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Antisense (Percheron) Therapeutics (ANP)

29 November 2023

Speculative Investment

Recommendation: Outperform

Looking forward to key DMD efficacy readout in 2024

Our View

Antisense (ANP) is currently enrolling patients in a randomised Phase IIb trial of its lead drug ATL1102 in non-ambulant patients with the fatal inherited muscle wasting disease Duchenne muscular dystrophy (DMD). It expects to report topline data in H2 CY24. Patients treated with ATL1102 in a small Phase IIa study showed encouraging improvement in upper limb function and preservation of muscle mass, in marked contrast to the progressive deterioration that is the hallmark of the disease.

The Phase IIb trial will primarily serve as a signal finding study to determine whether the drug has sufficient efficacy to be an effective treatment for DMD. It is likely to form the basis for discussions with potential partners who could undertake the final steps in clinical development and commercialise the drug. However, if the results of the Phase IIb trial are sufficiently positive, they could potentially be used to support an application for marketing approval of ATL1102.

At the recent AGM shareholders voted to change the company's name to Percheron Therapeutics. The name change is expected to be implemented in the next few weeks. It is proposed that the ticker will change to ASX.PER.

The company had $19.2m cash on 30 September, which gives it a funding runway beyond the expected release of topline data from the Phase IIb trial in H2 CY24.

We resume coverage with an **Outperform** recommendation and a valuation of $136m or **$0.15/sh**.

Key Points

ATL1102 stops the VLA-4 integrin connection that allows inflammatory cells to survive, proliferate and migrate across the vessel walls into the muscle tissue where they attack damaged and non-damaged muscle fibres. Its efficacy in reducing inflammation was confirmed in a previous randomised trial in multiple sclerosis, where it significantly reduced inflammation and disease progression.

Improved muscle function in DMD patients treated with ATL1102 – in a Phase IIa study that treated 9 boys with advanced DMD with ATL1102 for 24 weeks, the boys showed improvement or stabilisation in measures of upper limb strength and function. The most impressive result, in our view, was the average 0.9 point improvement in the validated Performance of the Upper Limb (PUL) 2.0 scale observed in ATL1102 treated patients, compared to the ongoing deterioration in PUL scores reported for DMD patients in the 7 other studies that we examined. The impressive PUL 2.0 results were supported by improvement or stabilisation of scores for the MyoSet test set, a validated suite of tools to assess strength and endurance of the upper limb.

Premium pricing for DMD products – the DMD market is expected to be worth US$27bn by 2030. As is common for rare diseases, DMD treatments command high prices. Prices in the US for DMD treatments approved in recent years (excluding gene therapies) range from US$81,000 to US$300,000 per patient per year, with list prices reportedly as high as US$892,000 per patient per year.

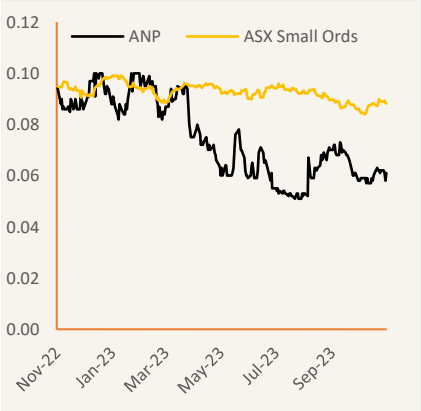
Eligible for a valuable Rare Pediatric Disease Priority Review Voucher - The FDA has granted rare pediatric disease (RPD) designation for ATL1102 for the treatment of DMD. This means that if ATL1102 gains FDA approval it may be eligible for the award of a priority review voucher (PRV), which can be freely traded and which have recently sold for around US$100m.

Our conflicts of interests are disclosed on the last page of this report.

Summary (AUD)

Market Capitalisation	**$54m**
Share price	**$0.06**
52 week low	$0.05
52 week high	$0.11
Cash as of 30 September 2023	$19.2m

Share price graph (AUD)



Key Financials (AUDm)

	FY23A	FY24F	FY25F
Revenue	1.6	4.3	2.2
R&D	(10.2)	(11.4)	(5.4)
SG&A	(3.1)	(3.0)	(3.1)
EBITDA	(11.7)	(10.1)	(6.3)
Reported NPAT	(11.4)	(10.0)	(6.2)
NPAT Adj.	(11.4)	(10.0)	(6.2)
EPS Adj. (c)	(1.7)	(1.3)	(0.7)
PE ratio (x)	n/a	n/a	n/a
DPS (c)	0.0	0.0	0.0
EV/Sales	n/a	n/a	n/a
EV/EBITDA (x)	n/a	n/a	n/a
ROE	n/a	n/a	n/a

Antisense Therapeutics - Summary of Forecasts — ANP $ 0.06

PROFIT & LOSS SUMMARY (A$m)

Year end June	FY23A	FY24F	FY25F	FY26F
Sales, royalties, milestones	0.0	0.0	0.0	0.0
Other (includes R&D tax rebate	1.6	4.3	2.2	1.0
Total Revenue	**1.6**	**4.3**	**2.2**	**1.0**
Growth (pcp)	-11.1%	173.4%	-50.0%	-55.6%
R&D Expenses	(10.2)	(11.4)	(5.4)	(2.4)
SG&A expenses	(3.1)	(3.0)	(3.1)	(3.2)
EBITDA	**(11.7)**	**(10.1)**	**(6.3)**	**(4.6)**
Dep'n/Other Amort'n	(0.1)	(0.0)	(0.0)	(0.0)
EBIT	**(11.8)**	**(10.1)**	**(6.3)**	**(4.6)**
Net Interest	0.4	0.1	0.1	0.1
Pre-Tax Profit	**(11.4)**	**(10.0)**	**(6.2)**	**(4.6)**
Tax Expense	0.0	0.0	0.0	0.0
Minorities	0.0	0.0	0.0	0.0
NPAT	**(11.4)**	**(10.0)**	**(6.2)**	**(4.6)**
Growth (pcp)	-	-	-	-
Adjustments	0.0	0.0	0.0	0.0
NPAT Reported	(11.4)	(10.0)	(6.2)	(4.6)

PER SHARE DATA*

Year end June	FY23A	FY24F	FY25F	FY26F
EPS (c) - Reported	**(1.7)**	**(1.3)**	**(0.7)**	**(0.5)**
Growth (pcp)	85.6%	-25.3%	n/a	n/a
EPS (c) - Adjusted	(1.7)	(1.3)	(0.7)	(0.5)
Growth (pcp)	85.6%	-25.3%	n/a	n/a
Gross CF per share (c)	(1.2)	(1.6)	(0.4)	(0.3)
NTA per share (c)	1.5	1.3	0.6	0.1
Dividend (c)	0.0	0.0	0.0	0.0
Franking	0.0	0.0	0.0	0.0

KEY RATIOS

Year end June	FY23A	FY24F	FY25F	FY26F
Current ratio (x)	4.5	5.0	2.9	1.4
Net Debt : Equity (%)	-109%	-84%	-106%	-221%
Net Debt: EBITDA (x)	0.9	0.9	0.9	0.6
ROE (%)	n/a	n/a	n/a	n/a
ROIC (%)	n/a	n/a	n/a	n/a
Dividend Payout Ratio (%)	n/a	n/a	n/a	n/a

VALUATION MULTIPLES

Year end June	FY23A	FY24F	FY25F	FY26F
Reported PE Ratio (x)	**n/a**	**n/a**	**n/a**	**n/a**
Adjusted PE Ratio (x)	**n/a**	**n/a**	**n/a**	**n/a**
Dividend Yield (%)	0.0%	0.0%	0.0%	0.0%
EV/Sales (x)	n/a	n/a	n/a	n/a
EV/EBITDA (x)	n/a	n/a	n/a	n/a
EV/EBIT (x)	n/a	n/a	n/a	n/a

CAPITAL RAISING ASSUMPTIONS

Year end June	FY23A	FY24F	FY25F	FY26F
Shares Issued (m)	0.0	232.8	0.0	0.0
Issue Price (A$)	0.12	0.05	0.10	0.10
Gross Cash Raised (A$m)	0.0	11.6	0.0	0.0

BALANCE SHEET SUMMARY

Year end June	FY23A	FY24F	FY25F	FY26F
Cash + Cash Equivalents	11.0	9.7	6.0	3.0
Receivables	1.7	4.4	2.2	1.0
Inventories	0.0	0.0	0.0	0.0
Other	0.1	0.1	0.1	0.1
Total Current Assets	12.7	14.1	8.3	4.1
Inventories	0.0	0.0	0.0	0.0
PP&E	0.2	0.1	0.1	0.1
Intangibles	0.0	0.0	0.0	0.0
Other	0.0	0.0	0.0	0.0
Total Non-Current Assets	0.2	0.1	0.1	0.1
TOTAL ASSETS	**12.8**	**14.3**	**8.4**	**4.1**
Accounts Payable	2.5	2.5	2.5	2.5
Borrowings	0.1	0.1	0.1	0.1
Provisions	0.2	0.2	0.2	0.2
Other	0.0	0.0	0.0	0.0
Total Current Liabilities	2.8	2.8	2.8	2.8
Borrowings	0.0	0.0	0.0	0.0
Provisions	0.0	0.0	0.0	0.0
Other	0.0	0.0	0.0	0.0
Total Non-Current Liabilities	0.1	0.1	0.1	0.1
TOTAL LIABILITIES	**2.9**	**2.9**	**2.9**	**2.9**
TOTAL EQUITY	**10.0**	**11.4**	**5.5**	**1.3**

CASH FLOW SUMMARY

Year end June	FY23A	FY24F	FY25F	FY26F
EBIT (excl Abs/Extr)	**(11.8)**	**(10.1)**	**(6.3)**	**(4.6)**
Add: Dep'n & Amort'n	0.1	0.0	0.0	0.0
Other non-cash items	3.0	0.3	0.3	0.4
Less: Tax paid	0.0	0.0	0.0	0.0
Net Interest	0.3	0.1	0.1	0.1
Change in Rec.	0.2	(2.7)	2.2	1.2
Change in Inv.	0.0	0.0	0.0	0.0
Gross Cashflows	(8.2)	(12.4)	(3.7)	(3.0)
Capex	(0.0)	0.0	0.0	0.0
Free Cashflows	(8.2)	(12.4)	(3.7)	(3.0)
Share Issue Proceeds	0.0	11.1	0.0	0.0
Other	(0.1)	0.0	0.0	0.0
Dividends Paid	0.0	0.0	0.0	0.0
Net Cash Flow	**(8.3)**	**(1.3)**	**(3.7)**	**(3.0)**
FX Effect on Cash	0.0	0.0	0.0	0.0

ANP valuation summary

	Probability (%)	Valuation (A$m)	Valuation A$/share)
ATL1102 RoW non-ambulant DMD	25%	45.9	0.05
ATL1102 US non-ambulant DMD	20%	54.2	0.06
ATL1102 Ambulant DMD	20%	32.7	0.04
RPD Priority Review Voucher	16%	4.4	0.00
SG&A	-	(11.7)	(0.01)
Portfolio total	-	125.4	0.14
Cash end FY24e	-	9.7	0.01
Total Valuation	-	136.1	0.15

Contents

Overview

Antisense Therapeutics is developing ATL1102 as a treatment for the inflammation that plays an important role in the loss of muscle function in boys with the inherited neuromuscular disease known as Duchenne muscular dystrophy (DMD).

ATL1102 blocks a key inflammatory signal which facilitates survival, proliferation and migration of white blood cells from the bloodstream to the site of inflammation. The inflammatory signal is integrin α4, also known as CD49d, the alpha subunit of VLA-4, an adhesion molecule expressed on most white blood cells. ATL1102 is an antisense oligonucleotide drug that triggers the breakdown of the messenger RNA (mRNA) that carries the instructions for integrin α4 from the nucleus to the site of protein synthesis, thereby inhibiting the expression of integrin α4 on the cell surface. ATL1102 is based on technology in-licenced from Ionis Pharmaceuticals (NASDAQ:IONS), which has commercialised 3 drugs based on its antisense technology.

ANP originally investigated ATL1102 as a treatment for multiple sclerosis (MS), but it pivoted to developing ATL1102 as a treatment DMD after a study published in 2015 identified CD49d as a promising therapeutic target in that condition. While DMD is an inherited disease caused by mutations in the dystrophin gene, the immune inflammatory response contributes to disease progression in DMD patients. Given the lack of effective therapies for this fatal condition, the company identified DMD as a promising commercial opportunity that potentially offered a faster route to market. Treatments for orphan diseases like DMD attract high prices and the global market for DMD drugs is expected to be worth US$27bn by 2030. The US FDA has granted ATL1102 Orphan Drug Designation and Rare Pediatric Disease Designation for the treatment of DMD.

In late 2019 and early 2020 ANP reported encouraging signs of efficacy from a single-arm Phase IIa study of ATL1102 in 9 boys with DMD who were confined to a wheelchair (ie non-ambulant). After 24 weeks of treatment with ATL1102, the boys showed improvement or slower-than-expected decline in measures of upper limb strength and function. There were also improvements in a number of biomarkers including reductions in targeted populations of inflammatory cells, preservation of muscle mass and reductions in the fat percentage in forearm muscles.

ANP is currently enrolling a randomised Phase IIb study of ATL1102 in 45 non-ambulant DMD patients at sites in Australia and Europe. The company expects to complete enrolment in Q1 CY24 and to report initial data in H2 CY24. We anticipate that this study could potentially be the basis for an application for conditional approval, if the results are sufficiently positive.

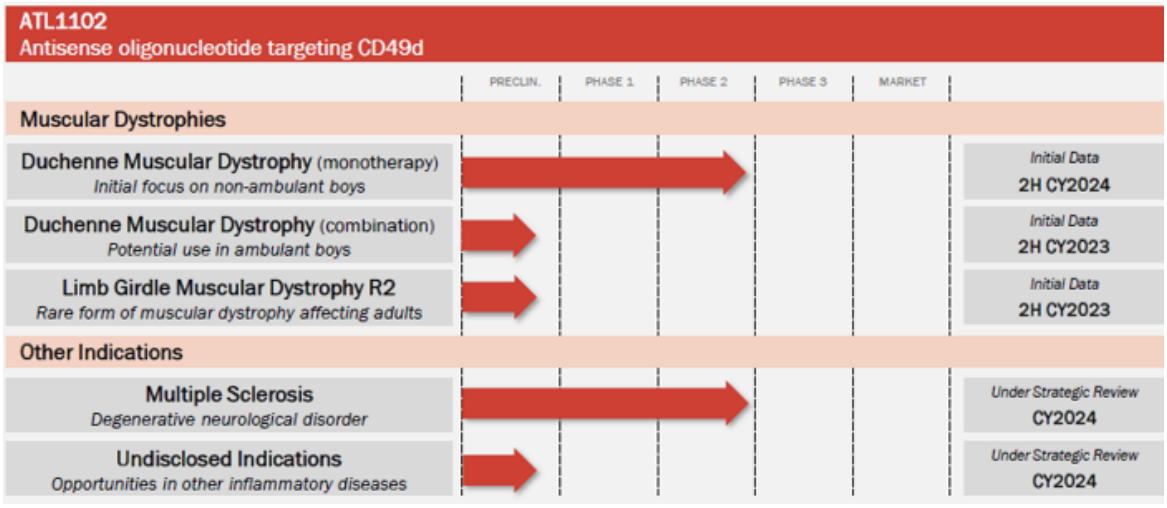
The company expects to complete dosing in an ongoing 9-month chronic monkey toxicity study before the end of the year, with results reported in H1 CY24. Successful completion of this is study is expected to be sufficient for the FDA to allow dosing of ATL1102 for 12 months or longer in the US (dosing in US studies is currently limited to a period of 6 months or less).

In September, ANP reported promising preclinical evidence of activity for ATL1102 in the rare genetic condition known as limb girdle muscular dystrophy R2 (LGMDR2) or dysferlinopathy. Treatment with ATL1102 partially normalised the function and physiology of calf muscles in a mouse model of LGMDR2. While further work needs to be done to confirm the potential, results to date suggest that ATL1102 could potentially be an effective treatment for the disease.

A separate study in a mouse model of DMD provided evidence indicating that the ATL1102 is likely to have beneficial effects in patients who are also being treated with “exon skipper” therapies to enhance production of the dystrophin protein.

ANP has previously investigated ATL1102 in MS and a second antisense drug ATL1103 in acromegaly, but these indications are no longer under active development.

Exhibit 1: Overview of Antisense’s pipeline



Source: Modified from Antisense presentation August 2023

ATL1102 showed encouraging efficacy in a Phase IIa study in non-ambulant DMD boys

ANP investigated the use of ATL1102 as a treatment for DMD in a Phase IIa study in 9 non-ambulant (wheelchair bound) patients at the Royal Children's Hospital, Melbourne. In this open label, single arm study, the boys were injected with 25mg of ATL1102 once per week for a period of 24 weeks, with 8 of the 9 subjects also continuing to receive corticosteroid treatment throughout the study period. The trial assessed the safety and tolerability of ATL1102 as well as its efficacy in terms of its effects on muscle strength and function. It also examined blood and imaging markers of inflammation and muscle damage.

Antisense reported top line data from the Phase IIa study in December 2019. The trial results were presented at the Muscular Dystrophy Association conference in 2020 and have been reported in a non-peer-reviewed article available on the medRxiv pre-print server[1]. ANP expects that the research will be published in a peer-reviewed scientific journal in H1 CY24. Exhibit 2 summarises some of the key results and includes the performance on each of the measures in similar patient populations in selected historical studies, for comparison.

ATL1102 was found to be safe and well tolerated and no participants withdrew from the study. The most commonly reported adverse events were mild redness and skin discolouration at the injection site, which either resolved or were close to resolution at the end of the study.

The outcomes in the key measures of efficacy in the study were very encouraging, with either improvement or slower-than-expected decline in measures of upper limb strength and function (PUL 2.0. MyoGrip, MyoPinch and Moviplate). Furthermore, there was a reduction in fat percentage in forearm muscles on MRI scans, whereas studies of the natural history of the disease show a steady increase as dying muscle fibres are replaced by fat. The impact on measures of respiratory function was inconclusive.

There were consistent reductions in the number of lymphocytes, including T cells, in the bloodstream over the 24-week treatment period, with a rebound to slightly above the starting levels at week 28. While the reduction in CD49 positive lymphocytes from baseline to week 24 did not reach statistical significance, there was a statistically significant rebound in the mean number of these T cells in the 4 weeks following the end of treatment. This demonstrated that ATL1102 modulated CD49+ lymphocytes in the blood during the treatment period, in line with the expected mode of action.

Professor Thomas Voit MD, Director, NIHR GOSH Biomedical Research Centre, UK, who is a widely published DMD researcher, observed that "Disease stabilisation or indeed improvement in functional scores in non-ambulant DMD boys is almost unheard of and a very encouraging result. This is even more meaningful as these results have been obtained using different independent measures and over a relatively short trial time of 24 weeks…"

While the efficacy results from the Phase IIa study are very encouraging, the small number of subjects means that the confidence intervals (error margins) of the estimates of efficacy are wide, so the degree of benefit is uncertain. Importantly, if the efficacy of ATL1102 is confirmed in the ongoing randomised Phase IIb-trial, it would become one of the few drugs with demonstrated efficacy in improving or preserving function in DMD patients. Very few of the approved drugs have been studied in non-ambulant patients.

Exhibit 2: Highlights of 6-month Phase IIa pilot study of ATL1102 in non-ambulant DMD patients

Endpoint	Description	ATL1102 Result	Historical Comparator
PUL2.0	*Performance of Upper Limb (PUL2.0) assesses the function of upper body muscles in 3 dimensions*	↑ 0.9 (-1.33 – 3.11)	↓ 2.0 (-2.95 – -1.05)
MyoGrip (dominant hand)	*MyoGrip assesses the clamping force of the fingers*	↑ 0.2 kg (-0.25 – 0.67)	↓ 0.5 kg (-1.01 – 0.00)
MyoPinch (dominant hand)	*MyoPinch assesses the pinch strength between thumb and forefinger*	→ 0.0 kg (-0.18 – 0.19)	↓ 0.4 (-0.53 – -0.22)
MoviPlate (dominant hand)	*MoviPlate assesses the fatigability of forearm muscles but is of uncertain significance in DMD*	↑ 1.9 (-6.08 – 9.85)	↑ 4.7 (2.01 – 7.40)
MRI - total lean muscle area	*Magnetic Resonance Imaging (MRI) is used to assess the amount of fat and lean muscle mass in the forearm*	↑ 13.9 mm^2 (-72.6 – 100.4)	↓ 32.1 mm^2 (-102.6 – 38.1)
Lymphocyte Counts	*Lymphocyte counts measure the ability of ATL1102 to modulate the immune system and reduce inflammation*	↓ 0.28 x 10^9 / L (-1.10 – 0.55)	↑ 0.47 x10^9 / L

Source: Antisense. Note Historical comparator data from Ricotti et al 2016 & Tachas et al 2020.

[1] Woodcock et al, unpublished. doi: https://doi.org/10.1101/2022.01.16.22269029

A closer look at the PUL 2.0 upper limb function score

The primary endpoint in ANP's Phase IIb trial is the difference in PUL 2.0 (Performance of the Upper Limb) scores between patients receiving ATL1102 or placebo.

PUL 2.0 is a validated 42-point functional scale specifically designed for assessing upper limb function in patients with DMD. The items within the scale were identified to be clinically meaningful, important to patients and relevant to everyday life. The scale involves scoring performance in a range of activities such as picking up coins, lifting and stacking cans, tracing a path, pushing on a light switch or lifting hands to their mouth; higher scores indicate better performance (Exhibit 3). The original PUL 1.2 scale (with a maximum score of 74) was developed at an international workshop in 2012 (Mercuri et al 2012); the ATL1102 Phase IIa trial assessed performance using the revised PUL 2.0 scale.[2]

A wide range of activities has been included in PUL 2.0 in order to avoid "ceiling" and "floor" effects and thus make it relevant to both ambulant and non-ambulant boys. The activities are shown in Exhibit 3.

Exhibit 3: PUL 2.0 measures 3 dimensions of upper limb – the shoulder, elbow and wrist/hand

Source: Taylor Collison Research, Capricor Therapeutics company announcement. NB The exhibit illustrates most items of the PUL 1.2 assessments, almost all of which are included in PUL 2.0; notable changes include that PUL 2.0 does not use light cans in the middle level items and that tearing paper becomes a wrist/hand (distal) level task in PUL 2.0. Shoulder level items include tasks performed both with and without weights.

Given that PUL 2.0 is the primary endpoint for the Phase IIb trial, it is positive for ANP that there was an impressive 0.9 point improvement in PUL 2.0 scores observed in ATL1102 treated patients in the Phase IIa study, compared to the deterioration in scores reported for non-ambulant DMD patients in other studies.

ANP has compared the PUL 2.0 scores in the ATL1102 study to scores from a matched control group from a natural history cohort of DMD patients in Rome, Italy, identified using the same inclusion criteria used to enrol patients in the ATL1102 Phase IIa study (the Rome cohort).

As Exhibit 4 shows, in the Rome cohort PUL 2.0 scores declined on average by 2.0 points over a 6-month period, compared to the 0.9 point improvement over the same period in the ATL1102 study. The difference between the change in PUL 2.0 scores in the ATL1102 trial and in the Rome cohort was statistically significant.

We have identified three other studies that reported changes in PUL 2.0 scores in non-ambulant DMD patients. In two of these studies, which reported the natural history of 90 and 128 boys, respectively, PUL 2.0 scores declined on average by 2.2 points over 12 months, equivalent to a decline of 1.1 points over a 6 month period[2,3]. In the third cohort, comprising 8 boys in the placebo arm of the HOPE-2 Phase II study of CAP-1002, PUL 2.0 scores declined by 2.3 points over 6 months.[4]

As Exhibit 4 shows, the average decline in these 4 cohorts of patients over a 6 month period was 1.6 points, which is comparable to the Rome Cohort and is a marked contrast to the 0.9 point improvement in the ATL1102 study.

[2] Mayhew et al. Developmental Medicine & Child Neurology 2020, 62: 633–639. DOI: 10.1111/dmcn.14361
[3] Pane et al 2023, Journal of Neuromuscular Diseases 10: 567–574. DOI 10.3233/JND-221556
[4] Capricor Therapeutics announcement

We also identified three additional studies that reported changes in the original PUL 1.2 scale over a 6 or 12 month period[5,6,7]. In each of those studies the mean PUL score also declined over the 6 or 12 month period.

Cross trial comparisons should always be treated with caution because there may be differences in the study design or unidentified differences between the patient populations. While bearing in mind this proviso, we find it very encouraging that mean PUL scores showed a progressive decline in the 7 other cohorts examined but improved in patients treated with ATL1102. In fact, PUL 2.0 scores improved or remained stable in 7 of the 9 subjects in the ATL1102 study.

The company also noted that there appears to be a relationship between changes in CD49d+ T cells and PUL 2.0 scores.

The fact that there were only 9 subjects in the ATL1102 trial means that the confidence intervals around the estimates of treatment effects are wide. The 95% confidence interval for the change in PUL 2.0 score ranges from a decline of 1.33 points to an improvement of 3.11 points. This tells us that if the study was repeated a large number of times that there is a 95% probability that the average change in PUL 2.0 score would lie in this range. It is encouraging that even at the bottom of the confidence interval (ie -1.33 points) the change in PUL 2.0 scores would be superior to the 1.6 point average decline in the four other cohorts included in Exhibit 4.

Exhibit 4: Change in total PUL 2.0 upper limb muscle function and strength score over 6 months

Measure	ANP change after 24 weeks treatment with ATL1102 (mean, 95% CI)	Mayhew et al 2020 12-month change pro rata to 6 months	Matched Rome cohort (WMS) (mean, SD)	Capricor CAP-1002 HOPE-2 Phase II 6 months (mean, SD)	Pane 2023 12-month change pro rata to 6 months*	Average of non-ATL1102 studies
PUL 2.0 score	0.9 (-1.33, 3.11)	-1.1	-2.0 (3.0)	-2.3 (1.5)	-1.1	-1.6

Source: Taylor Collison research. Note 95% CI= 95% confidence interval; SD= standard deviation; higher PUL 2.0 score= less disability. * based on weighted mean change at 12 months in non-ambulant boys calculated from the last 4 columns of Table 1 in Pane et al 2023, Journal of Neuromuscular Diseases 10: 567–574.

Potentially pivotal randomised Phase IIb trial of ATL1102 underway in Europe and Australia

ANP enrolled the first patient in a randomised Phase IIb study of ATL1102 in non-ambulant DMD patients in Europe and Australia in June 2023. Ten patients had been recruited as of 31 October; the company expects to compete recruitment in Q1 CY24 and to report topline data in H2 CY24.

The Phase IIb trial is testing once weekly sub cutaneous injections of the 25mg dose that was investigated in the Phase IIa trial, as well as a higher 50mg dose. The trial will enrol ~15 subjects at each of the two dose levels as well as an additional 15 subjects in a placebo arm, for a total of ~45 subjects.

After 6 months of treatment the trial will be unblinded and the effect on ATL-1102 on the PUL 2.0 primary endpoint and the secondary efficacy endpoints will be evaluated. Following this, participants will continue onto a further six-month extension treatment period, with placebo patients randomised to either the 25mg or 50mg ATL1102 groups. Exhibit 5 summarises the trial design.

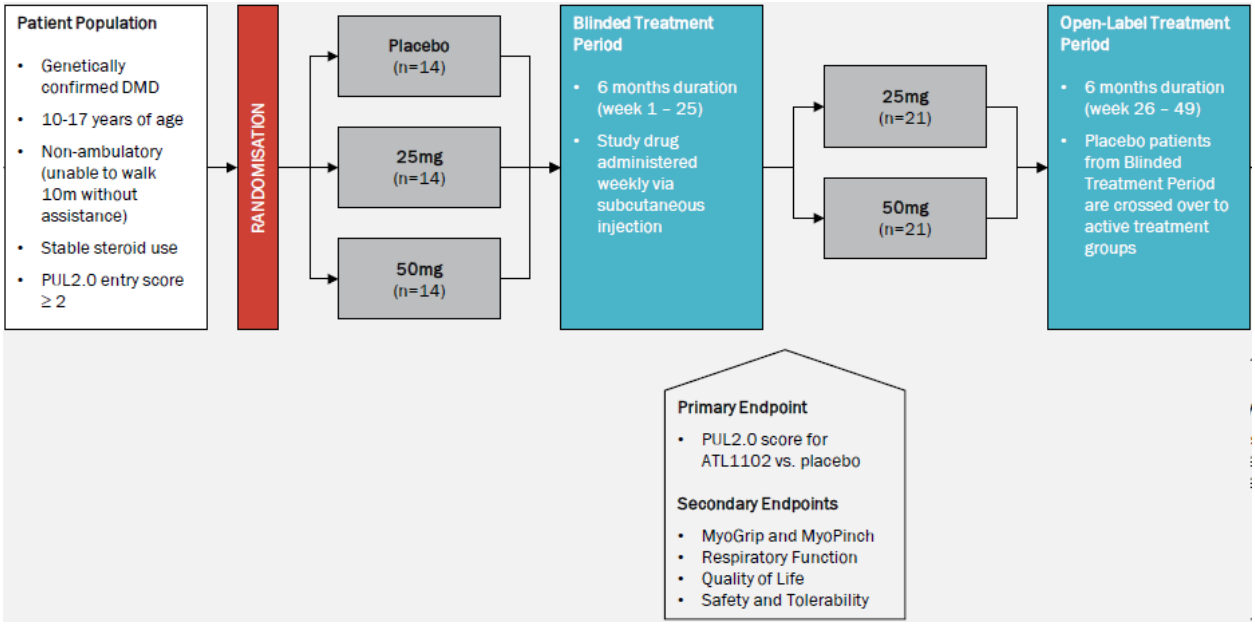
The company had initially proposed a larger Phase IIb/III trial that was to have treated ~114 subjects for 12 months. There was to have been an interim futility analysis after ~50 subjects had completed 24 weeks of dosing, but there would not have been an efficacy readout until the last subject had completed 12 months of dosing.

The revised trial design has reduced the cost and will bring forward the reporting of unblinded and statistically analysed efficacy data. The new strategy allows ANP to confirm drug efficacy and allow informed discussions with potential partners at an earlier date.

It is possible that if the study achieves statistical significance on the primary endpoint, as well as providing evidence of benefit on key secondary endpoints, that it could be the basis for applications for marketing approval in the US, Europe and Australia. However, we believe it more likely that the study will act as a signal finding trial to potentially provide evidence to support progressing to a larger Phase III trial.

[5] Ricotti et al 2016, PLosOne, 11(9) e0162542
[6] Ricotti et al 2019, Neuromuscular Disorders 29(4);261-268
[7] Pane et al 2015, Neuromuscular Disorders 25: 749–753

Exhibit 5: Key features of the randomised Phase IIb trial of ATL1102 in non-ambulant boys with DMD



Source: Modified from Antisense presentation

Potentially eligible for a valuable Pediatric Rare Disease Priority Review Voucher from the FDA

The Phase IIb trial is enrolling subjects in Australia and Europe partly because the US FDA requires additional animal safety data before it will allow patients in the US to be treated with ATL1102 for longer than six months. The company is conducting a 9-month chronic monkey toxicity study in order to satisfy this requirement and expects to report the results in H1 CY24. We expect successful completion of this is study to be sufficient for the FDA to allow ATL1102 to be studied in patients in the US for a longer term, such as 12 months.

The company has engaged with key opinion leaders, advocacy groups and regulatory consultants to determine the appropriate clinical development and regulatory path for ATL1102 in DMD in the US.

The US FDA has granted ATL1102 Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) for the treatment of DMD. The ODD would confer 7 years of market exclusivity for ATL1102 in the US, if the drug is approved. Separately, the RPD designation means that if ATL1102 gains FDA approval it would be eligible for the award of a priority review voucher (PRV) which can be used to shorten the FDA review process by 4 months.

Under the sunset clause in the current act authorising the RPD PRV program, ATL1102 would need to be approved by 30 September 2026 in order to qualify for a PRV. Given that the program has already been re-authorised by US lawmakers several times since it was first passed in 2012, we believe that the program is likely to be renewed again before the sunset clause takes effect, which would enable ATL1102 to be eligible for a PRV even if it is not approved until after the current September 2026 deadline. However, while we believe that further renewal of the program is likely, renewal is not guaranteed.

To date the FDA has awarded 22 PRVs for rare paediatric diseases. PRVs have sold for between US$65m and US$350m. Recent prices for PRVs have been around US$100m; for example, in July 2023 Sarepta Therapeutics sold its Elevidys PRV for US$102m.

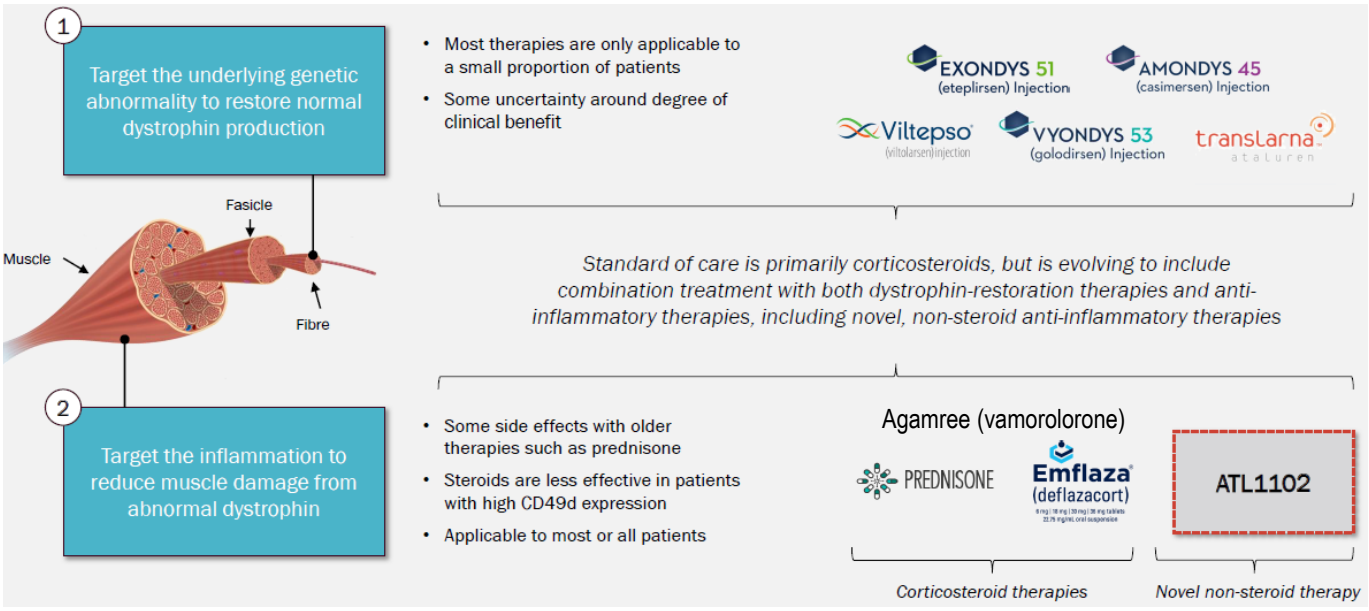
DMD overview

DMD is one of the most devastating and most common inherited neuromuscular diseases. It results from a defective gene responsible for producing the key muscle protein dystrophin. The gene mutation dramatically reduces production of the dystrophin protein, which strengthens and protects muscle fibres from damage as they contract and relax. Without dystrophin, muscle cells easily become damaged and die, resulting in progressive muscle weakness, loss of walking ability, heart and breathing failure and premature death. DMD has an X-linked recessive inheritance pattern because the gene for dystrophin is carried on the X chromosome. DMD almost exclusively affects boys, as they have only one copy of the X chromosome.

DMD has been estimated to affect one in 3,500[8] to 6,300[9] live male births. A recent meta-analysis estimated that the pooled global incidence of DMD was 19.8 per 100,000, equal to one in 5050 live male births[10]. The meta-analysis also estimated that that the global DMD prevalence was 7.1 cases per 100,000 males in the overall population. Based on this estimate of prevalence, we estimate that there are approximately 11,700 DMD patients in the US and 17,500 patients in the EU (including the UK). Despite the best available treatment, the need for wheelchair use can occur in the early teenage years, with an average age of 13. Between 36% and 48% of DMD patient are estimated to be non-ambulant.

The two main classes of drug therapies for managing aspects of the disease in DMD patients are shown in Exhibit 6 and are discussed in the sections below. Drugs to support cardiac function are also an important aspect of managing DMD, but they are not relevant to ATL1102 and so are not discussed here. The effects of the different classes of drugs tend to be complementary.[11]

Exhibit 6: ATL1102's potential place in the treatment of DMD



Source: Modified from Antisense presentation. Note: Therapies that target the underlying genetic abnormality are predominantly being tested in ambulant boys.

Drugs to manage inflammation are a key plank of DMD treatment

One of the key challenges in treating DMD is managing the inflammation-mediated muscle damage that contributes to the progression of the disease. Corticosteroids are the only treatment approved for the broad DMD population, but they are associated with significant side effects including bone fragility, suppression of the immune system and suppression of growth hormone production.

Despite the long-term safety concerns associated with steroids, the drugs have a multipronged mechanism. This mechanism significantly prolongs lifespan, so it is unlikely to be replaced in the near future. The key drugs in development to treat inflammation in DMD patients are shown in Exhibit 7.

ATL1102 aims to further reduce inflammation-mediated muscle damage over and above that achieved with corticosteroids, with 8 of the 9 patients being treated with corticosteroids in the completed Phase II trial. It could also potentially be used as an alternative to corticosteroid therapy in patients who are unable to tolerate the side effects of corticosteroids.

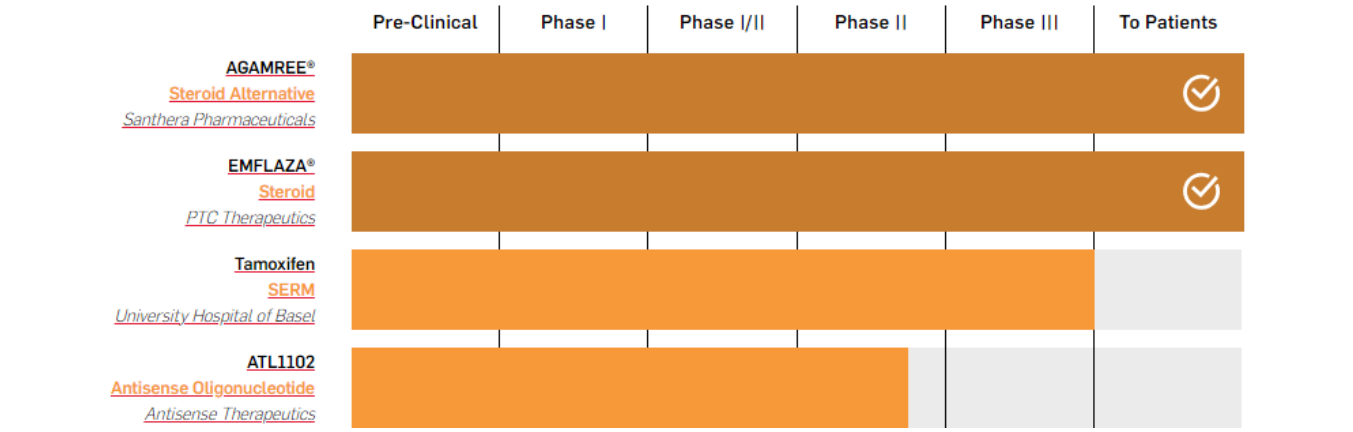
Emflaza (deflazacort), a corticosteroid that was approved by the FDA in 2017, was launched at a list price of US$89,000 per year. The Institute for Clinical and Economic Review (ICER) estimated that the annual cost of Emflaza for a 40kg boy in the US was US$81,400, based on the 2019 Federal Supply Schedule price.[12] Emflaza efficacy is comparable to standard corticosteroid therapy, but with an improved side effect profile.

[8] Emery 1991, Neuromuscul Disord 1 (1) 19- 29
[9] Ryder et al. 2017, Orphanet Journal of Rare Diseases 12:79
[10] Crisafulli et al. 2020, Orphanet Journal of Rare Diseases 15:141 https://doi.org/10.1186/s13023-020-01430-8
[11] Cordova et al 2018, Front. Genet. 9:114. doi: 10.3389/fgene.2018.00114
[12] Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy: Effectiveness and Value - Final report; p21

Agamree (vamorolone, ReveraGen/Santhera Pharmaceuticals) is a corticosteroid alternative that was approved by the FDA in October 2023. The drug retains the beneficial anti-inflammatory and muscle strengthening aspects of corticosteroids, while decreasing some of the undesirable side effects. A randomised Phase IIb study (NCT03439670) compared two doses of vamorolone to prednisolone (active control) and placebo in 121 boys aged 4-7. The trial showed that the higher dose of vamorolone resulted in similar improvements in motor function compared to the corticosteroid prednisone, but without the negative impact on growth that was seen in the prednisone-treated group. The company has not disclosed its intended pricing for Agamree.

The cancer drug Tamoxifen, a selective estrogen receptor regulator, was being investigated in a randomised Phase III study (NCT03354039) in 79 ambulant DMD patients and a small number of non-ambulant patients. However, in September 2021 it was reported that the study did not meet its primary endpoint of delaying disease progression.

Exhibit 7: Drugs that aim to reduce inflammation in DMD patients



Source: Parent Project DMD https://www.parentprojectmd.org/duchenne-drug-development-pipeline/

Two other drugs in development that are relevant to ATL1102 because they target inflammation and/or fibrosis in non-ambulant DMD patients but are not listed in Exhibit 7 are discussed below.

The first drug, **CAP-1002** (Capricor Therapeutics), is an intravenous (iv) cell therapy composed of cardiosphere-derived cells (CDCs), a type of heart progenitor cell with the ability to generate mature cardiac cells. CDCs have been shown to have anti-inflammatory, and immune modulatory properties. Capricor's HOPE-2 randomised Phase 2 trial (NCT03406780) assessed the safety and effectiveness of CAP-1002 in 20 boys and young men in advanced stages of DMD who were being treated with steroids. Subjects received either CAP-1002 (8 patients) or placebo (12 patients) every 3 months for one year. Over 12 months, PUL 2.0 scores declined by 1.3 points in boys treated with CAP-1002 vs a decline of 3.7 points in boys on placebo; the difference just missed statistical significance (p=0.053).

Capricor has enrolled the target of 58 subjects in Cohort A of the HOPE-3 randomised Phase III trial of CAP-1002 in non-ambulant DMD. It plans to report the outcome of the interim futility analysis in Q4 CY23 and expects to report the topline data from Cohort A in Q4 CY24. The primary endpoint of the trial is the change in PUL 2.0 at 12 months.

Capricor expects to commence enrolment of the planned 44 subjects in cohort B of the trial in Q4 CY23. Cohort B is a randomised, placebo-controlled study that aims to support approval of a second manufacturing site for CAP-1002.

Capricor has outlicenced the US and Japanese rights to CAP-1002 to Nippon Shinyaku. Terms include total upfront payments of US$42m, up to US$794m of potential milestone payments and a double digit royalty.

The second drug, **pamrevlumab** (FG-3019, FibroGen), an antibody drug that inhibits connective tissue growth factor (CTGF), was being studied in non-ambulant DMD patients. However, in June FibroGen announced that pamrevlumab failed to meet the primary endpoint (PUL 2.0 at 52 weeks) in a Phase III trial that recruited 99 non-ambulant boys with DMD.

We are not aware of any other drugs that are in late stage development in non-ambulant DMD patients.

Drugs that aim to restore or replace dystrophin production

The other significant class drugs are those that aim to replace or restore production of the dystrophin protein, in most cases by producing a shortened but still functional form of the protein. Drugs in this class either aim to counteract the effect of the mutation to allow the body to produce dystrophin (mainly exon skippers) or are gene therapies that aim to deliver shortened versions of the dystrophin gene to muscle cells. Exhibit 8 summarises the main drugs in this category, including four exon skipping drugs and one gene therapy that have received FDA approval.

While the exon skippers and other therapies that seek to address the genetic mutation may partially counteract the effect of the DMD mutations on dystrophin production and may slow disease progression, they are unlikely to provide a cure and are currently used to treat patients at the earliest stages of the disease. Drugs to treat the damaging effects of inflammation will still be needed. In fact, by extending the life expectancy of DMD patients, the treatments that target dystrophin production are likely to increase the addressable market for anti-inflammatory therapies such as ATL1102.

Exon skippers and other therapies aiming to restore dystrophin production

The exon skipping drugs are based on technology developed at the University of Western Australia. Antisense oligonucleotides or 'molecular patches' are used to mask the exon that contains the mutation that causes DMD in that patient, so that it is ignored during protein production. This allows a shortened but partially functional form of the dystrophin protein to be produced. The four exon skipping drugs that have been approved by the FDA are appropriate for mutations carried by around 29% of DMD patients. These drugs are used in young ambulant boys and are used on top of standard doses of corticosteroids to control inflammation.

The first of these drugs, Exondys 51 (eteplirsen), developed by Sarepta Therapeutics, which received accelerated approval from the FDA in 2016, induces skipping of exon 51. Approximately 13% of the total DMD population have gene deletions that are amenable to exon 51 skipping.

In 2019 the FDA granted accelerated approval to Vyondys 53 (golodirsen, Sarepta Therapeutics) to treat DMD patients who have a mutation of the dystrophin gene that is amenable to exon 53 skipping.

The FDA approved a third Sarepta exon skipping drug, Amondys 45 (Casimersen, SRP-4045) in 2021. Casimersen is a treatment for mutations that are amenable to exon 45 skipping.

According to Sarepta, Exondys 51, Vyondys 53 and Amondys 45 cost about $300,000 on average per patient a year, after taking into account rebates, discounts and compliance. However the list price appears to be substantially higher; an analysis of cost effectiveness by ICER estimated that the annual drug cost for treating a 40kg boy, including mark-up, was US$892,000.[13] Sarepta reported 2022 sales for Exondys 51, Amondys 45 and Vyondis 53 of US$512m, US$215m and US$117m, respectively.

Viltepso (viltolarsen, Nippon Shinyaku), another exon 53 skipper, was granted accelerated approval by the FDA in August 2020. Several other exon skippers are in development, as shown in Exhibit 8.

The approvals of these exon skipping drugs were controversial because the drugs have not been shown to produce clinical benefit but were approved on the basis of modest increases in dystrophin production. For example, in a 25-patient study, weekly treatment with Vyondys 53 for 48 weeks increased dystrophin levels from 0.10% of normal at baseline to 1.02% of normal.

Gene therapies to produce micro-dystrophins

The other class of drugs that aim to replace dystrophin production is gene transfer therapies that aim to deliver shortened but functional versions of the dystrophin gene to muscle tissue for the targeted production of a micro-dystrophin or mini-dystrophin protein.

Sarepta's Elevidys (SRP-9001) micro-dystrophin gene therapy was granted accelerated approval by the FDA in June 2023 for treating ambulatory patients aged 4 to 5 years. Elevidys uses a viral vector to transfect cardiac and skeletal muscle cells and express the micro-dystrophin gene. The approval was based on expression of Elevidys micro-dystrophin in skeletal muscle observed in treated patients.

The key clinical trial (Study 102) randomised 41 patients to received Elevidys (n=21) or placebo (n=20). Twelve weeks after Elevidys infusion, the quantity of micro-dystrophin in muscle biopsies was 41% of the level of wild-type dystrophin seen in biopsies from healthy boys.

In Study 102 there was a modest improvement in the 34-point North Star Ambulatory Assessment (NSAA) scores compared to placebo, but the difference was not statistically significant (p=0.37). There was a modest improvement in NSAA scores compared to placebo in patients aged 4-5 years, whereas in boys aged 6-7 the placebo group performed better than those treated with Elevidys.

In October, Sarepta reported that the EMBARK confirmatory Phase III trial in 125 boys aged 4-7 failed to meet its primary endpoint; while NSAA scores improved by 2.6 points in the Elevidys treated patients vs 1.9 points on placebo, the difference was not statistically significant (p=0.24). However, the results reportedly favoured Elevidys across all endpoints of the study, including achieving statistical significance on all pre-specified key secondary endpoints and in each age subgroup of the key secondary endpoints.

Despite the failure to meet the primary endpoint, Sarepta plans to file for a label expansion to treat "all DMD patients".

[13] Deflazacort, Eteplirsen, and Golodirsen for Duchenne Muscular Dystrophy: Effectiveness and Value - draft report; p49

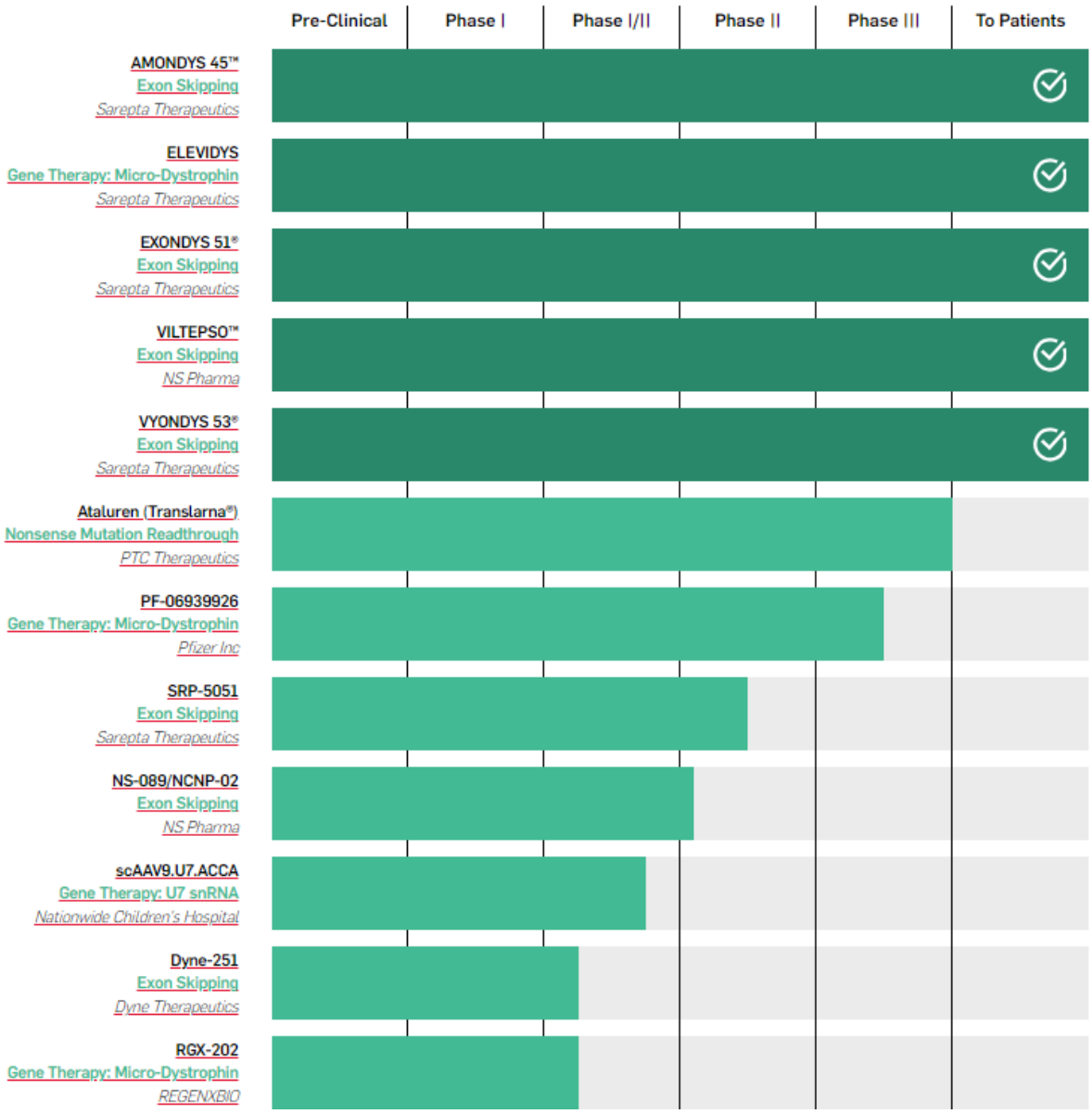
We suspect that Sarepta will need to confirm the efficacy of Elevidys in the ongoing ENVISION Phase III trial (NCT05881408) before it can convert the accelerated approval into a full traditional approval. ENVISION is enrolling 148 ambulant and non-ambulant DMD patients.

Elevidys generated sales of US$69m in its first quarter on the market. The drug is priced at US$3.2m per infusion.

The other gene therapy that is in late-stage development is Pfizer's PF-06939926. Pfizer expects to report results in H2 CY24 from the 99-patient CIFFREO Phase III trial of PF-06939926 (NCT04281485). The primary endpoint of the trial, which enrolled boys aged 4-7 years, is the change in NSAA at 52 weeks.

The gene therapy drugs in development use adenovirus vectors to transfect the muscles cells in patients. Elevidys is only approved for use in boys who have low levels of antibodies against the adenovirus vectors. A significant proportion of older boys carry antibodies against the adenovirus vectors, which may reduce the number of patients who are eligible for these treatments.

Exhibit 8: Selected drugs that aim to restore or replace dystrophin production



Source: Parent Project MD https://www.parentprojectmd.org/duchenne-drug-development-pipeline/

Inhibiting leukocyte integrins effective in inflammatory diseases

Therapies that inhibit the activity of leukocyte (white blood cell) integrins (as does ATL1102) have been shown to be effective as treatments for multiple sclerosis, psoriasis and inflammatory bowel disease. The two treatments that are currently marketed both target integrins that incorporate the same α4 integrin subunit that is inhibited by ATL1102.

Tysabri (Natalizumab, Biogen) is a monoclonal antibody against CD49d, the α4 subunit in α4β1 integrin (VLA-4). VLA-4 interacts with the vascular endothelium adhesion molecule VCAM-1, facilitating transmigration of white blood cells from the bloodstream to surrounding tissue. Tysabri is approved for treating multiple sclerosis and moderate to severe Crohn's disease.

Entyvio (vedolizumab), is a monoclonal antibody that binds to integrin α4β7. It was approved in the US and Europe in 2014 for the treatment of both moderate-to-severe ulcerative colitis and moderate-to-severe Crohn's disease.

Tysabri generates US$2.0bn of annual sales despite fatal infection risk

Tysabri is approved to treat relapsing forms of multiple sclerosis (MS) and moderate to severe Crohn's disease. However, treatment with Tysabri increases the risk of getting a rare brain infection called progressive multifocal leukoencephalopathy (PML) that usually leads to death or severe disability. There is no known treatment, prevention, or cure for PML, but stopping treatment with Tysabri at the earliest stages of the disease before clinical signs are evident leads to improved outcomes.

The risk of getting PML is higher in patients who:

- have been infected by the John Cunningham Virus (JCV).
- have received Tysabri for a long time, especially for longer than 2 years
- have received immunosuppressant medications before they started receiving Tysabri

JCV is a common virus that can cause PML in people who have weakened immune systems, such as people taking Tysabri. Patients can undergo a blood test to check if they have been infected with JCV before receiving Tysabri.

The risk of a patient who is being treated with Tysabri developing PML ranges from less than 1 in 10,000 for those who are negative for JCV virus antibodies to 1 in 1000 for patients who are JCV virus positive but have no other risk factors and 1 in 125 for patients in the highest risk categories. Because of the risk of PML, Tysabri is available only through a restricted distribution program. Despite the risk of PML and the restricted distribution program, Biogen reported Tysabri sales of **US$2.0bn** in 2022.

ATL1102 uses a different mechanism to Tysabri to reduce CD49d activity and there is no evidence to date that ATL1102 also increases the risk of PML, although it may do so. There was no evidence of JC virus activation in either of the Phase IIa trials of ATL1102 conducted to date. Given the very poor prognosis for DMD patients, we do not think that a modest risk of developing PML would deter patients from taking ATL1102, if future studies show the existence of such a risk. Only approximately 20-33% of 10-19 year olds carry JCV antibodies, so the majority of DMD patients would be expected to be at very low risk of PML.[14]

Patent applications could extend IP protection beyond 2040

In addition to patents covering the use of ATL1102 to treat MS, Antisense holds granted patents in the US (9,885,048) and Australia (20113017172) covering methods of using ATL1102 to reduce circulating leukocytes as observed in the Phase II DMD trial; these patents extend to 2031. It has filed US and international patent applications covering the use of ATL1102 to treat DMD, which would extend to 2039/2040 if granted. The patent term of these patents (if granted) could potentially be extended by up to 5 years once ATL1102 receives marketing approval. The key DMD patent applications are shown in Exhibit 9.

In 2023 the company filed a provisional patent application covering the combination of ATL1102 and dystrophin exon skipping drugs in the treatment of DMD, to protect the invention to 2044.

Exhibit 9: Key patent applications covering ATL1102 therapeutic uses and methods for treating muscular dystrophy

Territory	Application number	Status	Expiry (if granted)
US Continuation - in part	16/404561	Filed	2039
International	PCT/AU2018/051353	Filed	2039
International	PCT/AU2020/050445	Filed	2040
Australian provisional	2023900242	Provisional	2044

Source: Antisense annual report

[14] White and Khalili 2011 The Journal of Infectious Diseases; 203:578–586

Risks

Antisense is subject to clinical trial, regulatory and commercialisation risks common to all biotech companies. The key risk is the possibility that ATL1102 may fail to show statistically significant and clinically meaningful improvements in outcomes for DMD patients in future clinical trials. The encouraging indications of efficacy of ATL1102 in DMD were based on a small sample of only 9 patients in a single arm study, which increases the risk that the favourable results may have arisen due to chance rather than being due to the efficacy of the ATL1102 therapy.

Antisense had $19.2m cash on 30 September, which gives it a funding runway into 2025, beyond the anticipated reporting date of its Phase IIb trial in H2 CY24. However, if it is not able to attract a partner to fund further development of ATL1102, it may need to raise substantial funds if it chooses to continue the development of ATL1102 on its own. There is a risk that it may not be able to raise the funds at a reasonable price, or at all.

Tysabri (natalizumab), a drug that inhibits the activity of CD49b (via different mechanism to ATL1102), is known to increase the risk of the fatal condition known as PML. It is not known whether ATL1102 will increase the risk of PML. If any subjects in ATL1102 clinical trials develop PML, then this may result in interruption or suspension of clinical trials. Furthermore, if ATL1102 is shown increase the risk of developing PML, this may influence the uptake of ATL1102 (if approved).

Valuation

We resume coverage of Antisense with a valuation of $136m or 15c per share, based on a risk-adjusted discounted cash flow model, which includes our estimates of the future milestone payments and royalty streams for ATL1102, as listed in Exhibit 10.

We have extended our cash flow forecasts out to 2042, in the expectation that patents covering use of ATL1102 to treat DMD will be granted, but assume that sales will decline by 10% per year from 2040 onwards. The grant of Orphan Drug designation brings seven and twelve years of market exclusivity for the DMD indication in the US and Europe respectively. We assume a long-term exchange rate of US$0.70/A$ and apply a 12.5% discount rate.

We model Antisense out-licensing global rights to ATL1102 in a single transaction in 2025. We assume that the licence deal includes an upfront payment of US$42m and US$792m of milestone payments, based on the terms of the licencing agreement between Capricor Therapeutics and Nippon Shinyaku for CAP-1002. The Capricor deal is for the US and Japan only, but instead of assuming higher milestones for a global licensing deal, we assume the partner would pay for the cost of a Phase III trial (if required). We estimate that a 130-patient Phase III trial would cost ~US$17m (assuming US$130,000 per subject).

We assume that 40% (US$317m) of the milestone payments are for the achievement of clinical and regulatory milestones, with the remainder assumed to be based on sales hurdles. We split the US$42m upfront and US$317m clinical and regulatory milestones equally between European and US non-ambulant indications, adjusted with a 20-50% probability (50% probability of signing a license deal, 20% for US approval milestones). We do not include any potential sales-based milestones in our forecasts, and instead model a 20% gross royalty rate.

We assume that ANP will pay one third of any licensing revenue received to partner IONIS, in line with the company's statements about the previous licencing deal with Teva. We note that, alternatively, ANP could choose to establish its own sales force and commercialise ATL1102 itself, in which case it would pay a mid-single digit royalty on net sales to IONIS.

The likely pricing for ATL1102 is difficult to determine as we do not yet know how effective it will be at delaying disease progression in DMD patients. Plausible ex-manufacturer pricing in the US, net of discounts and rebates, ranges from US$81,400 for Emflaza modelled by ICER to the average of US$300,000 per patient per year cited by Sarepta for Exondys 51. We model the net price of ATL1102 being US$150,000 per patient/year in the US, a 50% discount to Exondys 51, but acknowledge that eventual pricing could be substantially higher or lower than this figure. In Europe and other territories, we model a net price of US$100,000 per year, two thirds of the assumed US price.

Exhibit 10 shows our market assumptions for ATL1102 for non-ambulant DMD patients in Europe and the US, separately, plus a separate rNPV for use in earlier-stage ambulant boys. We have offset the risk-adjusted trial cost against revenue for the first two indications; we assume a partner would fund clinical trials in ambulant boys.

Exhibit 10: Antisense risk-adjusted DCF base case valuation and assumptions

	Likelihood (%)	rNPV (A$m)	rNPV/share (A$)	Assumptions
ATL1102 RoW non-ambulant DMD	25%	45.9	$0.05	Peak sales US$225m. Prevalence of 17,500 DMD patients, 45% non-ambulant, 20% penetration; pricing US$100k/patient; launch FY29; patents expire 2040; assume receives 13% net royalty after pay-away to IONIS; out-license in 2025.
ATL1102 US non-ambulant DMD	20%	54.2	$0.06	Peak sales of US$280m. Assumes prevalence of 11,600 DMD patients, 45% non-ambulant, 25% penetration; pricing US$150k per patient; launch FY29; patents expire 2040; assume receives 13% net royalty.
ATL1102 Global ambulant DMD	20%	32.7	$0.04	Global peak sales in ambulant patients of US$580m; launch 2031; Prevalence, pricing and royalty assumptions as above; 55% ambulant, 20% penetration in ambulant patients.
RPD Priority Review Voucher	16%	4.4	$0.00	Assumes an 80% probability that the RPD PRV program is still in force at the time of FDA approval (if granted); PRV sold for US$100m, ANP receives 33% share, pays away 1/3 to IONIS.
SG&A		-11.7	-$0.01	
Portfolio total		125.4	$0.14	
Cash end FY24e		9.7	$0.01	
Enterprise total		136.1	$0.15	

Source: Taylor Collison research. Note: NPV adjusted for tax at an effective tax rate of 20%. We assume that the addressable markets grow at 3% per year

Key personnel

Antisense has a highly regarded board of directors with impressive pedigrees, including senior management and research positions at CSL, Biogen, Takeda and Sanofi. Of particular note non-executive Director and former Consultant Medical Director Dr Gil Price has had extensive experience as a Director at Sarepta Therapeutics.

Dr Charmaine Gittleson – Chair

Dr Gittleson has extensive international experience as a pharmaceutical physician and enterprise leader in pharmaceutical drug development, governance and risk management gained during her 15-year tenure (2005-2020) with global specialty biotechnology company CSL Limited (ASX: CSL). Dr Gittleson held the key leadership roles of: Senior Director, Head Safety and Clinical Development (2006-2010) in Melbourne Australia; Vice President Clinical Strategy (2010-2013) and Senior Vice President Clinical Development (2013-2017) in Pennsylvania United States; and Chief Medical Officer in Melbourne from 2017 until her recent retirement from corporate roles in 2020.

Dr James Garner – MD and CEO

James brings broad experience in drug development and commercialisation, acquired through regional and global roles in the biotech and pharmaceutical sector. His previous responsibilities have included leading phase I-IV clinical trials, product registration, reimbursement, and business development. He possesses strong executive leadership and management skills that have seen him achieve outstanding results over a twenty year career in the Pharmaceutical/Biotechnology industry including roles with Biogen, Takeda, Quintiles (an international clinical research organisation) and as Head of the Unit Development Office, AP R&D with Sanofi in Singapore. Most recently James was CEO of Kazia Therapeutics (ASX:KZA; NASDAQ:KZIA), where he rebuilt the organisation around a pipeline of novel assets and attracted significant financing via capital markets and nondilutive opportunities.

Dr Gil Price M.D. – Non-Executive Director

Dr. Price is a clinical physician trained in internal medicine with a long-standing focus in drug development, adverse drug reactions, drug utilization and regulation. Dr. Price is an experienced biotech executive and entrepreneur with a depth of expertise across clinical asset investment strategy, evaluation, financing and execution. Over the years Dr. Price has served on multiple boards of public, private and not-for-profit entities; from 2007 to 2016, he was a non-executive director of Sarepta Therapeutics, Inc.

Anthony Filippis - Chief Operating Officer

Anthony Filippis is an accomplished executive with over 25 years' experience as a senior leader, with deep understanding and knowledge of the biotechnology, pharmaceutical, healthcare and investment industries. Prior to joining Antisense Therapeutics in November 2022, Anthony was CEO and Managing Director at NeuroSciences Victoria, a role held since 2017. Anthony has international expertise and key skills in business development, corporate strategy, operations, and senior management in ASX-listed and private companies. He has a strong track record in negotiating transactions, partnering (in- and out-licensing), M&A and raising capital. Anthony will have responsibility for the negotiation and execution of partnering transactions, investor relations, and accessing additional development capital, with a strategic focus on global markets.

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