ASX Announcement

21 October 2020

Quarterly Update & Appendix 4C

- Received EMA feedback reflecting the prior scientific advice from the three EU national authorities on appropriateness of key trial design parameters
- US FDA & EMA Orphan Drug designation applications submitted for ATL1102 in DMD
- Received US FDA Rare Pediatric Disease Designation to ATL1102 for the treatment of DMD
- Presentation at Annual Congress of the World Muscle Society on post study analysis showing statistically significant improvement in PUL2.0 with ATL1102 treatment in non-ambulant boys with DMD compared to a matched natural history control

Antisense Therapeutics Limited (Antisense or Company) is pleased to provide its Appendix 4C and quarterly update for the period ended 30 September 2020.

EU & US regulatory agency interactions

During the quarter the Company continued to advance its interactions with regulatory agencies in EU and US.

In July, European Medicines Agency (EMA) provided the Company its feedback on the appropriateness of the key trial design parameters of dose duration, safety monitoring plan, endpoints, and potential pivotal status for the planned Phase IIb study of ATL1102 in non-ambulant boys with Duchenne muscular dystrophy (DMD) in Europe, which reflects the prior scientific advice received from the three European Union national authorities.

In August the Company submitted Orphan Drug Designation application for ATL1102 in DMD to EMA and US FDA.

In Europe, orphan drug designation status brings development and marketing incentives, such as reduced fees, scientific advice and market exclusivity for 10 years upon regulatory approval.

In US, the FDA provides incentives to help accelerate the development of products for rare diseases, which may include tax credits towards the cost of clinical trials, waiver of US prescription drug filing fees and orphan product exclusivity for seven years upon marketing authorisation.

As part of advancing US regulatory strategy, a request for a rare pediatric disease designation was submitted in conjunction with the ODD application. The FDA has granted the designation of ATL1102 as a drug for a rare pediatric disease following submission of data from Phase II clinical trial of ATL1102. Further, under the FDA's Rare Pediatric Disease Priority Review Voucher Program, a company that receives an approval for a product designated for a rare pediatric disease may qualify for a voucher that can be redeemed to receive an expedited priority marketing authorization review or sold to another party. In recent years, a secondary market for the vouchers has emerged allowing companies to use the sale of PRVs to recoup expenses undertaken for drug research and development and present them with additional source of non-dilutive capital to support further advancement of clinical development. Since 2009 when the first PRV was awarded the values for these vouchers have ranged between US$68 million and US$350 million.

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Post study analysis shows statistically significant improvement in PUL2.0 with ATL1102 treatment

ATL1102 was recently assessed in an open label Phase II study in adolescent non-ambulant patients with DMD.ATL1102 met the primary endpoint of the study with confirmation of the drug's safety and tolerability. ATL1102 also demonstrated strong effects on secondary endpoints including activity on the targeted CD49d immune cells consistent with the drug's proposed mechanism of action and outcomes on disease progression parameters that exceeded the Company's expectations with improvement or stabilization across different measures of muscle function and strength. The positive effects on disease progression were further supported by MRI data that suggested a stabilization in the percentage of fat fraction in the muscles and preservation of functional muscle mass.

In a poster presentation at the Annual Congress of the World Muscle Society results from a post study analysis were reported where the mean PUL2.0 data from the ATL1102 treated patients was compared with the 24-week PUL2.0 data of 39 assessments in 20 non-ambulant patients [mean age 15.61 (SD 2.02) years; 19 on corticosteroid] from a natural history database of DMD patients in Rome, Italy - the Rome cohort (RC). The RC were identified using the same inclusion criteria used to enrol patients in the ATL1102 Phase II study.

ATL1102 treated patients showed a statistically significant mean improvement in Total PUL2.0 scores (assessment of muscle function) at 24 weeks compared to a matched natural history control with a greater frequency of patients treated with ATL1102 showed improvement or maintenance of their Total PUL2.0 score relative to the natural history control group

PUL2.0 (Performance of Upper Limb Function) mean data from the ATL1102 Phase II trial was compared to a natural history control of matched non-ambulant boys on standard of care (corticosteroids) and showed a statistically significant improvement in Total PUL2.0 with a greater frequency of patients achieving an improvement or maintenance of their Total PUL2.0 scores relative to the matched natural history control group over 24 weeks.

The significantly improved results of ATL1102 treatment on PUL2.0 when compared to a matched RC group together with its positive effects on multiple additional disease progression parameters, positions ATL1102 as an exciting prospect for the treatment of DMD patients.

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:

- Virtual Investor Roadshow - Singapore & Hong Kong, 6 – 9 July 2020.
- 2020 Virtual Annual Conference - Parent Project Muscular Dystrophy, US, 22 July 2020
- StockPal Biotech & Healthcare Webinar, Singapore, 4 August 2020.
- 25th International Annual Congress of the World Muscle Society, UK, 1 October 2020

Cash Flow

As at 30 September 2020 the Company reported cash of $3.13m.

The Company continues to efficiently manage expenditure planned for continuation of the regulatory interactions with EMA and US FDA, submissions for Orphan Drug designation as well as advancement of potential new indications for ATL1102.

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 $173k. The payments related to salaries, directors' fees, and consulting fees on normal commercial terms.

The Company received cashflow boost credits totaling $37k from the Federal Government (in line with their response associated with Covid-19), as disclosed in item 1.7.

Mark Diamond, Chief Executive Officer of Antisense Therapeutics said: "The milestones we have achieved during the quarter including the FDA granting of the rare pediatric disease designation to ATL1102 reflect the significant development progress being made with ATL1102 and its substantial value creation potential. The favorable comparison of the results of ATL1102-treated patients with data from a matched group of DMD boys treated with corticosteroids presents as an important de-risking event for the ongoing development of ATL1102 for DMD, elevating the quality and significance of our data and increasing confidence in delivering on our goal of providing a new and enhanced therapy for boys with DMD."

This announcement has been authorised for release by the Board.

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Appendix 4C

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

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Name of entity	
Antisense Therapeutics Limited	
ABN	**Quarter ended ("current quarter")**
41 095 060 745	30 September 2020

Consolidated statement of cash flows		**Current quarter $A'000**	**Year to date (3 months) $A'000**
1.	**Cash flows from operating activities**		
1.1	Receipts from customers	-	-
1.2	Payments for		
	(a) research and development	(326)	(326)
	(b) product manufacturing and operating costs	-	-
	(c) advertising and marketing	(42)	(42)
	(d) leased assets	-	-
	(e) staff costs	(293)	(293)
	(f) administration and corporate costs	(307)	(307)
1.3	Dividends received (see note 3)	-	-
1.4	Interest received	2	2
1.5	Interest and other costs of finance paid	-	-
1.6	Income taxes paid	-	-
1.7	Government grants and tax incentives	37	37
1.8	Other	-	-
1.9	**Net cash from / (used in) operating activities**	**(929)**	**(929)**

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

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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	**Net cash from / (used in) investing activities**	**-**	**-**

3.	**Cash flows from financing activities**		
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	**Net cash from / (used in) financing activities**	**-**	**-**

4.	**Net increase / (decrease) in cash and cash equivalents for the period**		
4.1	Cash and cash equivalents at beginning of period	4,059	4,059
4.2	Net cash from / (used in) operating activities (item 1.9 above)	(929)	(929)
4.3	Net cash from / (used in) investing activities (item 2.6 above)	-	-
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	**Cash and cash equivalents at end of period**	**3,130**	**3,130**

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	130	359
5.2	Call deposits	3,000	3,700
5.3	Bank overdrafts	-	-
5.4	Other (provide details)	-	-
5.5	**Cash and cash equivalents at end of quarter (should equal item 4.6 above)**	**3,130**	**4,059**

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	173
6.2	Aggregate amount of payments to related parties and their associates included in item 2	-

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

7.	**Financing facilities** *Note: the term "facility' includes all forms of financing arrangements available to the entity.* *Add notes as necessary for an understanding of the sources of finance available to the entity.*	**Total facility amount at quarter end $A'000**	**Amount drawn at quarter end $A'000**
7.1	Loan facilities	-	-
7.2	Credit standby arrangements	-	-
7.3	Other (please specify)	-	-
7.4	**Total financing facilities**	-	-

7.5	**Unused financing facilities available at quarter end**	-

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.

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8.	Estimated cash available for future operating activities	$A'000
8.1	Net cash from / (used in) operating activities (Item 1.9)	(929)
8.2	Cash and cash equivalents at quarter end (Item 4.6)	3,130
8.3	Unused finance facilities available at quarter end (Item 7.5)	-
8.4	Total available funding (Item 8.2 + Item 8.3)	3,130
8.5	**Estimated quarters of funding available (Item 8.4 divided by Item 8.1)**	3

8.6 If Item 8.5 is less than 2 quarters, please provide answers to the following questions:

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?

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?

3. Does the entity expect to be able to continue its operations and to meet its business objectives and, if so, on what basis?

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: 21 October 2020

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

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[*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.

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