



# antisense

## THERAPEUTICS

(ASX: ANP)

**Investor Presentation - October 2021**

# Antisense Therapeutics Limited Investor Presentation - Disclaimer

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- the Joint Lead Managers and the Corporate Adviser will receive fees for acting in their capacity as joint lead manager or corporate adviser to the Offer (as applicable).

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## Withdrawal and cooling off

ANP reserves the right to withdraw, or vary the timetable for any part of the Offer without notice. Cooling off rights do not apply to the acquisition of New Shares and Options.

# Investment Highlights

## Clinical unmet need

1 in 3,500 boys suffer from DMD with limited treatment options

## Lead Program ATL1102 in DMD

In licensed from Ionis Pharmaceuticals

## Orphan Drug Designation

Granted in EU and US providing extended market exclusivity

## \$169M market cap

At 27 October 2021

## DMD market \$10bn by 2030

From Kamet Research

## Positive Phase II trial results

Across multiple measures of muscle strength and function

## Phase IIb/III trial in EU

Positive opinion adopted by PDCO (EMA)

## Capital Raising

To fund Phase IIb/III pivotal trial



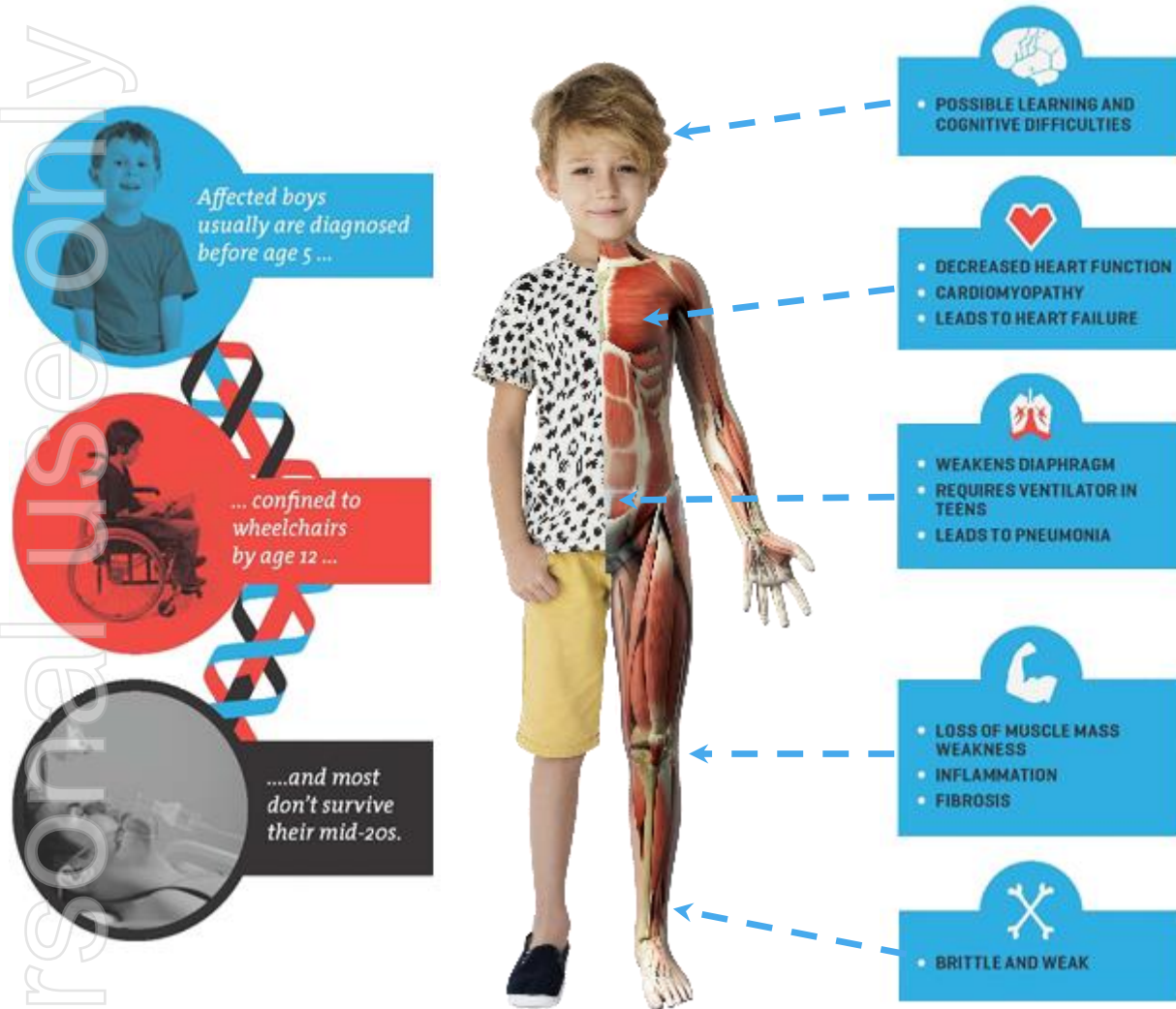
Lead programme - ATL1102
Final PDCO opinion received
Capital Raising to fund phase IIb/III clinical trial to futility analysis and commencement of marketing application preparations

- | Use of Funds Raised                            | Placement Only      | Placement & Entitlement Offer* |
|--|---------------------|--------------------------------|
| Phase IIb/III - Clinical Study costs           | \$7,000,000         | \$21,000,000                   |
| Open Label Extension Study initiation costs    | -                   | \$600,000                      |
| Drug Manufacturing costs                       | \$5,500,000         | \$7,400,000                    |
| Ongoing progression of other clinical programs | \$1,100,000         | \$1,100,000                    |
| Working Capital                                | \$5,000,000         | \$5,000,000                    |
| Capital Raising Costs                          | \$1,400,000         | \$1,700,000                    |
| <b>TOTAL</b>                                   | <b>\$20,000,000</b> | <b>\$36,800,000</b>            |

*Please refer to Slide 19 for further details.*

# What is DMD?

*DMD is a rapidly progressing genetic disease resulting in low QOL and 100% mortality into patients' 30's*



- Duchenne Muscular Dystrophy (DMD) is a devastating genetic muscular disease caused by loss of dystrophin with progressive muscle wasting & associated muscle injury leading to inflammation & fibrosis (100% mortality)
- Affects boys with an incidence of ~1 in 3,500 newborns<sup>1</sup> & prevalence of up to ~18,000 in US<sup>1</sup> and up to ~26,000 in EU<sup>2</sup>
- Key challenge in management of DMD patients is to reduce the inflammation and muscle fibre damage<sup>3</sup>
- Corticosteroids (CS) are the only therapy used to treat the inflammation in DMD<sup>4</sup> but have insufficient efficacy<sup>5</sup> & significant side effects including weight gain, reduced bone density & growth retardation<sup>3</sup>. CS not as effective in boys with a > number of T cells with high CD49d receptors<sup>6</sup>.
- ATL1102 is designed to inhibit CD49d expression on lymphocytes and is being developed as a treatment to reduce inflammation in DMD.

Source of Image Cure Duchenne

<sup>1</sup> McNeil et al, Muscle Nerve, 2010, 41(6): p. 740-5

<sup>2</sup> [http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/orphans/2015/05/human\\_orphan\\_001571.jsp&mid=WC0b01ac058001d12b](http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/orphans/2015/05/human_orphan_001571.jsp&mid=WC0b01ac058001d12b)

<sup>3</sup> Angelini and Peterle Acta Myologica 2012, XXXI: p. 9-15

<sup>4</sup> Rosenberg et al, Science Translational Medicine 2015, 7, p.299

<sup>5</sup> Miyatake et al Drug Design, Development & Therapy 2016, 10: p 2745–58

<sup>6</sup> Pinto-Mariz et al Skeletal Muscle 2015, 5: p. 45-55

# DMD Prevalence and Segmentation

DMD is a rapidly progressing genetic disease resulting in low QOL and 100% mortality into patients 30's

Affects 1 in 3,500<sup>1</sup> - 5,000<sup>5</sup> Male Births Worldwide

Up to Approximately

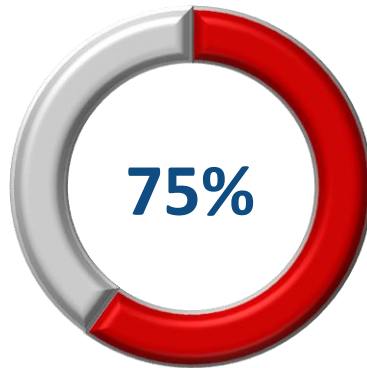
**18,000**<sup>1,3</sup>

patients in the US

Up to Approximately

**26,000**<sup>2,3</sup>

patients in the EU



Approx. 75% of diagnosed DMD patients are **>10 years old**<sup>4</sup>



Approx. 50% of all DMD patients are **non-ambulant**<sup>3</sup>

<sup>1</sup> McNeil et al, Muscle Nerve, 2010, 41(6): p. 740-5

<sup>2</sup> [http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/orphans/2015/05/human\\_orphan\\_001571.jsp&mid=WC0b01ac058001d12b](http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/orphans/2015/05/human_orphan_001571.jsp&mid=WC0b01ac058001d12b)

<sup>3</sup> DelveInsight - Duchenne Muscular Dystrophy (DMD) Market Insight, Epidemiology, and Market Forecast—2030 (August 2021)

<sup>4</sup> Romitti PA et al. Pediatrics. 2015

<sup>5</sup> Crisafulli et al. 2020, Orphanet Journal of Rare Diseases





# **ATL1102 Phase IIb/III clinical development plan**

## **Details of the Capital Raising**

### **Appendix A: Antisense additional information**

### **Appendix B: Key risks**

### **Appendix C: Offering jurisdictions**

# Lead program: ATL1102 in DMD - Profile Summary

## *ATL1102 well positioned for global success within high-growth multi-billion-dollar DMD market*

- **Novel and Differentiated MOA:** ATL1102 is the only therapy in development for DMD targeting CD49d to slow disease progression by reducing inflammation and resultant muscle damage with potential application across a broad range of inflammatory diseases
- **Efficacy Data:** Phase II shows activity across multiple measures of muscle strength and function with superiority to historical controls
- **Competitive Profile:** ATL1102's differentiated mechanism of action has the potential to be synergistic with other marketed compounds and those in development for DMD allowing for higher levels of market penetration. Additionally, ATL1102 has the potential to be used across all DMD subtypes.
- **Underserved Target Population:** Non-ambulant DMD patients who currently rely on corticosteroid therapy with associated significant side effects to treat muscle inflammation. New anti-inflammatory steroids, dystrophin restoration and micro dystrophin gene therapies are being trialled in predominantly ambulant boys.
- **IP Portfolio:** ATL1102 has been in-licensed from Ionis Pharmaceuticals Inc. (NASDAQ:IONS). Antisense Therapeutics has a substantial patent estate surrounding ATL1102 supplemented by Orphan Drug Designations in EU and US providing additional market exclusivity.
- **Potential Expedited Regulatory Pathway**      Potential for approval in EU based on positive results of a planned Phase IIb/III trial. Potential accelerated approval pathways in the US



# Clinical Development Plan

*European regulatory authorities have advised ATL1102 Phase IIb/III trial to be a potential pivotal study*

## EU Phase IIb/III Clinical Trial

- ANP will conduct a multi-centre, randomised, double-blind placebo-controlled Phase IIb/III study of ATL1102 in non-ambulant patients dosed with ATL1102 for 12 months at two dose levels as a potentially pivotal (approvable) trial with a follow-on open label extension phase.
- ANP has now received a positive final opinion for its ATL1102 Phase IIb/III Paediatric Investigational Plan (**PIP**) from EMA Paediatric Committee (**PDCO**).
- ANP has appointed globally renowned Clinical Research Organisation (CRO) Parexel to conduct and manage the Phase IIb/III study. Parexel was named “*Best Contract Research Organisation*” in Dec 2020 by Informa Pharma Intelligence.
- Parexel is currently conducting site evaluations to select the sites (>30) to take part in the Phase IIb/III study, which will recruit patients into the European trial once requisite trial application approvals are received for each jurisdiction.
- Professor Thomas Voit MD (Director of NIHR GOSH UCL Biomedical Research Centre, UK) will be the Coordinating Principal Investigator of the trial.



## US Regulatory Plans

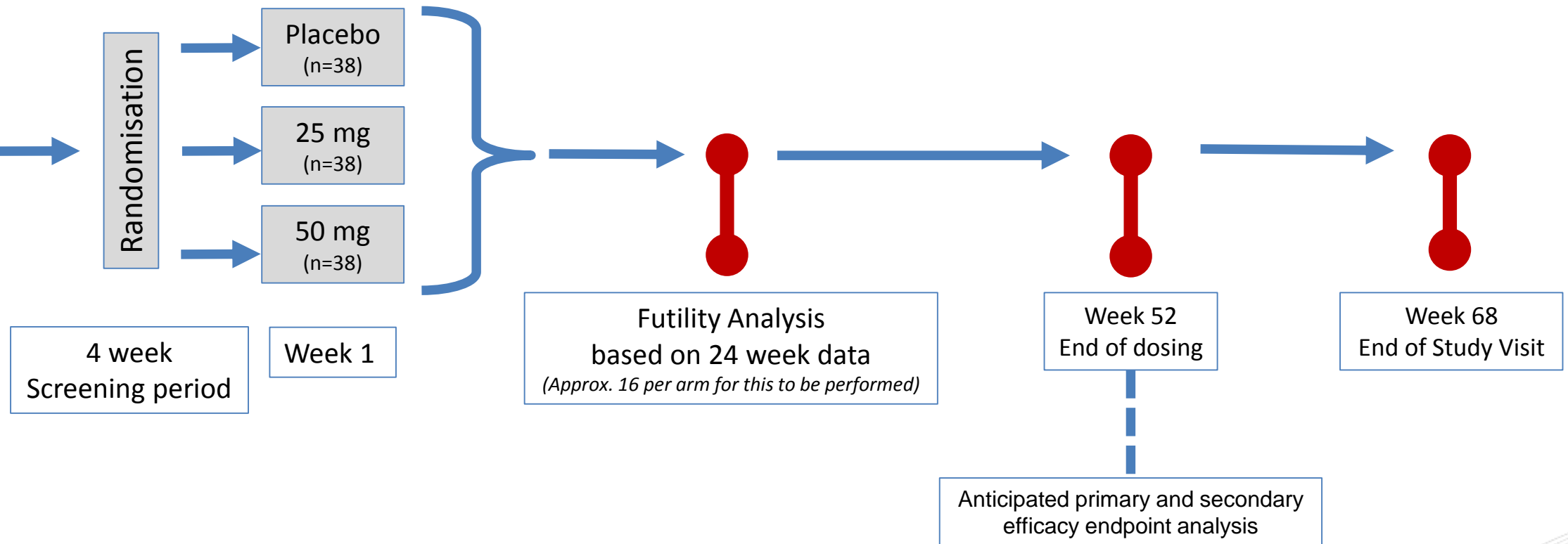
- Type C guidance meeting held with US FDA to discuss the further development of ATL1102 in DMD in the US. Meeting provided clarity on the requirements for a Phase IIb/III for approval in the US.
- Company is required to conduct a 9-month monkey toxicology study to support dosing beyond 6-months in the Phase IIb/III study. ANP expects to submit a protocol for the conduct of the study with the FDA for their approval.



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# EU Phase IIb/III Study Program

A multicentre, randomised, double-blind, placebo-controlled study to assess the efficacy, safety, and pharmacokinetic profile of two dose levels of ATL1102 to be conducted in 108 (114 randomised) DMD non-ambulant participants in ~9 countries across >30 trial sites.



# EU Phase IIb/III Study Program *(Continued....)*

## **Design:**

- A multicentre, randomised, double-blind, placebo-controlled study to determine the efficacy, safety, and pharmacokinetic profile of ATL1102 (25 mg and 50 mg) administered once weekly by subcutaneous (sc) injection for 52 weeks in non-ambulatory participants with DMD, compared to a placebo control
- Participants will be randomised to either weekly, sub-cutaneous dosing with 25 mg ATL1102, 50 mg ATL1102 or placebo in a 1:1:1 ratio with stratification by corticosteroid use
- The study is planned to be conducted in approx. 9 countries, ≥30 clinical trial sites in Europe

## **Sample size:**

- Up to 114 participants to be enrolled (38 per treatment arm) with 108 participants to complete the study

## **Target population:**

- Participants with DMD (confirmed by genetic testing) and are non-ambulatory, defined as unable to walk 10 meters without assistance or help
- 10 to 18 years of age, body weight of at least 25 kg
- PUL 2.0 Entry Item A score ≥2
- If on corticosteroid therapy, therapy was initiated at least six months prior to the baseline visit and a stable daily dose (≥0.25 mg/kg/day prednisone, prednisolone, or equivalent dose for deflazacort) for at least 3 months prior to baseline.



# EU Phase IIb/III Study Program *(Continued....)*

## **Primary objective:**

To evaluate the effect of ATL1102 on upper limb muscle function in non-ambulant participants with DMD as assessed by change in the Performance of Upper Limb Module for DMD 2.0 (PUL 2.0) score compared to placebo

## **Secondary objectives:**

To evaluate the effects of ATL1102:

- on upper limb muscle strength as assessed by change in percent predicted MyoGrip and MyoPinch compared to placebo
- in a responder analysis on the PUL 2.0 score compared to placebo
- on respiratory function as assessed by change in % predicted PEF and % predicted FVC compared to placebo
- on Quality of Life (PEDsQL assessments) in participants and their Study Partner (Parent/Guardian) compared to placebo
- safety and tolerability of ATL1102 including events associated with the Safety Monitoring Plan and Stopping Rules
- PK profile of ATL1102

## **Intended Futility Analysis:**

A futility analysis (FA) will be conducted (on approximately 16 patients per arm) on the 24-week PUL 2.0 data that assumes the 24-week result represents the difference between treatment group and placebo at 52 weeks. The independent Data Safety Monitoring Board (DSMB) will evaluate this FA with a go/no go decision to continue the treatment groups or the study itself.

# EU Phase IIb/III Study Program *(Continued....)*

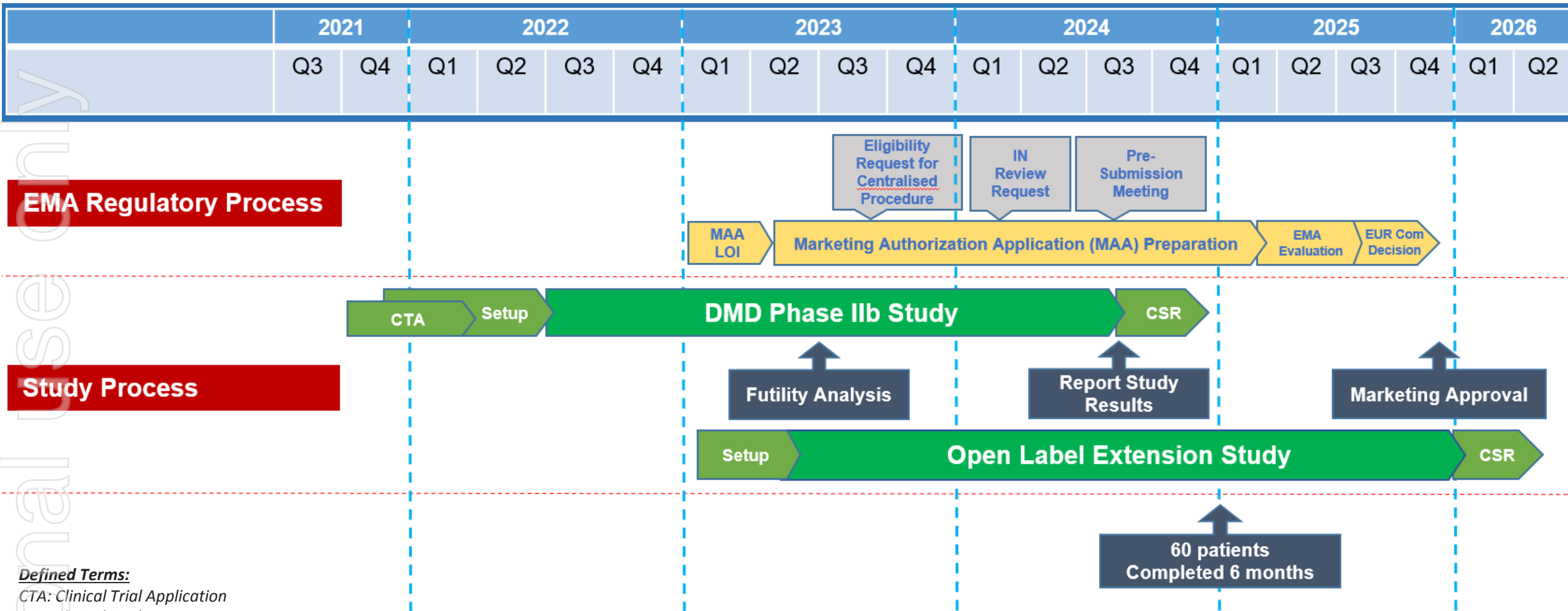
## **ATL1102 Open Label Extension Study:**

- All participants completing the Treatment Period of the Phase IIb/III study will be offered to enter an open label extension (OLE) study of ATL1102 based on the assumption of acceptable safety. The OLE study will serve to further increase the safety and efficacy database
- Participants not enrolling into the OLE study will complete the Post-treatment Follow-up period in the Phase IIb/III study

## **PCDO/EMA Additional Requirements:**

- A minimum number of participants not on corticosteroid therapy has been specified at 10% for the Phase IIb study
- Corticosteroid use should be considered as a covariate for the analyses, in relation to corticosteroid type and dosage
- Monitoring for effects on CD49d in the blood
- Events associated with Safety Monitoring Plan and Stopping Rules to be captured as secondary safety endpoints
- At least 60 participants should be followed for additional 6 months in the OLE study before MAA submission
- Participants entering the OLE study will continue on their initial dose level that they were randomised to in the Phase IIb/III study therefore, patients in the low dose treatment group (25 mg ATL1102 or placebo) will receive 25 mg ATL1102 and patients in the high dose treatment group (50 mg ATL1102 or placebo) will receive 50 mg ATL1102.

# Anticipated EU Phase IIb/III Study Timeline



## Defined Terms:

CTA: Clinical Trial Application

CSR: Clinical Study Report

LOI: Letter of Intent

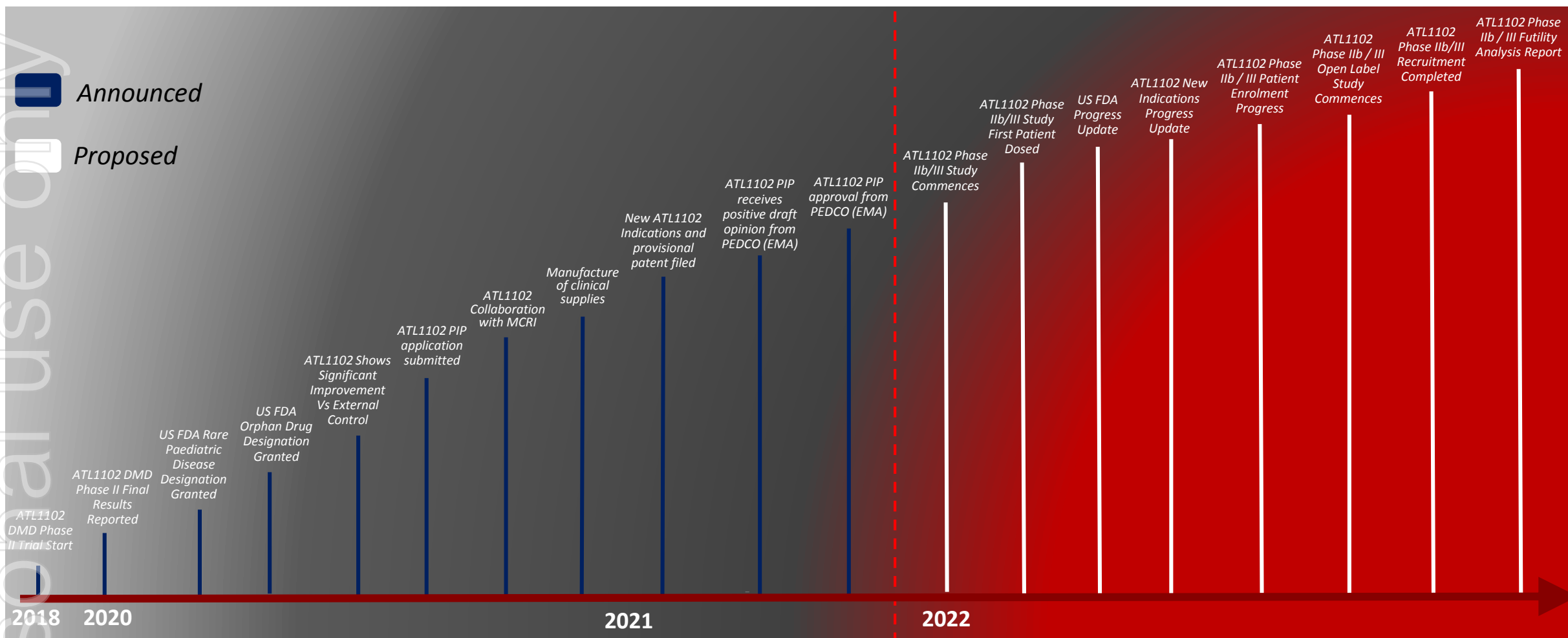
EUR Com Decision: European Commission Decision

Note: The above timeline is indicative and illustrative only and is not a forecast or projection or any assurance or guarantee that the indicated timelines will be met or that any individual milestone will be achieved in whole or in part. Refer to the Company's disclaimer on page 2 as well as the 'Key Risks' in Appendix A to this Presentation.



# Major Achievements and Upcoming Key Activities

*Regular progress and key market updates during the Phase IIb/III trial – multiple value creation opportunities*



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# Significant Market Opportunity

**ATL1102 - anti-inflammatory and immune modulating agent with potential for multiple clinical applications**

## ANTI-INFLAMMATORY

The market<sup>^</sup> size is expected global anti-inflammatory reach

**US\$191B by 2027**

(Fortune Business Insights)

<sup>^</sup>MS, Rheumatoid Arthritis, Asthma, Sinusitis Respiratory, IBD

## DMD THERAPIES

The global DMD drug market estimated to reach

**US\$4B by 2023 and**

**US\$10B by 2030**

(Kamet Research)

## CORTICOSTEROIDS

The global steroid market is forecast to attain value of

**US\$17 Billion in 2025**

(QY Research)

- Rare disease company **Sarepta Therapeutics Inc.** (NASDAQ: SRPT) (Mkt Cap **US\$7B**) 3<sup>rd</sup> Qtr 2021 DMD Sales Revenue was **US\$167M**
- In 2019 Roche acquired exclusive US rights for early stage gene therapy for DMD from Sarepta for **US\$1.15B** upfront **PLUS** **US\$1.7B** in milestone payments **PLUS** royalties on sales
- Cost of current DMD therapies:- Deflazacort (Emflaza) - CS approved in US only - avg cost ~**US\$93K<sup>1</sup>** per patient per year
  - Exondys 51 (exon-skipping/dystrophin restoration agent) avg cost in US ~**US\$750K<sup>1</sup>** per patient per year
  - Ataluren (Translarna) - stop codon skipping/dystrophin restoration) cost in EU ~**US\$320K<sup>1</sup>** per patient per year

<sup>3</sup> DelveInsight - Duchenne Muscular Dystrophy (DMD) Market Insight, Epidemiology, and Market Forecast—2030 (August 2021)

Note: Antisense's modelling is indicative and illustrative only and is not a forecast or projection of actual pricing of ATL1102 or the Company's ability to penetrate the depicted markets. A number of variable factors that will impact upon and influence actual pricing, market penetration and revenue and neither detailed and/or independent price modelling or audit has been undertaken.

## Assuming:

**\$200K per annum with 50% of boys non ambulant at any one time**  
**ATL1102 has a US\$4B market opportunity for DMD in US/EU**



**ATL1102 Phase IIb/III clinical development plan**

**Details of the Capital Raising**

**Appendix A: Antisense additional information**

**Appendix B: Key risks**

**Appendix C: Offering jurisdictions**



# Details of the Capital Raising

Offer Structure and Size	<ul style="list-style-type: none"> <li>Antisense is raising approximately \$20.0 million in a Placement to institutional and sophisticated investors (“<b>Placement</b>”). The placement will be conducted under the company’s existing placement capacity under ASX Listing Rule 7.1.</li> <li>Antisense also intends to undertake a 1 for 9.4 non-renounceable entitlement offer (“<b>Entitlement Offer</b>”) of fully paid ordinary shares to eligible shareholders. The Entitlement Offer is intended to raise approximately \$16.8 million and is not underwritten. Investors may also apply for shortfall shares under the Entitlement Offer.</li> <li>Shares issued under the Placement will be cum-entitlement and will be eligible to participate in the Entitlement Offer</li> <li>1 free-attaching unlisted new option will be issued for every 2 new shares issued under both the Placement and Entitlement Offer with an exercise price of \$0.48 per option.</li> </ul>
Offer Price	<ul style="list-style-type: none"> <li>The Offer price of A\$0.24 per share represents: <ul style="list-style-type: none"> <li>18.6% discount to last close of A\$0.295 on Wednesday 27 October 2021</li> <li>13.6% discount to the 15 day VWAP of \$0.278 up to and including Wednesday 27 October 2021</li> <li>4.9% discount to the 30 day VWAP of \$0.252 up to and including Wednesday 27 October 2021</li> </ul> </li> </ul>
Attaching Options	<ul style="list-style-type: none"> <li>1 free attaching new option issued for every 2 new shares issued under the Placement and Entitlement Offer with an exercise price of \$0.48 per option.</li> <li>The issue of the Placement options is subject to shareholder approval at Antisense’s AGM to be held on or around 15 December 2021.</li> <li>Options expire earlier of: <ul style="list-style-type: none"> <li>20 December 2024; or</li> <li>20 business days after the date on which the ATL1102 Phase IIb in DMD futility analysis results are announced to the ASX</li> </ul> </li> </ul>
Use of Proceeds	<ul style="list-style-type: none"> <li>As outlined on Page 19.</li> </ul>
Joint Lead Managers	<ul style="list-style-type: none"> <li>Morgans Corporate Limited (“Morgans”) and Wilsons Corporate Finance Limited (“Wilsons”) are engaged as Joint Lead Managers to the Placement</li> </ul>
Entitlement Offer	<ul style="list-style-type: none"> <li>Antisense will offer eligible Australian and New Zealand shareholders the opportunity to acquire new shares via an Entitlement Offer</li> <li>1 New Share for every 9.4 Shares held at the record date will be offered under the Entitlement Offer and will be issued at the same as the Offer price as the Placement. Investors may also apply for shortfall shares under the Entitlement Offer</li> <li>Shares issued under the Placement will be cum-entitlement and will be eligible to participate in the Entitlement Offer</li> <li>The Entitlement Offer is non-underwritten</li> <li>A Prospectus including further details of the Entitlement Offer will be dispatched to eligible shareholders in due course</li> </ul>
Ranking	<ul style="list-style-type: none"> <li>New shares issued will rank pari passu with existing shares from their date of issue</li> </ul>

# Detailed Use of Funds

- Funds raised via the Placement (\$20.0M) and the Entitlement Offer (\$16.8M), if fully subscribed, are intended to be used to fund the Phase IIb/III clinical trial through to futility analysis expected to be completed mid-CY2023.
- Should the Entitlement Offer not be fully subscribed, this may lead to a delay in the conduct of the Phase IIb/III study and depending on the amount raised, may require the Company to raise further capital or seek other funding in order to continue and/or complete the study. In this case, the Entitlement Offer monies received may be redeployed to prioritise other key activities including an US Toxicology Study, ATL1102 New Indications, Drug Manufacture and Business Development and Corporate initiatives to accelerate ATL1102 development plans.
- The 1:2 free-attaching options issued to participants of the Placement and Entitlement Offer, if fully exercised, will raise a further (\$36.8M) to fund the clinical program through to Phase IIb/III trial results in mid-2024 whilst also funding the Open Label Extension Study to the same point.

Use of Funds Raised	Placement	Placement & Entitlement Offer
Phase IIb/III - Clinical Study costs	*\$7,000,000	**\$21,000,000
Open Label Extension Study initiation costs	-	\$600,000
Drug Manufacturing Costs	\$5,500,000	\$7,400,000
Progression of R&D Programs	\$1,100,000	\$1,100,000
Working Capital	\$5,000,000	\$5,000,000
Capital Raising Costs	\$1,400,000	\$1,300,000
<b>TOTAL</b>	<b>\$20,000,000</b>	<b>\$36,800,000</b>

\* Anticipated costs up to trial application approval, ready for commencement of patient enrolment in the Phase IIb/III Study.

\*\* Phase IIb/III Study costs through to Futility Analysis.

# Capital Raising Timetable

Event	Date
Placement Bookbuild	Friday, 29 October
Announcement of Placement and Entitlement Offer Ordinary Shares Recommence Trading	Monday, 1 November
Settlement of Placement	Thursday, 4 November
Allotment & Trading of Shares Issued Under the Placement Lodgement of the Prospectus	Friday, 5 November
Lodgement of Notice of Annual General Meeting (AGM)	Tuesday, 9 November
Entitlement Offer Record Date	Wednesday, 10 November
Entitlement Offer Opens & Dispatch of the Prospectus	Monday, 15 November
Entitlement Offer Closes	Monday, 29 November
Allotment of Entitlement Offer Shares and Entitlement Offer Options	Friday, 3 December
Trading of Entitlement Offer Shares Commences	Monday, 6 December
Allotment of Placement Options	Thursday, 16 December
Holding Statements Dispatched	Friday, 17 December

*Note: All dates and times are indicative and subject to change without notice and have not been confirmed by the ASX. All times are Sydney time unless otherwise specified.*



**ATL1102 Phase IIb/III clinical development plan**

**Details of the Capital Raising**

**Appendix A: Antisense additional information**

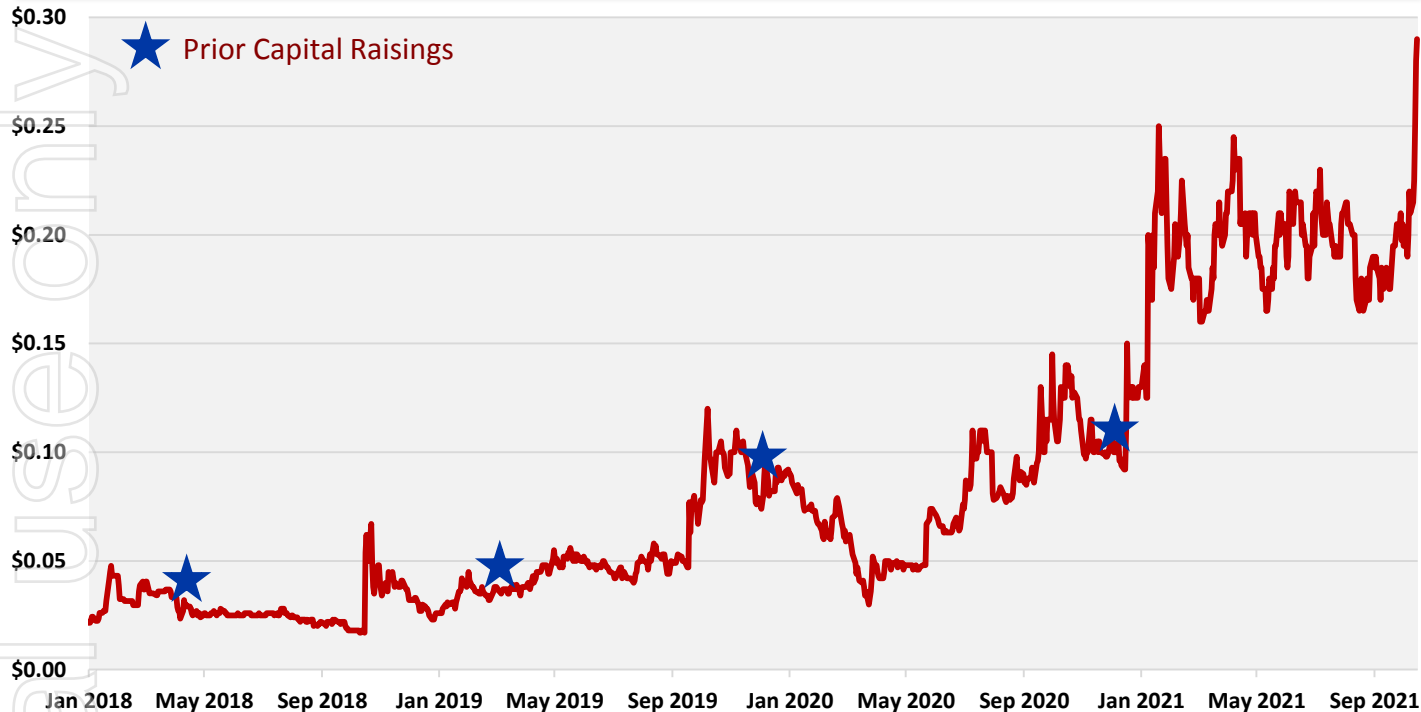
**Appendix B: Key risks**

**Appendix C: Offering jurisdictions**



# Antisense Corporate Overview

## Share Price Performance



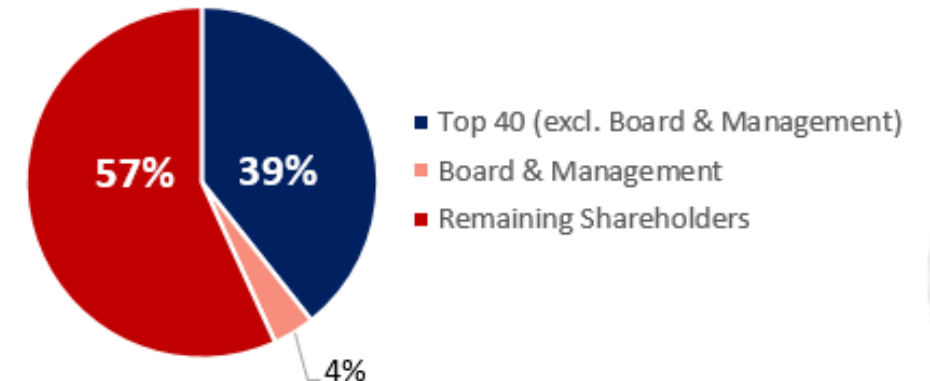
## Capital Raisings (since 2017)

- November 2020 – Placement & SPP - \$8.5M at \$0.10 per share
- December 2019 - Options Exercised - \$5.5M at \$0.08 per share
- March 2019 - Placement - \$1.6M at \$0.033 per share
- April 2018 - Placement & Entitlement Offer - \$5.0M at \$0.024 per share

## Company Details

- Market Capitalisation (27 Oct 2021) @ \$0.295 = \$169M
- Ordinary fully paid shares on issue = 574M
- Cash Balance as at 30 June 2021 = \$6M
- Largest shareholder – **Platinum Asset Management**

## Current Register



# Antisense - ATL1102

*Improved therapies are needed to ameliorate DMD severity & delay disease progression*

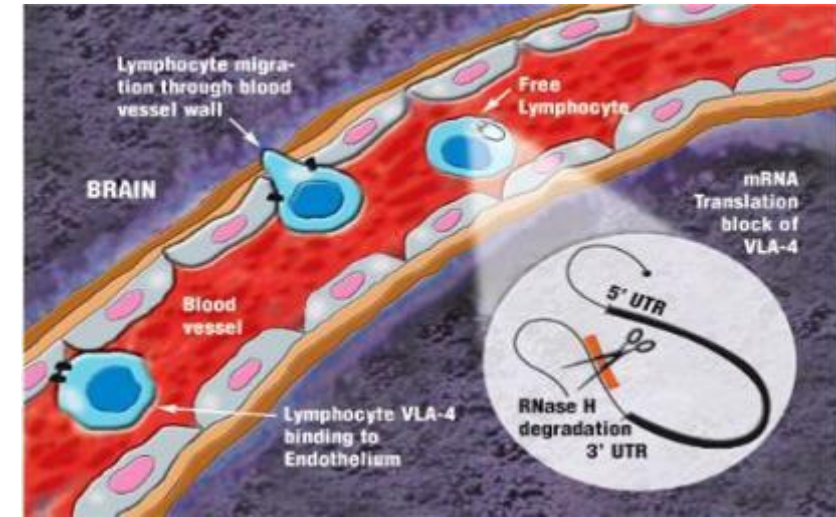
## ATL1102

- ATL1102 is a 2'MOE gapmer antisense oligonucleotide drug to integrin  $\alpha 4$  RNA (CD49d  $\alpha$  subunit of VLA-4), an adhesion molecule expressed on most human leukocytes
- ATL1102 is designed to inhibit CD49d expression on lymphocytes and thereby reduce their survival, activation and migration from the blood into sites of inflammation
- ATL1102 is an immunomodulatory antisense drug to human CD49d RNA which has completed a successful Phase IIa trial in Multiple Sclerosis (MS) patients  
[Limmroth V et al Neurology 2014, 11:83 (20) 1780-8]

## WHY ATL1102 FOR DMD?

### Pivotal scientific publication confirming CD49d as a potential target for DMD therapy

- DMD patients with greater number of circulating T cells with high levels of CD49d (CD49dhi) expression have both more severe & rapid progression of disease  
[Pinto-Mariz et al Skeletal Muscle 2015, 5: p45-55]
- Corticosteroids (CS) appear to have no effect on CD49dhi T cell numbers
- CS treatment does not modulate CD49d expression on T cells in MS
- Non-ambulant DMD patients have greatest number of CD49d high expressing T cells



Pinto-Mariz et al. Skeletal Muscle (2015) 5:45  
DOI 10.1186/s13395-015-0066-2

Skeletal Muscle

RESEARCH

Open Access

CD49d is a disease progression biomarker and a potential target for immunotherapy in Duchenne muscular dystrophy

Fernanda Pinto-Mariz<sup>1,2,3</sup>, Luciana Rodrigues Carvalho<sup>1</sup>, Alexandra Prifer De Queiroz Campos Araujo<sup>2</sup>, Wallace De Mello<sup>1</sup>, Márcia Gonçalves Ribeiro<sup>2</sup>, Maria Do Carmo Soares Alves Cunha<sup>2</sup>, Pedro Hernan Cabello<sup>6</sup>, Ingo Biedermann<sup>1</sup>, Elisa Meroni<sup>1</sup>, Isabelle Desquiere<sup>5</sup>, Mariana Vitor<sup>3</sup>, Erica Yeda<sup>3</sup>, Yves Allenbach<sup>6</sup>

antisense  
THERAPEUTICS

# ATL1102 – IP Protection

*Antisense has developed a substantial portfolio of intellectual property across multiple high-value markets*

## Orphan Drug Designation (ODD) Granted

- Protected by Commercial Exclusivity (data exclusivity and market exclusivity); post ATL1102 launch in DMD with orphan drug designation

*(Note: Exclusivity specifically relates to ODD)*

- 7 years USA and 10 years in Europe with potential to extend 2 year in EU

## Patent Portfolio

### ATL1102 Methods of reducing circulating leukocytes

Australia	2011301712	Patent Registered	2031*
Canada	2811228	Under Examination	2031
USA	9,885,048	Patent Registered	2031*

### ATL1102 Therapeutic uses and methods (for treating Muscular Dystrophy)

US Continuation-in-Part	16/404561	Filed 6 May 2019	2039*
International	PCT/AU2018/051353	Filed 18 December 2018	2039*
International	PCT/AU2020/050445	Filed 6 May 2020	2040*

PCT/AU2018/051353 has entered the national phase in Australia, Brazil, Canada, China, Japan, South Korea and New Zealand and the regional phase in Europe

*\*(Potentially extendible for up to 5 years)*

# ATL1102 – Phase II Study Results

## *“Positive effects across multiple measures of muscle structure, function & strength”<sup>1</sup>*

Open label Phase II trial in nine non-ambulant (wheelchair bound) boys 10-18 years of age with DMD conducted over 24 weeks of dosing:

- Primary endpoint met with confirmation of drug’s safety and tolerability
- Strong effects on secondary endpoints on activity markers and disease progression
  - Improvement or stabilisation across different measures of motor function & strength
  - Activity on the targeted CD49d immune cells consistent with drug’s proposed mechanism of action
  - MRI data suggests stabilisation of percentage of fat in muscles and preservation of functional muscle mass
- International KOLs are supportive of Phase IIb plans

<sup>1</sup> *“The data certainly suggests an overall ‘stabilisation’ in disease progression at the very least which of itself is a very positive clinical outcome. MRI data confirms the positive changes at a muscular/cellular level and supports the observed physical stabilisation/ improvements in muscle strength and function.*

*The consistency of positive clinically relevant effects of ATL1102 treatment across muscle measures of structure, strength and function are very pleasing and provide great encouragement for the treatment of non-ambulant patients with DMD.”*

**Professor Thomas Voit MD**

Director, NIHR GOSH Biomedical Research Centre, UK

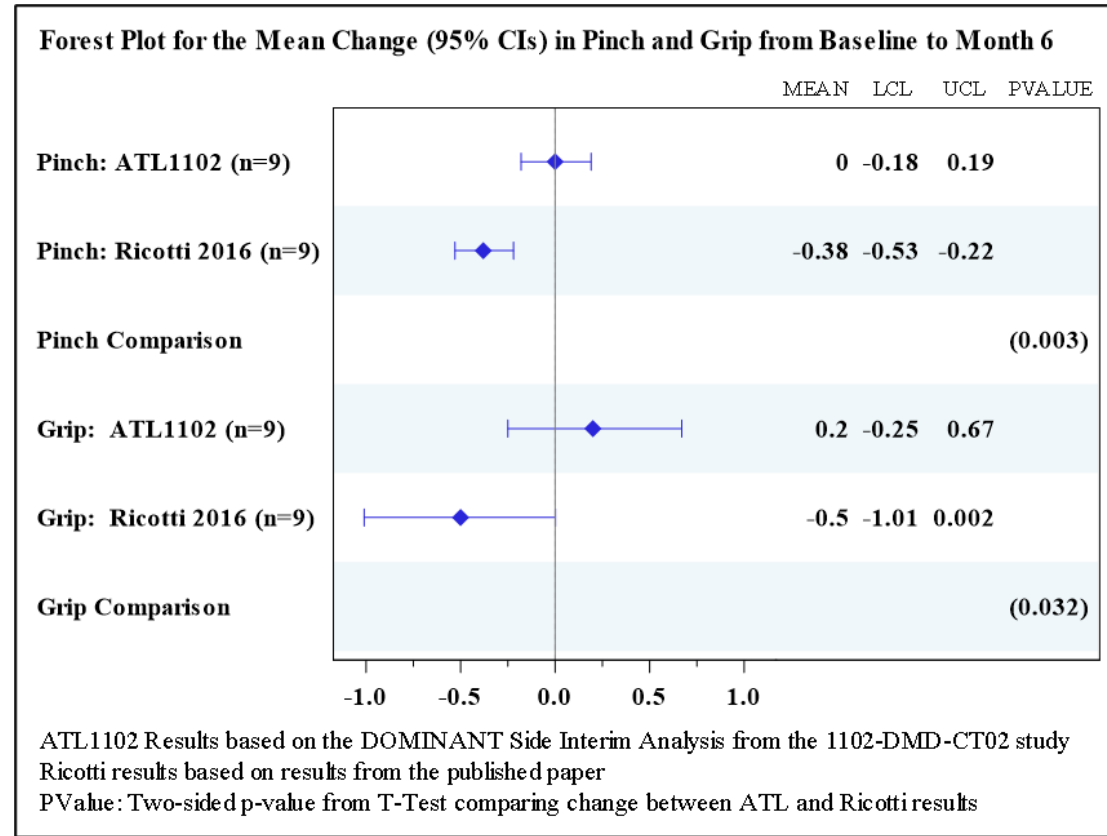


# ATL1102 Phase II Study Data *(Continued...)*

## Efficacy Parameters – Muscle Strength (MyoGrip & MyoPinch)

Comparison of Phase II Study Data with published literature **showing statistically significant improvements**. *(Ricotti et. al. 2016)*

When ATL1102 Phase II study grip and pinch strength data is compared with published historical data as a control, **ATL1102 generated statistically significant improvements** in pinch strength, and grip strength.



Ricotti et. al 2016 . PLoS One, 11(9) e0162542 (are historical results from a Non – Ambulant cohort of 9 DMD patients all on CS for 6 months).

# ATL1102 Phase II Study Data *(Continued...)*

## Efficacy Parameters – Muscle Structure (MRI)

Mean Change (95% CI) Screening/Baseline to Week 24/ 6 Months				
MRI Parameter	N	ATL1102 Study Data	N	Published Data*
MRI CENTRAL READING				
Fat Fraction (%)				
Volar Muscle	9	-0.57 (-7.8, 6.7)	7	0.7 (-1.8, 3.3)
Dorsal Muscles <sup>#</sup>	9	-0.88 (-3.4, 1.7)	7	5.5 (2.7, 8.3)
ECRLB-Br	9	-0.12 (-6.4, 6.2)	7	6.1 (3.1, 9.2)
Average Fat Fraction	9	-0.52 (-5.6, 4.6)	7	3.9 (1.9, 5.7)
Cross Sectional Muscle Area -total (mm <sup>2</sup> )	9	22.33 (-36.8, 81.4)	7	42.1 (-47.0, 131.2)
Remaining Muscle Area – total (mm <sup>2</sup> )	9	13.9 (-72.6, 100.4)	7	-32.1 (-102.6, 38.1)
MRI PROXIMAL READING - Average Fat Fraction (%) <sup>#</sup>	9	-2.14 (-7.6, 3.3)	7	4.5 (2.7, 6.3)
MRI DISTAL READING - Average Fat Fraction (%)	7	-5.14 (-18.7, 8.4) <sup>^</sup>	7	2.2 (-0.05, 4.5)

\*Ricotti et. al 2016. PLoS One, 11(9) e0162542 (results from Non – Ambulant cohort of 7 patients all but one on CS)

<sup>^</sup>Distal Reading is Average of Dorsal and Volar Muscle (ECRL-Br not measurable)

<sup>#</sup> T-Test analysis comparing the ATL1102 study data and the published data showed statistically significant differences for: MRI central reading mean change in percentage fat fraction from baseline to 6 months for the dorsal muscle group with a 2-sided p-value of 0.001. MRI proximal reading mean change in average fat fraction percentage with a 2- sided p-value of 0.018

Comparison of ATL1102 Phase II study data  
with data in published literature *(Ricotti et. al. 2016)*

*“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 MD,**

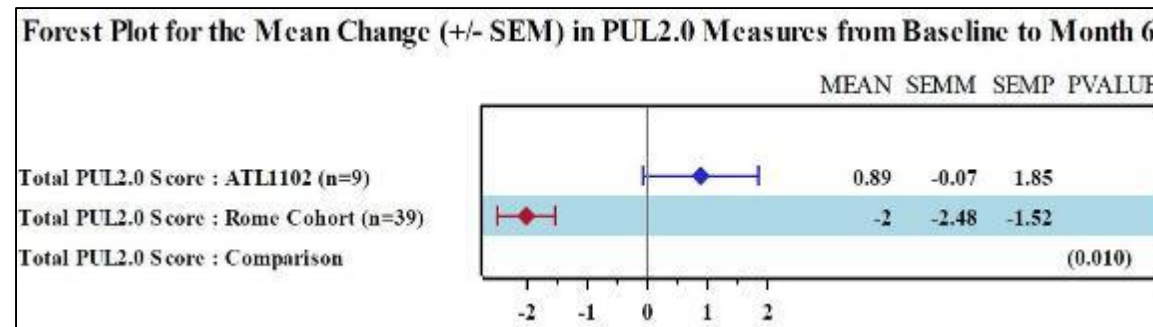
Researcher and Honorary Clinical Lecturer, Great Ormond Street  
Institute of Child Health University College London, UK

# ATL1102 Phase II Study Data *(Continued...)*

## Efficacy Parameters – Performance of Upper Limb Function (PUL2.0)

- ATL1102 data presented at the 25<sup>th</sup> International Annual Congress of the World Muscle Society in 2020
- ATL1102 treated patients demonstrated a statistically significant improvement in the mean (SD) PUL2.0 scores for the 24 week treatment compared to external control (Rome Cohort)

***ATL1102 Shows Statistically Significant Improvement vs Natural History Control in PUL 2.0 the registration endpoint for treatments in non-ambulant DMD***



*“The level of improvement achieved **is very positive** and clinically relevant. As Total PUL2.0 is the key efficacy endpoint for seeking drug approval in non-ambulant patients with DMD, the comparative data further indicates **ATL1102’s promising potential** to provide clinically meaningful benefits in the future treatment of non-ambulant DMD patients who have very limited treatment options.”*

**Professor Eugenio Mercuri,**

Professor of Pediatric Neurology at the Catholic University, Rome, Italy

# ATL1102 New Indications

## ***R&D collaboration with the Murdoch Children's Research Institute (MCRI)***



- ATL1102 modulates CD49d+ immune cells and disease processes in DMD and Multiple Sclerosis
- Several inflammatory muscle disease indications (like DMD) have been identified which may benefit from similar CD49d cell modulation for better treatment outcomes
- Murdoch Children's Research Institute (MCRI) is the largest child health research institute in Australia committed to making discoveries and developing treatments to improve child and adolescent health in Australia and around the world
- ANP and MCRI have collaborated to generate positive DMD animal (*mdx*) model data that showed antisense inhibition of CD49d reduces both the CD49d target in the muscle and muscle damage. This data was submitted in 2021 for publication
- Further study of antisense inhibition of CD49d effects in the *mdx* model in combination with other DMD treatments including the dystrophin restoration drugs to be conducted
- ASO inhibition of CD49d to also be assessed in another animal model of muscle disease where there are similar immune mediated inflammatory features to the *mdx* model
- 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
- Ability to deepen the product pipeline while adding further value to the ATL1102 asset



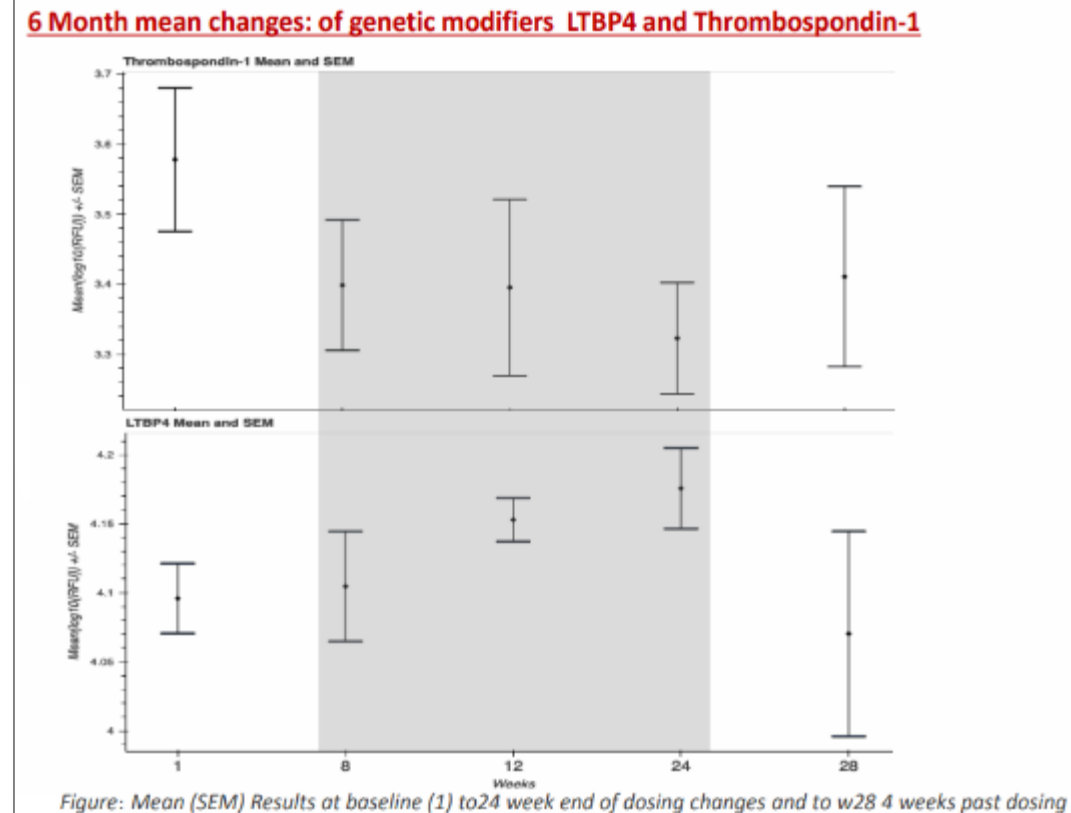
# ATL1102 New Indications *(Continued...)*

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

ATL1102 data presented at the 26<sup>th</sup> International Annual Congress of the World Muscle Society in 2021

- **Statistically significant mean** modulation at 24 weeks compared to baseline in Thrombospondin1 (TSP-1) **(-49%)\*** and Latent TGF-beta-binding protein 4 (LTBP4) **(20.7%)\*** levels, two proteins that modify the rate of loss of ambulation in DMD.
- **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.
- **New Provisional Patent** application filed covering this new data and applications of ATL1102 in new potential disease settings including diabetic, respiratory and age-related 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 **(18.0%)\*** is bound, and in CXCL16 **(29.9%)\*** which **can promote muscle regeneration**.

\* (False Discovery Rate:  $p$ -value  $< 0.0005$ )



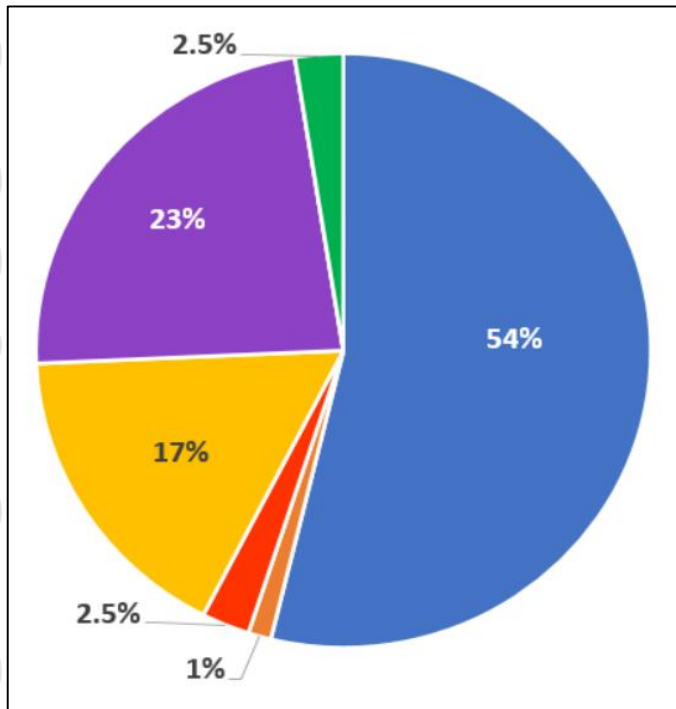
*The positive effects shown on the above proteins strengthen ATL1102's profile in the treatment of both non ambulant and ambulant DMD patients while positioning it as an exciting prospective therapeutic approach in other muscle and fibrotic conditions.*

# The DMD Market

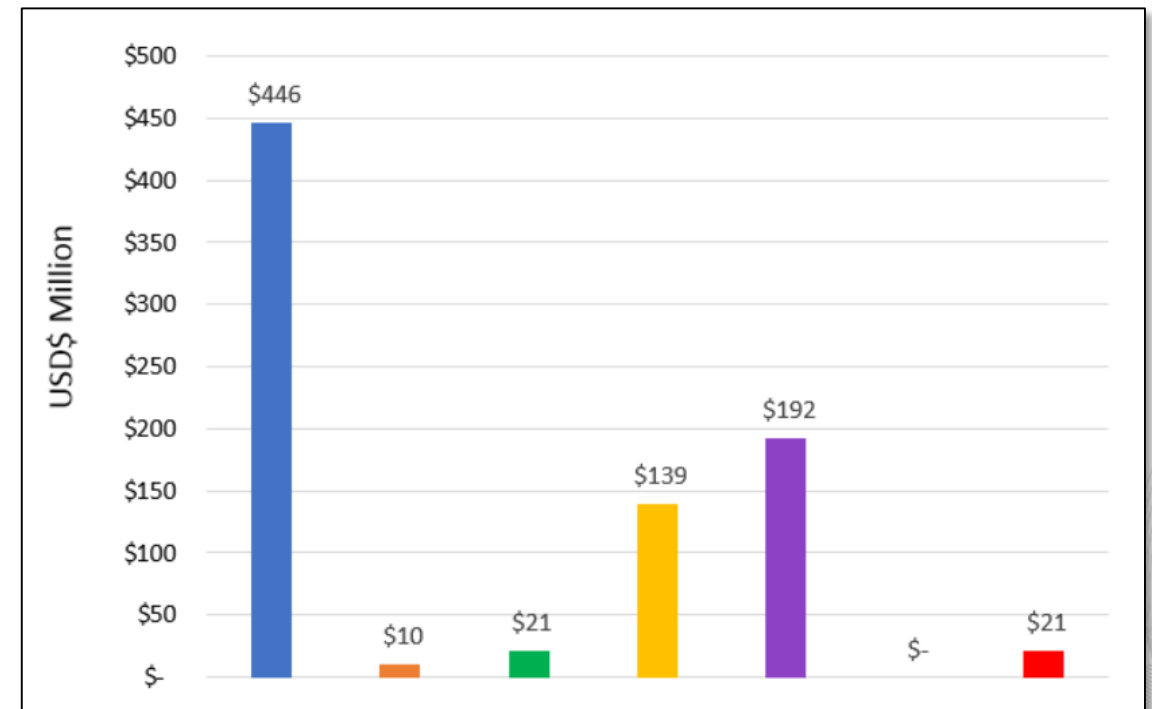
*The Global DMD drug market is expected to reach over US\$4 Billion by 2023 and US\$10 Billion by 2030<sup>1</sup>*

## DMD Market by therapies in 2020<sup>2</sup>

### 2020 DMD Market Share



### 2020 DMD Market Revenue



<sup>1</sup> [kametresearch.com/dmd-duchenne-muscular-dystrophy-market-analysis/](https://kametresearch.com/dmd-duchenne-muscular-dystrophy-market-analysis/)

<sup>2</sup> DelveInsight - Duchenne Muscular Dystrophy (DMD) Market Insight, Epidemiology, and Market Forecast—2030

# DMD Development Landscape

*Limited emerging competition in non-ambulant space*

*ATL1102 is a well-positioned differentiator from other products in development for treatment of DMD*

INTERVENTION MECHANISMS	ASSET	SPONSORS
INFLAMMATION & FIBROSIS	ATL1102	antisense THERAPEUTICS
	STERIODS Emflaza, VBP15	PTC, Santhera
	ANTI-FIBROTICS pamrevlumab	FibroGen
CARDIAC & CALCIUM REGULATION	CARDIAC DRUGS Ifetroban	Cumberland Pharma
	CALCIUM BALANCE Rimeporide	EspeRare Foundation
DYSTROPHIN REPLACEMENT & RESTORATION	GENE THERAPY	Pfizer
	EXON 51 /EXON 53/EXON 45 SKIPPING	Sarepta Therapeutics Nippon Shinyaku
	Ataluren READTHROUGH THERAPY	PTC Therapeutics
RESPIRATORY CELL ENERGY	A0364	Astellas Pharma
MUSCLE GROWTH & REGENERATION	CAP-1002 (intravenous cell therapy)	Capricor Inc
	Givinostat	Italfarmaco

- ATL1102 has a novel MOA to reduce inflammation in DMD patients
  - Anti-inflammatory steroids, dystrophin restoration technologies and gene therapies are being tested in predominantly ambulant patients
  - ATL1102's novel mechanism in targeting CD49d suggests potential for drug to be used in combination with steroid anti-inflammatory agents
- ATL1102 has potential to be synergistic with other projects in development reducing competitive pressure of other potential product launches
- ATL1102 has a mechanism that appears effective across all genetic subtypes of DMD - a key differentiator among the exon skipping therapies which increases the addressable patient pool

# DMD Market – Sarepta Comparison

## Sarepta Therapeutics Inc – Exondys 51, Vyondys 53 & Amondys 45

- First FDA approved treatment for DMD (2016)
- Exondys 51 only applicable for 13% of boys with the exon 51 mutation
- Vyondys 53, approved in 2019 for approx. 8% boys with exon 53 mutation
- Amondys 45, approved in 2021 for approx. 8% boys with exon 45 mutation
- Both received accelerated US approval (only) based on the surrogate endpoint of dystrophin increase in skeletal muscle observed in some treated patients.
- Sarepta market cap has grown from ~US\$60M (July2012) to **\$3B** on FDA approval of Exondys 51 to **~US\$7B** today.
- Exondys 51 cost per patient in the US is ~US\$750K per year.
- 3<sup>rd</sup> Quarter 2021 total net revenue for Sarepta ~ **US\$167M** in US only
- Roche acquired EU rights from Sarepta for early stage gene therapy for DMD for **US\$1.15B** upfront with **US\$1.7B** in milestones plus royalties on sales.
- **Mr William Goolsbee** - former Chairman of Sarepta, is a Non-Executive Director (NED) of Antisense Therapeutics.
- **Dr Gil Price** - former Non-Executive Director of Sarepta, is Antisense Therapeutics' NED & Consultant Medical Director.





# The Board of Directors

## *Highly experienced Board and Management with prior success in drug development and commercialisation*

### **DR. CHARMAINE GITTLESON** (Chairperson)

Dr. Gittleson is a senior executive with international experience as a pharmaceutical physician and enterprise leader in pharmaceutical drug development, governance and risk management gained during her 15-year tenure with global biotechnology company CSL Limited. At CSL, she had accountability for clinical research, medical safety, ethics for development, providing leadership across multiple therapeutic and rare disease areas.

### **DR. GARY PACE** (Non-Executive Director)

Dr. Pace has more than 40 years of experience in the development and commercialization of advanced tech. in biotech., pharmaceuticals, and medical devices. In 2003 Dr. Pace was awarded a Centenary Medal by the Australian Government “for service to Australian society in research and development”, and in 2011 was awarded Director of the Year (corporate governance) by the San Diego Directors Forum.

### **MR. ROBERT MOSES** (Non-Executive Director)\*

Mr. Moses draws on more than 40 years’ experience in the pharma/biotech industry. During the period 1993-2001, Mr. Moses played a central role in CSL’s development internationally. Prior to joining CSL, Mr. Moses was Managing Director of commercial law firm Freehills, Chairman and CEO of a NASDAQ listed medical service company, and Corporate Manager of New Business Development at ICI (now Orica).

### **DR. GRAHAM MITCHELL** (Non-Executive Director)

Dr. Mitchell was a former senior researcher at the Walter & Eliza Hall Institute, a Chief Scientist in Victorian Government Departments, and a Director of Research in the R&D Division of CSL Limited.

### **MR. MARK DIAMOND** (Managing Director & CEO)

Mark Diamond has over 30 years experience in the pharmaceutical and biotechnology industry. Before joining Antisense Therapeutics Limited as MD and CEO in 2001, Mr. Diamond was employed in the US as Director, Project Planning/Business Development at Faulding Pharmaceuticals. Prior to this he held the positions of Senior Manager, Business Development and In-licensing within Faulding’s European operation based in the UK and International Business Development Manager with Faulding in Australia.

### **DR. GIL PRICE** (Non-Executive Director & Medical Director)

Dr. Price is a clinical physician with a long-standing focus in drug development, adverse drug reactions, drug utilization and regulation. Dr. Price is an experienced biotech executive and entrepreneur with a depth of expertise across clinical asset investment strategy, evaluation, financing and execution. From 2007 to 2016, Dr. Price was a non-executive director of Sarepta Therapeutics, Inc., where he helped guide Sarepta’s transition to become a multi-billion dollar company with the first approved drug for DMD (sales approaching US\$400M annually).

### **MR. WILLIAM GOOLSBEE** (Non-Executive Director)\*

William (Bill) Goolsbee was founder, Chairman and Chief Executive Officer of Horizon Medical Inc. from 1987 until its acquisition by a unit of UBS Private Equity in 2002. Mr. Goolsbee was a founding Director of ImmunoTherapy Corporation in 1993, and became Chairman in 1995, a position he held until overseeing the successful acquisition of ImmunoTherapy by AVI Biopharma, Inc. (now Sarepta Therapeutics) in 1998.

# The Management Team

## **MR. MARK DIAMOND** (Managing Director & CEO)

Mark Diamond has over 30 years experience in the pharmaceutical and biotechnology industry. Before joining Antisense Therapeutics Limited as MD and CEO in 2001, Mr. Diamond was employed in the US as Director, Project Planning/Business Development at Faulding Pharmaceuticals. Prior to this he held the positions of Senior Manager, Business Development and In-licensing within Faulding's European operation based in the UK and International Business Development Manager with Faulding in Australia.

## **Mr GIL PRICE** (Consultant Medical Director)

Dr. Price is a clinical physician with a long-standing focus in drug development, adverse drug reactions, drug utilization and regulation. Dr. Price is an experienced biotech executive and entrepreneur with a depth of expertise across clinical asset investment strategy, evaluation, financing and execution. From 2007 to 2016, Dr. Price was a non-executive director of Sarepta Therapeutics, Inc., where he helped guide Sarepta's transition to become a multi-billion dollar company with the first approved drug for DMD (sales approaching US\$400M annually).

## **MS. NUKET DESEM** (Director of Clinical & Regulatory Affairs)

Ms Desem has over 25 years' experience in global regulatory affairs, clinical development and project management obtained through her roles within the pharmaceutical/biotechnology industry, including senior positions in various biotech companies. Ms Desem was previously employed at Antisense Therapeutics (2004–2010) as the Company's Development Director where part of her responsibility was the management of ANP's clinical trial programs. Major achievements in this role included the successful conduct and completion of the Company's multinational Phase IIa clinical trial of ATL1102 for the treatment of Multiple Sclerosis.

## **DR. GEORGE TACHAS** (Director of Drug Discovery & Patents)

Dr Tachas received his Ph.D from the University of Melbourne ('88) and a Diploma of Intellectual Property Law ('94). Dr Tachas Ph.D studies ('84-88) were in gene transfer, cloning and characterising of genes important in immunology at the Centre for Cancer and Transplantation, Uni. Melbourne. His post-doctoral studies were in molecular and cellular biology of vascular smooth muscle cells in cardiovascular disease as Head of Molecular Biology at the Cardiovascular Research Unit of Uni. Melbourne's Anatomy Department. Dr Tachas is inventor of using ATL1102 for the treatment of DMD.



**ATL1102 Phase IIb/III clinical development plan**

**Details of the Capital Raising**

**Appendix A: Antisense additional information**

**Appendix B: Key risks**

**Appendix C: Offering jurisdictions**

# Key risks

## INVESTMENT RISKS

This section highlights some of the risks that potential investors should consider prior to entering into the investment opportunity referred to in this Presentation. However, the following is not, and does not purport to be, a comprehensive statement of all relevant risks and is not listed in order of importance. Potential investors should seek their own financial or other professional advice in relation to the risks and must make their own assessment regarding an investment in the Company.

### Phase IIb/III Clinical trial program

There are a number of risks which relate specifically to the Phase IIb/III Clinical Trial program (including the open label study) which include but are not limited to:

- the Company obtaining access to adequate funding to undertake and complete the program;
- timing of regulatory submissions, feedback and approvals from the applicable regulatory or government agencies which may see delays and or possible rejection of the submissions;
- the Company obtaining adequate clinical supplies of drug compound to complete the Phase IIb/III Clinical Trial program;
- patient recruitment rates, timeframes, and access to sufficient patient numbers may adversely affect the conduct of the trial and the costs associated with the running of the trial; and
- risks associated with the conduct of clinical trials including meeting prespecified clinical endpoints and encountering serious safety or efficacy issues that may cause a slowing or halting of the study.

If any of these risks occur, it may have an impact on the Company's ability to conduct or continue the Phase IIb/III Clinical Trial program which may have an adverse effect on the Company's business, operations and the Company's product development efforts.

### Pharmaceutical Research and Development (R&D)

Pharmaceutical R&D involves scientific uncertainty and long lead times. Risks inherent in these activities include uncertainty of the outcome of the Company's research results; difficulties or delays in development of any of the Company's drug candidates; and general uncertainty related to the scientific development of a new medical therapy.

The Company's drug compounds require significant pre-clinical and human clinical development prior to commercialisation, which is uncertain, expensive and time consuming. There may be adverse side effects or inadequate therapeutic efficacy of the Company's drug candidates which would prevent further commercialisation. There may be difficulties or delays in the manufacturing or testing of any of the Company's drug candidates. There may also be adverse outcomes with the broader clinical application of the Antisense technology platform which could have a negative impact on the Company's specific drug development and commercialisation plans.

No assurance can be given that the Company's product development efforts will be successful, that any potential product will be safe and efficacious, that required regulatory and pricing reimbursement approvals will be obtained, that the Company's products will be capable of being produced in commercial quantities at an acceptable cost or at all, that the Company will have access to sufficient capital to successfully advance the products through development or to find suitable development or commercial partners for the development and/or commercialisation of the products and that any products, if introduced, will achieve market acceptance.

### Additional Capital Requirements

Pharmaceutical R&D activities require a high level of funding over a long period of time to complete the development and commercialisation of pharmaceutical products. There is no assurance that additional funding will be available to the Company in the future or be secured on acceptable terms. If adequate funds are not available, the Company's business will be materially and adversely affected. If the Company is unable to access capital to continue the development of its products, then this could adversely impact on the collaboration and licensing agreement with Ionis. If the Company is unable to meet certain performance obligations, it may lead to a dispute with Ionis. Unresolved disputes may in turn lead to potential termination of the license granted by Ionis to the Company to exploit relevant products, with the relevant product rights then returning to Ionis.

## Partnering and Licensing

Due to the significant costs in drug discovery and development it is common for biotechnology companies to partner with larger biotechnology or pharmaceutical companies to help progress drug development. While the Company has previously entered into such licensing agreements with pharmaceutical partners, there is no guarantee that the Company will be able to maintain such partnerships or license its products in the future. There is also no guarantee that the Company will receive back all the data generated by or related intellectual property from its licensing partners. In the event that the Company does license or partner the drugs in its pipeline, there is no assurance as to the attractiveness of the commercial terms nor any guarantee that the agreements will generate a material commercial return for the Company.

## Regulatory Approvals

Complex government health regulations, which are subject to change, add uncertainty to obtaining approval to undertake clinical development or obtaining marketing and pricing reimbursement approval for pharmaceutical products. Delays may be experienced in obtaining such approvals, or the regulatory authorities may require repeat of different or expanded animal safety studies or human clinical trials, and these may add to the development cost and delay products from moving into the next phase of drug development and up to the point of entering the market place. This may adversely affect the competitive position of products and the financial value of the drug candidates to the Company.

There can be no assurance that regulatory clearance will be obtained for a product or that the data obtained from clinical trials will not be subject to varying interpretations. There can be no assurance that the regulatory authorities will agree with the Company's assessment of future clinical trial results or with the suitability of the Company's regulatory submissions for clinical trial, early access or product marketing approval as applicable.

## Competition

The Company will always remain subject to the material risk arising from the intense competition that exists in the pharmaceutical industry. A material risk therefore exists that one or more competitive products may be in human clinical development now or may enter into human clinical development in the future. Competitive products focusing on or directed at the same diseases or protein targets as those that the Company is working on may be developed by pharmaceutical companies or other antisense drug companies including Ionis or any of its other collaboration partners or licensees. Such products could prove more efficacious, safer, more cost effective or more acceptable to patients than the Company product. It is possible that a competitor may be in that market place sooner than the Company and establish itself as the preferred product.

## Technology and Intellectual Property Rights

Securing rights to technology and patents is an integral part of securing potential product value in the outcomes of pharmaceutical R&D. The Company's success depends, in part, on its ability to obtain patents, maintain trade secret protection and operate without infringing the proprietary rights of third parties. There can be no assurance that any patents which the Company has in licensed or may own, access or control will afford the Company commercially significant protection of its technology or its products or have commercial application, or that access to these patents will mean that the Company will be free to commercialise its drug candidates. The granting of a patent does not guarantee that the rights of others are not infringed or that competitors will not develop technology or products to avoid the Company's patented technology or try to invalidate the Company's patents, or that it will be commercially viable for the Company to defend against such potential actions of competitors.

Accordingly, investment in companies specialising in drug development must be regarded as highly speculative. The Company strongly recommends that professional investment advice be sought prior to such investments.



# Key risks *(Continued...)*

## **Environmental Regulation and Performance**

The Company is involved in pharmaceutical research and development, much of which is contracted out to third parties, and it is the Director's understanding that these activities do not create any significant/material environmental impact. To the best of the Company's knowledge, the scientific research activities undertaken by, or on behalf of, the Company are in full compliance with all prescribed environmental regulations.

## **Reimbursement Approvals and Government Policy**

Changes to the laws, regulations, standards and practices applicable to the industry in which the Company operates (for example, drug approval regulations and government R&D rebates) may increase costs and limit the Company's proposed scope of activity. The Company has little or no control over these risks. Consequently, there can be no firm assurance that the Company can effectively limit these risks, which could materially adversely affect its business, financial condition and results of operations.

The research, development, manufacture, marketing and sale of products using the Company's technology are subject to varying degrees of regulation by a number of government authorities in Australia and overseas. Products developed using the Company's technology, must undergo a comprehensive and highly regulated development and review process before receiving approval for marketing. The process includes the provision of clinical data relating to the quality, safety and efficacy of the products for their proposed use.

Products may also be submitted for reimbursement approval. The availability and timing of that regulatory and/or reimbursement approval may have an impact upon the uptake and profitability of products in some jurisdictions. Furthermore, any of the products utilising the Company's technology may be shown to be unsafe, non-efficacious, difficult or impossible to manufacture on a large scale, uneconomical to market, compete with superior products marketed by third parties or not be as attractive as alternative treatments.

## **Management Actions**

The Directors will, to the best of their knowledge, experience and ability (in conjunction with the management team) endeavour to anticipate, identify and manage the risks inherent in the activities of the Company, but without assuming any personal liability, with the aim of eliminating, avoiding and mitigating the impact of risks on the performance of the Company and its securities.

The Company is dependent on the principal members of its scientific and development team, the loss of whose services could materially adversely affect the Company and may impede the achievement of its research and development objectives. Given the nature of the Company's activities, its ability to maintain its program is dependent on its ability to attract and maintain appropriately qualified personnel either within the Company or through contractual arrangements. If one or more of the Company's key personnel was unwilling or unable to continue in their current roles, there is a risk that the Company may be unable to recruit a suitable replacement on commercially acceptable terms or at all.

The loss of any key personnel, without suitable and timely replacement, may significantly disrupt the operations of the Company's business and impede the Company's ability to implement its business plans. This may, in turn, have a materially adverse effect on both the financial performance and future prospects of the Company. The Company may also incur significant costs in recruiting and retaining new key personnel.

Further, the Company's current size affects its ability to provide substantial training and development opportunities to its key managers and personnel. Extensive ongoing development opportunities are not feasible for a small biotechnology company such as Antisense. The Company has sought to address this risk by hiring sufficiently qualified and skilled management and scientific development staff.

## **Litigation, Claims and Disputes**

The Company may be subject to litigation and other claims and disputes in the course of its business, including contractual disputes with suppliers or customers, employment disputes, indemnity claims, and occupational and other claims. There is a risk that any such litigation, claim or dispute could materially adversely impact the Company's operating and financial performance due to the significant cost and time invested by management in investigating, commencing, defending and/or settling such matters. Any claim against the Company, if proven, may also have a sustained negative impact on its operations, financial performance, financial position and reputation.

The Company is not currently engaged in litigation and, as at the date of this Presentation, the Directors are not aware of any legal proceedings pending or threatened against, or any material legal proceedings affecting, the Company.

## **Share Market Conditions**

The market price of securities can fall as well as rise and may be subject to varied and unpredictable influences on the market for equities. Neither the Company nor the Directors warrant the future performance of the Company or any return on an investment in the Company.

## **Economic Factors**

The operating and financial performance of the Company is influenced by a variety of general economic and business conditions including the levels of consumer confidence and spending, business confidence and investment, employment, inflation, interest rates, foreign exchange rates, access to debt and capital markets, fiscal policy, monetary policy and regulatory policies. A prolonged deterioration in any number of the above factors may have a material adverse impact on the Company's business and financial performance including its ability to fund its activities.

## **COVID-19 Pandemic**

The COVID-19 pandemic has to date created significant economic and social challenges in Australia and around the world. There is continued uncertainty in relation to the ongoing impacts of the pandemic which, to date, have included a general contraction in output, increased levels of unemployment, restrictions on movement and includes the potential to impact recruitment of patients into clinical trials and the welfare of those patients being impacted by COVID over the extended trial period. In response, the Company has concentrated on preserving cash and long term shareholder value while maintaining focus on service of new and existing clients.

The Company will continue to closely monitor developments related to COVID-19 and is cognisant of its duty to responsibly manage and, where possible mitigate the risks posed by the global pandemic.

## **Taxation**

Relevant tax laws and treaties and their interpretation and applicability change from time to time. There is the risk that these changes could adversely and materially affect the Company's profitability and prospects.

## **Unforeseen Expenses**

While the Company is not aware of any expenses that may need to be incurred that have not been taken into account, if such expenses were subsequently incurred, the expenditure proposals of the Company may be adversely affected.





**ATL1102 Phase IIb/III clinical development plan**

**Details of the Capital Raising**

**Appendix A: Antisense additional information**

**Appendix B: Key risks**

**Appendix C: Offering jurisdictions**

# International Offer Restrictions

## International Offer Restrictions

This document does not constitute an offer of new ordinary shares ("New Shares") of the Company in any jurisdiction in which it would be unlawful. In particular, this document may not be distributed to any person, and the New Shares may not be offered or sold, in any country outside Australia except to the extent permitted below.

### New Zealand

This document has not been registered, filed with or approved by any New Zealand regulatory authority under the Financial Markets Conduct Act 2013 (the "FMC Act").

The New Shares are not being offered or sold in New Zealand (or allotted with a view to being offered for sale in New Zealand) other than to a person who:

- is an investment business within the meaning of clause 37 of Schedule 1 of the FMC Act;
- meets the investment activity criteria specified in clause 38 of Schedule 1 of the FMC Act;
- is large within the meaning of clause 39 of Schedule 1 of the FMC Act;
- is a government agency within the meaning of clause 40 of Schedule 1 of the FMC Act; or
- is an eligible investor within the meaning of clause 41 of Schedule 1 of the FMC Act.

### Hong Kong

**WARNING:** This document has not been, and will not be, registered as a prospectus under the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong, nor has it been authorised by the Securities and Futures Commission in Hong Kong pursuant to the Securities and Futures Ordinance (Cap. 571) of the Laws of Hong Kong (the "SFO"). Accordingly, this document may not be distributed, and the New Shares may not be offered or sold, in Hong Kong other than to "professional investors" (as defined in the SFO and any rules made under that ordinance).

No advertisement, invitation or document relating to the New Shares has been or will be issued, or has been or will be in the possession of any person for the purpose of issue, in Hong Kong or elsewhere that is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to New Shares that are or are intended to be disposed of only to persons outside Hong Kong or only to professional investors. No person allotted New Shares may sell, or offer to sell, such securities in circumstances that amount to an offer to the public in Hong Kong within six months following the date of issue of such securities.

The contents of this document have not been reviewed by any Hong Kong regulatory authority. You are advised to exercise caution in relation to the offer. If you are in doubt about any contents of this document, you should obtain independent professional advice.

# International Offer Restrictions *(Continued...)*

## Singapore

This document and any other materials relating to the New Shares have not been, and will not be, lodged or registered as a prospectus in Singapore with the Monetary Authority of Singapore. Accordingly, this document and any other document or materials in connection with the offer or sale, or invitation for subscription or purchase, of New Shares, may not be issued, circulated or distributed, nor may the New Shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore except pursuant to and in accordance with exemptions in Subdivision (4) Division 1, Part XIII of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA"), or as otherwise pursuant to, and in accordance with the conditions of any other applicable provisions of the SFA.

This document has been given to you on the basis that you are (i) an "institutional investor" (as defined in the SFA) or (ii) an "accredited investor" (as defined in the SFA). If you are not an investor falling within one of these categories, please return this document immediately. You may not forward or circulate this document to any other person in Singapore.

Any offer is not made to you with a view to the New Shares being subsequently offered for sale to any other party. There are on-sale restrictions in Singapore that may be applicable to investors who acquire New Shares. As such, investors are advised to acquaint themselves with the SFA provisions relating to resale restrictions in Singapore and comply accordingly.

## United Kingdom

Neither this document nor any other document relating to the offer has been delivered for approval to the Financial Conduct Authority in the United Kingdom and no prospectus (within the meaning of section 85 of the Financial Services and Markets Act 2000, as amended ("FSMA")) has been published or is intended to be published in respect of the New Shares.

The New Shares may not be offered or sold in the United Kingdom by means of this document or any other document, except in circumstances that do not require the publication of a prospectus under section 86(1) of the FSMA. This document is issued on a confidential basis in the United Kingdom to "qualified investors" within the meaning of Article 2(e) of the UK Prospectus Regulation. This document may not be distributed or reproduced, in whole or in part, nor may its contents be disclosed by recipients, to any other person in the United Kingdom.

Any invitation or inducement to engage in investment activity (within the meaning of section 21 of the FSMA) received in connection with the issue or sale of the New Shares has only been communicated or caused to be communicated and will only be communicated or caused to be communicated in the United Kingdom in circumstances in which section 21(1) of the FSMA does not apply to the Company.

In the United Kingdom, this document is being distributed only to, and is directed at, persons (i) who have professional experience in matters relating to investments falling within Article 19(5) (investment professionals) of the Financial Services and Markets Act 2000 (Financial Promotions) Order 2005 ("FPO"), (ii) who fall within the categories of persons referred to in Article 49(2)(a) to (d) (high net worth companies, unincorporated associations, etc.) of the FPO or (iii) to whom it may otherwise be lawfully communicated (together "relevant persons"). The investment to which this document relates is available only to relevant persons. Any person who is not a relevant person should not act or rely on this document



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