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Antisense Therapeutics

Sensing big things ahead

ANP is an Australian biotechnology company focused on the development and commercialisation of a drug class called antisense therapeutics for the treatment of diseases where there is a need for improved therapies.

ANP's lead candidate, ATL1102, is a Phase 2 asset that has data demonstrating its safety, tolerability, and efficacy across a range of diseases, including Duchenne's Muscular Dystrophy (DMD) and Multiple Sclerosis (MS). Due to its unique mechanism of action and anti-inflammatory properties, ANP has also earmarked potential application in the treatment of other inflammatory conditions.

ANP recently appointed ex-Sarepta (SRPT.NAS) director Dr Gil Price as its US based Medical Director to provide an on-the-ground presence to advance US clinical development activities and commercialisation plans.

In this report, we describe what ANP does, highlight the clinical results achieved, and detail a list of companies that serve as relevant comparison, noting that the average enterprise value (EV) for similar stage Australian life science companies is A$172.3m, compared with ANP at A$13.2m (Factset).

COMPANY SNAPSHOT

Reuters/Bloomberg:	ANP.AX / ANP AU
Market cap:	US$12.3m
	A$20.5m
Current price:	A$0.042
Average daily turnover:	US$51k
	A$85k
Current shares o/s	488.7m
Free float:	100%

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

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. It has been reported 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 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.

Phase 2a DMD trial top-line results

ANP released its top-line results in December 2019, confirming a strong safety profile and positive drug effects on disease progression endpoints on the low dosage testing. The trial was conducted over 24 weeks using the ATL1102 drug in nine non-ambulant (wheelchair bound) boys and showed to be generally safe and well tolerated with no serious adverse events or concerns raised from the Data Safety Monitoring Board. Secondary endpoints were to assess drug activity (impact on immune cell numbers) as well as test to see if the treatment had any measurable impacts on the functional capacity in the patients via Performance of Upper Limb Test (PUL2.0) and upper limb strength via MyoGrip and MyoPinch assessments. The Company then compared the results achieved in the study to similar functional assessments on DMD boys in a 2016 study by Ricotti et al in non-ambulant DMD boys on corticosteroids who were also monitored for 6 months. The results of the drug activity measures showed a consistent reduction of CD49d lymphocytes from baseline followed by statistically significant return to starting levels post treatment in a subset of cells expressing CD49d. This data is supportive of its intended mode of action to modulate the targeted T cells in the patient's system. Looking across the function tests, the results suggest an overall improvement in disease progression with all functional tests showing either less of a decline than boys observed within the Ricotti study, and in some cases showing a physical improvement in functional capabilities. Results are pending on new efficacy data on additional disease progression endpoints with the final trial data to be reported in April.

Listen to ANP's CEO talk about recent developments

We spoke to CEO Mark Diamond for his views on a number of recent announcements as well as developments over the next 12 months. Click here

Antisense Therapeutics

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 in 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.

Key drivers

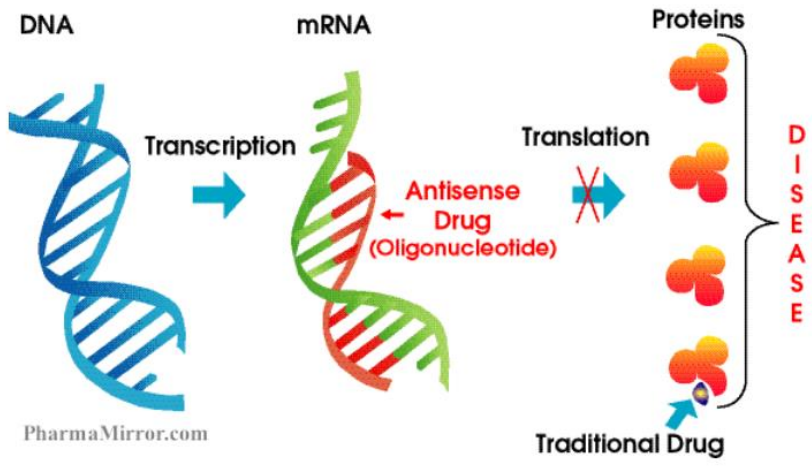
There are a number of key drivers including:

- Two Phase II compounds (ATL1102 & ATL1103) with strong clinical efficacy which have been peer reviewed in a number of scientific journals;
- Pending Phase II in DMD final results with new efficacy data on additional disease progression endpoints;
- Phase IIb in DMD and potential for conditional approval on completion;
- Work starting on new indications, given the success of ATL1102 in DMD;
- Compounds targeting underserved markets;
- Advancing US clinical development activities and commercialisation plans; and
- Potential for partnering to further develop compounds.

Mechanism of action

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.

Figure 1: Mechanism of action



SOURCES: PharmaMirror.com

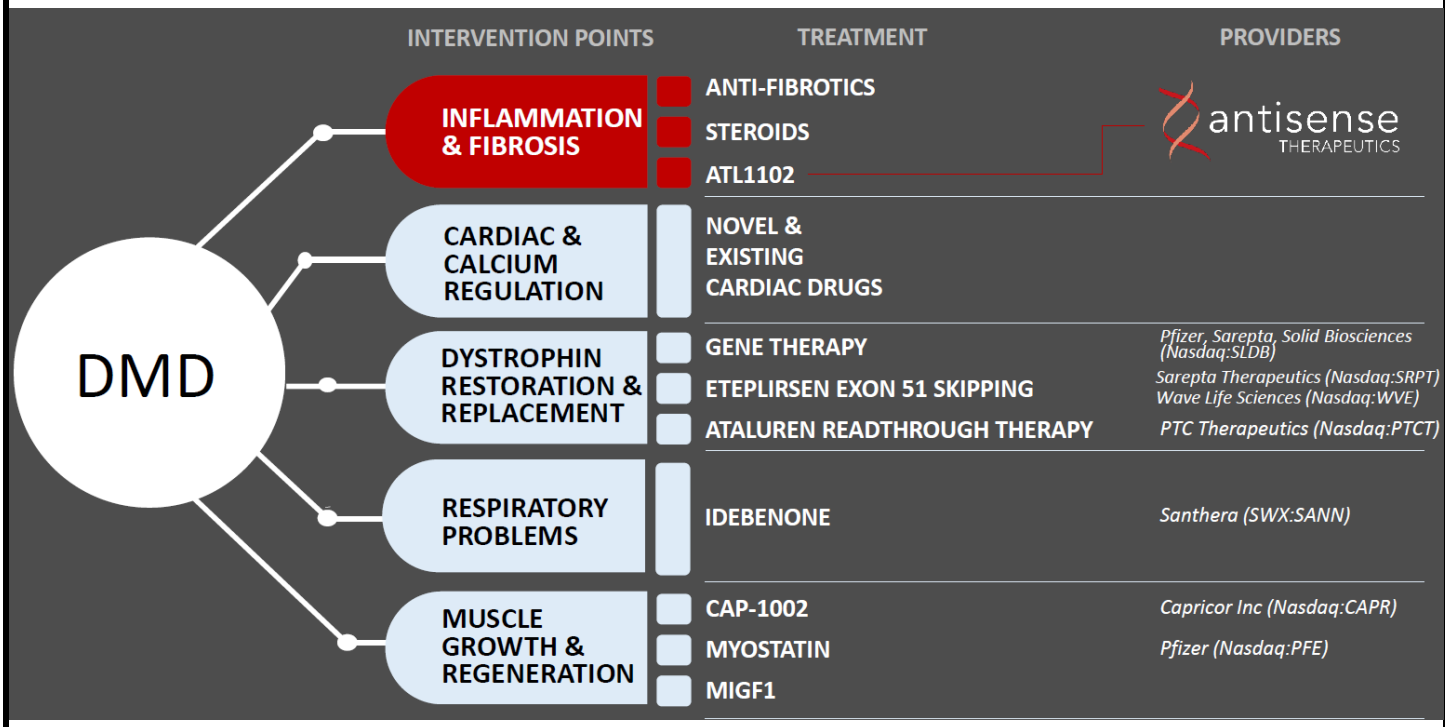
How ATL1102 works

Antisense drugs block the production of the target protein. In the case of ATL1102, it is designed to reduce levels of CD49d (also known as VLA-4).

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, management expects if approve, for the treatment of DMD ATL1102 would likely be applied in concert with other treatments (combination therapy) to combat the inflammation aspect.

Figure 2: 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 a known to be a factor in the progression or severity of the diseases 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 (preliminary data – full data set expected April 2020):

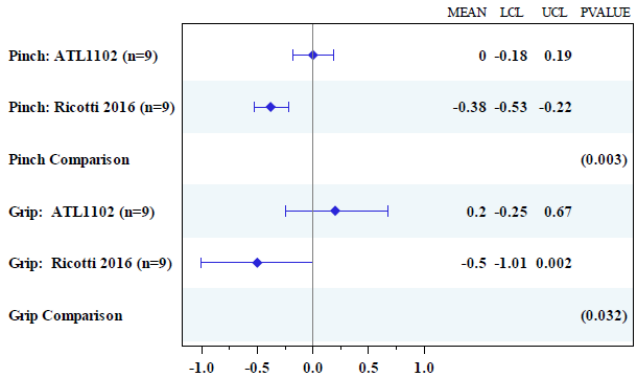
- Safe (generally well tolerated). No serious adverse events or patient withdrawals. Minor issues such as injection site erythema, skin discolouration and injection site pain.
- Immunomodulatory effect observed. CD49d levels trending downward during treatment phase and returned to starting levels post dosing phase.
- Demonstrated mean improvement of 0.9 in PUL2.0 test (upper limb function) and 7 or the 9 boys either demonstrated increases or no change in PUL2 0 compared to the losses reported in the published literature comparator dataset.
- Similar improvements seen across MyoGrip and MyoPinch tests.
- Respiratory data was inconclusive and required substantially more subjects over a longer time frame to produce interpretable changes.

Figure 3: Lymphocyte mean # of cells at week 24

White blood cell type (X10^9 cells per litre)	Mean # and Change from baseline			Median % change from baseline	
	Baseline	24 weeks (end of dosing)	28 weeks	24 weeks (end of dosing)	28 weeks
Lymphocytes (mostly CD3+ T cells)	3.68	-0.28	+0.19	-4.22%	+11.81%
CD3+ T cells (mostly CD3+ CD4+ and CD3+ CD8+ T cells)	2.93	-0.18	+0.25	0.86%	+17.11%
CD3+ CD49d+ T cells (CD4+CD49d+ and CD8+CD49d+ cells)	2.44	-0.28	+0.11*	-9.78%	+9.93%
CD4+ T cells	1.57	-0.15	+0.11	-1.12%	+16.50
CD4+ CD49d+ T cells	1.20	-0.19	+0.01	-16.7%	+1.73
CD4+ CD49d++ T cells (are the high CD49d expressing CD4+ T cells)	0.24	-0.01	+0.01	-11.1%	+7.58
CD8+ T cells	1.22	-0.02	+0.14	-2.62%	+17.99
CD8+ CD49d+T cells	1.17	-0.05	+0.11	-5.79%	+13.37
CD8+ CD49d++ T cells (5 of 9 patients had these cells at baseline) (are the high CD49d expressing CD8+T cells)	-	-	-	-6.17%	+14.12

SOURCES: COMPANY REPORTS

Figure 4: 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 IIa 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.

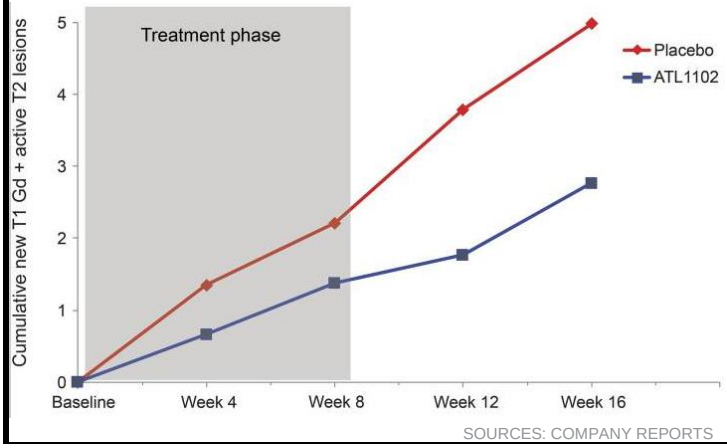
Clinical data:

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:

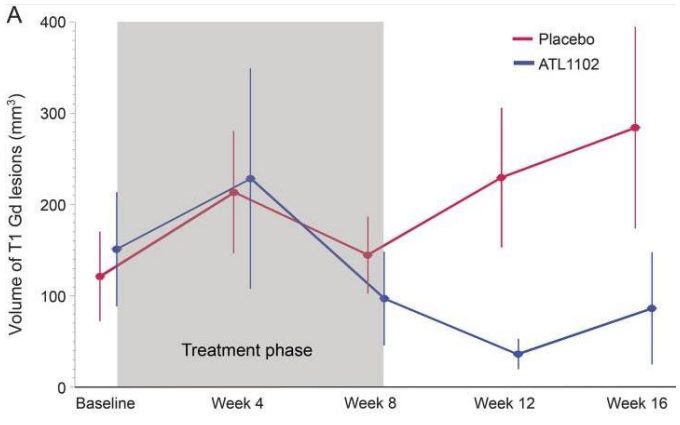
- Adverse events that were more frequent under ATL1102 included mild to moderate injection site reactions and a tendency for decreased platelet counts which were reversible after treatment interruption.
- ATL1102 significantly reduced the cumulative number of new active lesions by 54.4% compared to placebo (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 5: Cumulative new lesions



SOURCES: COMPANY REPORTS

Figure 6: Volume of lesions



SOURCES: COMPANY REPORTS

Other applications of ATL1102

Following the reported clinical trial results in the Phase II 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 IIb trial in DMD, the Company 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).

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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).

Acromegaly – Ph II 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). There are a number of diseases that 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 II 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.

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 an 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$5.1m at 31 Dec-19) and operating cash outflow (A$1.4m for 6-months ended Dec-19), 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 look to either partner, 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.

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Comparable companies

We have selected 13 Australian listed life science companies that are currently undertaking Phase I or Phase II clinical development. The average enterprise value (EV) of this cohort is A$172.3m compared with ANP's EV of A$13.2m (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$10.9bn.

Sarepta's treatment is only applicable to 13% of boys with the exon 51 mutation, while ANP's ATL1102 is aimed as a much broader application, being developed to treat the inflammation that is caused by the loss of dystrophin, exacerbating tissue damage and causing fibrosis in DMD patients — an aspect of the disease that currently can only be managed with corticosteroids and affects all DMD patients.

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 overleaf.

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Figure 7: Comparable domestic and international companies

Ticker	Company name	Business description	Phase assets	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	Ph2	13.27	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.	Ph2	254.18	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 Melbourne, Australia.	Ph2 / Ph3	91.33	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 2	937.61	Stem-cell	Cardiovascular Graft-versus-host
IVX-AU	Invion Ltd.	Invion Ltd. is a clinical-stage life sciences company, which engages in the research and development of treatments for market opportunities in a variety of cancer indications, chronic inflammatory, and autoimmune diseases. It focuses on the manufacturing and development plans for Photosoft, identification and engagement of specialist advisors, and progressing preclinical and clinical development planning. The company was founded on October 11, 2000 and is headquartered in Melbourne, Australia.	Ph1	38.39	Photosensitisers	Oncology
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 in January 2017 and is headquartered in Melbourne, Australia.	Ph 3	221.32	Molecular targeted radiation	Oncology
CYP-AU	Cynata Therapeutics Limited	Cynata Therapeutics Ltd. engages in the development and commercialization of therapeutic products. It focuses on therapeutic stem cell platform technology. The company was founded on March 12, 2003 and is headquartered in Carlton, Australia.	Ph1	72.38	Stem-cell	GvHD Osteoarthritis
OPT-AU	Opthea Limited	Opthea Ltd. is engaged in the development of biological therapeutics for cancer and other serious diseases. It is a biologics drug developer building on significant intellectual property portfolio around Vascular Endothelial Growth Factor C and D angiogenic molecules and R3. The company focuses on vascular endothelial growth factors to develop truly novel therapies to extend and improve the lives of cancer sufferers. Opthea was founded on October 17, 1984 and is headquartered in South Yarra, Australia.	Ph3	437.51	VEGF	Wet Age-related Macular Degeneration
NOX-AU	Noxopharm Ltd.	Noxopharm Ltd. engages in the research and development of drugs. It focuses on sensitizing cancer cells to radiotherapy and chemotherapy. The company was founded by Graham Kelly on October 27, 2015 and is headquartered in Gordon, Australia.	Ph1	30.77	S1P Inhibitor	Oncology
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	8.26	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	61.15	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	26.99	Anthracyclines	Oncology
RCE-AU	Recce Pharmaceuticals Ltd.	Recce Pharmaceuticals Ltd. is engaged in the research and development of pharmaceutical drugs that kill germs. It produces antibiotics that are effective in attacking disease-causing Gram-positive and Gram-negative bacteria. The company was founded on April 11, 2007 and is headquartered in Sydney, Australia.	Ph1	37.22	Synthetic antibiotics	Blood inflections
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	22.70	PI3K modulation	Oncology
Average				**172.29**		
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	10,912.53	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	31.97	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	312.89	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	3,656.99	Ataluren Readthrough Therapy	Duchennes Muscular Dystrophy
SANN-CH	Santhera Pharmaceuticals Holding A	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	176.01	Idebenone	Duchennes Muscular Dystrophy
Median				**312.89**		
Average				**3,018.08**		

SOURCES: MORGANS, FACTSET

Significant shareholdings

Figure 8: Top 10 shareholders

Name	Ownership
Top 40 holders	*57.74%*
Australian Ethical Investment	18.50%
Platinum Asset Management	6.27%
Leon Serry	6.15%

SOURCES: MORGANS, COMPANY REPORTS

Board and Scientific Advisory Board

Figure 8: 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 9: 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

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Stockbroking, Corporate Advice, Wealth Management	
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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

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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

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Northern Territory

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Tasmania

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Western Australia

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Stockbroking, Corporate Advice, Wealth Management	
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South Australia

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Analyst owns shares in the following mentioned company(ies): Antisense Therapeutics

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