

# Positioning Antisense for Success in 2024

Presentation to Annual General  
Meeting of Shareholders

Dr James Garner  
Chief Executive Officer

Melbourne, Australia  
15 November 2023

# Forward-Looking Statements

This presentation contains **forward-looking statements** within the meaning of the safe-harbor provisions of the Private Securities Litigation Reform Act of 1995. These statements do not relate strictly to historical or current facts and may be accompanied by words such as ‘could,’ ‘would,’ ‘may,’ ‘potentially,’ ‘suggest,’ ‘believes,’ ‘expects,’ ‘should,’ ‘intends,’ ‘plans,’ ‘forecasts,’ and similar words or expressions.

Such statements involve substantial risks and uncertainties, not all of which may be known at the time. All statements contained in this presentation, other than statements of historical fact, including without limitation statements regarding our strategy, research and development plans, collaborations, future operations, future financial position, future revenues, projected costs, pricing, prospects, plans, and objectives of management, are forward-looking statements. Not all forward-looking statements in this presentation are explicitly identified as such.

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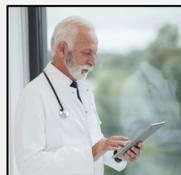
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# Agenda

- Update on ATL1102 Development Program
- Developments in Duchenne Muscular Dystrophy Landscape
- Biotech Market Environment
- The Year Ahead for Antisense Therapeutics

# Good progress is being made across all aspects of ATL1102 development, with current work focused on five key areas



## Deliver Phase IIb Clinical Trial in DMD

*Provide high-quality data to enable partnering, excite new investors, and engage with regulators*

- All four countries open to recruitment
- 10 patients randomised by 31 Oct; more in screening
- No safety concerns identified by DSMB; good engagement from patients and investigators

### Next Milestones

Complete recruitment: 1Q CY2024  
Data: 2H CY2024



## Complete 9-Month Toxicology Study

*Remove impediments to conducting clinical trials and seeking marketing approval in US, thereby de-risking ATL1102*

- Nine-month dosing phase of study due to complete by end of CY2023

### Next Milestones

Complete 'in life' 2H CY2023  
Data: 2H CY2024



## Optimise for Future Regulatory Approval

*Identify potential regulatory and manufacturing needs in key markets and advance plans to optimise ATL1102 program*

- New team members recruited in FY2023 with extensive international experience in drug development
- International CRO engaged to review dossier

### Next Milestones

FDA engagement CY2024



## Envisage Potential Expansion

*Evaluate opportunities to expand use of ATL1102 within DMD, in other forms of muscular dystrophy, and in other diseases*

- Collaborations have generated new data in DMD (in combination with existing therapies) and in LGMDR2

### Next Milestones

Publication / presentation of combination data and LGMDR2 data  
CY2024



## Publish Our Data

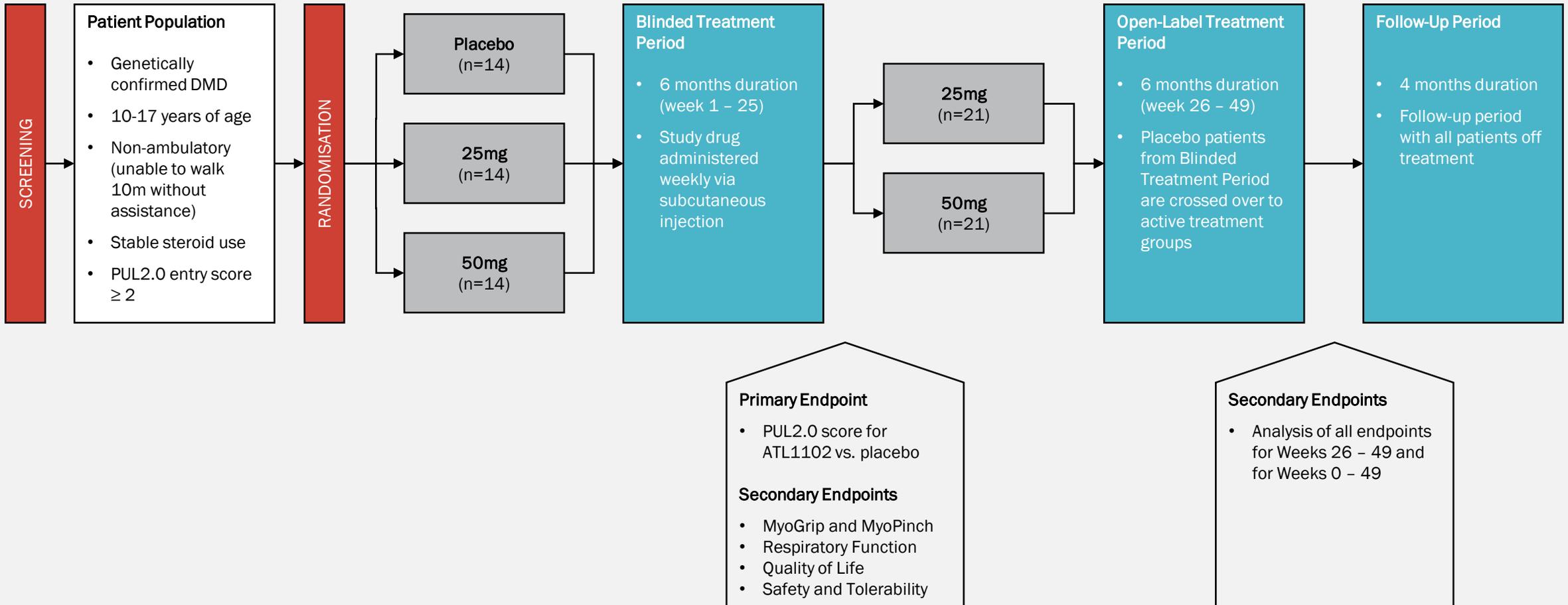
*Share ATL1102's impressive dataset with the world via peer-reviewed journals and scientific conferences*

- Phase IIa publication submitted for publication
- Key scientific conferences targeted for CY2024

### Next Milestones

Publication / presentation of Phase IIa data  
1Q CY2024

# The ongoing, double-blind phase IIb clinical study has been designed to provide definitive evidence of efficacy for ATL1102 in non-ambulant boys with DMD



# The previous phase IIa study of ATL1102 in DMD provided highly encouraging evidence of efficacy in this challenging disease

Key Study Parameters
<b>Population</b>
Non-ambulant boys with confirmed Duchenne muscular dystrophy, aged 10-18
<b>Sample Size</b>
n = 9
<b>Intervention</b>
ATL1102, 25mg weekly via sc injection for 24 weeks
<b>Primary Endpoint</b>
Safety and tolerability
<b>Secondary Endpoints</b>
Lymphocyte count Upper limb function Upper limb strength Forearm muscle MRI
<b>Location and Timing</b>
Melbourne, Australia 2018 - 2020

Study Results (Efficacy) [at 6 months]			
Endpoint	Description	ATL1102 Result	Historical Comparator
 <b>PUL2.0</b>	Performance of Upper Limb (PUL2.0) assesses the function of upper body muscles in 3 dimensions	<b>↑ 0.9</b> (-1.33 - 3.11)	<b>↓ 2.0</b> (-2.95 - -1.05)
 <b>MyoGrip</b> (dominant hand)	MyoGrip assesses the clamping force of the fingers	<b>↑ 0.2 kg</b> (-0.25 - 0.67)	<b>↓ 0.5 kg</b> (-1.01 - 0.00)
 <b>MyoPinch</b> (dominant hand)	MyoPinch assesses the pinch strength between thumb and forefinger	<b>→ 0.0 kg</b> (-0.18 - 0.19)	<b>↓ 0.4</b> (-0.53 - -0.22)
 <b>MoviPlate</b> (dominant hand)	MoviPlate assesses the fatigability of forearm muscles but is of uncertain significance in DMD	<b>↑ 1.9</b> (-6.08 - 9.85)	<b>↑ 4.7</b> (2.01 - 7.40)
 <b>MRI - total lean muscle area</b>	Magnetic Resonance Imaging (MRI) is used to assess the amount of fat and lean muscle mass in the forearm	<b>↑ 13.9 mm<sup>2</sup></b> (-72.6 - 100.4)	<b>↓ 32.1 mm<sup>2</sup></b> (-102.6 - 38.1)
 <b>Lymphocyte Counts</b>	Lymphocyte counts measure the ability of ATL1102 to modulate the immune system and reduce inflammation	<b>↓ 0.28 x 10<sup>9</sup> / L</b> (-1.10 - 0.55)	<b>↑ 0.47 x 10<sup>9</sup> / L</b>

Study Results (Safety)
Side effects of ATL1102 limited to non-serious injection site reactions, with no patients requiring withdrawal from treatment

Source: [IR Woodcock et al. \(2022\) medRxiv 2022.01.16.22269029](#); [V Ricotti et al. \(2016\) PLoS ONE 11\(9\): e0162542](#); [G Tachas et al. \(2020\) Neuromuscul. Disord. 30\(S1\):S129-130](#)

Note: Comparison between studies is never perfectly like-for-like and functional endpoints would typically require further confirmation in a randomised, placebo-controlled trial

# Phase IIb study start-up has been moderately slower than expected, but recruitment is accelerating and Antisense is deploying mitigations

Clinical trial recruitment is becoming more challenging across the industry

	2008 - 2011	2016 - 2019
Median recruitment duration	13 months	18 months
Median recruitment rate	0.6 patients per site per month	0.4 patients per site per month
Median number of sites	51 sites	64 sites

Note: metrics based on analysis of 3,652 industry-sponsored phase III studies from 2008-2019 across all indications

Antisense has moved quickly to support recruitment to the ATL1102 study

1. Simplification of screening procedures in protocol to reduce drop-out rate
2. Planned opening of additional sites in UK and Turkey
3. Engagement meetings with investigators and site study teams (virtual and in person)
4. Outreach to muscular dystrophy patient groups and not-for-profits to stimulate recruitment
5. Consideration of an additional country

# Agenda

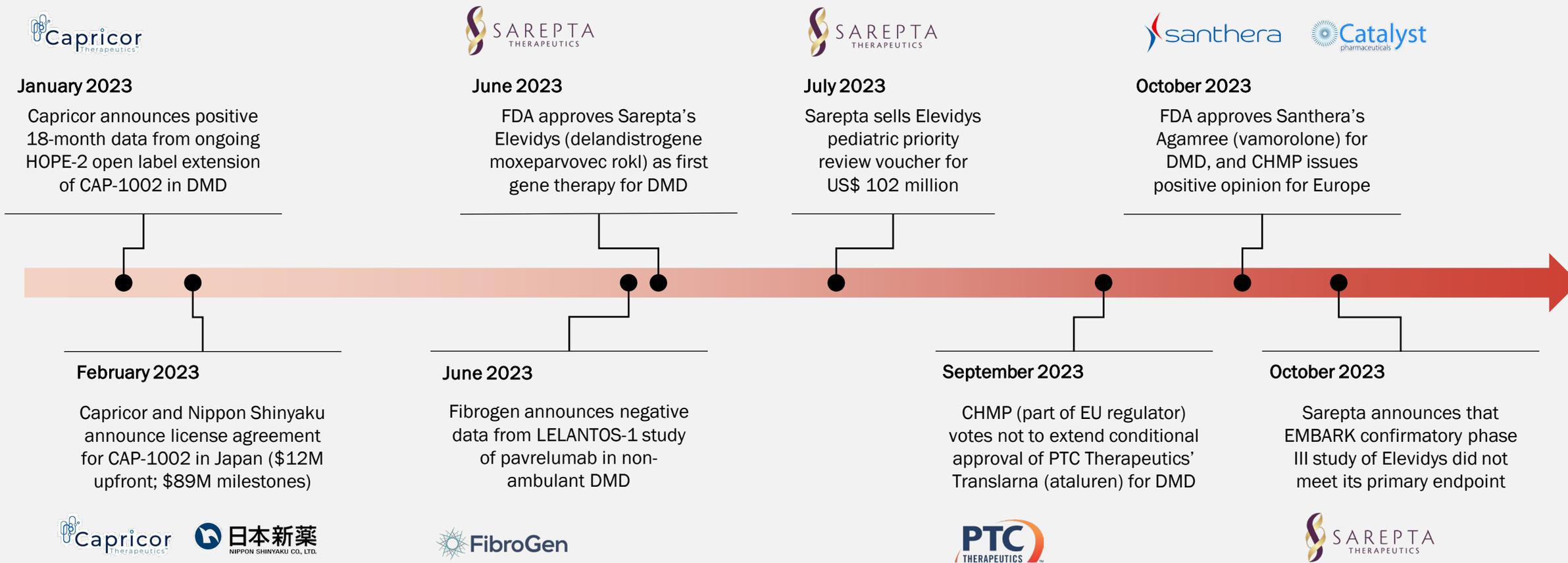
- Update on ATL1102 Development Program

- Developments in Duchenne Muscular Dystrophy Landscape

- Biotech Market Environment

- The Year Ahead for Antisense Therapeutics

# 2023 has been a busy year for Duchenne's treatment, illustrating both the opportunities and challenges of this disease area



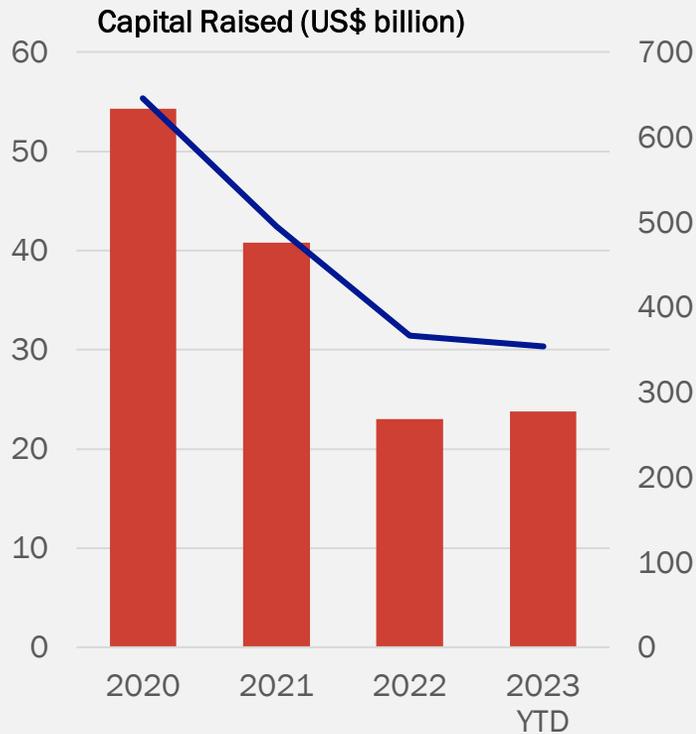
Source: Company press releases and SEC filings, Antisense analysis

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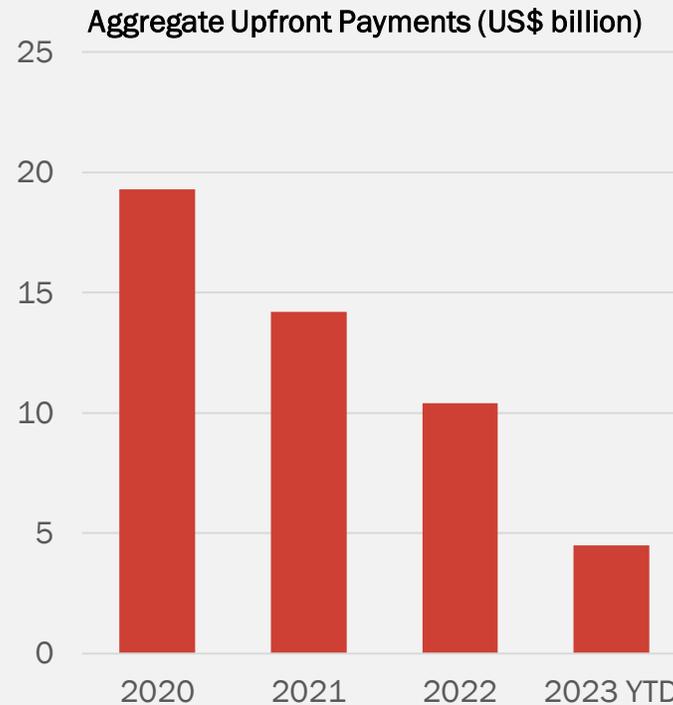
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# The last 2-3 years have been an immensely challenging period for biotech companies, but there are early signs of potential recovery in 2024

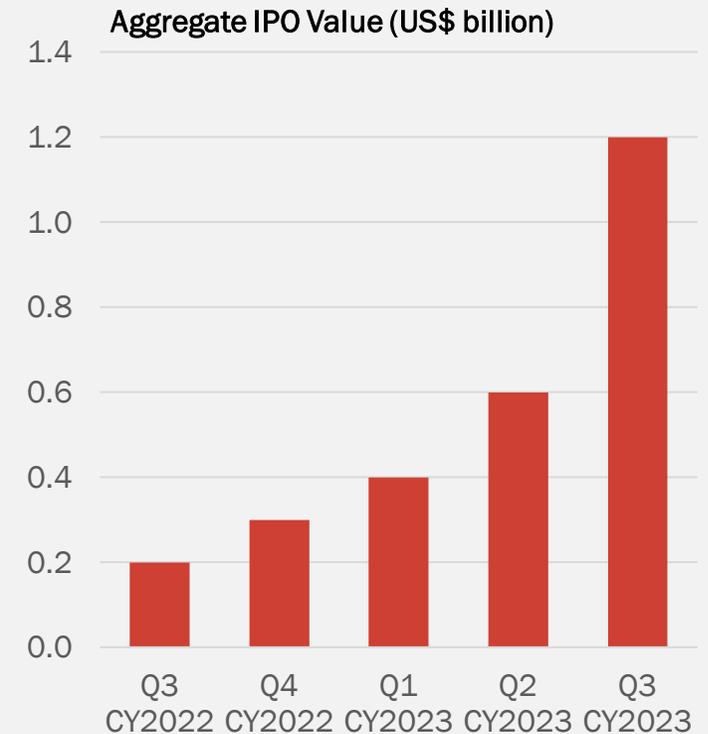
Access to finance has been very challenging...



...and partnering deal flow has been at its lowest point since 2018...

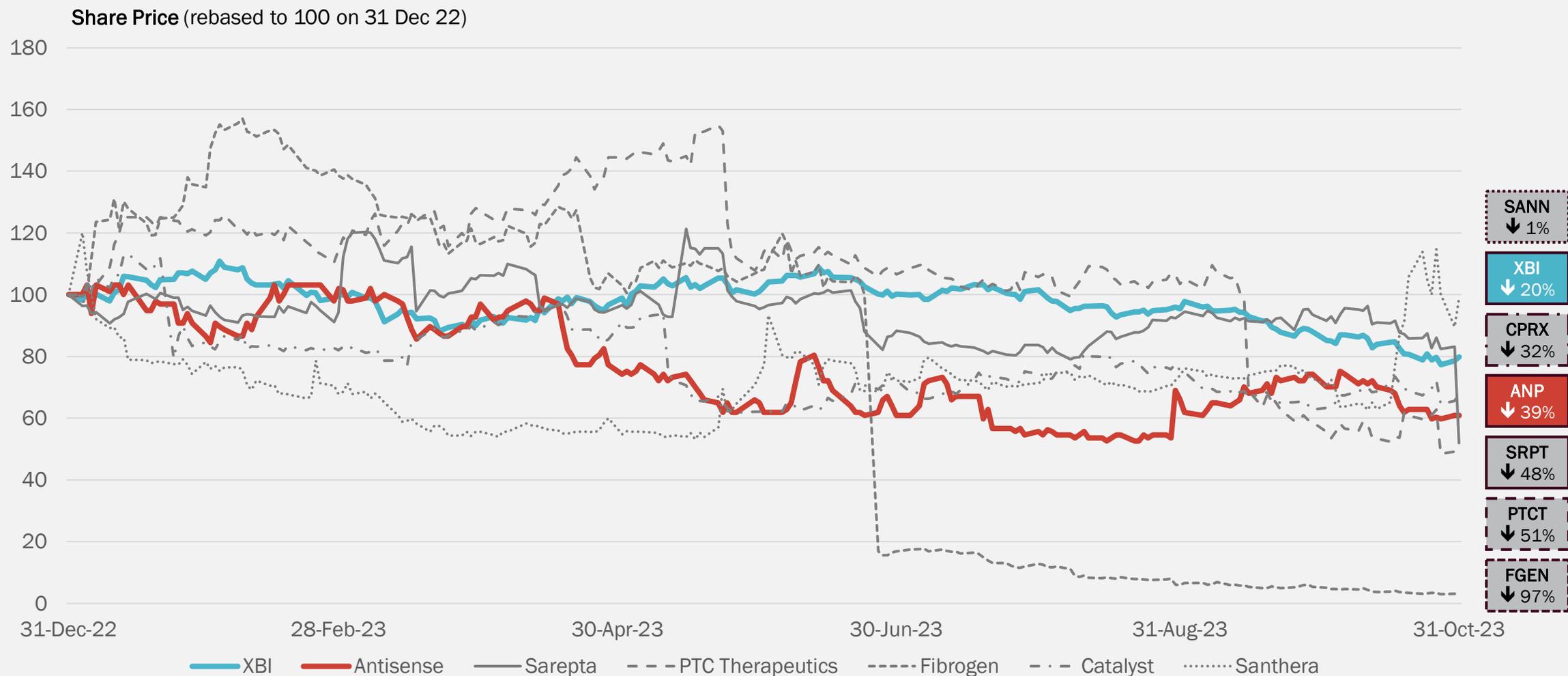


...but IPO activity is returning, signalling a potential turnaround



Source: DealForma; JP Morgan, Q3 2023 Biopharma Licensing and Venture Report

# Some companies focused on Duchenne's have been impacted by negative readouts, but investors remain positive about the future of this disease area



# Antisense's financing round in 3Q CY2023 demonstrates investor confidence and has positioned the company to execute the phase IIb study

Date	Issuer	Mkt Cap (\$M)	Raise (\$M)	Offer Price (\$)	Type	Discount (%)	Options	Performance (%)	
19 May 2023	CPH	Creso Pharma Limited	18.7	2.5	0.012	Placement	23.8%	1:1 at \$0.08	-75.0%
19 May 2023	IPD	Impedimed Limited	333.0	20.0	0.130	Placement + SPP	16.1%	n/a	3.9%
23 May 2023	RSH	Respiri Limited	31.3	6.5	0.034	Convertible Note + SPP	10.5%	1:2 at \$0.065	-14.7%
2 Jun 2023	IMM	Immutep Limited	319.2	80.0	0.260	Placement + Entitlements	13.1%	n/a	9.6%
23 Jun 2023	EBR	EBR Systems Limited	238.6	30.0	0.910	Placement	7.1%	n/a	-14.3%
11 Jul 2023	AT1	Atomo Diagnostics Limited	29.7	1.3	0.036	Placement + SPP	30.8%	n/a	-38.9%
<b>18 Jul 2023</b>	<b>ANP</b>	<b>Antisense Therapeutics Limited</b>	<b>43.5</b>	<b>8.4</b>	<b>0.050</b>	<b>Placement + SPP</b>	<b>23.1%</b>	<b>n/a</b>	<b>22.0%</b>
24 Jul 2023	ONE	Oneview Healthcare Limited	128.0	20.0	0.180	Placement + SPP	25.0%	n/a	30.6%
16 Aug 2023	IMU	Imugene Limited	603.8	65.0	0.084	Placement + SPP	10.6%	1:1 at \$0.118	25.0%
24 Aug 2023	OPT	Opthea Limited	280.3	80.0	0.460	Placement + Entitlements	23.3%	1:2 at \$0.80	-28.3%
29 Aug 2023	NXS	Next Science Limited	173.6	18.9	0.420	Placement	35.4%	n/a	-29.8%
7 Sep 2023	RCE	Reece Pharmaceuticals	115.8	6.0	0.650	Placement + Entitlements	32.3%	n/a	-29.2%
19 Oct 2023	MAP	Microba Limited	111.3	20.0	0.230	Placement + Entitlements	28.1%	n/a	0.0%
26 Oct 2023	AVR	Anteris Technologies Limited	287.2	40.0	20.0	Placement	3.1%	n/a	0.0%
30 Oct 2023	PAR	Paradigm Biopharmaceuticals Limited	173.3	30.0	0.430	Placement + Entitlements	30.1%	3:4 at \$0.65	-10.5%
<b>Median (companies &lt;\$200M in market cap)</b>						<b>26.6%</b>		<b>-12.6%</b>	

Source: Morgans Corporate Limited

# Agenda

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# The company has been working hard to raise its profile in the investor and clinician communities, with intensive plans for CY2024

**Antisense Therapeutics**  
**FY24: The biggest year of its life**

• ANP has reported its FY23 results. No surprises here given quarterly updates. Cash balance is key, which following successful institutional placement and SPP was recently received. Fully-funded to PhD outcome.

• Focus remains solely on near-term catalysts including recruiting milestones, toxicology study, and PhD top-line results all due to reach out within FY24.

• No changes to our valuation which remains at AS\$2.25 ps.

• We view ANP as having one of the best risk/return profiles in the space, with clear near-term catalysts, strong board and management team, and scientific support for success.

**Event**

• R&D expenses of AS\$10.2m (+124% in the pop). Increase in spend was in line with expectations with PhDs commencement, pre-clinical studies, and toxicology works. R&D rebate -11% to AS\$1.9m was marginally lower than we forecast, with -52% of R&D expenses eligible in FY22 for rebate.

• Net loss increases to AS\$11.4m (+96% in the pop) due to R&D activities, all other expense lines largely unchanged in the pop.

• Following the recent institutional placement and SPP delivered -AS\$11.5m, the company has -AS\$22.2m in cash (AS\$11.0m as at FY23).

**Analysis**

• Minimal additional detail in the result however all timelines appear to remain on track. Key risk here is recruitment rates which the company view as tracking ahead of expectations. We will continue to track progress on this front.

• The one interesting detail in the commentary that caught our eye was around toxicology progress, noting 16 of 40 doses had by 30 June been administered and tolerated. Not surprising considering the safety profile in humans to date (based on shorter dosing regimen) and clearly the detail in bloodwork will be the judge of this, but comforting to know there doesn't appear to be any showstopping adverse events and the animals appear in good order. Give further comfort on positive results. The results are key to unlock the US opportunity. Results due out HCY24.



Antisense Therapeutics - executive interview with James Garner, chief executive

**EDISON**  
 BRILLIANT KNOWLEDGE

**EXECUTIVE INTERVIEW**

James Garner  
 CEO  
 Antisense Therapeutics

**Bio Investor Forum**

1:45 PM - 2:00 PM  
 Antisense Therapeutics Limited

Company Description: Antisense Therapeutics Limited (ASX: ANP; OTC: A1101) is an Australian biotechnology company focused on the development of novel high-value therapies for orphan diseases.

James Garner, Chief Executive Officer, Antisense Therapeutics Limited

Antisense Therapeutics (ASX:ANP) - Webinar Presentation

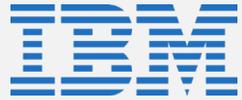
Antisense Therapeutics (ASX: ANP) is a late-clinical stage biotech company, focused on development of novel high-value therapies for orphan diseases

- Lead program is **ATL1302**, an antisense oligonucleotide treatment for **Duchenne muscular dystrophy (DMD)**, a rare disease.
  - Intrathecal double-blind, placebo controlled phase 1b trial ongoing
  - Positive clinical data from pre-clinical phase 1a study
  - Most validated technology with multiple FDA approved therapies in various conditions
- ATL1302 is a late-stage asset with substantial commercial opportunity**
  - 1 Approaching 200,000 DMD patients worldwide
  - Existing therapies priced up to US\$ 200K per treatment year; total market estimated at ~US\$ 40B per annum (~US\$ 80B to US\$ 120B)
  - ATL 1302 potentially applicable to almost all DMD patients, not just those with specific genetic mutations (exon skipping)
  - Potential applications for ATL 1302 in other disease areas
- Antisense using unique **exon skipping** technology
  - Highly-experienced Board and management team
  - Recent institutional institutional financing of US\$ 20M, plus three Fundraise Plan proceeds of \$3.2M, means the company will fund its ongoing operations
  - Lean, virtual operating model

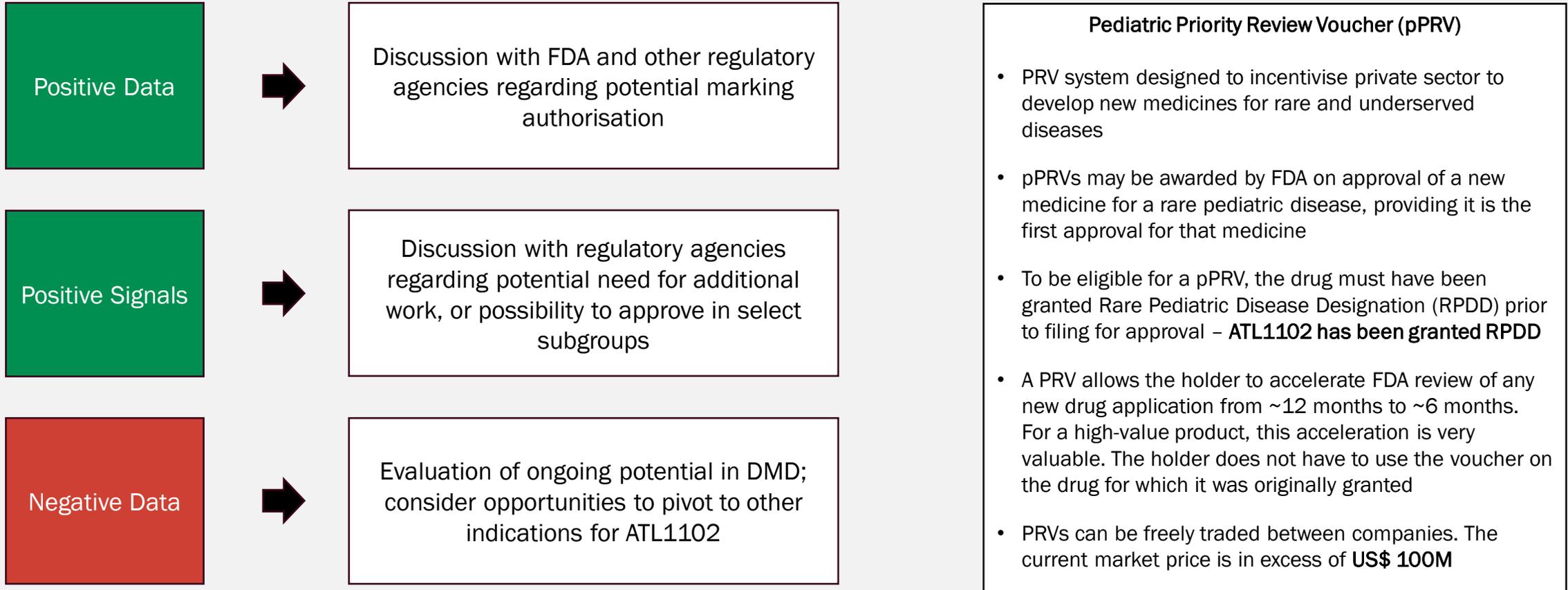
Dr James Garner  
 CEO  
 Antisense Therapeutics (ASX:ANP)

- ### Key Objectives for CY2024
- 1 US and Australian roadshows to raise investor awareness
  - 2 Deeper engagement with analysts and equity research
  - 3 Further retail-focused efforts, including additional 'Open House' presentations
  - 4 Publication of data in peer-reviewed journals, and presentations at key international conferences
  - 5 Rebuild of company website

Many companies rebrand as their business evolves; Antisense will become Percheron Therapeutics, launching a new chapter in the history of the company

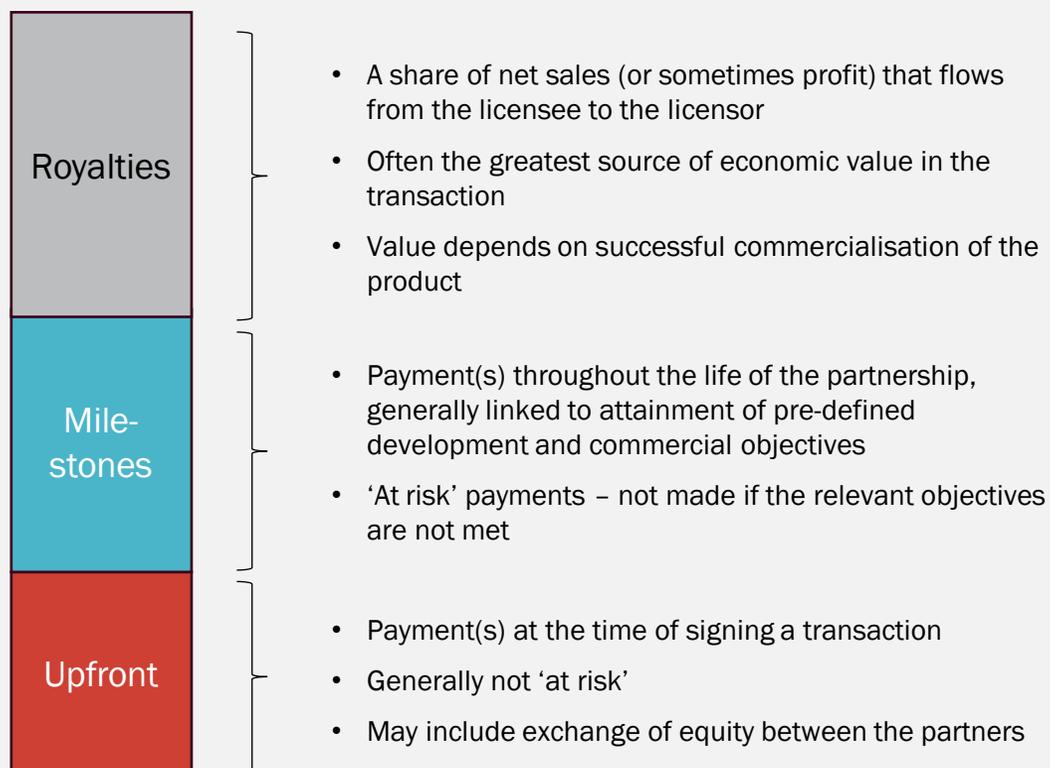


# Six-month data from the ongoing phase IIb trial of ATL1102 will define the likely path to market for the drug



# The company has initiated a broad outreach program to identify and cultivate potential future partners for ATL1102

## Illustrative Composition of Typical Pharma Partnering Transactions



## Benchmarks for Phase II Rare Disease Partnering Transactions (2016 – 2023) (n=47)

	Low	Median	High
Upfront Cash (US\$ M)	1	18	900
Milestones (US\$ M)	3	200	1,700
Royalties	9%	15%	40%

*The ability and commitment of a partner to develop and commercialise the product can be at least as important as the economic terms*

Source: DealForma; Antisense analysis

# Antisense is rich in near-term news flow, with the potential for multiple value-driving catalysts over the next 18 months

CY2023		
Commence recruitment to international phase IIb study of ATL1102 in Duchenne muscular dystrophy	1H CY2023	✓
Initial data from preclinical study in Duchenne muscular dystrophy in combination with ESTs (muscle function)	1H CY2023	✓
Further data from preclinical study in Duchenne muscular dystrophy in combination with ESTs (dystrophin & transcriptomic data)	2H CY2023	✓
Data from preclinical study in limb girdle muscular dystrophy R2 at Murdoch Children's Research Institute	2H CY2023	✓
CY2024		
<i>Full recruitment to international phase IIb study of ATL1102 in Duchenne muscular dystrophy</i>	1H CY2024	
Completion of 'in life' phase of 9-month non-human primate toxicology study	1H CY2024	
Publication in peer-reviewed journal of full data from phase IIa study of ATL1102 in Duchenne muscular dystrophy	1H CY2024	
Initial data from international phase IIb study of ATL1102 in Duchenne muscular dystrophy	2H CY2024	

*italics* = updated guidance



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