

November 11, 2021

EMERGING COMPANY

SPECULATIVE BUY (no change)

Stock code:	ANP AU
Price:	A\$0.21
12-month target price:	A\$0.58
Previous target price:	A\$0.45
Up/downside to target price:	176.2%
Dividend yield:	0.00%
12-month TSR*:	176.2%
Market cap:	A\$138.1m
Average daily turnover:	A\$0.60m
Index inclusion:	N/A

* Total stock return – Up/downside to target price + 12-month forward dividend yield.

Price performance

(%)	1M	3M	12M	3Y
Absolute	0	5	82.6	366.7
Rel ASX/S&P200	-1.4	6.8	65.5	341.3



Financial summary

	Jun-21A	Jun-22F	Jun-23F	Jun-24F
Revenue (A\$m)	0.63	1.40	2.14	2.00
EBITDA Norm (A\$m)	-7.95	-11.27	-24.86	-27.00
Net Profit (A\$m)	-8.06	-11.14	-24.23	-26.82
EPS Norm (A\$)	-0.015	-0.017	-0.033	-0.035
EPS Growth Norm (%)	16.9%	13.0%	94.5%	5.2%
P/E Norm (x)	NA	NA	NA	NA
DPS (A\$)	0.000	0.000	0.000	0.000
Dividend Yield (%)	0%	0%	0%	0%
EV/EBITDA (x)	NA	NA	NA	NA
Gearing (Net Debt/EBITDA)	0.76	2.91	0.40	0.75

Source: Company data, Morgans estimates

Related research

[ANP \(SPEC BUY - TP A\\$0.45\) - 26 Aug 2021](#)

[ANP \(SPEC BUY - TP A\\$0.44\) - 12 Aug 2021](#)

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

– Antisense Therapeutics

Antisense Therapeutics

Leaps and stepping stones

- ANP has received the final positive opinion from the European regulator to commence pivotal trials in for its ATL1102 drug in DMD. Ratification to follow.
- With the green light, preparatory works including site selection and recruitment can commence shortly in Europe with first patient dosing expected in mid CY22.
- In order to fund the program, ANP completed a A\$20m institutional placement at A\$0.24 along with a 1 for 2 free-attaching options. ANP is aiming to raise a further A\$16.8m through a 1 for 9.4 rights issue on the same terms.
- The capital allows the company to complete its pivotal DMD program in EU up to a major valuation inflection point being a key go / no-go futility analysis at the mid-point of the trial, while the options (if exercised) will raise sufficient capital to cover trial costs (Ph2b/3 + open label extension phase).
- We viewed the stock as being handicapped by the obvious funding requirements. With this now removed, the story ahead appears primed with a thickening catalyst pipeline with EU trial updates, FDA progression, and indication expansion.
- Given the pipeline of news ahead, FY22 is shaping up to be a big year and we are looking to add to positions as the program progresses. Our risked price target increases to A\$0.58 (from A\$0.45) and we retain our Speculative Buy recommendation.

Where to from here?

- With EU trial funding concerns now largely removed, ANP has provided a clear roadmap post-EMA approval and highlights the line of sight into a number of major value inflection points over the short to medium term.
- We look to mid-CY23 as the next major catalyst being a futility analysis, which will provide a flag on whether a clear drug effect is taking place or not.
- Short-term catalysts (next 12 months) are: trial application, site activations, first patient dosing, FDA progression update, new indication progress, continuous enrollment updates, open label study commencement, and final recruitment.

To partner or not to partner?

- Eventually, but not yet – and even then it all comes down to value.
- One of the major expenses post approval in mass-market indications is marketing. With a relatively small and well-connected population, ANP is unlikely to require the support of a pharma with large sales forces and reach in order to quickly penetrate key markets. We view EU as a prime market to “go-it-alone” given the addressable market and population density.
- A partner typically provides funding, sales force, and external validation which ultimately de-risks the asset. However, we view relinquishing a significant portion of potential DMD revenues as short-sighted, at least until ANP have additional indications to accelerate development on and broaden the pipeline.
- However, looking forward we view ANP as a prime target for a number of players, particularly in gene-therapy where inflammation is believed to be a drug class effect which raises an immune response to the treatment.
- When do we think this will happen? With cashed up competitors, anytime is possible although we think the key lies in firstly de-risking the US program (tox study) and outcomes of further studies investigating the combination with ATL1102 and dystrophin restoration drugs.

Fair value and un-risked valuation upgrades

- Our fair-value valuation increases to A\$0.58 (from A\$0.45).
- Our unrisked valuation rises to A\$2.62 (from A\$1.82).

Investment view

- Continue to add to positions on any weakness. We continue to view ANP as one of the best risk/reward plays in the healthcare space. Speculative Buy.

Price catalysts

- FDA clinical and tox submission (late CY21), new indications (CY22), trial recruitment (CY22), trial commencement (CY22), futility analysis (CY23).

Risks

- Prolonged delays in trial commencement / Failure of DMD in Ph2b/3 program.

Antisense Therapeutics

as at November 11, 2021

Rating	SPECULATIVE BUY	Price (A\$):	0.21
Market cap (A\$m):	138.1	12-month target price (A\$):	0.58
Shares outstanding (m):	574.0	Up/downside to target price (%):	176.2
Free float (%):	100.0	Dividend yield (%):	0.00

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.

<p>Market considerations for ATL1102</p> <div style="display: flex; justify-content: space-around;"> <div style="background-color: #002060; color: white; padding: 10px; border-radius: 10px; width: 45%;"> <p style="text-align: center;">ANTI-INFLAMMATORY</p> <p style="text-align: center;"><i>The market size is expected global anti-inflammatory reach</i></p> <p style="text-align: center;">US\$191B by 2027</p> <p style="text-align: center;"><small>(Fortune Business Insights)</small></p> <p style="text-align: center;"><small>*MS, Rheumatoid Arthritis, Asthma, Sinusitis Respiratory, IBD</small></p> </div> <div style="background-color: #002060; color: white; padding: 10px; border-radius: 10px; width: 45%;"> <p style="text-align: center;">CORTICOSTEROIDS</p> <p style="text-align: center;"><i>The global steroid market is forecast to attain value of</i></p> <p style="text-align: center;">US\$17 Billion in 2025</p> <p style="text-align: center;"><small>(QY Research)</small></p> </div> </div> <div style="background-color: #c00000; color: white; padding: 10px; border-radius: 10px; margin-top: 10px; text-align: center;"> <p>DMD THERAPIES</p> <p><i>The global DMD drug market estimated to reach</i></p> <p>US\$4B by 2023 and</p> <p>US\$10B by 2030</p> <p><small>(Kamet Research)</small></p> </div>	<p>Major upcoming milestones</p> <ul style="list-style-type: none"> FDA clinical and tox submission (late CY21) New indications (CY22) Trial recruitment (CY22) Trial commencement (CY22) Futility analysis (CY23).
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Source: Antisense Therapeutics

Source: Antisense Therapeutics

INTERVENTION MECHANISMS	ASSET	SPONSORS
INFLAMMATION & FIBROSIS	ATL1102	antisense
	STERIODS Emflaza, VBP15	PTC, Santhera
	ANTI-FIBROTICS pamrevlumab	FibroGen
CARDIAC & CALCIUM REGULATION	CARDIAC DRUGS Ifetroban	Cumberland Pharma
	CALCIUM BALANCE Rimeporide	EspeRare Foundation
DYSTROPHIN REPLACEMENT & RESTORATION	GENE THERAPY	Pfizer
	EXON 51 /EXON 53/EXON 45 SKIPPING	Sarepta Therapeutics Nippon Shinyaku
	Ataluren READTHROUGH THERAPY	PTC Therapeutics
RESPIRATORY CELL ENERGY	A0364	Astellas Pharma
MUSCLE GROWTH & REGENERATION	CAP-1002 (intravenous cell therapy)	Capricor Inc
	Givinostat	Italfarmaco

Source: Antisense Therapeutics

ATL1102 mechanism of action

Mechanism of translation inhibition by ASOs

translation initiation factor

ASO mRNA

- ASO binds near start codon
- ASO sterically blocks translation initiation machinery or ribosome

Source: CureFFI.org

Core market data assumptions

EU MARKET DATA	#	Comments
Population of target market ('000s)	26.0	DMD population EU (ANP)
Non-ambulant population	50.0%	Assumes only non-ambulant
Number of Cases Forecast for Year 1 ('000s)	13.0	
Annual Population Growth	0.70%	Worldbank, 2016
Peak Market Penetration	25.0%	Low assumption
Revenue Per Unit (\$US)	\$ 150,000	Analysis of competing therapies
Market Ramp Time to Peak Penetration (Yrs)	7	Analysis of competing therapies
Hold peak	5	Avg peak sales time for new therapies
Life cycle of drug	20	Avg life cycle for new therapies
Royalty Rate	100.0%	Retain in-house
Total partnership milestone package	0	As above

Source: Morgans estimates

ATL1102 mechanism of action

Key Drivers

- Licensing 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 estimates

Trial structure and estimated timelines

ANP's Ph2/3 EU trial remains largely the same as the Ph2a in terms of target population, administration route, and primary / secondary endpoints. Major changes revolves around a significantly more robust trial design, increased patient coverage, and the addition of a mid-point futility analysis. Below we highlight the major changes in **green**, commentary in **red**:

Design: Gold standard, higher dose cohort, broader patient coverage

- **Multicentre, randomised, double-blind, placebo-controlled** study to determine the efficacy, safety, and pharmacokinetic profile of ATL1102 (25 mg and 50 mg) administered once weekly by subcutaneous (sc) injection for **52 weeks** in non-ambulatory participants with DMD, compared to a placebo control. **Gold standard trial setup. This compares to Ph2a being a single arm trial compared against a natural history (NH) study in typical degradation seen over the same time frame. The dosage schedule is also extended to 12 months (from 6 months) – we view this is a major benefit with the delta between NH and active arms continued to increase into the six month measure. Based on Ph2a results - longer dosing is likely see greater variance = higher efficacy measures.**
- Participants will be randomised to either weekly, sub-cutaneous dosing with 25 mg ATL1102, **50 mg ATL1102 or placebo** in a 1:1:1 ratio with stratification by corticosteroid use. **We view the addition of a “lower” higher dose (being 50mg rather than 100mg) as a sign of confidence that the existing data generated on the 25mg range was strong enough as a standalone.**
- The study is planned to be conducted in approx. **9 countries, ≥30 clinical trial sites** in Europe. **Management suggests this will take approximately 6-9 months to complete recruitment. We view the broad site coverage should accelerate recruitment timelines with an average of <4 patients per site. For perspective, the single-site Ph2a trial recruitment of 9 boys took ~9 months to complete. Avg time 30.3 days per patient. Therefore in our view there is upside to this timeline.**

Sample size: Powered to achieve statistical significance

- Up to 114 participants to be enrolled (38 per treatment arm) with **108 participants** to complete the study. **Powering undisclosed but given protocols have been agreed to with the EMA therefore we have no major concerns.**
- **Target population: No significant changes to target population**
- Participants with DMD (confirmed by genetic testing) and are non-ambulatory, defined as unable to walk 10 meters without assistance or help.
- 10 to 18 years of age, body weight of at least 25 kg.
- PUL 2.0 Entry Item A score ≥ 2 .
- If on corticosteroid therapy, therapy was initiated at least six months prior to the baseline visit and a stable daily dose for at least 3 months prior to baseline.

Primary endpoint: No major changes

To evaluate the effect of ATL1102 on upper limb muscle function in non-ambulant participants with DMD as assessed by change in the Performance of Upper Limb Module for DMD 2.0 (PUL 2.0) score compared to placebo.

Secondary endpoints: No major changes

To evaluate the effects of ATL1102:

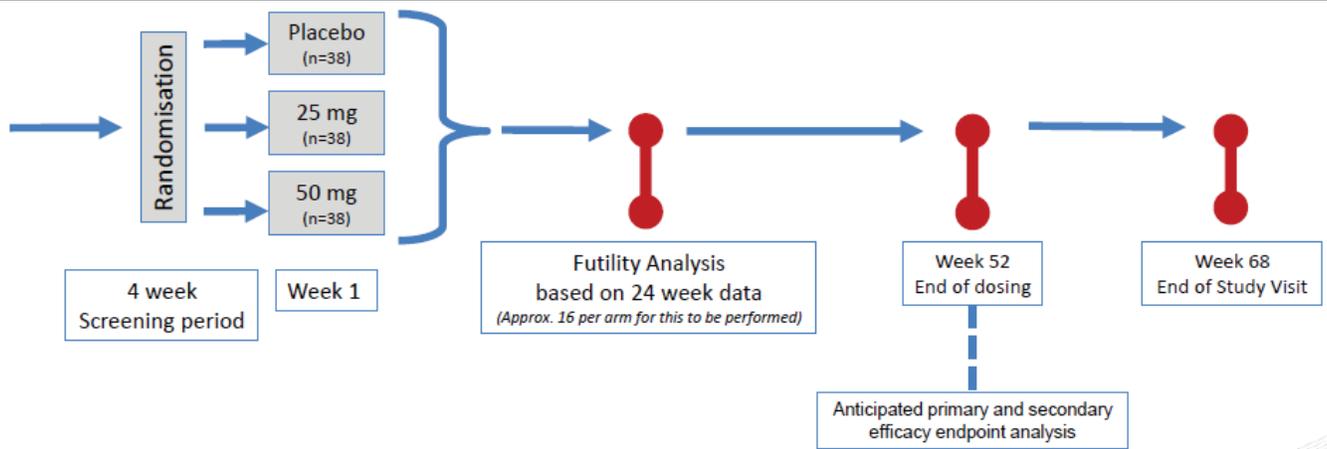
- on upper limb muscle strength as assessed by change in percent predicted MyoGrip and MyoPinch compared to placebo;
- in a responder analysis on the PUL 2.0 score compared to placebo;
- on respiratory function as assessed by change in % predicted PEF and % predicted FVC compared to placebo;

- on Quality of Life in participants and their Study Partner (Parent/Guardian) compared to placebo;
- safety and tolerability of ATL1102 including events associated with the Safety Monitoring Plan and Stopping Rules; and
- PK profile of ATL1102

Intended futility analysis: Major catalyst. ETA mid CY23

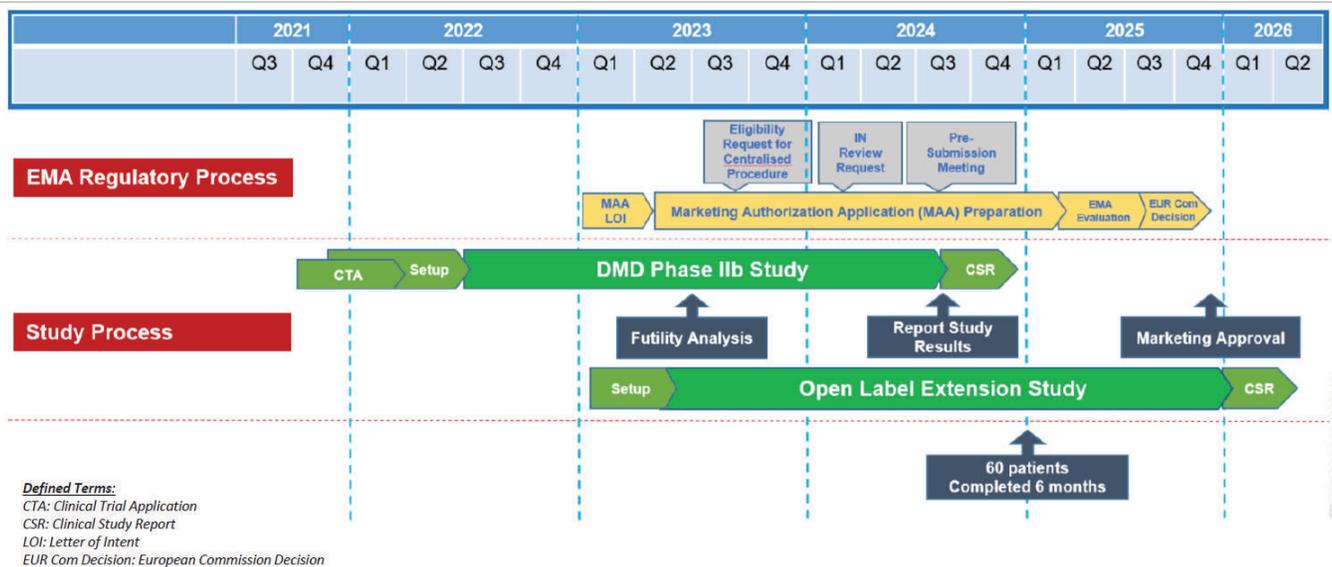
A futility analysis (FA) will be conducted (on approximately 16 patients per arm) on the 24-week PUL 2.0 data that assumes the 24-week result represents the difference between treatment group and placebo at 52 weeks. The independent Data Safety Monitoring Board (DSMB) will evaluate this FA with a go/no go decision to continue the treatment groups or the study itself. **We view this as the next major catalyst. These analyses to allow the DSMB to review the data to ensure the data remains on track to achieve a result. While investors and the Company will not get a read-through (kept within DSMB), we view a green-light as a strong indication of clear separation between the active and placebo groups and will act as a major re-rate catalyst if successful.**

Figure 2: EU Ph2b/3 study program



Source: Antisense Therapeutics Investor Presentation – Nov 2021

Figure 3: ANP’s anticipated EU Ph2b/3 study timeline



Source: Antisense Therapeutics Investor Presentation – Nov 2021

Use of funds

As of now – ANP has guaranteed A\$20m through a sophisticated and institutional placement, sufficient to fund ANP’s program up to the trial application approval and other preparatory works (drug manufacture, regulatory, etc) up to first patient enrolment. The remaining funding (~A\$16.8m) through the entitlement offer will be used to fund the ongoing Ph2b/3 trial costs through to the critical futility analysis timepoint.

The funding of the remaining facets of the program (open-label extension, regulatory submissions and approvals) is anticipated to be raised upon exercise of the 1:2 free-attaching options (exercise price A\$0.48) to raise a further A\$36.8m. These options are expected to expire late CY24 or 20 business days post futility analysis (estimated mid CY23).

Figure 4: Use of funds

Use of Funds Raised	Placement	Placement & Entitlement Offer
Phase IIb/III - Clinical Study costs	*\$7,000,000	**\$21,000,000
Open Label Extension Study initiation costs	-	\$600,000
Drug Manufacturing Costs	\$5,500,000	\$7,400,000
Progression of R&D Programs	\$1,100,000	\$1,100,000
Working Capital	\$5,000,000	\$5,000,000
Capital Raising Costs	\$1,400,000	\$1,300,000
TOTAL	\$20,000,000	\$36,800,000

Source: Antisense Therapeutics Investor Presentation – Nov 2021

Figure 5: ANP options valuation (Black Scholes Valuation Model)

ASSUMPTIONS	
Cost of Option	\$0.00
Share Price (Offer Price)	\$0.240
Exercise Price	\$0.48
Start Date	17/12/2021
Acceleration Date (futility analysis)	31/03/2023
Period to Exercise (in years)	1.28
Risk Free Rate of Return	0.3%
Volatility factor (sd ²)	80.0%
Dividend pa per share	0.0%
Dividend adjusted share price	\$0.240
Present value of exercise price	\$0.478
Value of each Option	\$0.037
Per ordinary issued (times 1/2)	\$0.019

Source: Morgans estimates, company data

ANP is currently trading below the ~A\$0.22 total value in the placement and rights issue (shares issued at A\$0.24 plus the additional value of the free options being A\$0.037 (or A\$0.019) based on the 1:2 offering).

The rights went ex 10-Nov-21 and closes on the 29th of November. Given the current trading levels below placement value, we view a shortfall as likely. In this case, ANP have ~ 3 months to allocate any shortfall stock. Over this time, we would expect a number key catalysts to pass including FDA toxicology progress and potentially progress on new indications.

Other happenings in the space

Sarepta (SRPT-US): raises US\$500m in mid-October in addition to its US\$1.6bn cash balance. Interesting raise considering the significant cash balance. Use of funds were “*principally for the continuation of, and initiation of further, clinical trials, commercialisation, manufacturing, business development activities, including the potential licensing or acquisition of complementary products, technologies and entities, and other general corporate purposes.*”

Pfizer (PFE-US): expecting delays in its pivotal DMD study, now expecting to read out in CY23 due to safety concerns in patients with certain genetic mutations causing muscle weakness / heart inflammation. Interesting to note the company highlighted that these types of serious adverse events (SAE) being potentially inherent in gene replacement therapies, supported by other SAEs across other programs. Supports the notion that inflammation is potentially a by-product of the therapy, indicating inflammation risk as a drug-class effect.

Modelling changes

Major changes include formally segregating the base-case scenarios for EU and US jurisdictions in-line with our view of divergent models for each jurisdiction and rolling through the capital raise and updated timelines.

EU base case: ~52cps valuation

- **DMD population:** Maintains assumption for addressable market in only ambulant population (50%). Upside to 100% based on off-label use if approved / expansion with further studies around efficacy for ambulant populations.
- **Peak market penetration:** maintain our base-case assumption for 25% of peak market penetration across eligible boys. Analysis of SRPT’s Exondys 51 drug since launch in 2016 shows penetration of roughly ~25%.
 - Addressable patients for Exondys = 13% of the up to 18,000 patients in the US= 2,340 patients
 - Exondys 2020 revenue= \$446m
 - Treatment costs in the US ~\$750k / patient / year
 - Penetration is \$446m / \$750k = 594 patients of the 2,340 = 25%.
- We view the upside potential for ATL1102 higher due to lower cost, low side-effect profile, and functional efficacy results.
- **Treatment cost per patient:** Base case of US\$150k p.a. based on low-end of DMD drug pricing in space. Discount to US pricing of US\$200k. Upside to ~US\$400k p.a. with confirmation of strong efficacy read post Ph2b/3 efficacy.
- **Royalty rate 100%:** The major point of difference to US model is that we assume 100% royalty rate in EU. We model ANP as retaining all marketing rights for EU given the current/planned funding underway. We view EU as a prime market given the population density and well-connected DMD population with a small number of key opinion leaders (KOLs).
- **Trial costs:** Assumes ANP bearing all trial and regulatory costs in-line with updated presentation.
- **Trial risks:** With the view that the EU Ph2b/3 trial serves as a registrational trial – we adjust our average trial progression rates of metabolic disease indications to the mid-point between Ph2 progression rates of 52% and Ph3 of 79%. Risked progression to approval now sits at 60.9% (from 48.3%).

Figure 6: EU market assumptions

MARKET DATA	#	Comments
Population of target market ('000s)	26.0	DMD population EU (ANP)
Non-ambulant population	50.0%	Assumes only non-ambulant
Number of Cases Forecast for Year 1 ('000s)	13.0	
Annual Population Growth	0.70%	Worldbank, 2016
Peak Market Penetration	25.0%	Low assumption
Revenue Per Unit (\$US)	\$ 150,000	Analysis of competing therapies
Market Ramp Time to Peak Penetration (Yrs)	7	Analysis of competing therapies
Hold peak	5	Avg peak sales time for new therapies
Life cycle of drug	20	Avg life cycle for new therapies
Royalty Rate	100.0%	Retain in-house
Total partnership milestone package	0	As above

Source: Morgans estimates, company data

US base case: ~6cps valuation as standalone

- **DMD population:** Maintains assumption for addressable market in only ambulant population (50%). Upside to 100% based on off-label use if approved / further studies around efficacy for ambulant populations.
- **Peak market penetration:** maintain our base-case assumption for 25% of peak market penetration across eligible boys. Analysis of SRPT's Exondys 51 drug since launch in 2016 shows penetration of roughly ~25%. Upside for ATL1102 significantly higher due to lower cost, low side-effect profile, and functional efficacy results.
- **Treatment cost per patient:** Base case of US\$200k p.a. based on low-end of DMD drug pricing in space. Higher prices achieved in US vs EU. Upside to ~US\$500k p.a. with confirmation of strong efficacy read post Ph2b/3 efficacy.
- **Royalty rate 20%:** On low-end of assumption. Typical royalty rates on mass-market indications usually range from high single digit to low double digit ranges. However, given the expected knowledge density through KOL's resulting in lower spend required to market the drug and results/progress from pivotal EU study - prior to US studies significantly de-risking the US regulatory pathways - we view a significantly de-risked pathway for a potential partner. The lower risk due to EU progress, low regulatory/trial cost, and lower marketing cost. Our high range estimate grow to 40%.
- **Trial costs:** Assumes partner to bear all potential regulatory and trial costs.
- **Milestone payments:** US\$350m. Based on half of average biotech partnering deal values of US\$700m / relatively small TAM / rarity of disease. Compared to NEU total milestone deal in 2019 of US\$455m (2 indications but high trial cost and low upfront). Lack of clinical applications for DMD and data on typical licensing deals within the space. The only major deal demonstrates upside potential with US\$2.85bn deal between Sarepta and Roche (US\$1.15bn upfront) in 2019.
- **Trial risks:** We maintain average Ph2 progression risks at 52% with the view to move it in-line with the EU assumption once FDA IND clinical hold is lifted and FDA trial protocols are accepted. Trial risks remain at 48.3%.

Figure 7: US market assumptions

MARKET DATA	#	Comments
Population of target market ('000s)	18.0	DMD population US (ANP)
Regulatory approval weight	100.0%	
Non-ambulant population	50.0%	Assumes only non-ambulant
Number of Cases Forecast for Year 1 ('000s)	9.0	
Annual Population Growth	0.70%	Worldbank, 2016
Peak Market Penetration	25.0%	Low assumption
Revenue Per Unit (\$US)	\$ 200,000	Analysis of competing therapies
Market Ramp Time to Peak Penetration (Yrs)	7	Analysis of competing therapies
Hold peak	5	Avg peak sales time for new therapies
Life cycle of drug	20	Avg life cycle for new therapies
Royalty Rate	20.0%	Base-case assumption
Total partnership milestone package (\$US)	350	Base-case assumption

Source: Morgans estimates, company data

Capital raise: We have updated our capital raise assumptions given the recent placement and rights issuance. While the rights issuance is not underwritten, the Company has three months to place any shortfall. Given the current proximity to offer price and potential short-term catalysts – we assume for now that any shortfall will be successfully placed.

We had previously modelled a A\$30m raise at 15cps (+200m SOI) in FY22. The placement and rights issuance at 24cps (+153m shares) is significantly less dilutive than prior assumptions and delivers an extra A\$6.8m in funding. We have also assumed the exercise of options at 48cps in FY24 to raise an additional A\$36.8m while adding 76.7m shares to the register. In total, we have added an additional 230m SOI raising A\$73.6m cash consideration over FY22-24.

Changes to forecasts

With further clarity post EMA approval, we are able to more accurately estimate trial costs and expected timelines. Major changes to previous forecasts are around trial commencement and associated costs.

The updated timelines shows 9 month delay to trial commencement in EU (trial commencement costs pushed to FY23) versus our initial forecasts and we back-end weight our initial US partnering assumptions to FY26 (from FY24). We have also assumed base case R&D rebates applicable to ANP's programs and limited to exploratory R&D works in Australia.

The changes to near-term forecasts are offset by the reduction in risk-weighted revenues expected to be generated from FY27 onwards and better-than-forecast capital raising assumptions.

Figure 8: Changes to forecasts (A\$m)

	FY22 (old)	FY22 (new)	% Δ	FY23 (old)	FY23 (new)	% Δ	FY24 (old)	FY24 (new)	% Δ
Revenue	1.4	1.4	0.0%	5.7	2.1	-62.8%	45.2	2.0	-95.6%
EBITDA	-22.9	-11.3	50.9%	-8.6	-24.9	-188.1%	30.1	-27.0	-189.8%
NPAT	-22.8	-11.1	51.1%	-8.3	-24.2	-190.6%	21.1	-26.8	-227.0%
EPS	-3.4	-1.7	49.4%	-1.1	-3.3	-209.2%	2.7	-3.5	-228.4%
DPS	0.0	0.0	n.a.	0.0	0.0	n.a.	0.0	0.0	n.a.

Source: Morgans estimates, company data

Valuation

High and low cases

We have valued ANP using a risked DCF 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 which is based on an unsuccessful trial. Valued on cash backing and net assets.

Risked valuation (A\$0.58): DCF methodology. Risked assumptions based on average historical success rates for metabolic drugs potential marketability of

asset. Assumes retention of all marketing rights and distribution in EU / partner US jurisdiction and associated costs. Multiple assumptions including addressable market, drug pricing, peak penetration rates, potential partnership deal and structures.

De-risked valuation (A\$2.62): DCF methodology. Trial risked assumptions set to 100% on EU and US approval and assumes a successful commercial launch. ANP to retain EU distribution rights and US jurisdiction partner to fund FDA registration and marketing. Assumes broad access across DMD population (both ambulant and non-ambulant). Further upside potential lies in upward adjustments to our high range for peak penetration rates, pricing, licensing deal metrics, and royalty rate structures.

Price target

We use our risked valuation of 58 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 note our recommendation of a Speculative Buy highlights the risk/reward nature of the asset.

Queensland

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Australian Capital Territory	
Canberra	+61 2 6232 4999

Victoria

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Stockbroking, Corporate Advice, Wealth Management	
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Domain	+61 3 9066 3200
Geelong	+61 3 5222 5128
Hawthorn	+61 3 9900 4350
South Yarra	+61 3 9006 9955
Southbank	+61 3 9037 9444
Traralgon	+61 3 5176 6055
Warrambool	+61 3 5559 1500

Western Australia

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Perth	+61 8 6462 1999

South Australia

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Stockbroking, Corporate Advice, Wealth Management	
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