ASX Announcement

29 July 2021

# Quarterly Activities Report & Appendix 4C

- **Type C guidance meeting held with US FDA for ATL1102 in DMD**
- **ATL1102 EMA PIP feedback**
- **US FDA Fast Track Designation request**
- **Manufacture of clinical supplies**
- **ANP and MCRI new R&D collaboration for ATL1102**
- **Board Chair transition**

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

## Type C guidance meeting held with the US Food and Drug Administration (FDA) to discuss development for ATL1102 in DMD

The FDA feedback from the Type C meeting held during the quarter confirmed that the findings at 25mg/week in ANP's Phase II, open-label study conducted at Royal Children's Hospital in Melbourne, Australia are adequate to support larger studies.

Importantly, the FDA noted that the proposed design of the Phase IIb/III study (as a single, randomized double blind, placebo-controlled study) and the primary endpoint (PUL2.0) appear acceptable. Secondary endpoints of muscle strength as assessed by MyoGrip, MyoPinch, and predicted forced vital capacity (FVC), also appear reasonable as was the 52-week study duration, non-ambulant patient population and number of subjects relative to statistical power assumptions. Provided appropriate safety-monitoring recommendations are adopted by ANP, the FDA said it could consider the exploration of higher doses of ATL1102 beyond 25mg/week subject to adequate justification.

With regard to the non-clinical requirements, the FDA expects the Company to conduct a nine-month monkey toxicology study to support the Phase IIb/III study. The agency stated, however, that because of the seriousness of the indication, ANP may initiate the 12-month Phase IIb/III human clinical study prior to submission of a nine-month toxicology study.

ANP is consulting with its US based regulatory advisors on the appropriate next steps to advance the Phase IIb/III study design and development plans for the US.

## European Medicines Agency Paediatric Investigation Plan (PIP) feedback received for ATL1102 in DMD

As part of its review of the PIP, the Paediatric Committee (PDCO) of the European Medicines Agency (EMA) provided feedback outlining additional information requirements on the Company's planned Phase IIb clinical trial in non-ambulant DMD boys.

ANP is addressing the PDCO information requirements and anticipates finalising the trial design with PDCO later in Q3'CY21, ahead of submitting the clinical trial application shortly thereafter for the Phase IIb trial of ATL1102 in non-ambulant DMD patients to be conducted in Europe.

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 potential ambulant DMD patient studies). ANP through its interactions with PDCO, is looking to ensure that its planned clinical studies will be run in accordance with PDCO expectations for future product approval.

**US FDA Fast Track Designation request**

Given 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, the Company has submitted a FastTrack Designation Request (https://tinyurl.com/fwhemd95) with the FDA. The Company continues to work with its expert US based regulatory advisors on appropriate next steps to advance the ATL1102 DMD program in the US.

**Manufacture of ATL1102 clinical supplies for Phase IIb trial in DMD**

The manufacture of a batch of ATL1102 active pharmaceutical ingredient (API) for the Phase IIb trial was undertaken in North America by Nitto Denko Avecia (Avecia). Upon completion of the manufacture of this batch of API, the material was then shipped to Contract, Parenteral (injectable) Drug Product Manufacturer Pyramid Laboratories (Pyramid) in Costa Messa, Southern California and formulated into injectable product for use in the Phase IIb trial. Importantly, both Avecia and Pyramid have commercial capabilities to support advanced clinical trials and subsequent commercial supply.

**Antisense Therapeutics and Murdoch Children's Research Institute enter into new R&D collaboration to further explore ATL1102 use in multiple muscle diseases**

During the quarter the Company has entered into a new Research and Development collaboration with the Murdoch Children's Research Institute's (MCRI) scientific researchers, Dr Peter Houweling and Associate Professor Shireen Lamande, to further investigate the potential of ATL1102 to deliver breakthrough treatment for the control of immune mediated inflammatory muscle damage in muscle diseases where there is an acknowledged need for more effective and safer treatments.

ATL1102 has been shown to be clinically active in Multiple Sclerosis and Duchenne muscular dystrophy (DMD) patients while antisense inhibition of CD49d has also previously demonstrated activity in multiple disease animal models. The MCRI researchers and ANP have additionally undertaken experimental work that showed antisense inhibition of CD49d in the X chromosome-linked muscular dystrophy (mdx) mouse model of DMD reduces both the CD49d target in the muscle and muscle damage. This data is expected to be submitted for publication in 2021.

Having achieved positive results in the mdx animal model now allows for the further study of antisense inhibition of CD49d effects in the mdx model in combination with other DMD treatments including the dystrophin restoration drugs to assess the potential of the combination to improve therapeutic outcomes. This work is to be conducted in the 2nd half of 2021 and is funded through ANP's existing cash reserves.

In addition, antisense inhibition of CD49d will be assessed in another animal model of muscle disease where there are similar immune mediated inflammatory features to the mdx model, where it has demonstrated positive effects.

ANP is also planning for ATL1102 to be assessed in ANP's ex-vivo cell expression and modeling systems by studying patient blood samples taken from children afflicted by a range of muscle diseases to explore ATL1102's potential activity in these conditions, where there is a clear need for effective therapies. Subject to participant recruitment this work is to be initiated in the 2H'CY21.

As previously advised, the broader immunomodulatory effects of ATL1102 are being investigated by ANP through the analysis of blood (plasma) samples retained from the Company's Phase II trial of ATL1102 in DMD patients. ANP is presently completing this plasma analysis and is expecting this new data to provide insights on the mode of action and broader biological activity of ATL1102. ANP is planning to file for additional patent protection with this new data ahead of its proposed presentation at an appropriate scientific conference in 2H'CY21.

## Board Chair transition

On 28 July 2021 and subsequent to the reporting period, the Company announced the transition of Non-Executive Director Charmaine Gittleson M.D to become the Chair of the ANP Board and as part of the transition, Bob Moses, ANP Chairman since 2001, announced his intention to retire from the Board at the conclusion of the Company's 2021 Annual General Meeting after which he will continue to support the Company in a consultant capacity.

The announcement noted that transitioning of the Chair role is in recognition of the ANP's maturation from a drug discovery to late-stage clinical development group with near term commercialisation aspirations and that Dr Gittleson's extensive international experience in global pharmaceutical drug development and registration across multiple therapeutic and rare disease areas, is precisely the leadership and experience required to steward the Company through this next phase of the Company's growth.

## 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:

- ShareCafé Small Cap "Hidden Gems" Webinar, Australia, 14 May 2021.
- Wilsons Rapid Insights 2021 Conference, Sydney, Australia, 26 May 2021
- Virtual Investor Roadshow - Singapore & Hong Kong, 7 – 9 June 2021
- Virtual Investor Roadshow - Sydney & Melbourne, 23 – 25 June 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/):

"FDA Feedback as Good As It Gets" - Marc Sinatra, Corporate Connect

"Positive FDA feedback on pivotal trial design" - Dennis Hulme, Taylor Collison

"Harmony on the table" - Shane Storey / Melissa Benson, Wilsons Equity Research

## Cash Flow

As at 30 June 2021 the Company reported cash of $6.02 million.

The Company continues to efficiently manage expenditure planned for continuation of the regulatory interactions with EMA and US FDA, manufacturing of ATL1102 drug compound as well as advancement of potential new indications for ATL1102. During the quarter the expenditure incurred on those activities amounted to $1.53 million with the major portion of the costs associated with the ATL1102 manufacturing campaign referred to above now having been expensed.

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 $153,000. The payments related to salaries, directors' fees and consulting fees on normal commercial terms.

*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



| **Name of entity** | |
|---|---|
| Antisense Therapeutics Limited | |
| **ABN** | **Quarter ended ("current quarter")** |
| 41 095 060 745 | 30 June 2021 |

| **Consolidated statement of cash flows** | | **Current quarter $A'000** | **Year to date (12 months) $A'000** |
|---|---|---|---|
| **1.** | **Cash flows from operating activities** | | |
| 1.1 | Receipts from customers | - | - |
| 1.2 | Payments for | | |
| | (a) research and development ** | (1,531) | (3,909) |
| | (b) product manufacturing and operating costs | - | - |
| | (c) advertising and marketing | (56) | (242) |
| | (d) leased assets | - | - |
| | (e) staff costs | (321) | (1,194) |
| | (f) administration and corporate costs | (381) | (1,275) |
| 1.3 | Dividends received (see note 3) | - | - |
| 1.4 | Interest received | 2 | 5 |
| 1.5 | Interest and other costs of finance paid | - | - |
| 1.6 | Income taxes paid | - | - |
| 1.7 | Government grants and tax incentives | - | 688 |
| 1.8 | Other (provide details if material) | - | - |
| **1.9** | **Net cash from / (used in) operating activities** | **(2,287)** | **(5,928)** |

** Includes ATL1102 drug compound manufacturing costs

| **2.** | **Cash flows from investing activities** | | |
|---|---|---|---|
| 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 (12 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** | **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) | - | 8,500 |
| 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 | - | (611) |
| 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** | - | **7,889** |

| **4.** | **Net increase / (decrease) in cash and cash equivalents for the period** | | |
|---|---|---|---|
| 4.1 | Cash and cash equivalents at beginning of period | 8,307 | 4,059 |
| 4.2 | Net cash from / (used in) operating activities (item 1.9 above) | (2,287) | (5,928) |
| 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 (12 months) $A'000 |
|---|---|---|---|
| 4.4 | Net cash from / (used in) financing activities (item 3.10 above) | - | 7,889 |
| 4.5 | Effect of movement in exchange rates on cash held | - | - |
| **4.6** | **Cash and cash equivalents at end of period** | **6,020** | **6,020** |

| 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 | 120 | 307 |
| 5.2 | Call deposits | 5,900 | 8,100 |
| 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)** | **6,020** | **8,307** |

| 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 | 153 |
| 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**<br>*Note: the term "facility' includes all forms of financing arrangements available to the entity.*<br>*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.

| | | |
|---|---|---|
| **8.** | **Estimated cash available for future operating activities** | **$A'000** |
| 8.1 | Net cash from / (used in) operating activities (item 1.9) | 2,287 |
| 8.2 | Cash and cash equivalents at quarter end (item 4.6) | 6,020 |
| 8.3 | Unused finance facilities available at quarter end (item 7.5) | - |
| 8.4 | Total available funding (item 8.2 + item 8.3) | 6,020 |
| 8.5 | **Estimated quarters of funding available (item 8.4 divided by item 8.1)** | 3 |

*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.*

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:

*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.*

## 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 July 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.