

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

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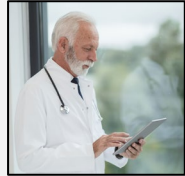
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Agenda

- 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

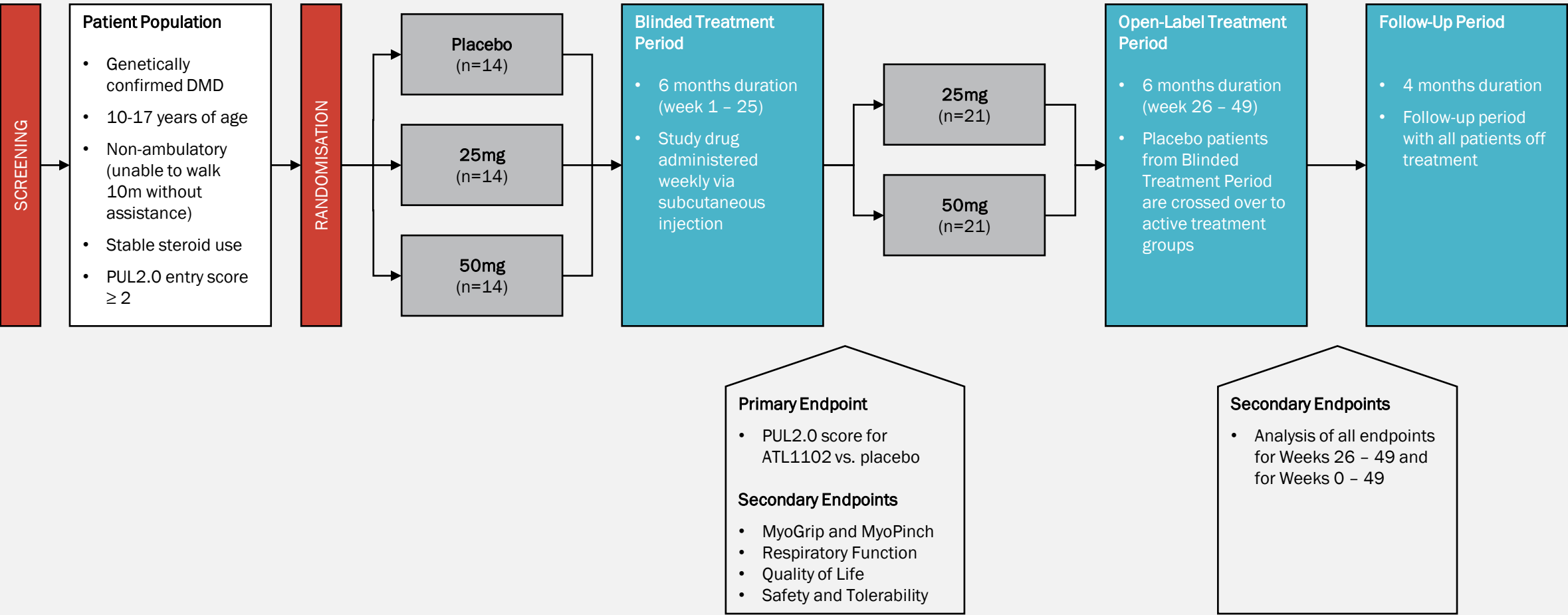
Share ATL1102's impressive dataset with the world via peer-reviewed journals and scientific conferences

- 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		Study Results (Efficacy) [at 6 months]			
Population		Endpoint	Description	ATL1102 Result	Historical Comparator
Non-ambulant boys with confirmed Duchenne muscular dystrophy, aged 10-18		 PUL2.0	Performance of Upper Limb (PUL2.0) assesses the function of upper body muscles in 3 dimensions	 0.9 (-1.33 – 3.11)	 2.0 (-2.95 – -1.05)
Sample Size		 MyoGrip (dominant hand)	MyoGrip assesses the clamping force of the fingers	 0.2 kg (-0.25 – 0.67)	 0.5 kg (-1.01 – 0.00)
Intervention		 MyoPinch (dominant hand)	MyoPinch assesses the pinch strength between thumb and forefinger	 0.0 kg (-0.18 – 0.19)	 0.4 (-0.53 – -0.22)
Primary Endpoint		 MoviPlate (dominant hand)	MoviPlate assesses the fatigability of forearm muscles but is of uncertain significance in DMD	 1.9 (-6.08 – 9.85)	 4.7 (2.01 – 7.40)
Safety and tolerability		 MRI - total lean muscle area	Magnetic Resonance Imaging (MRI) is used to assess the amount of fat and lean muscle mass in the forearm	 13.9 mm ² (-72.6 – 100.4)	 32.1 mm ² (-102.6 – 38.1)
Secondary Endpoints		 Lymphocyte Counts	Lymphocyte counts measure the ability of ATL1102 to modulate the immune system and reduce inflammation	 0.28 x 10 ⁹ / L (-1.10 – 0.55)	 0.47 x10 ⁹ / L
Location and Timing		Study Results (Safety)			
Melbourne, Australia 2018 - 2020		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

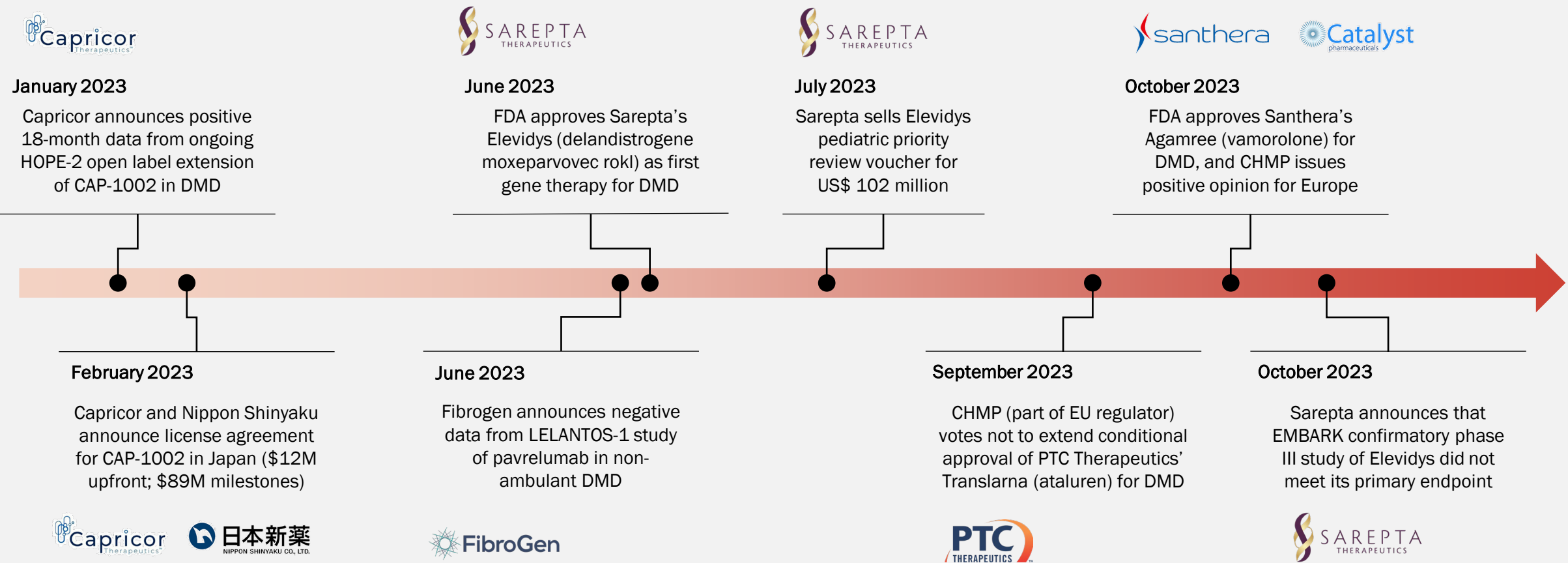
- 1. Simplification of screening procedures in protocol to reduce drop-out rate
- 2. Planned opening of additional sites in UK and Turkey
- 3. Engagement meetings with investigators and site study teams (virtual and in person)
- 4. Outreach to muscular dystrophy patient groups and not-for-profits to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

Source: M Brøgger-Mikkelsen et al. (2022) PLoS ONE 17(7): e0271819

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



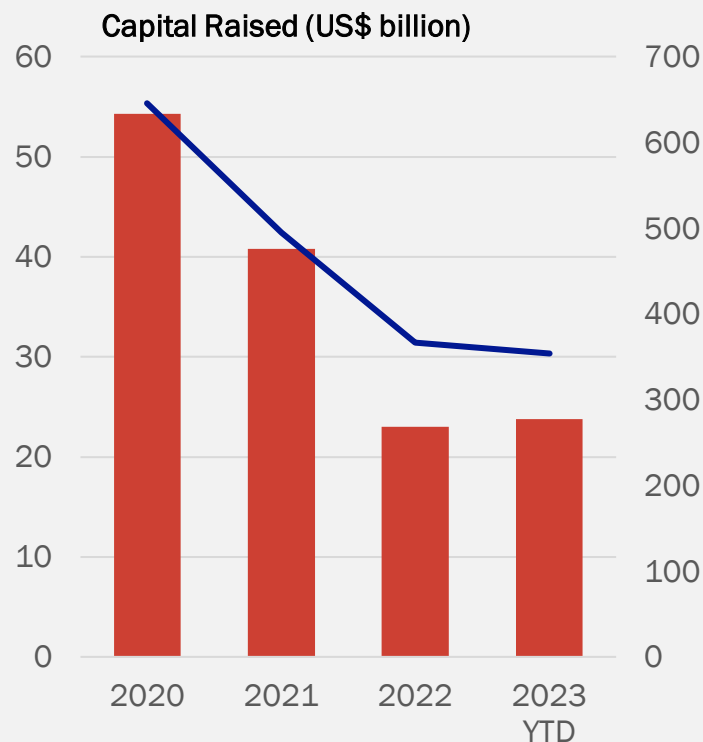
Source: Company press releases and SEC filings, Antisense analysis

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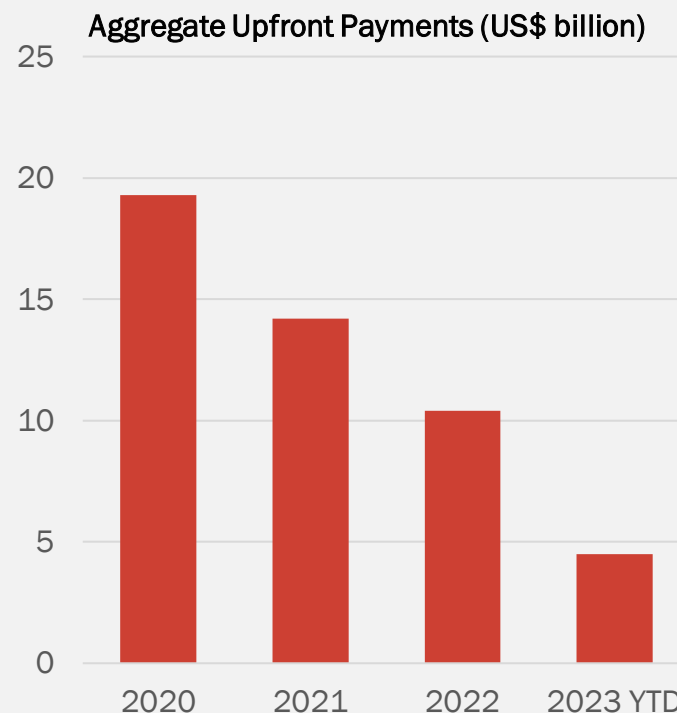
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The last 2-3 years have been an immensely challenging period for biotech companies, but there are early signs of potential recovery in 2024

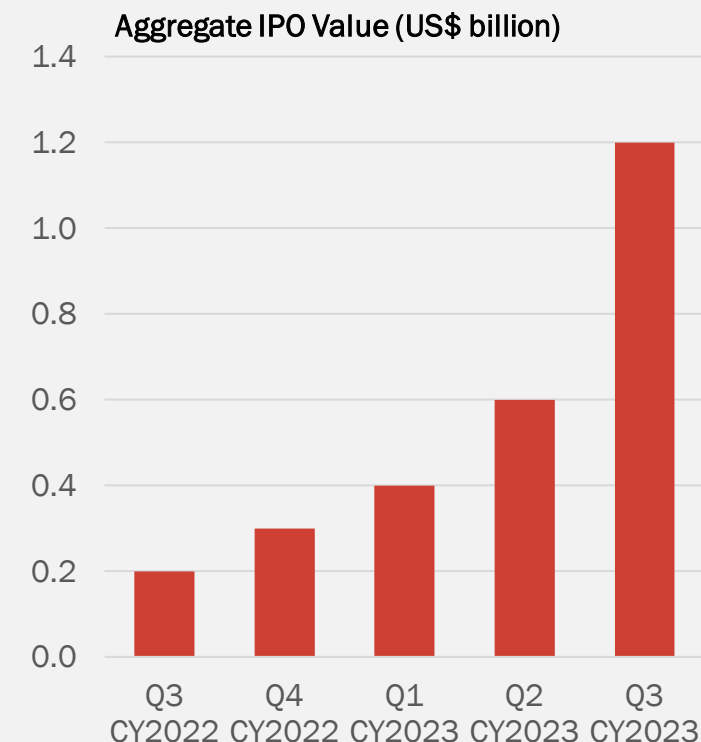
Access to finance has been very challenging...



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

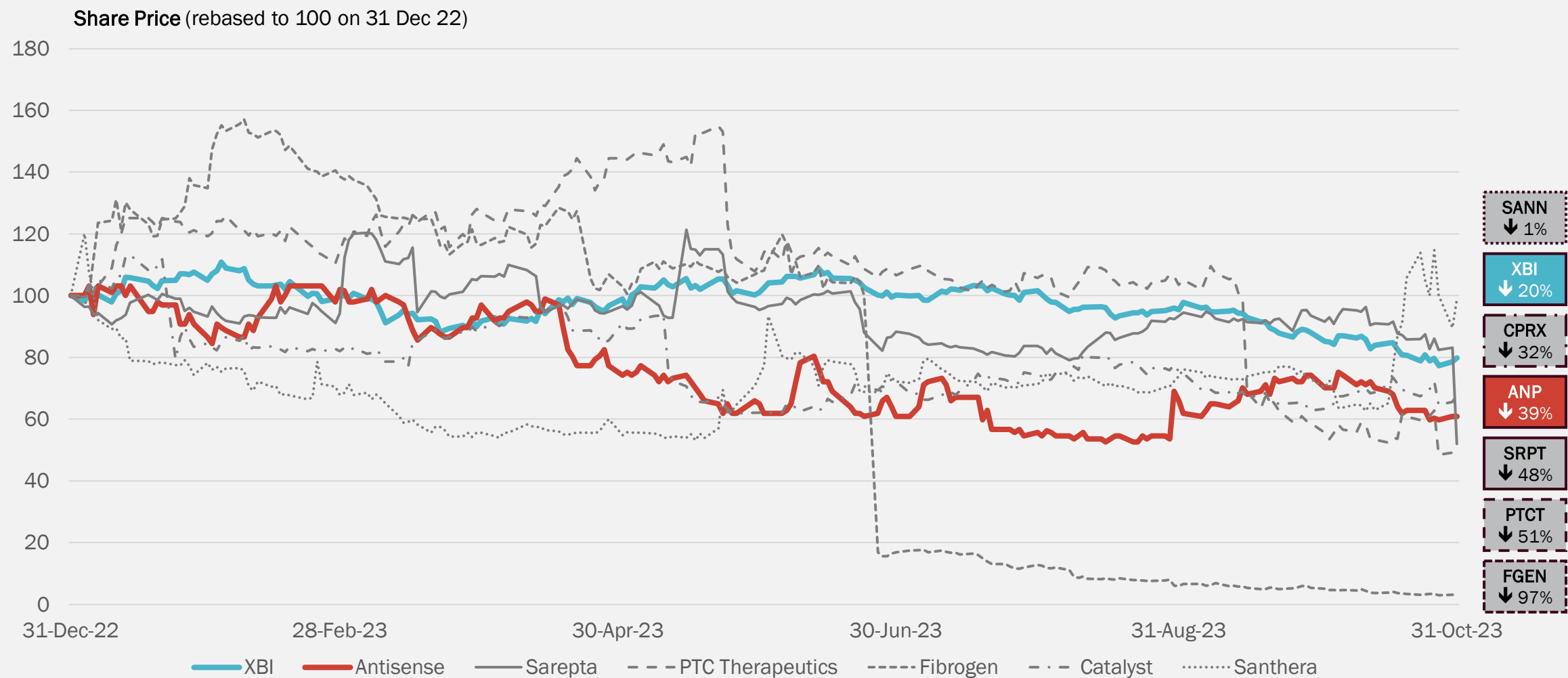


...but IPO activity is returning, signalling a potential turnaround



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

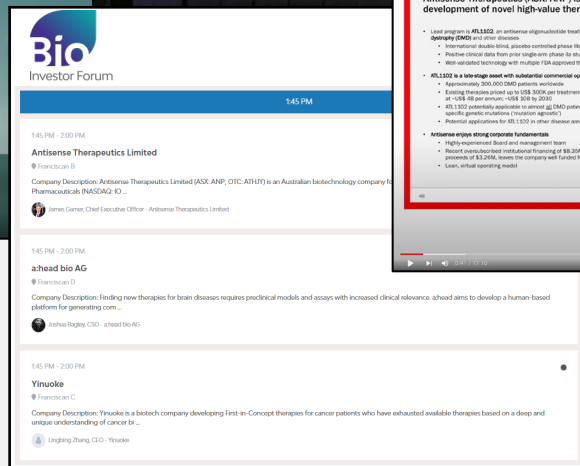
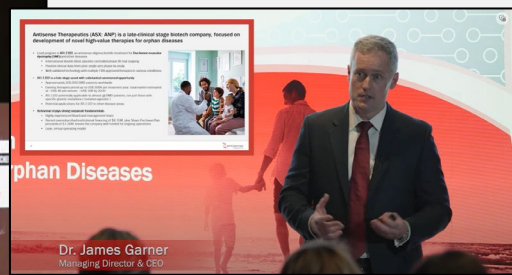
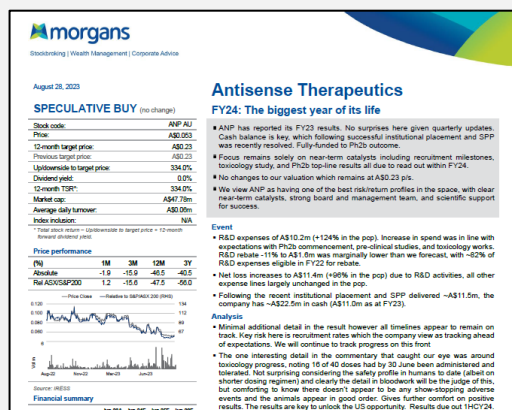
Date	Issuer		Mkt Cap (\$M)	Raise (\$M)	Offer Price (\$)	Type	Discount (%)	Options	Performance (%)
19 May 2023	CPH	Creso Pharma Limited	18.7	2.5	0.012	Placement	23.8%	1:1 at \$0.08	-75.0%
19 May 2023	IPD	Impedimed Limited	333.0	20.0	0.130	Placement + SPP	16.1%	n/a	3.9%
23 May 2023	RSH	Respiri Limited	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%
18 Jul 2023	ANP	Antisense Therapeutics Limited	43.5	8.4	0.050	Placement + SPP	23.1%	n/a	22.0%
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%
Median (companies <\$200M in market cap)							26.6%		-12.6%

Source: Morgans Corporate Limited

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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



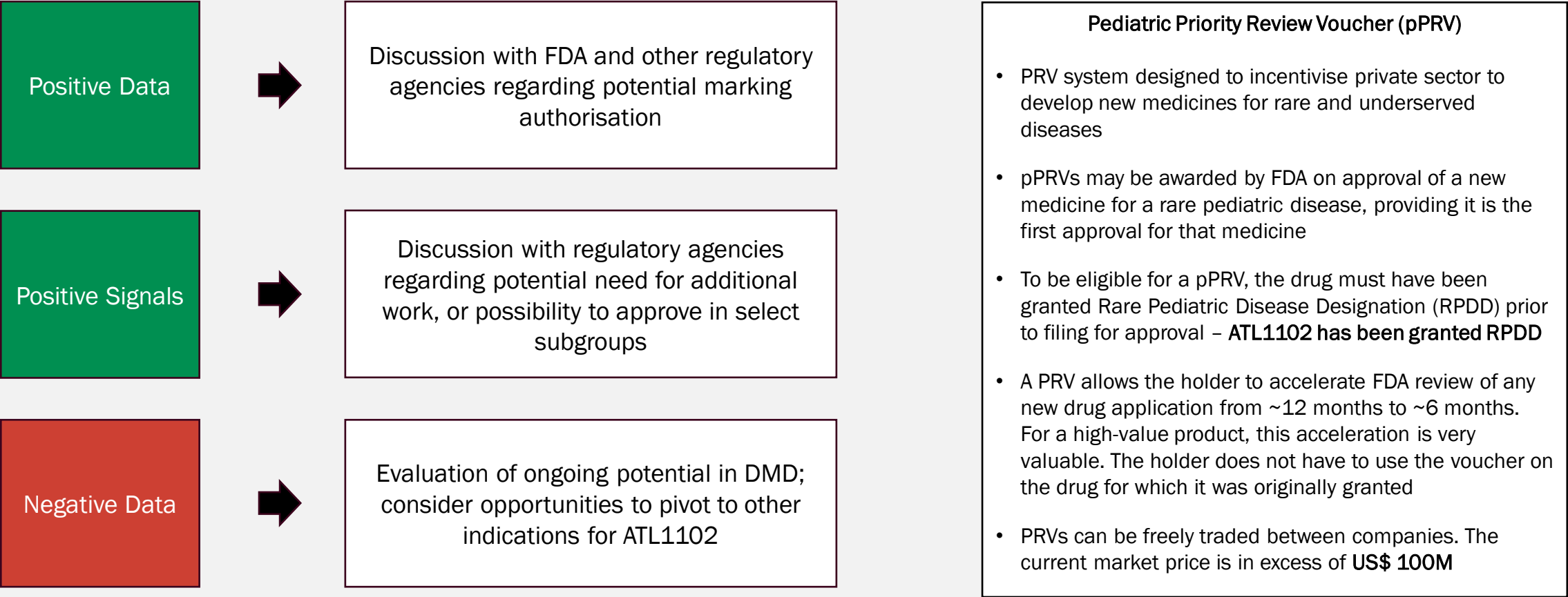
Key Objectives for CY2024

- 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

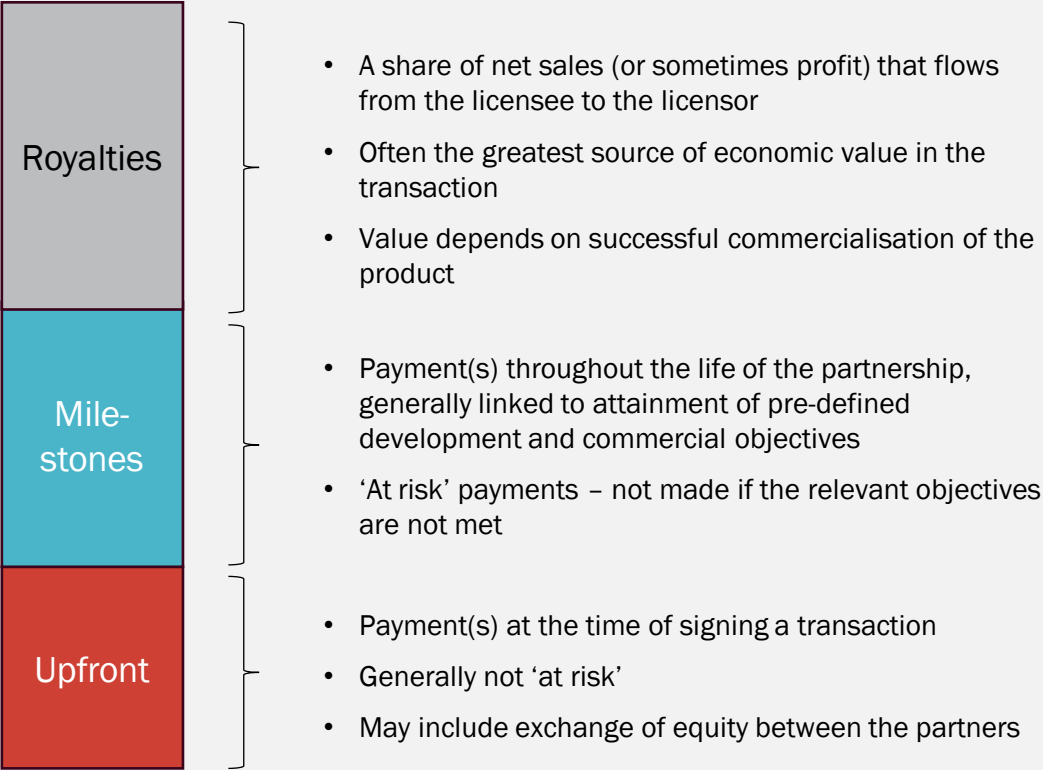


Six-month data from the ongoing phase IIb trial of ATL1102 will define the likely path to market for the drug



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

Illustrative Composition of Typical Pharma Partnering Transactions



Source: DealForma; Antisense analysis

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

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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