confirm further development plans for the additional indication.

Another key assumption is the licensing of NNZ-2591 on announcement of positive Phase 2 data in CY23. NEU has a number of options in terms of realising the value of NNZ-2591 and may choose a different development pathway, depending on the Phase 2 results. While there is strong preclinical data to support efficacy of NNZ-2591 there is, as yet, no clinical efficacy data in patients. This has been accounted for in the use of the industry average risk weighting. However, trials are usually a binary event.

Approval by the FDA in Rett Syndrome is the key short term valuation driver. It should be noted that NNZ-2591 in its announced targeted treatment populations, offers five times the potential market size of Rett Syndrome and therefore in longer term, if approved, is likely to be the key value driver.

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