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THERAPEUTICS

(ASX: ANP)

**AGM Presentation
December 2021**

Forward Looking Statements

This presentation contains forward-looking statements regarding the Company's business & the therapeutic & commercial potential of its technologies & products in development. Any statement describing the Company's goals, expectations, intentions or beliefs is a forward-looking statement & should be considered an at-risk statement. Such statements are subject to certain risks & uncertainties, particularly those risks or uncertainties inherent in the process of developing technology & in the process of discovering, developing & commercializing drugs that can be proven to be safe & effective for use as human therapeutics, & in the endeavor of building a business around such products & services. Actual results could differ materially from those discussed in this presentation. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the Antisense Therapeutics Limited Annual Report for the year ended 30 June 2021, which is available from the Company or at www.antisense.com.au.

Charmaine Gittleson MD
Board Chair

ANP Corporate Video

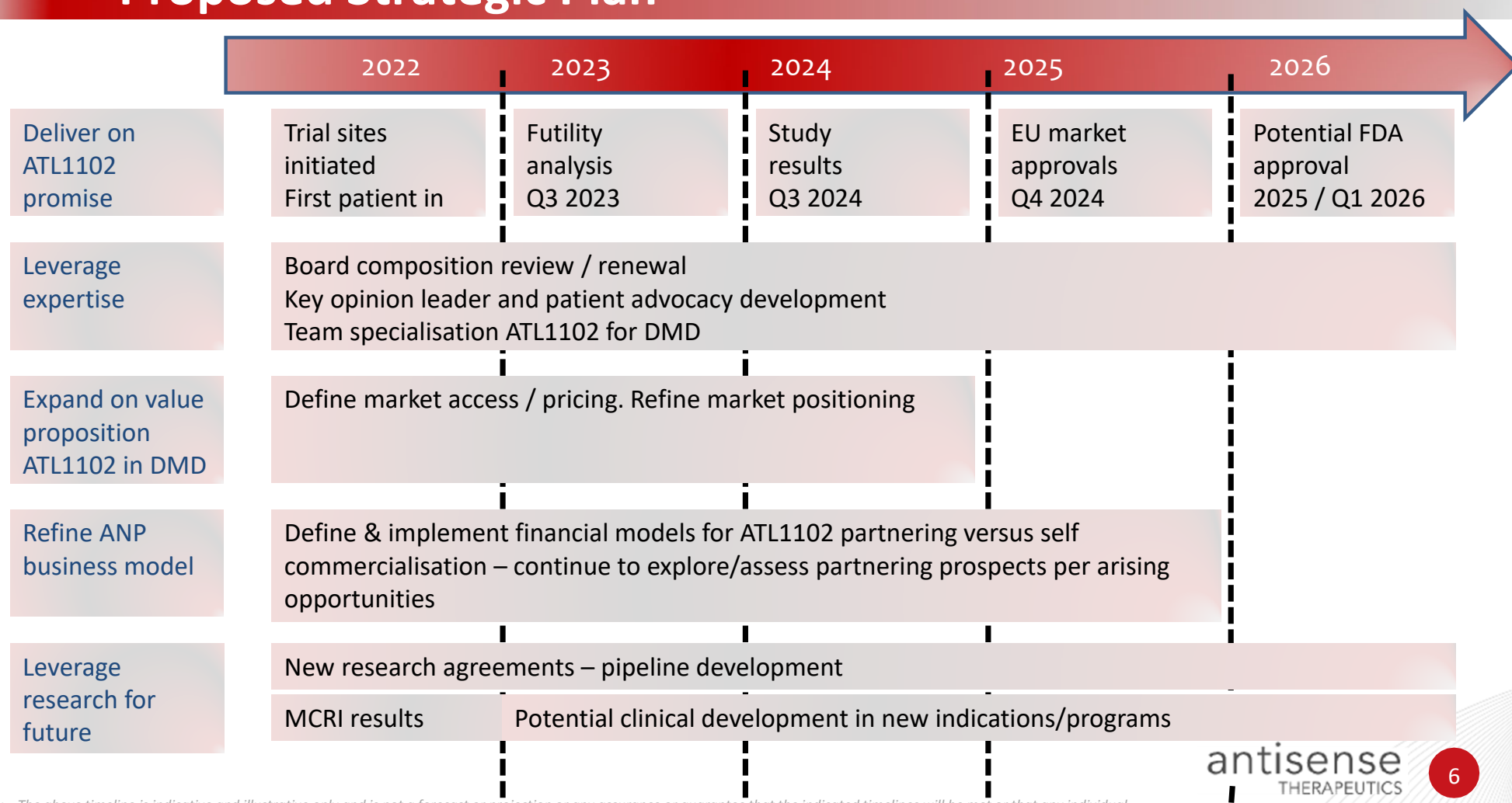


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Key Strategic Factors for ANP Success



Proposed Strategic Plan



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 the Prospectus dated 5 November 2021.

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Mark Diamond
Managing Director and CEO

Antisense Corporate Overview

Share Price Performance

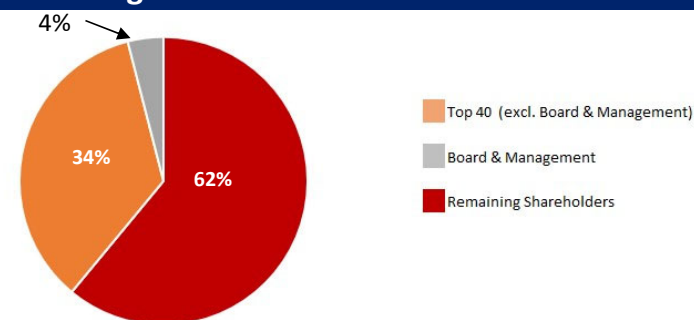


- **Analyst coverage** <https://www.antisense.com.au/broker-other-reports>
 - Shane Storey – Wilsons Equity Research
 - Dennis Hulme – Taylor Collison
 - Marc Sinatra – Corporate Connect
 - Ian Wilkie – Morgans
 - Mark Pachacz - Bioshares

Company Details

- Market Capitalisation @ \$0.185 = \$122M**
 - Ordinary fully paid shares on issue = 658M**
 - Cash Balance as at 30 September 2021 = \$4.7M*
 - Largest shareholder – **Platinum Asset Management (5.23%)**
- * On 5th November the Company announced it had raised **\$20M** in a private placement and an Entitlement Offer to shareholders to raise a further \$16.8M (if fully subscribed)
- ** Includes the Placement shares

Current Register



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Lead Program: ATL1102 in DMD - Profile Summary

ATL1102 clear differentiation in DMD space positions it 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 EU

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**) and Medicines and Healthcare products Regulatory Agency (MHRA) in the UK.
- Professor Thomas Voit MD (Director of NIHR GOSH UCL Biomedical Research Centre, UK) will be the Coordinating Principal Investigator of the trial
- ANP has appointed globally renowned Clinical Research Organisation (CRO) Parexel to conduct and manage the Phase IIb/III study
- Parexel is finalising site evaluations via site inspections to select the sites (>30) in the United Kingdom, Netherlands, Germany, Italy, France, Belgium, Spain, Bulgaria and Turkey.
- Clinical trial agreements to be executed with all the trial sites and separate trial applications made to national competent authorities of all participating countries
- ATL1102 clinical supplies for the trial are also being prepared for shipment to Europe for packaging and labelling into study medication kits.
- Following above activities patient recruitment would be expected to commence in mid-2022



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EU Clinical Trial Process ATL1102



Sponsor - ANP

Develops protocol and clinical development plan

Obtains National Competent Authority / country and EMA advice on protocol

Requests Input and approval Paediatric Development Committee (PDCO) of EMA and MHRA



Regulator

Trial protocol

PDCO/EMA
Paediatric Investigational Plan

1

2

DEC '21



Contract Organisations

Conducts country and site feasibility & qualifies sites

Sets up clinical trial documents and data bases

Imports and releases study drug

3a

Clinical trial application (CTA) package

Site ethics submission package

Hospital legal contracts

3b

Clinical Trial approval (final protocol)

Site ethics approval (final protocol)

Site contracts completed

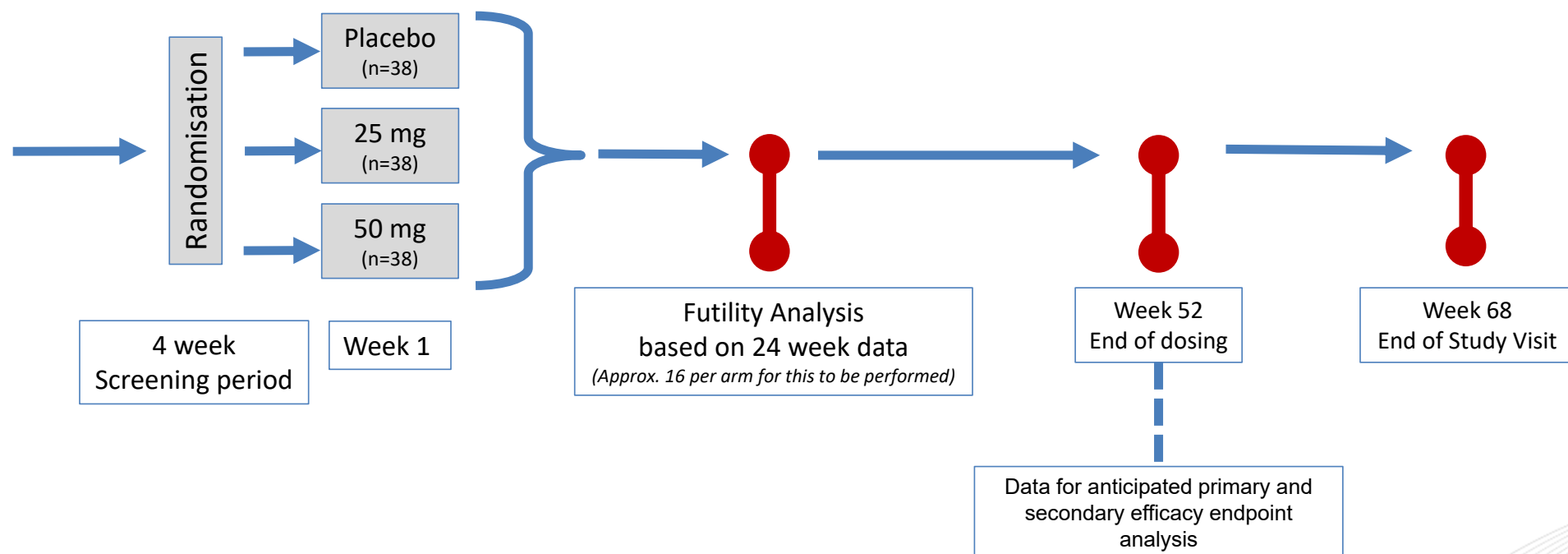
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MID '22

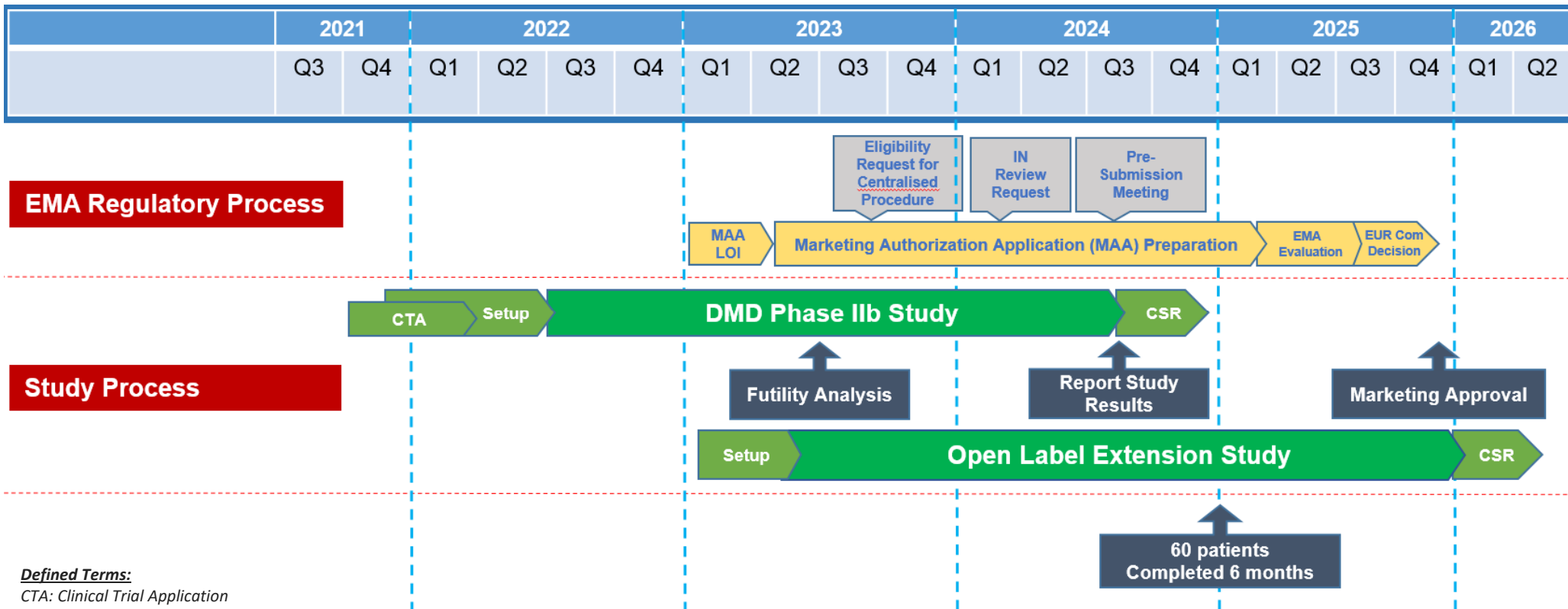
Site initiation and recruitment begins

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 with an open label extension phase.



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

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Clinical Development US

US Regulatory Plans

- Protocol synopsis submitted to the FDA for a nine-month chronic monkey toxicology study to support the dosing of patients with ATL1102 beyond six months in US for DMD or any other clinical application of ATL1102 - expecting feedback in 1Q'22.
- Given the apparent high-level alignment between EMA and FDA on Phase IIb/III study requirements, opportunity for ANP to engage with the FDA to streamline the regulatory processes
 - ANP considers that it has potential optionality in its actions with FDA including to take the EU Phase IIb/III data to the FDA to be assessed as supportive data for a future marketing application or should the data warrant it, possibly an approval of ATL1102 for DMD without further trials
 - FDA interactions to explore the optionality highlighted above are to continue in parallel with the conduct of Phase IIb/III pivotal trial in Europe. The timing of the initiation of the nine-month toxicology study will be dependent on these continued interactions with the agency.
- Should the Phase IIb/III pivotal trial in Europe be successful, ANP believes it could be in a position to receive a rare pediatric disease priority review voucher (PRV) if it obtains FDA approval for ATL1102 in the DMD indication (as the drug's first approval) before September 30, 2026. The Company may choose to sell its PRV. From 2017 - 2021, sales of PRVs ranged between US\$80 - \$150 million.

FDA Interactions ATL1102



Sponsor - ANP

Protocol and clinical development plan

Nine month toxicology design submitted to FDA for review



Regulator - FDA

IND

Six month study
25 mg ATL1102
(partial hold)

Rare Paediatric Disease
Designation
Orphan Drug Designation

Type C meeting

DEC '21

Nine month non-human primate toxicology study
design feedback expected 2022

Discuss protocol and development plan 2022
(12 month study, PUL2.0, 25 mg & 50mg ATL1102)

Optionality for ATL1102

Lift partial clinical hold
Fast track application permissible

Introduces opportunity to seek
an aligned final clinical protocol
and clinical development plan

Introduce potential for
submitting EU data to FDA as
pivotal or supportive data

ATL1102 New Indications

- Focus on advancing ATL1102 for DMD towards commercialisation whilst expanding the clinical application of ATL1102 beyond DMD
 - capitalising on the investment to date and the extensive data package generated on this asset
 - to deepen the product pipeline to add further value for shareholders and to diversify possible indication risk
- Several inflammatory muscle disease indications (like DMD) have been identified as initial focus
 - to leverage established core competencies (rare disease experience, scientific partnerships and collaborations e.g. MCRI, KoL's etc)
 - selected based on analysis that the diseases may benefit from CD49d cell modulation for better treatment outcomes
 - commercially attractive space (like DMD) with limited competition, premium pricing, and orphan drug development incentives
 - opportunity to generate valuable IP from animal studies to underpin development/commercialisation (including supporting future orphan drug designation applications)
 - potential for ANP to move rapidly into the clinic based on positive animal data or out-license

ATL1102 New Indications *(continued..)*

- Collaboration with Murdoch Children's Research Institute (MCRI) the largest child health research institute in Australia
 - Study 1 - *mdx* model in combination with other DMD agents including the dystrophin restoration drugs
 - Study 2 - another animal model of muscle disease
 - ✓ New batches of antisense drug to mouse CD49d have been manufactured
 - ✓ New batch of Dystrophin restoration drugs has been manufactured
 - ✓ Ethics Committee (EC) approval received and animals ordered by the MCRI
 - Study to start in Q1'22 with the first data due Q3'22.
 - ATL1102 to be assessed by studying patient blood samples taken from children afflicted by a range of muscle diseases
 - RCH clinic was closed to research due to Covid-19 restrictions and is expected to open in Q1'22
- Proteomics analysis of retained plasma samples from ATL1102 Phase II DMD trial undertaken to identify proteins affected to provide further insight into the drug's mode of action and biological activity
 - ✓ Statistically significant modulation in two DMD disease modifier proteins supporting ATL1102 in ambulant DMD and fibrotic conditions - Data presented at 2021 World Muscle Society conference
 - Additional proteomics data generated from the DMD study to feature in an abstract to be submitted to an International Conference with potential for presentation in 1H'22

Capital Management

- Placement monies received \$20 million
 - ANP is funded into 2H'23 calendar year including costs associated with setup of the Phase IIb/III DMD trial in Europe
- Entitlement offer to raise \$16.8 million, closing 17 December 2021
 - Use of funds: progressing Phase IIb/III DMD trial in Europe to Futility Analysis
 - This funding would be required by mid 2022 prior to trial commencement
- Ongoing assessment of capital requirements – Company has track record of judiciously applying funds to deliver on key value creation catalysts

Balance of funds not received via Entitlement Offer can be sourced via one or more below alternatives:

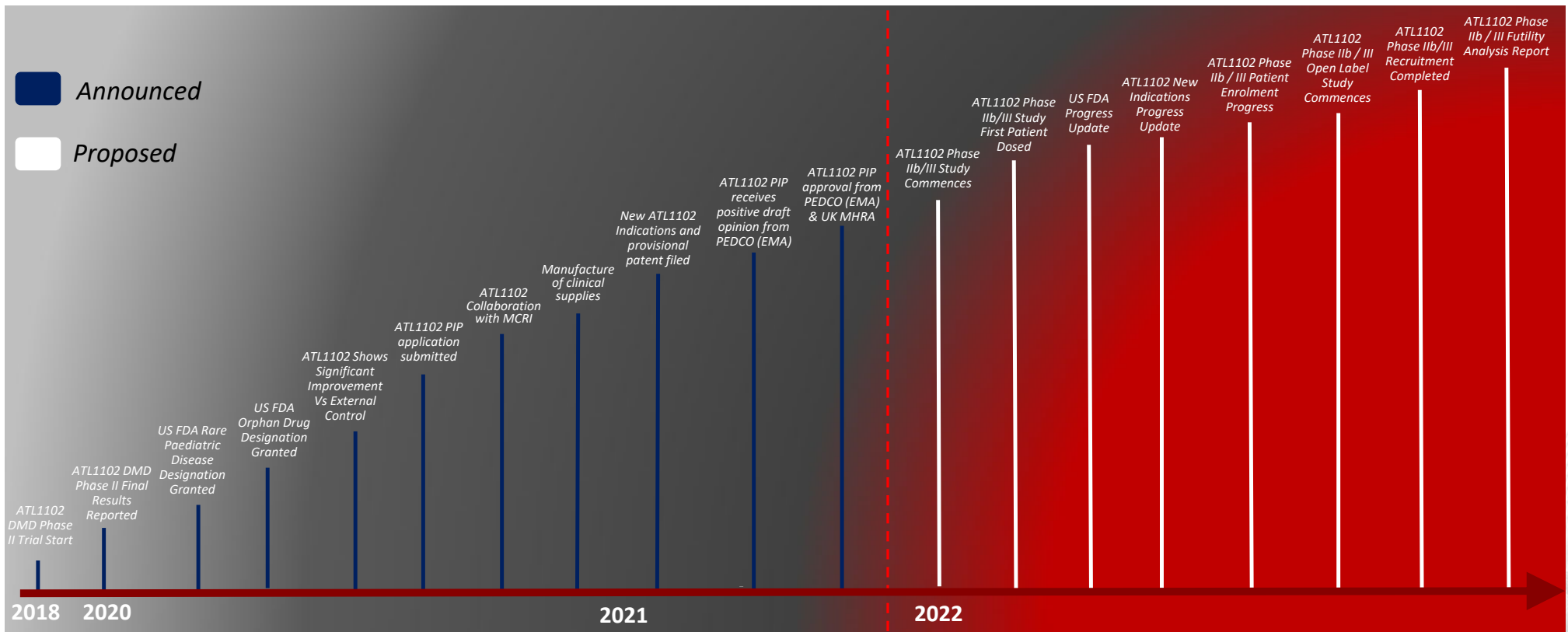
- Placement of the of the Entitlement Offer shortfall within three months following Closing Date
- Debt funding via a loan or a convertible instrument
- Potential equity via partnering / collaboration initiatives

Commercialisation considerations

- Company is focused on preparations for EU Phase IIb/III trial initiation
- Trial with its OLE phase is set up as a registration (approval) study
- Company is gearing up to potentially take ATL1102 all the way to through approval in the Europe and possibly to market (e.g. Sarepta in the US)
 - Major value creation potential (transition from R&D cash burner valued on discounted blue sky sales potential to cash positive Pharma Co valued on PE)
 - EU 5 major markets – approx. 180 DMD treatment centers with 750 HCP's that allows for very focused sales and marketing that is manageable for a smaller Co
 - Premium pricing in the space provides very attractive gross margins further supporting the vertical integration concept
- ANP is incentivized under the Ionis agreement to take ATL1102 to market
 - ANP retain greater amount of commercial proceeds vs partnering with single digit royalty on sales payable to Ionis if ANP commercialize
- ANP may consider partnering on a geographical and/or indications basis
- Board will look to advance in the best interest of shareholders

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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