Antisense Therapeutics

COMPANY SNAPSHOT

Reuters/Bloomberg:	ANP.AX / ANP AU
Market cap:	US$21.4m
	A$32.7m
Current price:	A$0.067
Average daily turnover:	US$51k
	A$85k
Current shares o/s	488.7m
Free float:	100%

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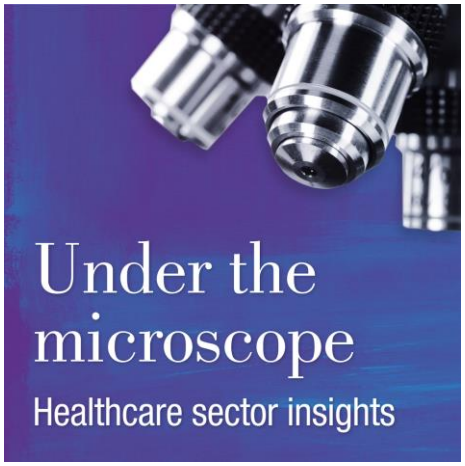
– Antisense Therapeutics



Ph2a DMD results successful

ANP is an Australian biotechnology company focused on the development and commercialisation of a drug class called antisense therapeutics with a number of therapies currently targeting Duchenne's Muscular Dystrophy (DMD), Multiple Sclerosis (MS), and Acromegaly.

ANP recently released positive results from its final Ph2a DMD trial, successfully reaching its primary endpoint of safety/tolerability, and exceeding expectations for the secondary endpoints of efficacy.

An unexpected observation in the results included MRI scans which showed a stabilisation of fat fraction percentage and the preservation of functional muscle mass amongst the boys, suggesting the drug may be also having a disease modifying impact on muscle dystrophin loss alongside its primary mechanism of action.

In this report, we highlight the clinical results achieved, and refresh the list of companies that serve as relevant comparison, noting that the average EV for similar stage Australian life science companies is ~A$300m, compared with ANP at A$23.0m.

Detailed Ph2 results show some interesting efficacy measures

ANP released its final report for its Ph2a DMD trial, confirming the ATL-1102 safety profile and demonstrating strong effects across its secondary endpoints on activity markers and disease progression which exceeded expectations. The final report (top-line data released in December 2019) presented more detail as well as a number of new efficacy measures and observations that suggest the drug may be having potential disease modifying effects on muscle strength and function in addition to its primary target of reducing inflammation resulting in muscle fibre damage and degradation in patients. The data from the study shows an apparent improvement in muscle strength based on the observed mean change from baseline after 24 weeks of dosing with ATL1102 as assessed by MyoGrip (+0.2) and MyoPinch (+0.0) tests compared to the loss of muscle strength reported in the literature in similar patient populations. The data is also suggestive of an improvement in muscle function as assessed by the Performance of Upper Limb Test (PUL 2.0), where 7 of the 9 participants have demonstrated clinically meaningful improvements or stabilisation in their PUL 2.0 scores (+0.9) from baseline after 24 weeks of dosing with ATL1102. MRI data suggests stabilisation of percentage of fat in muscles and preservation of functional muscle mass confirming a positive change at a muscular/cellular level and supports the observed physical stabilisation / improvements in muscle strength and function. "Based on the MRI data from the study, the observed stabilisation in the percentage fat fraction with ATL1102 treatment would not be expected in the natural course of disease in DMD even under corticosteroid treatment. Furthermore, the stabilisation of fat fraction percentage combined with the observed maintenance / increase of remaining muscle area is suggestive that ATL1102's effect could preserve the contractile muscle mass." – Dr Valeria Ricotti.

Next steps – reg submissions, new indications, partnerships

ANP's scientific advisory board has recommended to advance the drug into a Phase 2b trial. The Company awaits the European Medicines Agencies (EMA) scientific advice due mid-year which will enable ANP to prepare for its Ph2b submission for Europe and the UK for DMD. Concurrently, ANP is also preparing a submission for Orphan Drug Designation in the US and EU. Given the strength of the results shown in the trial and on the advice from its scientific advisory board, ANP stated it is now also investigating other potential diseases including other muscular dystrophies and neurological conditions which are inadequately controlled by corticosteroids. In regards to potential partnership or license agreements, the Company stated "as we gain further certainty from the regulatory process on the parameters of the next trial, which may lead to early market approval, we will assess funding and trial management options and also engage in discussions with interested potential partners ahead of commencement of Phase 2b."

Listen to ANP's CEO talk about trial results

We spoke to CEO Mark Diamond to get his views on the trial result announcement. Click here.

Comparable companies

In our last desk note for ANP (see here), we provided a comp table of listed companies that were undertaking Phase I or Phase II clinical development. The current average enterprise value (EV) of this ASX listed cohort is ~A$300m and A$450m in the international comps compared with ANP's EV of A$23.0m.

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	23.02	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	430.19	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	153.44	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	1,990.40	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	43.89	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	299.52	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	62.15	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	682.25	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	31.38	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	15.07	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	49.02	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	31.50	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	70.00	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	19.10	PI3K modulation	Oncology
Average				**298.30**		
Global comps (DMD)						
SRPT-US	Sarepta Therapeutics, Inc.	Sarepta Therapeutics, Inc. is a commercial-stage biopharmaceutical company, which is engaged in the discovery and development of therapeutics for the treatment of rare diseases. The company was founded on July 22, 1980 and is headquartered in Cambridge, MA.	Commercial	15,328.21	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	150.90	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	452.50	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	4,410.43	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	193.57	Idebenone	Duchennes Muscular Dystrophy
Median				**452.50**		
Average				**4,107.12**		

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Analyst owns shares in the following mentioned company(ies): Antisense Therapeutics
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29.01.19