

## Quarterly Activities Report & Appendix 4C

- **ANP to participate in development of DMD Guidance for FDA**
- **Update on US Regulatory Plans for ATL1102 in DMD**
- **New proteomics data supports ATL1102's broader clinical potential**
- **Positive feedback received on Paediatric Investigation Plan**

Antisense Therapeutics Limited (Antisense or Company) is pleased to provide its Appendix 4C and quarterly update for the period ended 30 September 2021.

### **ANP to participate in PPMD development of new Community-Led Duchenne Guidance for FDA**

In August 2021, Antisense Therapeutics accepted an invitation and nominated its US-based Non-Executive Director & Medical Director, Gil Price, MD to serve as a member of the Pharmaceutical Advisory Board (PAB) for the development of the New Duchenne Guidance by Parent Project Muscular Dystrophy (PPMD) for the US FDA.

Working closely with the Steering Committee and Working Group Chairs, comprised of individuals representing the patient advocate, caregiver, clinician, researcher, academic, and pharmaceutical industry, the PAB will focus on ensuring perspectives from companies with an interest Duchenne community are represented throughout the guidance.

PPMD successfully developed the first-ever patient group initiated draft guidance for companies developing treatments for Duchenne. Submitted to the FDA in June 2014, the work was a key resource informing companies and FDA about the evolving drug development landscape for Duchenne muscular dystrophy (DMD), as well as the patient focused views of benefit expectations and risk tolerance of the community. PPMD initiative has since become a landmark not only in the Duchenne community, but across rare disease communities exemplifying the value patients and caregivers can bring to drug development.

PPMD has now begun the process for modernizing the landmark Community-Led Guidance of 2014 document to ensure it reflects many advancements in knowledge, understanding, care, clinical trials and approvals over the recent years. Similar to the 2014, PPMD has formed a Steering Committee and Working Groups of over 80 stakeholders. This will help drive even more innovation as well as carve a path toward the ultimate goal of accessible therapies for all patients.

PPMD is the largest most comprehensive non-profit organization in the United States focused on finding a cure for DMD - their mission is to end DMD ([www.parentprojectmd.org](http://www.parentprojectmd.org)).

### **Update on US Regulatory Plans for ATL1102 in DMD**

Further to the outcomes reported following ANP's Type C meeting with the FDA and the FDA's positive feedback on the design parameters for a US Phase IIb/III study (refer Company's 1 June 2021 ASX announcement), the Company has continued to work with its expert US based regulatory advisors on appropriate next steps to advance the ATL1102 DMD program in the US. Accordingly, ANP expects to submit the protocol synopsis to the FDA for a nine-month chronic monkey toxicology study to support the dosing of patients beyond 6 months in the US.

## **Statistically significant modulation in two DMD disease modifier proteins supports potential of ATL1102 in ambulant DMD and fibrotic conditions**

In September 2021, new ATL1102 Phase II non-ambulant DMD patient plasma protein data was presented at the 26th International Annual Congress of the World Muscle Society in the late breaking news poster titled "ATL1102 treatment in non-ambulant boys with DMD modulates Latent TGF-beta-binding protein 4, and thrombospondin-1, two disease genetic modifiers of ambulant DMD, and CXCL16"

Planned as part of the Phase II study, a large-scale protein analysis (known as a proteomics analysis) of retained blood plasma samples from the non-ambulant DMD patients treated with ATL1102 was undertaken to identify the proteins affected in the blood so as to provide further insight into the mode of action and biological activity of ATL1102.

- Statistically significant mean modulation at 24 weeks compared to baseline in Thrombospondin-1 (TSP-1) and Latent TGF-beta-binding protein 4 (LTBP4) levels, two proteins that modify the rate of loss of ambulation in DMD. ATL1102 modulation of these two DMD disease modifier proteins known to impact TGF- $\beta$  and the rate of loss of ambulation in DMD patients supports ATL1102's potential use in ambulant patients with DMD, and as an agent to reduce fibrosis in other human diseases.
- Increase at 24 weeks in plasma VCAM-1 supportive of the ATL1102 mechanism of action of reducing CD49d on the surface of cells to which soluble VCAM-1 is bound, and in CXCL16 which can promote muscle regeneration appear to align with the positive effects on muscle structure observed under MRI in the ATL1102 Phase II trial. These plasma proteins were increased such that they approached the median levels seen in an external control dataset of health adults, supporting the beneficial nature of the outcomes in ATL1102 treated DMD patients.
- Positive effects on LTBP4 and TSP-1 positions ATL1102 as an exciting prospect for the treatment of both non-ambulant and ambulant patients with DMD and the treatment of other muscle and fibrotic conditions.

The protein changes observed in the plasma of the ATL1102 treated non ambulant DMD patients in the Phase II study is also consistent with the drug's positive effects on muscle function and strength reported in the ATL1102 Phase II trial.

Analysis of the plasma protein data is ongoing in order to further elucidate ATL1102's biological effects and to position the drug's development in disease settings. The Company will continue to report on any material developments from this ongoing data analysis and associated commercial opportunities.

Based on the positive outcomes from the protein analysis reported above, Australian Provisional Patent Application No. 2021903024 was filed 20 September 2021 with claims covering applications of ATL1102 in new potential disease settings including diabetic, respiratory and age-related diseases to support the Company's future commercial and partnering plans for ATL1102.

For further details refer to Company's 24 September 2021 ASX announcement.

## **Positive feedback received on Paediatric Investigation Plan**

During the quarter the Company received a draft opinion recommending the agreement with its Paediatric Investigation Plan (PIP) for the development of ATL1102 for Duchenne muscular dystrophy (DMD) from the Paediatric Committee (PDCO) of the European Medicines Agency (EMA). The draft positive opinion was then discussed for adoption by PDCO during their meeting on 15 October 2021.

A paediatric investigation plan is a development plan aimed at ensuring that the necessary data is obtained through studies in children. Approval of the PIP is required to support the authorisation of a medicine for

children in the European Union (EU). The PIP addresses the entire paediatric development program for ATL1102 in DMD (including future ambulant DMD patient studies). ANP through its interactions with PDCO, is looking to ensure that its planned clinical studies including its Phase IIb clinical trial of ATL1102 in non-ambulant DMD boys, will be run in accordance with PDCO expectations for future product approval.

Further details on the Phase IIb trial design including the expected timing for trial application and approval for the Phase IIb trial of ATL1102 in non-ambulant DMD patients to be conducted in Europe will be communicated to the market following adoption of the final opinion by PDCO.

The Company continues to advance its preparations for its planned Phase IIb clinical trial in Europe and has selected global Clinical Research Organisation Parexel to conduct the study. <https://www.parexel.com/>

The Company advised that the work program has commenced and Parexel is currently conducting site evaluations to select the sites (>30) to take part in the Phase IIb study, which will recruit patients into the European trial once requisite trial application approvals are received for each jurisdiction.

### **Ongoing engagement with DMD community, investors and pharmaceutical companies**

The Company continued its communication and active engagement with key opinion leaders, potential collaborators, investors and commercial partners as a key operational priority. During the quarter the Company presented and participated at the following events:

- Scandinavian Alliance, Investor Webinar - Stockholm, Sweden, 15 July 2021
- Spark Plus Healthcare Day Webinar - Singapore, 27 July 2021.
- Virtual Investor Roadshow - Singapore & Hong Kong, 1 – 3 September 2021
- Scandinavian Alliance, Investor Webinar - Stockholm, Sweden, 30 September 2021

### **Broker Research & Other Reports**

Several leading Australian healthcare research analysts have released positive research notes on the Company during the quarter (reports are available on ANP website: <https://www.antisense.com.au/broker-other-reports/>):

"The chicken and the egg" - Iain Wilkie / Scott Power, Morgans Financial

"Not so Fast Track" - Shane Storey / Melissa Benson, Wilsons Equity Research

"Big year, but even bigger one ahead" - Iain Wilkie / Scott Power, Morgans Financial

"Monkey business" - Shane Storey / Melissa Benson, Wilsons Equity Research

Desk Note – Cyprus Sia, Spark Plus Pte Ltd, Singapore (this report is only available by request to s708 investors)

"Mechanism strengthened; reach expanded" - Shane Storey / Melissa Benson, Wilsons Equity Research

## **Cash Flow**

As at 30 September 2021 the Company reported cash of \$4.73 million.

The Company continues to efficiently manage expenditure planned for continuation of the regulatory interactions with EMA and US FDA, preparations for the conduct of Phase IIb clinical trial of ATL1102 in DMD in Europe as well as advancement of potential new indications for ATL1102. During the quarter the net expenditure incurred on those activities amounted to \$1.29 million.

During the quarter the Company made payments to related parties of the entity and their associates as disclosed in Item 6 of the Appendix 4C amounting to \$219,330. The payments related to salaries, directors' fees and consulting fees on normal commercial terms.

*This announcement has been authorised for release by the Board.*

### **For more information please contact:**

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## Appendix 4C

### Quarterly cash flow report for entities subject to Listing Rule 4.7B

**Name of entity**

Antisense Therapeutics Limited

**ABN**

41 095 060 745

**Quarter ended ("current quarter")**

30 Sept 2021

<b>Consolidated statement of cash flows</b>	<b>Current quarter \$A'000</b>	<b>Year to date (03 months) \$A'000</b>
<b>1. Cash flows from operating activities</b>		
1.1 Receipts from customers	-	-
1.2 Payments for		
(a) research and development **	(518)	(518)
(b) product manufacturing and operating costs	-	-
(c) advertising and marketing	(47)	(47)
(d) leased assets	-	-
(e) staff costs	(338)	(338)
(f) administration and corporate costs	(384)	(384)
1.3 Dividends received (see note 3)	-	-
1.4 Interest received	1	1
1.5 Interest and other costs of finance paid	-	-
1.6 Income taxes paid	-	-
1.7 Government grants and tax incentives	-	-
1.8 Other (provide details if material)	-	-
<b>1.9 Net cash from / (used in) operating activities</b>	<b>(1,286)</b>	<b>(1,286)</b>

\*\* Includes ATL1102 drug compound manufacturing costs

<b>2. Cash flows from investing activities</b>		
2.1 Payments to acquire or for:		
(a) entities	-	-
(b) businesses	-	-
(c) property, plant and equipment	-	-
(d) investments	-	-
(e) intellectual property	-	-
(f) other non-current assets	-	-

Consolidated statement of cash flows		Current quarter \$A'000	Year to date (03 months) \$A'000
2.2	Proceeds from disposal of:		
	(a) entities	-	-
	(b) businesses	-	-
	(c) property, plant and equipment	-	-
	(d) investments	-	-
	(e) intellectual property	-	-
	(f) other non-current assets	-	-
2.3	Cash flows from loans to other entities	-	-
2.4	Dividends received (see note 3)	-	-
2.5	Other (provide details if material)	-	-
2.6	<b>Net cash from / (used in) investing activities</b>	-	-

<b>3.</b>	<b>Cash flows from financing activities</b>		
3.1	Proceeds from issues of equity securities (excluding convertible debt securities)	-	-
3.2	Proceeds from issue of convertible debt securities	-	-
3.3	Proceeds from exercise of options	-	-
3.4	Transaction costs related to issues of equity securities or convertible debt securities	-	-
3.5	Proceeds from borrowings	-	-
3.6	Repayment of borrowings	-	-
3.7	Transaction costs related to loans and borrowings	-	-
3.8	Dividends paid	-	-
3.9	Other (provide details if material)	-	-
3.10	<b>Net cash from / (used in) financing activities</b>	-	-

<b>4.</b>	<b>Net increase / (decrease) in cash and cash equivalents for the period</b>		
4.1	Cash and cash equivalents at beginning of period	6,020	6,020
4.2	Net cash from / (used in) operating activities (item 1.9 above)	(1,286)	(1,286)
4.3	Net cash from / (used in) investing activities (item 2.6 above)	-	-

Consolidated statement of cash flows		Current quarter \$A'000	Year to date (03 months) \$A'000
4.4	Net cash from / (used in) financing activities (item 3.10 above)	-	-
4.5	Effect of movement in exchange rates on cash held	-	-
4.6	<b>Cash and cash equivalents at end of period</b>	<b>4,734</b>	<b>4,734</b>

5.	Reconciliation of cash and cash equivalents at the end of the quarter (as shown in the consolidated statement of cash flows) to the related items in the accounts	Current quarter \$A'000	Previous quarter \$A'000
5.1	Bank balances	4,734	120
5.2	Call deposits	-	5,900
5.3	Bank overdrafts		-
5.4	Other (provide details)		-
5.5	<b>Cash and cash equivalents at end of quarter (should equal item 4.6 above)</b>	<b>4,734</b>	<b>6,020</b>

6.	Payments to related parties of the entity and their associates	Current quarter \$A'000
6.1	Aggregate amount of payments to related parties and their associates included in item 1	219
6.2	Aggregate amount of payments to related parties and their associates included in item 2	-
<i>Note: if any amounts are shown in items 6.1 or 6.2, your quarterly activity report must include a description of, and an explanation for, such payments.</i>		

<b>7. Financing facilities</b> <i>Note: the term "facility" includes all forms of financing arrangements available to the entity.</i> <i>Add notes as necessary for an understanding of the sources of finance available to the entity.</i>	<b>Total facility amount at quarter end \$A'000</b>	<b>Amount drawn at quarter end \$A'000</b>
7.1 Loan facilities	-	-
7.2 Credit standby arrangements	-	-
7.3 Other (please specify)	-	-
7.4 <b>Total financing facilities</b>	-	-
7.5 <b>Unused financing facilities available at quarter end</b>		-
7.6 Include in the box below a description of each facility above, including the lender, interest rate, maturity date and whether it is secured or unsecured. If any additional financing facilities have been entered into or are proposed to be entered into after quarter end, include a note providing details of those facilities as well.		

<b>8. Estimated cash available for future operating activities</b>	<b>\$A'000</b>
8.1 Net cash from / (used in) operating activities (item 1.9)	(1,286)
8.2 Cash and cash equivalents at quarter end (item 4.6)	4,734
8.3 Unused finance facilities available at quarter end (item 7.5)	-
8.4 Total available funding (item 8.2 + item 8.3)	3,448
8.5 <b>Estimated quarters of funding available (item 8.4 divided by item 8.1)</b>	3
<i>Note: if the entity has reported positive net operating cash flows in item 1.9, answer item 8.5 as "N/A". Otherwise, a figure for the estimated quarters of funding available must be included in item 8.5.</i>	
8.6 If item 8.5 is less than 2 quarters, please provide answers to the following questions:	
8.6.1 Does the entity expect that it will continue to have the current level of net operating cash flows for the time being and, if not, why not?	
Answer:	
8.6.2 Has the entity taken any steps, or does it propose to take any steps, to raise further cash to fund its operations and, if so, what are those steps and how likely does it believe that they will be successful?	
Answer:	
8.6.3 Does the entity expect to be able to continue its operations and to meet its business objectives and, if so, on what basis?	
Answer:	
<i>Note: where item 8.5 is less than 2 quarters, all of questions 8.6.1, 8.6.2 and 8.6.3 above must be answered.</i>	



## Compliance statement

- 1 This statement has been prepared in accordance with accounting standards and policies which comply with Listing Rule 19.11A.
- 2 This statement gives a true and fair view of the matters disclosed.

Date: 29 October 2021 .....

Authorised by: By the Board .....  
(Name of body or officer authorising release – see note 4)

## Notes

1. This quarterly cash flow report and the accompanying activity report provide a basis for informing the market about the entity's activities for the past quarter, how they have been financed and the effect this has had on its cash position. An entity that wishes to disclose additional information over and above the minimum required under the Listing Rules is encouraged to do so.
2. If this quarterly cash flow report has been prepared in accordance with Australian Accounting Standards, the definitions in, and provisions of, *AASB 107: Statement of Cash Flows* apply to this report. If this quarterly cash flow report has been prepared in accordance with other accounting standards agreed by ASX pursuant to Listing Rule 19.11A, the corresponding equivalent standard applies to this report.
3. Dividends received may be classified either as cash flows from operating activities or cash flows from investing activities, depending on the accounting policy of the entity.
4. If this report has been authorised for release to the market by your board of directors, you can insert here: "By the board". If it has been authorised for release to the market by a committee of your board of directors, you can insert here: "By the [name of board committee – eg Audit and Risk Committee]". If it has been authorised for release to the market by a disclosure committee, you can insert here: "By the Disclosure Committee".
5. If this report has been authorised for release to the market by your board of directors and you wish to hold yourself out as complying with recommendation 4.2 of the ASX Corporate Governance Council's *Corporate Governance Principles and Recommendations*, the board should have received a declaration from its CEO and CFO that, in their opinion, the financial records of the entity have been properly maintained, that this report complies with the appropriate accounting standards and gives a true and fair view of the cash flows of the entity, and that their opinion has been formed on the basis of a sound system of risk management and internal control which is operating effectively.