Transformative Phase 3 Results

Results Support Commercial Activities

NEU's North America (NAM) partner, Acadia Pharmaceuticals (NASDAQ:ACAD) has announced strong top-line results of its Phase 3 trial in Rett Syndrome. All three endpoints were met with clinical significance of p values of <0.0175. Generally, p values of <0.05 are regarded as positive. In MST's view, the strength of the results supports US regulatory approval, good market uptake and favourable drug pricing. It also enhances NEU's power in licensing negotiations for ex-NAM rights.

US Approval/Market Entry to Trigger Revenues

FDA approval and US market entry are expected to trigger payments of ~A$111m over CY22/CY23, with double-digit royalties on net sales to follow. NEU is expected to confirm the licensing rights for ex-NAM markets over CY22. MST forecasts a US$20m as a sign on payment. The strength of the results brings upside risk to MST forecasts.

NNZ-2591 to come

NEU's Phase 2 trials of its second drug NNZ-2591 in three additional neurodevelopmental syndromes were planned to start in late CY21. FDA queries regarding the trial protocols have the program on 'hold'. NEU expects to resolve the queries over early CY22. From an efficacy perspective, based on its testing to date, NEU believes that NNZ-2591 offers a greater clinical potential than trofinetide. MST's forecasts assume a licensing deal over late CY22 with a sign-on US$20m payment.

Financials, Valuation, Risks, Sensitivities

We value NEU at $5.05 per share on a 12-month forward risk-adjusted DCF basis (previously $3.58ps). On the strength of the positive top-line trial results, the probability of approval is 95% (previously 60%) for Rett Syndrome. Market share is forecast to peak at 15% (previously 10%). The probability of approval of 25% and other valuation assumptions for NNZ-2591 in its targeted conditions are unchanged.

The valuation includes assumptions of the probability of approval and commercial performance. The forecasts are subject to the usual sensitivities/risks regarding trial delay, competitor activity, market approval, pricing, patient uptake, product supply and reimbursement. They present upside and downside risks to MST valuation assumptions.

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. Top-line results of the Phase 3 trial of trofinetide in Rett Syndrome showed strong clinical significance. NNZ-2591 is planned to enter Phase 2 trials in CY22.

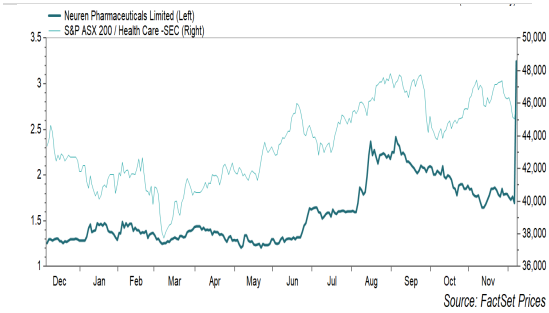
Board and management are well credentialled with in-depth experience in drug development and commercialisation.

Company data	
Stock	ASX: NEU
Primary Exchange	ASX
Price	A$3.20
Market Cap	A$413m
Valuation	A$651m
Valuation ps	A$5.05(dil)
Net cash (30/09/21)	A$33.6m
Shares on issue	126m
Options/Rights	3m

Next steps

- Q1CY22 ACAD Pre-NDA meeting with FDA
- H1CY22 Commence NNZ-2591 Phase 2 trials
- CY22 New Drug Application submission to FDA

Share price performance (12 months)



Source: FactSet Prices

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Exhibit 1 – NEU Financial Summary

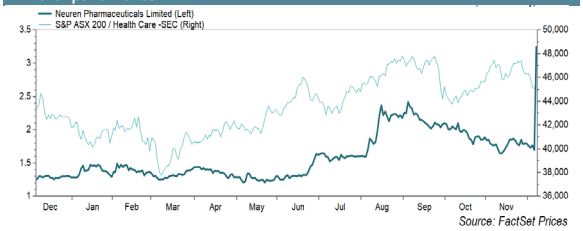
Neuren Pharmaceuticals Limited — NEU-AU

Year end 31 December

MARKET DATA

Share Price	**A$**	**3.20**
52 week high / low	A$	3.56 - 1.19
Valuation (12 month forward)	**A$**	**5.05**
Market capitalisation	**A$m**	**413**
Shares on issue	m	126
Options	m	3
Other equity	m	-
Potential shares on issue (diluted)		129

12 month performance



Source: FactSet Prices

INVESTMENT FUNDAMENTALS		FY20	FY21	FY22E	FY23E	FY24E
EPS Reported (undiluted)	¢	(8.6)	(11.1)	30.2	23.7	36.3
EPS Underlying (undiluted)	**¢**	**(8.6)**	**(11.1)**	**30.2**	**23.7**	**36.3**
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$)		FY20	FY21	FY22E	FY23E	FY24E
Forecast year end shares	m	118	126	126	126	126
Market cap (Y/E / Spot)	**$m**	**376.3**	**403.1**	**403.1**	**403.1**	**403.1**
Net debt /(cash)	$m	(24.2)	(36.2)	(74.3)	(104.1)	(149.8)
Enterprise value	**$m**	**352.2**	**366.9**	**328.8**	**299.0**	**253.2**
EV/Sales	x	431.0	116.0	3.9	5.9	3.3
EV/EBITDA	**x**	**(37.7)**	**(33.4)**	**5.7**	**6.9**	**3.8**
EV/EBIT	x	(37.7)	(32.9)	6.1	7.3	4.0
Net debt / Enterprise Value	x	(0.1)	(0.1)	(0.2)	(0.3)	(0.6)
Gearing (net debt / EBITDA)	**x**	**2.6**	**3.3**	**(1.3)**	**(2.4)**	**(2.2)**
Operating cash flow per share	$	(0.1)	(0.1)	0.3	0.3	0.4
Price to operating cash flow	**x**	**(46.6)**	**(35.7)**	**9.5**	**12.5**	**8.1**
Free cash flow	**$m**	**(8.1)**	**(11.5)**	**38.1**	**29.8**	**45.7**
Free cash flow per share	$	(0.07)	(0.09)	0.30	0.24	0.36
Price to free cash flow	**x**	**(46.6)**	**(35.2)**	**10.6**	**13.5**	**8.8**
Free cash flow yield	**%**	**-2.1%**	**-2.8%**	**9.5%**	**7.4%**	**11.3%**
Book value / share	$	0.21	0.29	0.61	0.84	1.21
Price to book (NAV)	**x**	**15.6**	**11.1**	**5.3**	**3.8**	**2.7**
NTA / share	$	0.21	0.29	0.61	0.84	1.21
Price to NTA	**x**	**15.6**	**11.1**	**5.3**	**3.8**	**2.7**
EBITDA margin	%	n/m	n/m	68%	86%	86%
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	58.5	25.1	27.3

PROFIT AND LOSS (A$)		FY20	FY21	FY22E	FY23E	FY24E
Total Revenue & Other Income	$m	0.8	3.2	84.8	50.5	77.7
COGS	$m	-	-	-	-	-
Gross margin	**$m**	**0.8**	**3.2**	**84.8**	**50.5**	**77.7**
Corporate costs	$m	(10.2)	(14.2)	(27.1)	(7.1)	(10.8)
EBITDA	**$m**	**(9.3)**	**(11.0)**	**57.7**	**43.5**	**66.9**
Depreciation & amortisation	$m	-	(0.1)	(4.2)	(2.5)	(3.9)
EBIT	$m	(9.3)	(11.1)	53.5	41.0	63.0
Net interest	$m	0.1	0.2	0.9	1.6	2.3
Pretax Profit	$m	(9.2)	(10.9)	54.4	42.6	65.3
Tax expense	$m	-	-	(16.3)	(12.8)	(19.6)
Minorities	$m	-	-	-	-	-
Underlying NPAT	**$m**	**(9.2)**	**(10.9)**	**38.1**	**29.8**	**45.7**

BALANCE SHEET (A$)		FY20	FY21	FY22E	FY23E	FY24E
Cash	$m	24.2	36.2	74.3	104.1	149.8
Receivables	$m	0.8	-	3.5	2.1	3.2
Inventory	$m	-	-	2.1	1.3	1.9
PPE	$m	0.0	0.1	0.1	0.1	0.1
Intangibles	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Total Assets	**$m**	**25.0**	**36.4**	**80.0**	**107.6**	**155.1**
Accounts Payable	$m	0.8	-	3.5	2.1	3.2
Borrowings	$m	-	-	-	-	-
Leases	$m	-	-	-	-	-
Provisions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Total Liabilities	**$m**	**0.8**	**-**	**3.5**	**2.1**	**3.2**
Shareholder's Equity	**$m**	**24.2**	**36.4**	**76.6**	**105.5**	**151.9**

CASH FLOW (A$)		FY20	FY21	FY22E	FY23E	FY24E
Receipts from customers	$m	-	-	53.3	40.5	77.7
Payments to suppliers and employees	$m	(1.4)	(7.8)	(4.1)	(3.8)	(4.6)
R&D	$m	(7.8)	(5.4)	(23.0)	(3.2)	(6.2)
Govt Grants, Rebates & Milestones	$m	0.9	1.7	31.5	10.0	-
Interest	$m	0.2	0.2	0.9	1.6	2.3
Tax	$m	-	-	(16.3)	(12.8)	(19.6)
Operating cash flow	**$m**	**(8.1)**	**(11.3)**	**42.3**	**32.3**	**49.6**
Capex	$m	(0.0)	(0.2)	(4.2)	(2.5)	(3.9)
Acquisitions	$m	-	-	-	-	-
Other	$m	-	-	-	-	-
Investing cash flow	**$m**	**(0.0)**	**(0.2)**	**(4.2)**	**(2.5)**	**(3.9)**
Borrowings (Net)	$m	-	-	-	-	-
Equity	$m	19.1	23.3	-	-	-
Dividend	$m	-	-	-	-	-
Financing cash flow	**$m**	**19.1**	**23.3**	**-**	**-**	**-**
Change in Cash / FX	$m	11.1	11.8	38.1	29.8	45.7
Year end cash	**$m**	**24.2**	**36.2**	**74.3**	**104.1**	**149.8**

Source: NEU Reports, MST Estimates

Strong Trial Results to Drive Regulatory & Market Activities

Acadia Pharmaceuticals (NASDAQ:ACAD), NEU's licensing partner, has reported positive top-line results from its pivotal Phase 3 Lavender™ study. The trial evaluated the efficacy and safety of trofinetide in 187 girls and young women aged 5-20 years with Rett syndrome. The 12-week placebo-controlled study demonstrated statistically significant improvement over placebo for both co-primary endpoints, Rett Syndrome Behaviour Questionnaire (RSBQ) and the Clinical Global Impression–Improvement (CGI-I). The key secondary endpoint, the Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist–Social composite score (CSBS-DP-IT–Social) also reported an unequivocal result with a p value of 0.0064.

Trial Key Take-aways

The Lavender trial data are very supportive from both regulatory and market perspectives.

ENDPOINT		TYPE	ASSESSMENT	p VALUE	EFFECT SIZE
Rett Syndrome Behaviour Questionnaire	RSBQ	Primary	Caregiver	**0.0175**	0.37
Clinical Global Impression – Improvement	CGI-I	Primary	Physician	**0.003**	0.47
CSBS-DP-IT Social Composition Score	CSBS-DP-IT	Secondary	Caregiver	**0.0064**	0.43

- The strength of the data with clinical significance across the three trial endpoints leaves little room for debate around trofinetide's efficacy. Regulators usually do not require the customary two trials, pivotal and confirmatory, in rare diseases. However, when trial results are not definitive a second study can be requested. The strength of the Phase 3 data is likely to support FDA approval on the single Lavender trial.
- Trial patient cohorts were well balanced with respect to age and disease status which is also supportive of the results.
- Consistency of data was also a key feature. The Phase 3 results trended those reported in the Phase 2 trial results and data emerging from the Lilac extension trial. There was also a consistency of the results across the different age groups, severity of disease and subscores of the RSBQ. All will be important to the regulators and supportive of broader label indications.
- ACAD is conducting a pharmacokinetic study in 2–5-year-olds to support the inclusion of the younger patients in the label indication.
- >95% of the participants continued to the Lilac extension trial which signals patient/carer support.
- The strong results are also likely to be supportive of pricing negotiations and market uptake. There are no approved treatments for Rett Syndrome and it is commonly severely debilitating.
- Adverse effects were generally not significant. Diarrhea was the main cause of patients who withdrew from the trial. The diarrhea was attributed to an interaction of trofinetide with laxative medications. ACAD has designed a protocol to manage these patients to minimize the effect.
- The mechanism of action of trofinetide in addressing the condition's underlying nervous system defects supports a disease modifying ability but this effect is yet to be confirmed.

FDA approval and market entry to trigger licensing revenues

Under its licensing agreement with ACAD, NEU is entitled to receive milestone payments based on FDA approval and US market entry. The agreement also allows for NEU to receive one third of the market value of a Rare Paediatric Disease Priority Review Voucher, if awarded. In total, NEU may receive ~A$111m over CY22 &CY23. On market launch, NEU will receive double digit royalties on net sales, with potential sales-based milestone payments of US$350m.

The FDA has awarded the trial Fast Track status. The designation allows for a six-month review of the trial data by the FDA rather than the customary period of ten months. In addition, trofinetide has an FDA orphan drug status which confers a market exclusivity period of seven years.

Ex-NAM Markets

NEU retains the rights for the ex-NAM markets. Under its agreement with ACAD, the data of the trial and associated development activities can be used as part of the regulatory data packages for ex-NAM markets. With the confirmation of positive results, NEU is expected to confirm its marketing plans over the following months. Trofinetide has Orphan Drug Status in the EU, conferring 12 years market exclusivity. The extended market protection is likely to be valued by potential partners for the EU markets.

Fragile X - Next Steps

The ACAD agreement also includes the use of trofinetide in Fragile X syndrome. A Phase 2 trial which was undertaken by NEU included 82 females who were treated over 28 days. Despite the relatively short treatment period, trofinetide at the high dose (70 mg/kg twice daily) demonstrated a consistent pattern of clinical improvement, observed in both clinician and caregiver assessments. With the positive news of trofinetide efficacy in Rett Syndrome, MST assumes ACAD will continue the development of the Fragile X indication.

Implications for NNZ-2591

The Rett Syndrome trial results may also be supportive of NNZ-2591. While the exact mechanism of action of trofinetide is not fully understood, the two drugs are based on two related molecules. Trofinetide and NNZ-2591 are synthetic versions or analogues of glycine-proline-glutamate (GPSE) or glypromate (GPE) and cyclic-glycine-proline (cGP) peptides, respectively. Both GPE and cGP are believed to play important roles in regulating the activity of Insulin Growth Factor 1(IGF-1).

IGF-1 is critical for brain development and bodily functions such as movement and cognition. Studies have shown that different neural diseases and brain trauma often trigger the same pathological effects at the cellular and molecular levels. The close relationship of the two peptides in IGF-1 factor control and the shared underlying pathology of the targeted neurodevelopmental conditions offer some support that NNZ-2591 will be also effective in these types of conditions.

Potential Value Drivers in CY21/22

Q1 CY22	Pre-NDA (New Drug Application) submission by ACAD
CY22	NDA submission
CY22	Phase 2 trials of NNZ-2591 in three conditions
CY22	Licensing agreements/upfront payments for trofinetide ex-NAM
CY22/23	FDA approval of trofinetide in Rett Syndrome and market entry with milestone payments
CY22/23	ACAD to announce plans for the development of trofinetide in Fragile X Syndrome
CY22/23	Licensing agreement for NNZ-2591 post positive Phase 2 trials

Valuation and Key Risks

We value NEU at $5.05 per share on a 12-month forward risk-adjusted DCF basis (previously $3.58ps). On the strength of the positive top-line trial results, we have increased the probability of approval to 95% (previously 60%) for Rett Syndrome. We have also increased market share across the different markets to peak at 15% (previously 10%). The probability of approval of 25% and other valuation assumptions for NNZ-2591 in its targeted conditions are unchanged.

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 may affect the ongoing clinical trials in NNZ-2591.

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