Antisense Therapeutics

Small changes for a big difference

SPECULATIVE BUY

Current price:	A$0.125
Target price:	A$0.377
Previous target:	A$
Up/downside:	202.0%
Reuters:	ANP.AX
Bloomberg:	ANP AU
Market cap:	US$55.88m
	A$71.75m
Average daily turnover:	US$0.30m
	A$0.41m
Current shares o/s	574.0m
Free float:	100.0%

Source: Bloomberg

Price performance	1M	3M	12M
Absolute (%)	25	19	50.6
Relative (%)	25.4	8.2	52.5

Iain WILKIE
T (61) 7 3334 4521
E iain.wilkie@morgans.com.au

Scott POWER
T (61) 7 3334 4884
E scott.power@morgans.com.au

Dr Derek JELLINEK
T (61) 2 9043 7904
E derek.jellinek@morgans.com.au

Analyst(s) own shares in the following stock(s) mentioned in this report:

– Antisense Therapeutics

- Antisense Therapeutics (ANP) is an advanced stage drug developer focused on inflammation in patients suffering from a number of rare genetic diseases with large unmet clinical needs.
- The treatment targets mRNA to inhibit the expression of specific genes linked to rapid and severe inflammation, often accelerating functional muscle mass losses in patients and potentially hindering effectiveness of first-line treatments.
- While the Ph2a DMD trial was small (inherent of rare disease indications), it exceeded expectations, showing improvements in disease progression versus a matched natural history control on standard of care.
- ANP is now heading into a milestone rich period with commencement of a pivotal Ph2b trial, data on additional indications, possible monetising of non-core indications, and potential awarding of regulatory incentives and priority status.
- We view the stock as materially undervalued given the data produced to date, potential for partnership deals, and likely out-licensing of indications beyond its core focus in DMD. We initiate coverage with a risk-weighted price target of A$0.38 per share and Speculative Buy recommendation.

What is Duchenne's Muscular Dystrophy?

DMD is an X chromosome-linked disease that affects 1 in 3,600 to 6,000 male births and occurs as a result of mutations in the dystrophin gene. The mutation causes a substantial reduction in the body's production of the dystrophin protein which is used to strengthen and protect muscle fibres as they contract and relax. Ongoing deterioration in muscle strength affects lower limbs leading to impaired mobility, and also upper limb function, leading to further loss of function and self-care ability. Studies show that patients with higher levels of immune T cells (lymphocytes – central role in immune response) expressing high levels of CD49d have more severe and progressive disease and are wheelchair bound by the age of 10 despite being on corticosteroids. With no intervention, the mean age of life expectancy is approximately 19 years.

Strong results for DMD – moving to Phase 2b

ANP's Ph2a trial in non-ambulant boys suffering from DMD showed the treatment to be safe and well tolerated. While the trial was small (n=9), the data produced on the secondary efficacy endpoints in these patients showed statistically significant improvements in muscle function assessments versus natural history studies for disease progression. A larger trial in EU and US is expected to commence late CY21. The company is expected to further define its regulatory pathway in the coming months.

Investment view

We initiate coverage on ANP with a Speculative Buy recommendation and risk-weighted target price of A$0.38 per share at the bottom end of our A$0.38 to A$1.82 per share valuation range. While considerable trial risk and hurdles remain, we view ANP as one of the best risk/reward plays in the healthcare space given the data produced to date and heading into a catalyst rich 36 month period. Downside risk to our price target is failure in the DMD Ph2b program (value is 2 cps) and prolonged delays in trial recruitment and commencement.

Financial Summary	Jun-19A	Jun-20A	Jun-21F	Jun-22F	Jun-23F
Revenue (A$m)	2.98	3.65	6.35	2.85	0.00
Operating EBITDA (A$m)	0.00	-6.19	-7.99	-22.79	-8.03
Net Profit (A$m)	0.26	-5.23	-7.81	-22.69	-7.74
Normalised EPS (A$)	0.001	(0.012)	(0.015)	(0.033)	(0.010)
Normalised EPS Growth	510%	(1847%)	28%	125%	(71%)
FD Normalised P/E (x)	189.8	NA	NA	NA	NA
DPS (A$)	-	-	-	-	-
Dividend Yield	0%	0%	0%	0%	0%
EV/EBITDA (x)	NA	NA	NA	NA	NA
P/FCFE (x)	NA	NA	NA	NA	NA
Net Gearing	(103%)	(89%)	(108%)	(118%)	(117%)
P/BV (x)	18.69	13.42	14.23	8.05	21.56
ROE	7%	(142%)	(163%)	(261%)	(91%)
% Change In Normalised EPS Estimates					
Normalised EPS/consensus EPS (x)			1.84	2.21	

SOURCE: MORGANS, COMPANY REPORTS

Antisense Therapeutics

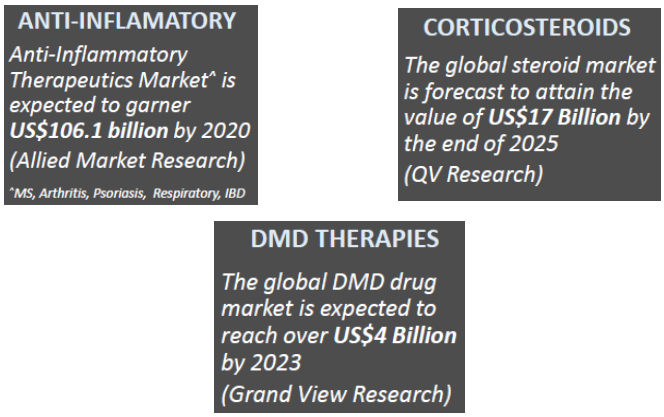
as at January 7, 2021

Market cap (A$m):	71.75	Rating:	SPECULATIVE BUY
Shares outstanding (m):	574.0	Price (A$):	0.125
Free float (%):	100.0	Target price (A$):	0.377
Website:	https://antisense.com.au	Upside/downside to target price (%):	202.0

Company description

Antisense Therapeutics Limited, a biopharmaceutical company, engages in the research and development of novel antisense pharmaceuticals in Australia. Its product pipeline comprises ATL1102, an antisense inhibitor of CD49d that has completed Phase IIa for the treatment of multiple sclerosis, Duchennes Muscular Dystrophy, acromegaly, asthma, and other inflammatory indications. The company's product pipeline also includes ATL1103, a second generation antisense drug designed to block growth hormone receptor expression thereby reducing levels of the hormone insulin-like growth factor-I in the blood, as well as to treat diseases associated with excessive growth hormone action that has completed Phase II clinical trial.

Market considerations for ATL1102



SOURCE: ANTISENSE THERAPEUTICS

Near-term milestones (CY21)

* ATL1102 drug product manufacture

* ATL1102 in new indications

* US FDA DMD progress

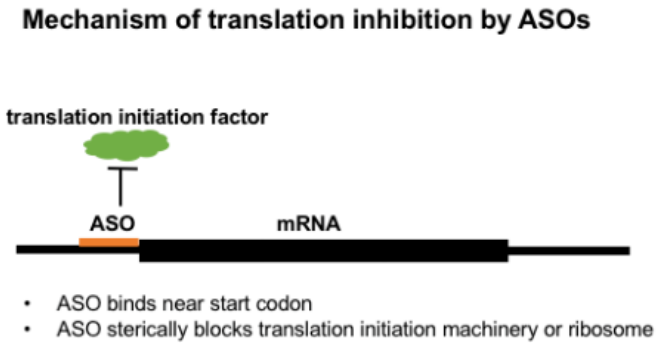
* ATL1102 DMD Ph2b EU trial prep and initiation

SOURCE: ANTISENSE THERAPEUTICS

Product pipeline

SOURCE: ANTISENSE THERAPEUTICS

ATL1102 mechanism of action



SOURCE: CureFFi.org

MARKET DATA	#
Population of target market ('000s)	48.0
Regulatory approval weight	25.0%
Non-ambulant population	50.0%
Number of Cases Forecast for Year 1 ('000s)	6.0
Annual Population Growth	0.70%
Peak Market Penetration	50.0%
Revenue Per Unit ($US)	$ 150,000
Market Ramp Time to Peak Penetration (Years	5
Hold peak	10
Life cycle of drug	20
Royalty Rate	20.0%

SOURCE: MORGANS

Key Drivers

Licencing deal value for late stage assets

Potential for early commercialisation

Key risks:

Timing / execution risks

Trial risks

Alternative therapies

COVID-19 related impact

Funding risk

SOURCE: MORGANS

Figure 1: Financial summary

Antisense Therapeutics

Income statement	2019A	2020A	2021F	2022F	2023F
Milestone payments	0.0	0.0	0.0	0.0	0.0
Royalty	0.0	0.0	0.0	0.0	0.0
R&D rebate	3.0	3.6	6.3	2.9	0.0
Total revenue	**3.0**	**3.6**	**6.3**	**2.9**	**0.0**
EBITDA	**0.0**	**-6.2**	**-8.0**	**-22.8**	**-8.0**
Associate income	0.0	0.0	0.0	0.0	0.0
Depreciation	0.0	0.0	0.0	0.0	0.0
EBITA	0.0	-6.2	-8.0	-22.8	-8.0
Amortisation/impairment	0.0	0.0	0.0	0.0	0.0
EBIT	**0.0**	**-6.2**	**-8.0**	**-22.8**	**-8.0**
EBIT(incl associate profit)	0.0	-6.2	-8.0	-22.8	-8.0
Net interest expense/FX	0.0	0.3	1.0	-0.2	-0.1
Pre-tax profit	**0.3**	**-5.2**	**-8.2**	**-22.9**	**-8.3**
Income tax expense	0.0	0.0	0.0	0.0	0.0
After-tax profit	0.3	-5.2	-8.2	-22.9	-8.3
Minority interests	0.0	0.0	0.0	0.0	0.0
NPAT	**0.3**	**-5.2**	**-8.2**	**-22.9**	**-8.3**
Significant items	0.0	0.0	0.0	0.0	0.0
NPAT post abnormals	0.3	-5.2	-8.2	-22.9	-8.3

Cash flow statement	2019A	2020A	2021F	2022F	2023F
EBITDA	0.0	-6.2	-8.0	-22.8	-8.0
Other cash items	0.0	0.0	0.0	0.0	0.0
Net interest (pd)/rec	-0.3	-1.0	0.2	0.1	0.3
Taxes paid	0.0	0.0	0.0	0.0	0.0
Change in working capital	-2.7	3.3	0.9	0.0	-1.5
Cash flow from ops (1)	**-2.9**	**-4.0**	**-6.9**	**-22.7**	**-9.2**
Capex (2)	0.0	-0.1	0.0	0.0	0.0
Disposals/(acquisitions)	0.0	0.0	0.0	0.0	0.0
Other investing cash flow	0.0	0.0	0.0	0.0	0.0
Cash flow from invest (3)	**0.0**	**-0.1**	**0.0**	**0.0**	**0.0**
Incr/(decr) in equity	1.5	5.2	8.3	30.0	0.0
Incr/(decr) in debt	0.0	0.0	0.0	0.0	0.0
Ordinary dividend paid	0.0	0.0	0.0	0.0	0.0
Preferred dividends (4)	0.0	0.0	0.0	0.0	0.0
Other financing cash flow	0.0	0.0	0.0	0.0	0.0
Cash flow from fin (5)	**1.5**	**5.2**	**8.3**	**30.0**	**0.0**
Forex and disc ops (6)	0.0	0.0	0.0	0.0	0.0
Inc/(decr) cash (1+3+5+6)	-1.4	1.1	1.4	7.3	-9.2
Equity FCF (1+2+4)	-2.9	-4.1	-6.9	-22.7	-9.2

Balance sheet	2019A	2020A	2021F	2022F	2023F
Cash & deposits	2.9	4.1	5.5	14.6	5.4
Trade debtors	0.6	0.7	1.0	0.5	0.0
Inventory	0.0	0.0	0.0	0.0	0.0
Investments	0.0	0.0	0.0	0.0	0.0
Goodwill	0.0	0.0	0.0	0.0	0.0
Other intangible assets	0.0	0.0	0.0	0.0	0.0
Fixed assets	0.0	0.1	0.1	0.1	0.1
Other assets	0.0	0.0	0.0	0.0	0.0
Total assets	**3.7**	**5.4**	**7.1**	**15.7**	**6.0**
Short-term borrowings	0.0	0.0	0.0	0.0	0.0
Trade payables	0.6	0.3	1.6	2.8	0.9
Long-term borrowings	0.0	0.0	0.0	0.0	0.0
Provisions	0.3	0.5	0.5	0.5	0.5
Other liabilities	0.0	0.0	0.0	0.0	0.0
Total liabilities	**0.9**	**0.8**	**2.1**	**3.3**	**1.4**
Share capital	63.9	69.1	77.4	107.4	107.4
Other reserves	0.0	2.4	2.4	2.4	2.4
Retained earnings	-61.1	-67.0	-74.8	-97.5	-105.3
Other equity	0.0	0.0	0.0	0.0	0.0
Total equity	**2.8**	**4.6**	**5.0**	**12.4**	**4.6**
Minority interest	0.0	0.0	0.0	0.0	0.0
Total shareholders' equity	2.8	4.6	5.0	12.4	4.6
Total liabilities & SE	3.7	5.4	7.1	15.7	6.0

Closing price (A$)	0.125	Price target (A$)	0.38
Valuation metrics			
Methodology -DCF-PER Comp		Target Price	$0.38
DCF valuation inputs			
Rf	3.50%		
Rm-Rf	5.10%		
Beta	1.80		
CAPM (Rf+Beta(Rm-Rf))	12.7%		
E/EV*Ke+D/EV*Kd(1-t)		NPV cash flow (A$m)	211.2
Equity (E/EV)	100.0%	Minority interest (A$m)	0.0
Debt (D/EV)	0.0%	Net debt (A$m)	-5.5
Interest rate	5.00%	Investments (A$m)	0.0
Tax rate (t)	30.0%	Equity market value (A$m)	216.7
WACC	12.7%	Diluted no. of shares (m)	574.0
		DCF valuation	$0.38

Multiples	2019A	2020A	2021F	2022F	2023F
Enterprise value (A$m)	68.8	67.7	66.3	57.2	66.4
EV/Sales (x)	n.a.	n.a.	n.a.	n.a.	n.a.
EV/EBITDA (x)	n.a.	-10.9	-8.3	-2.5	-8.3
EV/EBIT (x)	-72420.8	-10.9	-8.3	-2.5	-8.3
PE (pre-goodwill) (x)	201.4	-11.8	-9.2	-4.4	-12.9
PEG (pre-goodwill) (x)	0.8	0.0	0.0	0.1	0.1

At target price	2019A	2020A	2021F	2022F	2023F
EV/EBITDA (x)	-72420.8	-10.9	-8.3	-2.5	-8.3
PE (pre-goodwill) (x)	608.1	-35.7	-27.7	-13.2	-38.8

Per share data	2019A	2020A	2021F	2022F	2023F
No. shares	420.1	488.8	574.0	796.0	796.0
EPS (cps)	0.1	-1.1	-1.4	-2.9	-1.0
EPS (normalised) (c)	0.1	-1.1	-1.4	-2.9	-1.0
Dividend per share (c)	0.0	0.0	0.0	0.0	0.0
Dividend payout ratio (%)	0.0%	0.0%	0.0%	0.0%	0.0%
Dividend yield (%)	0.0%	0.0%	0.0%	0.0%	0.0%

Growth ratios	2019A	2020A	2021F	2022F	2023F
Sales growth	n.a.	n.a.	n.a.	n.a.	n.a.
Operating cost growth	n.a.	n.a.	29.0%	185.3%	-64.8%
EBITDA growth	n.a.	n.a.	-29.1%	-185.1%	64.8%
EBITA growth	n.a.	n.a.	n.a.	n.a.	n.a.
EBIT growth	n.a.	n.a.	n.a.	n.a.	n.a.
NPAT growth	n.a.	n.a.	n.a.	n.a.	n.a.
Pre-goodwill NPAT growth	n.a.	n.a.	n.a.	n.a.	n.a.
Pre-goodwill EPS growth	n.a.	n.a.	n.a.	n.a.	n.a.
Normalised EPS growth	n.a.	n.a.	n.a.	n.a.	n.a.

Operating performance	2019A	2020A	2021F	2022F	2023F
Asset turnover (%)	0.0	0.0	0.0	0.0	0.0
EBITDA margin (%)	n.a.	n.a.	n.a.	n.a.	n.a.
EBIT margin (%)	n.a.	n.a.	n.a.	n.a.	n.a.
Net profit margin (%)	n.a.	n.a.	n.a.	n.a.	n.a.
Return on net assets (%)	0.0	-136.0	-158.5	-184.5	-174.1
Net debt (A$m)	-2.9	-4.1	-5.5	-14.6	-5.4
Net debt/equity (%)	-103.3	-89.1	-108.4	-118.1	-116.8
Net interest/EBIT cover (x)	0.0	23.7	7.7	-123.9	-73.5

Internal liquidity	2019A	2020A	2021F	2022F	2023F
Current ratio (x)	3.3	5.1	2.6	4.4	3.9
Receivables turnover (x)	0.0	0.0	0.0	0.0	0.0
Payables turnover (x)	0.0	14.7	8.6	10.4	4.4

SOURCE: MORGANS RESEARCH, COMPANY

Background

Company overview

Antisense Therapeutics Limited (ANP) is an Australian biopharmaceutical drug discovery and development company whose mission is to create, develop and commercialise novel antisense therapeutics for a variety of drug candidates including ATL1102 for Duchenne Muscular Dystrophy (DMD) and Multiple Sclerosis (MS), and ATL1103 for Acromegaly. ANP has a long-standing partnership with world leading antisense drug development and commercialisation company Ionis Pharmaceuticals (IONS.NAS – US$7.5bn market cap). ANP's drug candidates were licensed from Ionis in 2001 (then called Isis Pharmaceuticals) and form the basis of ANP's clinical and commercialisation strategy. ANP has exclusive world-wide licenses to the drug for all disease applications.

What is Duchenne's Muscular Dystrophy?

DMD is an X chromosome-linked disease that affects 1 in 3600 to 6000 male births and occurs as a result of mutations in the dystrophin gene. The mutation causes a substantial reduction in the body's production of the dystrophin protein which is used to strengthen and protect muscle fibres as they contract and relax. Ongoing deterioration in muscle strength affects lower limbs leading to impaired mobility, and also upper limb function, leading to further loss of function and self-care ability. The need for wheelchair use can occur in early teenage years for patients on corticosteroids (earlier for those not on steroids) with a mean age of 13, with respiratory, cardiac, and cognitive dysfunction also emerging. Studies have shown that patients with higher levels of immune T cells (lymphocytes – central role in immune response) expressing high levels of CD49d have more severe and progressive disease and are wheelchair bound by the age of 10 despite being on corticosteroids. With no intervention, the mean age of life expectancy is approximately 19 years. Current standard of care for the treatment of inflammation in these boys is long-term use of corticosteroids, however efficacy is limited and often associated with significant side effects.

Key value drivers

There are a number of key value drivers including:

- Two Phase 2 compounds (ATL1102 & ATL1103) with strong clinical efficacy, which have been peer reviewed in a number of scientific journals;
- Phase 2b in DMD and potential for conditional approval on completion;
- Eligibility for Rare Pediatric Disease Priority Review Voucher (PRV) upon FDA approval. Current market value for these in secondary markets is ~US$100m;
- Preliminary work starting on new indications, given the success of ATL1102 in DMD;
- High quality board with extensive experience in DMD;
- Potential for partnering to further develop compounds; and
- Compounds targeting rare diseases – typically command high prices.

Key risks

ANP is still in the clinical trial / development stage and as such is running at an operating loss. Due to the potential application of the drug across multiple diseases, management has stated its intention to pursue licensing arrangements for a number of other indications in order to extend its cash runway for its core applications in DMD and MS.

ANP is exposed to standard risks within the drug development space:

- **Technology risk** – Inherent risks in the process of developing technology and in the process of discovering, developing and commercialising drugs that can be proven to be safe and effective for use as human therapeutics;
- **Competitor risks** – While the indications ANP is currently targeting are relatively underserved from a therapeutic perspective, there remains a number of alternative therapies that may already exist or are under development that could impinge on ANP's ability to commercialise a product; and
- **Funding risks** – Given ANP's modest cash balance (A$11.6m at 2020 AGM) and operating cash outflow (A$4m for 12-months ended Jun-20), there remains a high likelihood that further capital may be required to continue operations and further trials. ANP noted in recent announcements that it may explore non-dilutive grant funding, or to license out one or more of its potential applications to extend its cash runway and fund the development of its programs.

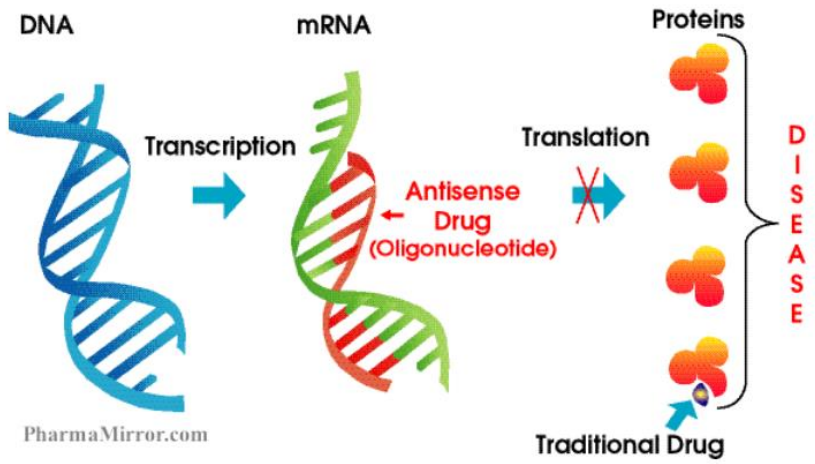
Mechanism of action (MOA)

ANP's primary drug ATL1102 is a second-generation antisense inhibitor of CD49d, a subunit of the VLA-4 (Very Late Antigen-4) receptor found on the surface of lymphocytes (a type of white blood cell). Antisense drugs (designed to bind to complementary messenger RNA, or mRNA, a molecule central to translating DNA into protein) have been used over the years to treat such diseases as cancer, amyotrophic lateral sclerosis (ALS), diabetes, and those with an inflammatory component like asthma and arthritis.

By blocking CD49d production, ATL1102 is thought to reduce the VLA-4 receptor on the surface of lymphocytes (white blood cells involved in the immune response). VLA-4 normally allows lymphocytes to move from the blood vessels into a tissue (which can lead to inflammation) such as the central nervous system (CNS) in MS.

In the case of DMD, patients with high levels of circulating T cells with high levels of CD49d have been found to have more severe and rapid progression of disease (Pinto-Mariz 2015). By reducing these levels within patients, ANP aims to reduce the inflammatory response that is common in all DMD patients. Given its unique anti-inflammatory mechanism and properties, if approved for the treatment of DMD, ATL1102 would likely be applied in concert with other treatments (combination therapy) to combat the inflammation aspect.

Figure 2: Broad overview of how antisense therapies work



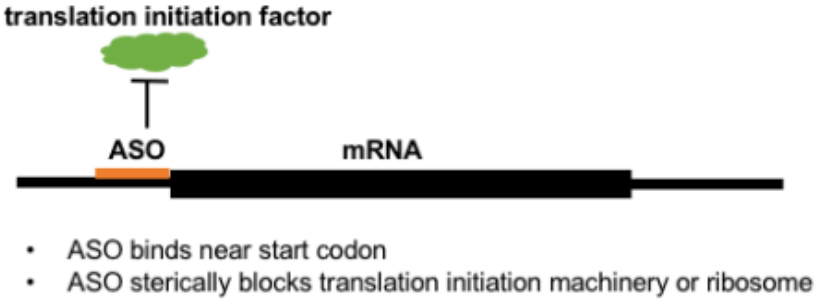
SOURCES: PharmaMirror.com

There are four main types mechanisms of actions (MoA) for antisense oligonucleotides (ASOs) based on its sequence and chemistry. The sequence determines where in the RNA the ASO will bind while the chemistry determines whether the enzymes which cleave the RNA strand or not.

1. **RNA knockdown –** targets mRNA degradation using double-stranded RNA inducing sequence-specific gene silencing. Applications in CNS diseases such as Huntington's Disease, ALS, spinocerebellar ataxia, etc).
2. **Splice Modulation** – molecular manipulation of premessenger RNA splicing engineered to yield genetic correction. Binds in or near an exon of interest and occludes a splice enhancer or represses binding site, sterically blocking splicing machinery within an exon. Otherwise known as exon skipping. Uses in DMD (Sarepta's Etiplerson product) and other spinal atrophies.
3. **Inhibiting translation** – works by binding near the start of mRNA codon and sterically blocks translation initiation machinery or ribosome. It prevents the ribosome from translating the RNA, reducing the target protein levels but maintains RNA levels.
4. **Increasing translation** – complex and still theoretical but aims to increase protein abundance. Works by blocking translation in upstream open reading frames to then promote translation at canonical open reading frames. The process disrupts the inhibitory stem-loop structures and allows more translation initiation factors to bind.

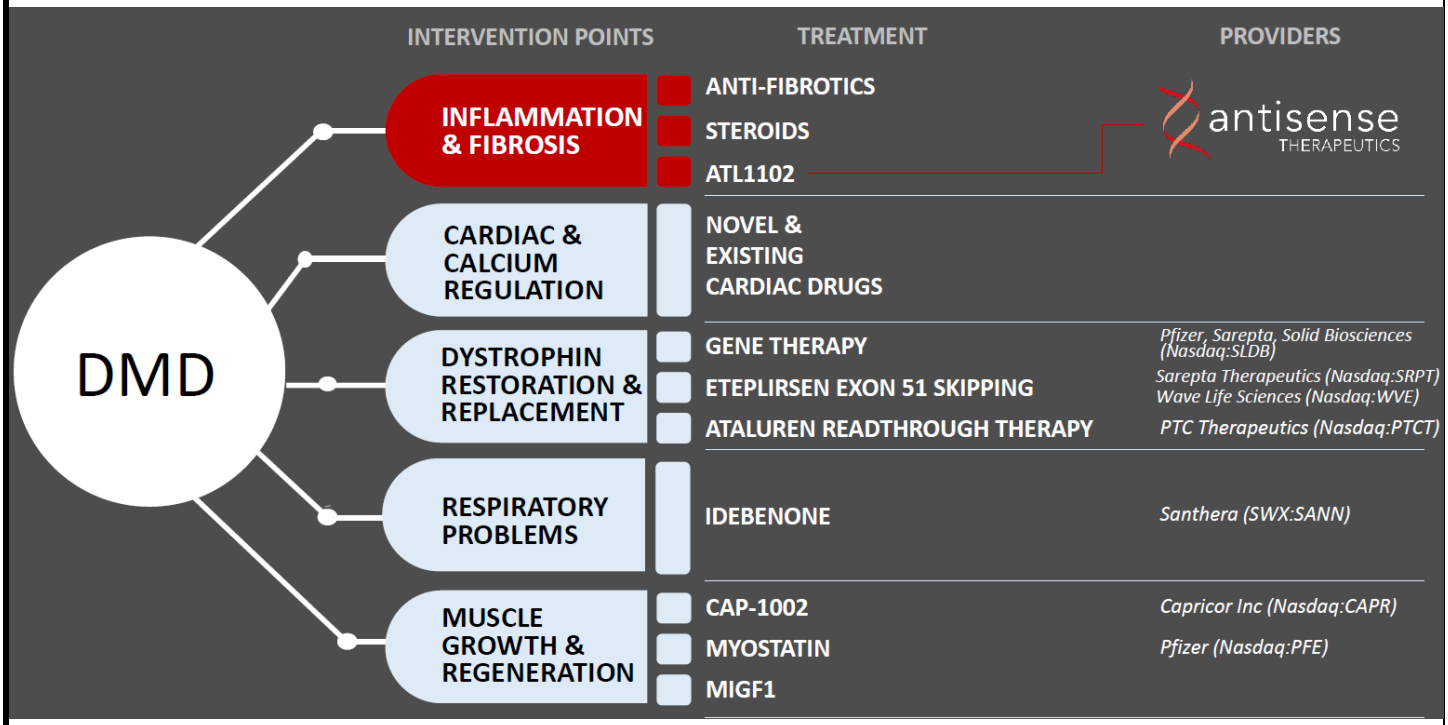
ANP's ATL1102 works through the inhibiting mechanism (MoA #3 above) which binds to the start codon for the gene expression in CD49d antigens. ANP's clinical study results showed consistency in mean reductions in the number of lymphocytes including T-lymphocytes expressing CD49d, with blood levels of this CD49d antigen returning to normal levels after cessation of treatment.

Figure 3: Mechanism of translation inhibition by ASOs



SOURCES: CureFFI.org

Figure 4: ANP's potential role in the treatment of DMD



SOURCES: COMPANY REPORTS

Applications

Due to its mechanism of action, ANP has targeted indications where CD49d is known to be a factor in the progression or severity of the disease and where inflammation remains a critical factor.

Duchenne's Muscular Dystrophy (DMD) – Heading into Ph2b/Ph3

ANP's lead indication (where there is a high unmet clinical need and strong causal link between CD49d and inflammation) is in DMD patients. ANP's studies in DMD patients have shown that the drug is safe and tolerable, shown evidence of modulation in the blood of the target immune cells, and also promising signs of efficacy on a low dose (albeit in a small trial cohort).

Clinical data:

Phase 2a (open-label trial)
Drug: ATL1102
Dosage: 25mg, 1x per week for 24 weeks
n=9, non-ambulatory
Primary Endpoint: Safety and tolerability
Secondary Endpoints:

- Lymphocyte-modulatory response
- PK profile
- Functional capacity
- Respiratory capacity
- Quality-of-life

Results:

ANP released its final report for its Ph2a DMD trial in May 2020, confirming the ATL-1102 safety profile and demonstrating strong effects across its secondary endpoints on activity markers and disease progression which exceeded expectations. The final report (top-line data released in December 2019) presented more detail as well as a number of new efficacy measures and observations that suggest the drug may be having potential disease modifying

effects on muscle strength and function in addition to its primary target of reducing inflammation resulting in muscle fibre damage and degradation in patients.

The data from the study shows an apparent improvement in muscle strength based on the observed mean change from baseline after 24 weeks of dosing with ATL1102 as assessed by MyoGrip (+0.2) and MyoPinch (+0.0) tests compared to the loss of muscle strength reported in the literature in similar patient populations.

The data is suggests an improvement in muscle function as assessed by the Performance of Upper Limb Test (PUL 2.0), where 7 of the 9 participants demonstrated clinically meaningful improvements or stabilisation in their PUL 2.0 scores (+0.9) from baseline after 24 weeks of dosing with ATL1102. MRI data suggests stabilisation of percentage of fat in muscles and preservation of functional muscle mass confirming a positive change at a muscular/cellular level and supports the observed physical stabilisation / improvements in muscle strength and function.

"Based on the MRI data from the study, the observed stabilisation in the percentage fat fraction with ATL1102 treatment would not be expected in the natural course of disease in DMD even under corticosteroid treatment. Furthermore, the stabilisation of fat fraction percentage combined with the observed maintenance / increase of remaining muscle area is suggestive that ATL1102's effect could preserve the contractile muscle mass." – Dr Valeria Ricotti.

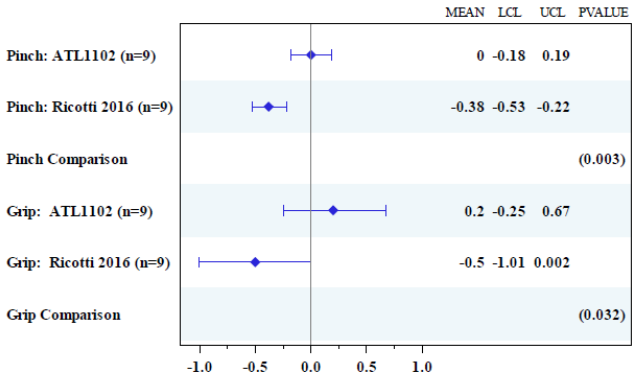
Figure 5: Lymphocyte mean # of cells at week 24

	Change from Baseline to Week 24			
Patient No.	PUL 2.0	MyoGrip (dom) (% Pred)	MyoPinch (dom) (% Pred)	MoviPlate Score (dom)
01-001	+1	-1	0	-1
01-002	+1	0	0	+1
01-003	0	0	-1	0
01-004	+1	0	+1	+1
01-006	-1	0	0	+1
01-008	+1	0	+1	0
01-009	0	-1	-1	+1
01-010	0	0	0	-1
01-011	-1	0	-1	+1
Clinically Meaningful Stabilisation/Improvement	7/9 Participants	7/9 Participants	6/9 Participants	7/9 Participants
Mean Change (95% CI):	0.9 (-1.33, 3.11)	-0.7 (-2.33, 0.90)	-1.0 (-3.56, 1.63)	1.9 (-6.08, 9.85)

A clinically meaningful annual change was considered to be: for the PUL2.0 total score = 4 points, for the MyoGrip and MyoPinch = 3% and for the MoviPlate = 10 points.
A decline was captured as −1, unchanged measurements as 0 and improvement as +1.

SOURCES: COMPANY REPORTS

Figure 6: Mean Change (95% CIs) in Pinch and Grip



ATL1102 Results based on the DOMINANT Side Interim Analysis from the 1102-DMD-CT02 study
Ricotti results based on results from the published paper
PValue: Two-sided p-value from T-Test comparing change between ATL and Ricotti results

SOURCES: COMPANY REPORTS

Multiple Sclerosis (MS) - Phase 2

ANP's study in MS showed promise with strong clinical efficacy reported in a Phase IIa clinical trial of ATL1102 where the drug was dosed at 400mg per week for 8 weeks. The positive trial results were reported in the Journal of Neurology (Limmroth, V. et al Neurology). While the trial was deemed a success, dosage issues were subsequently raised by the FDA in its review of the Company's Investigational New Drug Application (NDA) for a 6 month dosing study in MS patients, with the Company advising that the issue related to an adverse event seen in a pre-clinical monkey toxicology study. The Company stated there was a common scientific view that this adverse finding in monkeys was not a risk factor for humans as highlighted by its absence in the Phase 2a clinical trial. The FDA, however, determined to place a clinical hold on ATL1102, and subsequently changed to a partial hold to allow the study to continue at a starting dosage of 25mg / week (Note: the same 25mg per week dose that has shown activity in the DMD). The Company has continued to file new patent applications to protect the use of ATL1102. Recently international patent application PCT/AU 2018/050598 titled 'Methods for treating multiple sclerosis using antisense oligonucleotides' advanced to the national phase in the US, Australia, New Zealand, Canada and

Europe. When granted this patent family would provide protection for the use of ATL1102 in MS until 2038, potentially extendible for a further 5 years in the US, Australia and Europe.

MS drug sales in 2018 were US$23bn and forecast to grow to US$39bn by 2026 (Fortune Business Insights). The Company is consulting with clinical experts on the appropriate next steps for clinical development in MS while also re-engaging with pharmaceutical companies active in the MS space to discuss partnering opportunities. The Company is following up potential sources for non-dilutive grant funding for a Phase IIb clinical trial of ATL1102 in MS patients.

Clinical data:

Phase 2 (Double-blinded, placebo controlled, randomised)

Drug: ATL1102

Dosage: 2 x 200mg per week for 7 weeks (3 x 200mg dose 1st week)
n=74, Relapse Remitting Multiple Sclerosis (RRMS)

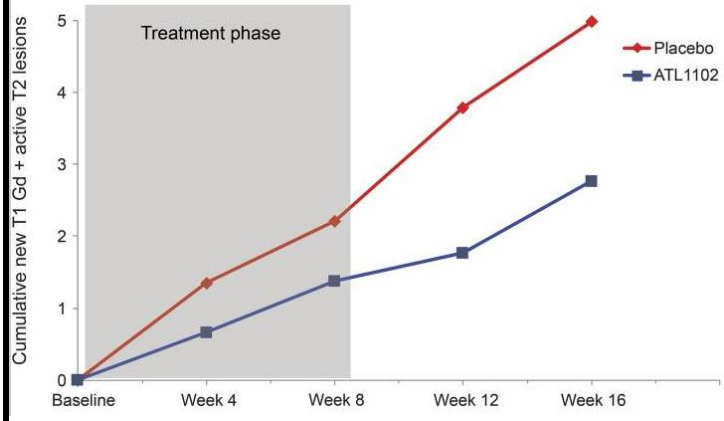
Primary Endpoint: Cumulative number of new active lesions on Magnetic Resonance Imaging (MRI), corrected for the number of enhancing lesions at baseline at 4, 8 and 12 weeks after intervention commencement vs placebo.

Secondary Endpoints: Cumulative volume of gadolinium-enhancing lesions on MRI, corrected for the volume of enhancing lesions at baseline at 4, 8 and 12 weeks after intervention commencement vs placebo

Results:

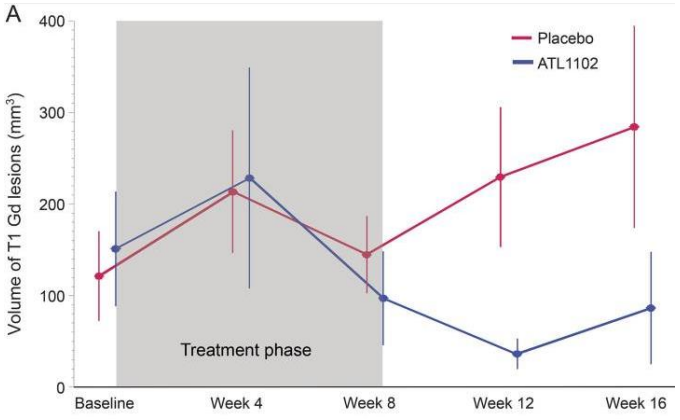
- Adverse events that were more frequent under ATL1102 included mild to moderate injection site reactions and a tendency for decreased platelet counts which were reversible after treatment interruption.
- ATL1102 significantly reduced the cumulative number of new active lesions by 54.4% compared to placebo (6.2 placebo, 3.0 ATL1102; p=0.01).
- A reduction of 66.7% (p=0.002) was observed in the cumulative number (weeks 4,8,12) of new T1-Gd lesions with ATL1102.
- A reduction in 1-Gd lesions was also observed under ATL1102 but did not reach significance (589.4 mm3 placebo, 358.0 mm3 ATL1102; p=0.1068).

Figure 7: Cumulative new lesions



SOURCES: COMPANY REPORTS

Figure 8: Volume of lesions



SOURCES: COMPANY REPORTS

Other applications of ATL1102

Following the reported clinical trial results in the Phase 2 clinical trial of ATL1102 in DMD that affirmed the safety and immunomodulatory activity of the drug on CD49d T cells in the blood with clinical benefits on muscle strength and function,

in parallel with progressing plans for the Phase 2b trial in DMD, ANP announced it is actively exploring clinical development opportunities in other indications where inflammation plays a key role in disease progression. ATL1102 is an antisense inhibitor of CD49d, a subunit of VLA-4 (Very Late Antigen-4).

Antisense inhibition of CD49d expression has demonstrated activity in a number of animal models of inflammatory disease including asthma, arthritis and MS. ANP has made multiple references to its potential for use in other neuroinflammatory and muscular dystrophy disorders given the expected antisense platform and CD49d target based advantages in these applications.

In 2019 ANP filed patent applications to support clinical development and commercialisation of ATL1102 in muscular dystrophies in addition to DMD (PCT/AU2018/051353 & US16/404561).

We expect news on this front over the next 6-12 months and given time / focus / cost constraints, believe it likely that ANP will look to monetise (out-license) some or all of these indications in order to fund and accelerate the development of its DMD program.

Acromegaly – Ph 2 completed (ATL1103)

ATL1103 (also referred to as atesidorsen) is an antisense drug designed to block growth hormone receptor (GHr) expression thereby reducing levels of the hormone insulin-like growth factor-I (IGF-I) in the blood and is a potential treatment for diseases associated with excessive growth hormone action.

By inhibiting GHr production, ATL1103 has shown to reduce IGF-I levels in the blood (serum). A number of diseases are associated with excess GH and IGF-I action. These diseases include acromegaly, an abnormal growth disorder of organs, face, hands and feet. ATL1103 is in clinical development as a treatment for acromegaly. Normalising serum IGF-I levels is the therapeutic goal in the treatment of acromegaly. ANP conducted a Phase 2 trial of ATL1103, meeting its primary efficacy endpoint by showing a statistically significant average reduction in sIGF-1 levels. The results of the Phase II trial were published in the Journal, the European Journal of Endocrinology (Trainer et al, Eur J Endocrinol, 2018 May 22 - 179: 97-108). The Company also conducted a high dose study of ATL1103 in adult patients with acromegaly in Australia. The US FDA and European Commission have granted Orphan Drug designation to ATL1103 for treatment of acromegaly.

While ANP's current development focus is directed towards the clinical development of ATL1102 in DMD, the Company has stated that circumstances could present in the future where the Company has the capacity and justification to continue to invest in further clinical development of ATL1103 and continues to pursue the potential out-licensing of ATL1103 to support and fund its ongoing clinical development.

Forecasts and model assumptions

Due to the early-stage nature of ANP's clinical assets and the risks surrounding clinical trials, we have valued the company on failure and success, as well as variations to the risk profile upon successful outcomes.

We have run a risk-adjusted model based on each case, anticipated partnership agreements and milestone payments to bring the product through to commercialisation, and subsequent royalty generation from the DMD program. While we note ANP has a number of possible indications, we remain focused on the DMD asset which we believe has the clearest near-term value and which the market is tracking and prescribing value to.

Focus on US / EU sales for DMD indication: We focus on the US and EU markets only (~90% of global market) due to maturity of market, clearer regulatory pathways, and strong medicare / health insurance coverage systems.

There are a number of companies with the view that gene therapy is the solution to treat the underlying disease which may render ATL1102 obsolete. We disagree

on two fronts: 1) ATL1102 is likely to work in combination with other therapies to treat inflammation characteristics of disease, and 2) limited efficacy and safety claims in the space to date – unlikely to see an approved product with broad claims (i.e. Sarepta's etiplersen products only amendable to ~13% of patients who carry a specific gene). There are also concerns amongst gene therapies about duration of efficacy.

Within our model, we have assumed:

- ANP to partner after a Ph2b trial success in Europe. We have modelled ATL1102 as achieving conditional approval in the EU upon successful Ph2b results.
- Addressable market for ATL1102 of ~22k cases pa;
    - Based on ANP estimation of US/EU DMD populations of 48k patients

      Multiplied by:
    - 50% prevalence of patients being non-ambulant

      Multiplied by:
    - 25% population due to ANP formalising clinical trial for EMA at this stage.
- US$150k price per treatment (Morgans Estimates – in line with DeflazaCorp corticosteroid drug ~US$150k) of which ANP to receive an ongoing 20% royalty from sales (Morgans estimates – low estimate); and
- Forecast ANP to incrementally grow up to 50% of market penetration over a 5 year period of a 20 year life cycle of drug.

Considering DMD affects 1-in-3,600 boys: 1bn (broadly # humans in EU and US) x 50% (males) / 3600 (DMD prevalence) x 50% (non-ambulant) = 70k total addressable population in target markets, we consider ANP's total addressable market assumptions of~31% to be relatively conservative**.**

While we view it highly likely that ATL1102 would also be used in ambulant boys (same market size) as an off-label use – we remain focused on the immediate-term market potential and reserve this as further upside.

At this stage we only focus on the DMD asset with other indications (MS / Acromegaly) providing upside to our valuation scenario as the clinical development path becomes clearer and derisked.

We have also assumed a A$30m capital raise in FY22 to fund costs of the EU trial.

Figure 9: ANP market assumptions

MARKET DATA	#	Comments
Population of target market ('000s)	48.0	DMD population US and EU (ANP)
Regulatory approval weight	25.0%	EU / US split
Non-ambulant population	50.0%	
Number of Cases Forecast for Year 1 ('000s)	6.0	
Annual Population Growth	0.70%	Worldbank, 2016
Peak Market Penetration	50.0%	
Revenue Per Unit ($US)	$ 150,000	ANP - analysis of competing therapies
Market Ramp Time to Peak Penetration (Years	5	Study of peak penetration rates. Blockbuster drugs
Hold peak	10	
Life cycle of drug	20	

SOURCE: MORGANS RESEARCH, COMPANY

We have applied a discount to our forecast cashflows to reflect historical success rates of drugs at various stages of FDA / EMA approval and the likelihood of ATL1102 achieving commercial success. Achievement of clinical stage success would result in risk mitigation in line with the phase level in the table below (i.e. royalty risk mitigation discounts reduce from 48% to 7%).

Figure 10: Success rates by therapeutic area: Benchmarks

Therapeutic Area	Phase 1	Phase 2	Phase 3	Approval	Cumulative
Arthritis & Pain	77%	38%	78%	89%	20%
Central Nervous System	66%	46%	62%	78%	15%
Cardiovascular	63%	43%	76%	84%	17%
Gastrointestinal	67%	49%	71%	86%	20%
Immunology	65%	45%	65%	82%	15%
Infectious Diseases	71%	51%	80%	97%	28%
Metabolism	48%	52%	79%	93%	18%
Oncology	77%	44%	62%	85%	18%
Respiratory Diseases	63%	41%	60%	77%	12%

SOURCE: TuftsUniversity, 2010; Villiger, 2012

We have assumed a total spend of A$35m for ANP to bring ATL1102 to market in Europe which includes a Ph2b trial and subsequent open-label follow-on trial which will likely require a capital raise in the short to medium term. These expenses are split over FY22 / 23 / 24 at which point we model ANP to enter into a partnership agreement to commercialise, scale, market, and distribute ATL1102.

At this stage, given the sizeable and potential near-term commercialisation opportunity we view ANP as more likely to be a takeover target. However, given the subjective and variable nature for prices paid for these types of assets, we will continue to model ANP under a partnership model which we believe provides the best risk-weighted outcome for shareholders.

We forecast office and admin costs to increase by 5% over the last reported period pa.

Milestone payment: The size and structure of a potential milestone payment is one of the largest drivers to ANP's short/medium term value. The up-front amount, timing and deal terms of the subsequent milestone payments vary materially from deal to deal.

We assume ANP will partner to fund registration, marketing, and manufacturing for rollout across EU upon successful EMA trial and then bare costs of US pivotal trials. We have forecast a potential total deal value worth US$350m. We have a high front-end load to our milestone payments due to decreased risk of commercialisation (typically ~10%). We forecast 25% upfront, and 75% on marketing approval and commercialisation milestones.

We have applied a progressive probability model based on studies on historical success rates and are set out in Figure 11 below.

Figure 11: Forecast milestone payments and payment risks

Phase	Progression risk	M'stone	MS $	rMS $
Start	100%	350		
Pre-clinical	100%			
Phase 1	100%			
Phase 2	52%	0%	0	
Phase 3	100%	n.a.		
Approved	93%	25%	88	81
Commer	100%	75%	263	263

MS = Milestone value
rMS = Milestone value post progression risk applied

SOURCE: MORGANS RESEARCH

Valuation

High and low cases

We have valued ANP using a risked valuation methodology although highlight both low and high cases which considers the risks associated with clinical stage assets.

Low case (A$0.02): Sum of parts methodology. Based on unsuccessful trial. Valued on cash backing and net assets.

Risked valuation (A$0.38): DCF methodology. Risked assumptions based on average historical success rates for metabolic drugs and only European approval. Assumes only EU approval. ANP to partner for EU distribution rights and global pharma to run US trial. Multiple assumptions including addressable market, drug pricing, peak penetration rates, potential partnership deal and structures.

De-risked valuation (A$1.82): DCF methodology. Risked assumptions set to 100%, EU and US approval. Commercial success. ANP to partner for EU distribution rights and global pharma to run US trial. Multiple assumptions including addressable market, drug pricing, peak penetration rates, potential partnership deal and structures.

Price target

We use our risked valuation of 38 cps as our base case upon which we set our price target. While we view these scenarios and risk factors as conservative, we note the wide range of possibilities varying from trial failure up to approval in all major jurisdictions and any risk adjustments along the way.

Given the wide range of possibilities and subsequent valuation, we have initiated coverage on ANP with a Speculative Buy recommendation to highlight the risk/reward nature of the asset.

Sensitivity analysis

We have conducted a sensitivity analysis across our model assumptions. We have highlighted six forecast assumptions which we believe are most sensitive to changes and would result in the greatest variance to our forecasts. It should be noted that this sensitivity analysis treats each assumption in isolation. Furthermore, the changes in the key variables are not intended to be indicative of the complete range of variables that may be experienced. In practice, changes in variables may offset each other or be additive and it is likely that ANP management would proactively respond to any adverse changes.

Due to the nature of the pre-approved pharmaceutical assets and reliance on regulatory approval to be able to generate material cashflows, the value of the business is predominantly based on future revenues. For this reason, rather than place emphasis on impact to NPAT within a particular financial year, we focus on the impact to our weighted valuation.

Figure 12: Sensitivity analysis

Assumption variable	Current value	Change in value	Change in valuation
Selling price of ATL1102	US$150k	+/- $US50k	+ / - 6cps
Immediate patient population #	6,000	+/- 10%	+ / - 2cps
Discount rate	12.68%	+/- 1%	+ / - 3cps
Milestone total deal value	US$350k	+/- 10%	+ / - 3cps
Royalty rate	20%	+/- 5%	+ / - 7cps
Peak market penetration	50%	+/- 10%	+ / - 4cps

SOURCE: MORGANS RESEARCH, COMPANY

Comparable companies

As a further check to our risked valuation, we highlight 13 Australian listed life science companies that are currently undertaking Phase 2 or Phase 3 clinical development. The average enterprise value (EV) of this cohort is A$438m compared with ANP's EV of A$57.1m (Factset).

If we review the global setting, ANP's closest peer is Sarepta Therapeutics (NASDAQ:SRPT), a DMD focused company whose market cap was US$60m in 2012 when the company began clinical development of Exondys 51 – a treatment for increasing dystrophin levels in boys with the exon 51 mutation. Exondys 51 gained FDA approval in 2016, with last quarter sales in the US approaching US$100m, now Sarepta has a market cap of US$15.2bn.

Sarepta's treatment is only applicable to 13% of boys with the exon 51 mutation, while ANP's ATL1102 is aimed at a much broader application.

Former Chairman of Sarepta, William (Bill) Goolsbee, is a director on ANP's Board. Antisense recently announced appointment of a US-based Consultant Medical Director Dr Gil Price (also ex-Sarepta Therapeutics Director) to accelerate development activities in the US and to engage with Key Opinion Leaders, DMD Advocacy Groups, industry and capital markets participants.

A full list of comparable companies is detailed in Figure 13 overleaf.

Figure 13: Comparable domestic comps

Ticker	Company name	Business description	Phase	EV ($m AUD)	Method of action	Target indication
ANP-AU	Antisense Therapeutics Limited	Antisense Therapeutics Ltd. engages in the research and development of novel antisense pharmacceuticals. It operates through the ATL1102, and ATL1103 segments. The ATL1102 segment represents the second generation antisense inhibitor of CD49d, the alpha subunit of very late antigen-four. The ATL1103 segment refers to atesidorsen is an antisense drug designed to block growth hormone receptor expression thereby reducing levels of the hormone insulin. The company was founded on November 13, 2000 and is headquartered in Toorak, Australia.	Ph2	57.61	Antisense Therapy	Duchennes Muscular Dystrophy Multiple Sclerosis Acromegaly
Australian listed drug development companies						
PAR-AU	Paradigm Biopharmaceuticals Ltd.	Paradigm Biopharmaceuticals Ltd. is a biopharmaceutical company, engages in researching and developing therapeutic products for human use. It is a drug repurposing company which seeks to find new uses for old drugs. Paradigm Biopharmaceuticals was founded on May 2, 2014 and is headquartered in Melbourne, Australia.	Ph3	384.89	Inflammation	Osteoarthritis
NEU-AU	Neuren Pharmaceuticals Limited	Neuren Pharmaceuticals Ltd. is a biopharmaceutical company, which engages in the development of new therapies for brain injury, neurodevelopment and neurodegenerative disorders. The company was founded on December 17, 2001 and is headquartered in Camberwell, Australia.	Ph2 / Ph3	122.05	Neurotrophic peptides	Retts Syndrome Fragile X Syndrome Pitt Hopkins Syndrome Phelan-McDermid Syndrome Angelman Syndrome
MSB-AU	Mesoblast Limited	Mesoblast Ltd. is a biopharmaceutical company, which engages in the research, development, and market of mesenchymal lineage adult stem cell technology platform. Its medicines target the cardiovascular diseases, spine orthopedic disorders, oncology and hematology, immune-mediated, and inflammatory diseases. The company was founded by Itescu Silviu on June 8, 2004 and is headquartered in Melbourne, Australia.	Ph 3	1,310.57	Stem-cell	Cardiovascular Graft-versus-host
TLX-AU	Telix Pharmaceuticals Ltd.	Telix Pharmaceuticals Ltd. engages in the development and commercialization of several clinical-stage oncology assets. It focuses on cancer care, specifically in prostate, renal or kidney and glioblastoma or brain cancer. Its products include TX250, TX591, and TX101. The company was founded by Andreas Kluge and Christian P. Behrenbruch in November 2015 and is headquartered in Melbourne, Australia.	Ph 3	972.32	Molecular targeted radiation	Oncology
OPT-AU	Opthea Limited	Opthea Ltd. is a clinical stage biopharmaceutical company that engages in the development of biological therapeutics for the treatment of progressive retinal diseases. It targets to treat neovascular age-related macular degeneration (wet AMD) and diabetic macular edema, which causes visual impairment among elderly and diabetic patients. The company was founded on October 17, 1984 and is headquartered in South Yarra, Australia.	Ph3	467.66	VEGF	Wet Age-related Macular Degeneration
BOT-AU	Botanix Pharmaceuticals Limited	Botanix Pharmaceuticals Ltd. engages in the development of therapeutics for the treatment of skin diseases. It focuses on the treatment of patients battling with acne, psoriasis, and atoptic dermatitis. The company also develops pharmaceutical ingredient known as cannabidol, which seeks to treat epilepsy, pain, arthritis, and schizophrenia. Botanix Pharmaceuticals was founded by Roger New and Glen Travers in July 2000 and is headquartered in Northbridge, Australia.	Ph2	92.82	Inflammation	Dermatology
BIT-AU	Biotron Limited	Biotron Ltd. is a clinical stage Australian biotechnology company. It engages in developing and commercializing a novel small molecule approach that has the potential to treat a number of serious viral diseases. The company's technology targets viroporin proteins, which are key to enabling the pathogenicity of a number of viruses including hepatitis C, HIV-1, Dengue, Zika, Influenza and Respiratory Syncytial Virus. Its proprietary primary bacterial cell-based screening platform enables rapid screening for target viroporin proteins. The company was founded in February 1999 and is headquartered in North Ryde, Australia.	Ph2	44.72	Viroporin inhibitor	HIV
RAC-AU	Race Oncology Ltd.	Race Oncology Ltd is a pharmaceutical company, whose business model is to pursue later stage drug assets, principally in the cancer field. Its first important asset is a chemotherapy drug, called Bisantrene, which is used as the first line of treatment for Acute Myeloid Leukaemia and many other cancers. The company was founded on February 15, 2011 and is headquartered in Melbourne, Australia.	Ph2	186.94	Anthracyclines	Oncology
KZA-AU	Kazia Therapeutics Ltd	Kazia Therapeutics Ltd. engages in the pharmaceutical drug research and development. Its pipeline includes two clinical-stage drug development candidates such as GDC-0084, and Cantrixil. The company was founded by Graham Edmund Kelly in March 1994 and is headquartered in Sydney, Australia.	Ph2	85.00	PI3K modulation	Oncology
Average				**407.44**		

SOURCES: MORGANS, FACTSET

Figure 14: Comparable global comps

Ticker	Company name	Business description	Phase	EV ($m AUD)	Method of action	Target indication
Global comps (DMD)						
SRPT-US	Sarepta Therapeutics, Inc.	Sarepta Therapeutics, Inc. is a commercial-stage biopharmaceutical company, which is engaged in the discovery and development of therapeutics for the treatment of rare diseases. The company was founded on July 22, 1980 and is headquartered in Cambridge, MA.	Commercial	15,221.03	Exon Skipping	Duchennes Muscular Dystrophy
SLDB-US	Solid Biosciences Inc.	Solid Biosciences, Inc. engages in the development of treatments for patients with Duchenne muscular dystrophy. It develops gene therapies, disease modifying therapies, and assistive devices for the cure of DMD. The company was founded by Ilan Ganot, Andrey J. Zarur, Matthew Arnold, Annie Ganot and Gilad David Hayeem in March 2013 and is headquartered in Cambridge, MA.	Ph1	438.19	Gene Therapy	Duchennes Muscular Dystrophy
WVE-US	Wave Life Sciences Ltd.	Wave Life Sciences Ltd. is a biotechnology company, which engages in the development of proprietary synthetic chemistry drug. It focuses on the design, development, and commercialization of nucleic acid-based therapeutics. The company was founded by Gregory L. Verdine and Takeshi Wada on July 23, 2012 and is headquartered in Singapore.	Ph1/2a	170.64	Exon Skipping	Duchennes Muscular Dystrophy
PTCT-US	PTC Therapeutics, Inc.	PTC Therapeutics, Inc. is a biopharmaceutical company, which engages in the discovery and commercialization of clinically-differentiated medicines. It focuses on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology. The company was founded by Allan Steven Jacobson and Stuart W. Peltz on March 31, 1998 and is headquartered in South Plainfield, NJ.	Ph3	5,583.11	Ataluren Readthrough Therapy	Duchennes Muscular Dystrophy
SANN-CH	Santhera Pharmaceuticals Holding AG	Santhera Pharmaceuticals Holding AG engages in the development and commercialization of products for the treatment of neuromuscular and pulmonary diseases. Its product, Raxone, focuses on the treatment of Leber's hereditary optic neuropathy. The company was founded 1998 and is headquartered in Pratteln, Switzerland.	Ph2	111.82	Idebenone	Duchennes Muscular Dystrophy
Median				**438.19**		
Average				**4,304.96**		

SOURCES: MORGANS, FACTSET

Board and Scientific Advisory Board

Figure 15: Board of Directors

Position	Name	Description
NE Chairman	Robert Moses	Formerly Corporate Vice President of CSL Limited. Mr. Moses draws on more than 40 years' experience in the pharmaceutical/biotechnology industry.
MD/CEO	Mark Diamond	Over 30 years' experience in the pharmaceutical & biotechnology industry. Formerly Director, Project Planning/Business Development at Faulding Pharmaceuticals in the USA, Senior Bus Dev Manager within Faulding's European operation & International Business Development Manager with Faulding in Australia.
NED	Dr Graham Mitchell	Joint Chief Scientist for the Victorian Government Department of Environment & Primary Industries. Formerly Director of Research in the R&D Division of CSL Limited.
NED	Dr Gary Pace	Dr Pace has more than 40 years' international experience in the development & commercialisation in biotechnology/pharmaceuticals industries. Long-term board level experience with both multi-billion & small cap companies.
NED	William Goolsbee	Founder, Chairman & CEO of Horizon Medical Inc. 1987 –2002 until acquisition by UBS Private Equity. Founding Director then Chairman of ImmunoTherapy Corporation until acquisition by AVI Biopharma, Inc. (now Sarepta Therapeutics). Former Chairman of privately held BMG Pharma LLC & Metrodora Therapeutics.

SOURCES: MORGANS, COMPANY REPORTS

Figure 16: Scientific Advisory Board (SAB)

Position	Name	Description
Principle Investigator	Dr Ian Woodcock MD	Royal Children's Hospital (RCH) Neuromuscular Fellow, Melbourne Australia
Co-Investigator	Professor Monique Ryan MD	Director Neurology Department, Head of Royal Children's Hospital, Neuromuscular ClinicRCH, MCRI, Melbourne Australia
	Professor Steve Wilton Ph.D	Western Australian Neuroscience Research Institute (NRI), Foundation Chair in Molecular Therapy at Murdoch University, Perth, Western Australia: Patent holder on target sequence of Sarepta's drug eteplirsen & additional exon-skipping drugs
	Professor Sue Fletcher PhD	Principal Research Fellow, NRI Murdoch University, Perth, Western Australia: Patent holder on target sequence of Sarepta's drug eteplirsen & additional exon-skipping drugs
	Dr Gillian Butler-Browne PhD	Director, Centre of Research in Myology, Sorbonne Universités, INSERM, Paris, France: Expert in inflammatory muscle disease, author of CD49d Skeletal Muscle 2015 research paper
SAB Chairman	Mr William Goolsbee	Antisense Therapeutics Ltd, non-executive director: Chairman, Sarepta Therapeutics, 2010-2014, Developers of eteplirsen for the treatment of DMD

SOURCES: MORGANS, COMPANY REPORTS

Queensland

Brisbane	+61 7 3334 4888
Stockbroking, Corporate Advice, Wealth Management	
Brisbane: Edward St	+61 7 3121 5677
Brisbane: Tynan Partners	+61 7 3152 0600
Brisbane: North Quay	+61 7 3245 5466
Bundaberg	+61 7 4153 1050
Cairns	+61 7 4222 0555
Caloundra	+61 7 5491 5422
Gladstone	+61 7 4972 8000
Gold Coast	+61 7 5581 5777
Holland Park	+61 7 3151 8300
Ipswich/Springfield	+61 7 3202 3995
Kedron	+61 7 3350 9000
Mackay	+61 7 4957 3033
Milton	+61 7 3114 8600
Newstead	+61 7 3151 4151
Noosa	+61 7 5449 9511
Redcliffe	+61 7 3897 3999
Rockhampton	+61 7 4922 5855
Spring Hill	+61 7 3833 9333
Sunshine Coast	+61 7 5479 2757
Toowoomba	+61 7 4639 1277
Townsville	+61 7 4725 5787

New South Wales

Sydney	+61 2 9043 7900
Stockbroking, Corporate Advice, Wealth Management	
Sydney: Grosvenor Place	+61 2 8215 5000
Sydney: Reynolds Securities	+61 2 9373 4452
Sydney: Currency House	+61 2 8216 5111
Armidale	+61 2 6770 3300
Ballina	+61 2 6686 4144
Balmain	+61 2 8755 3333
Bowral	+61 2 4851 5555
Chatswood	+61 2 8116 1700
Coffs Harbour	+61 2 6651 5700
Gosford	+61 2 4325 0884
Hurstville	+61 2 8215 5079
Merimbula	+61 2 6495 2869
Mona Vale	+61 2 9998 4200
Neutral Bay	+61 2 8969 7500
Newcastle	+61 2 4926 4044
Orange	+61 2 6361 9166
Port Macquarie	+61 2 6583 1735
Scone	+61 2 6544 3144
Wollongong	+61 2 4227 3022

Victoria

Melbourne	+61 3 9947 4111
Stockbroking, Corporate Advice, Wealth Management	
Brighton	+61 3 9519 3555
Camberwell	+61 3 9813 2945
Domain	+61 3 9066 3200
Geelong	+61 3 5222 5128
Richmond	+61 3 9916 4000
South Yarra	+61 3 8762 1400
Southbank	+61 3 9037 9444
Traralgon	+61 3 5176 6055
Warrnambool	+61 3 5559 1500

Australian Capital Territory

Canberra	+61 2 6232 4999

Northern Territory

Darwin	+61 8 8981 9555

Tasmania

Hobart	+61 3 6236 9000

Western Australia

West Perth	+61 8 6160 8700
Stockbroking, Corporate Advice, Wealth Management	
Perth	+61 8 6462 1999

South Australia

Adelaide	+61 8 8464 5000
Exchange Place	+61 8 7325 9200
Norwood	+61 8 8461 2800
Unley	+61 8 8155 4300