

Developing High Impact Therapies for Orphan Diseases

Corporate Overview

August 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.

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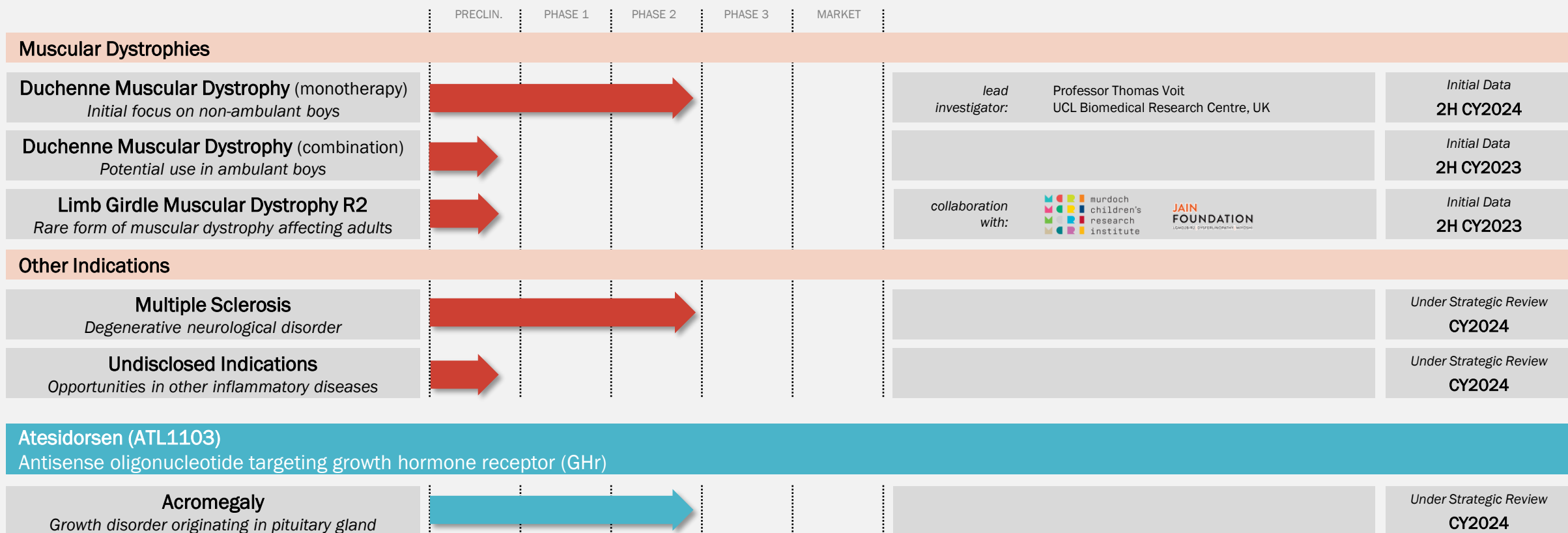
Antisense Therapeutics (ASX: ANP) is a late-clinical stage biotech company, focused on development of novel high-value therapies for orphan diseases

- Lead program is **ATL1102**, an antisense oligonucleotide treatment for **Duchenne muscular dystrophy (DMD)** and other diseases
 - International double-blind, placebo-controlled phase IIb trial ongoing
 - Positive clinical data from prior single-arm phase IIa study
 - Well-validated technology with multiple FDA approved therapies in various conditions
- **ATL1102 is a late-stage asset with substantial commercial opportunity**
 - Approximately 300,000 DMD patients worldwide
 - Existing therapies priced up to US\$ 300K per treatment year; total market estimated at ~US\$ 4B per annum; ~US\$ 10B by 2030
 - ATL1102 potentially applicable to almost all DMD patients, not just those with specific genetic mutations ('mutation agnostic')
 - Potential applications for ATL1102 in other disease areas
- **Antisense enjoys strong corporate fundamentals**
 - Highly-experienced Board and management team
 - Recent oversubscribed institutional financing of \$8.35M, plus Share Purchase Plan proceeds of \$3.26M, leaves the company well funded for ongoing operations
 - Lean, virtual operating model

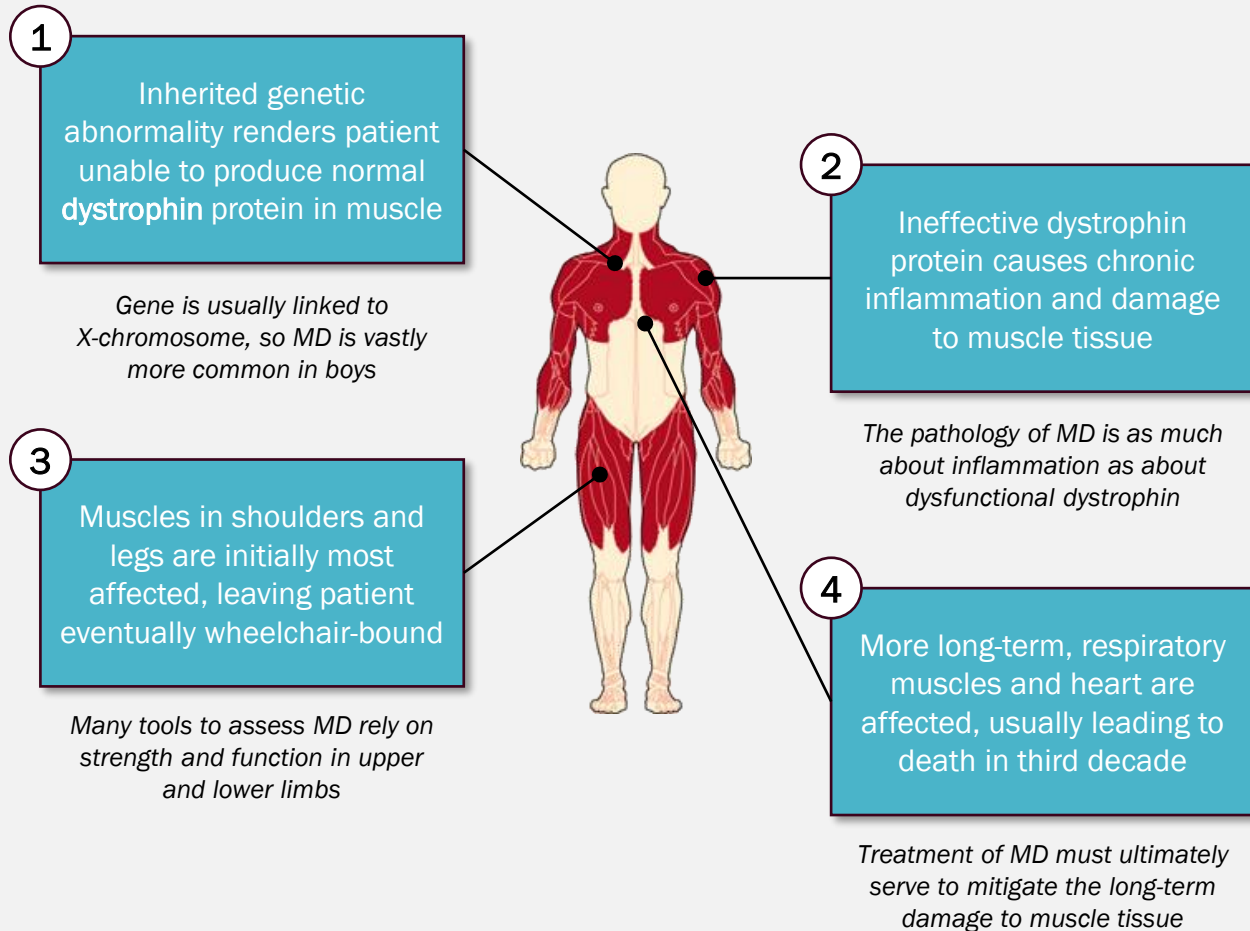


Antisense's pipeline comprises potential first-in-class assets for genetic diseases with high unmet clinical need

ATL1102 Antisense oligonucleotide targeting CD49d



Duchenne muscular dystrophy (DMD) is an incurable genetic condition that affects approximately 300,000 children and young adults worldwide



Duchenne muscular dystrophy (DMD) represents ~50% of MD cases	Incidence is approximately 6 in 100,000 births	DMD also associated with cognitive dysfunction, brittle bones, and other degenerative effects
Usually diagnosed by Age 5	Typically wheelchair-bound by Age 12	Life expectancy 20s

There are two fundamental approaches to the pharmacological treatment of DMD: (1) target the underlying genetic abnormality, and (2) target its effects

1
Target the underlying genetic abnormality to restore normal dystrophin production

- Most therapies are only applicable to a small proportion of patients
- Some uncertainty around degree of clinical benefit

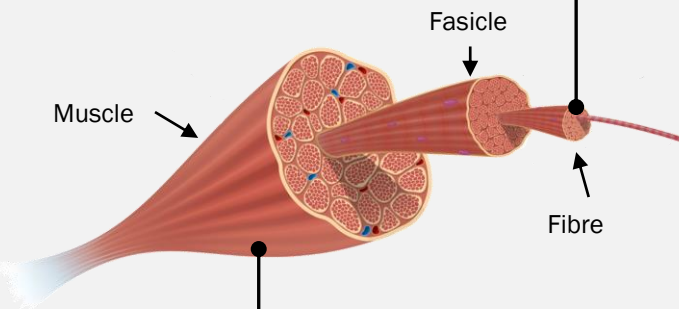
EXONDYS 51
(eteplirsen) Injection

AMONDYS 45
(casimersen) Injection

Viltepso
(viltolarsen) injection

VYONDYS 53
(golodirsen) Injection

transLarna
ataLuren



Standard of care is primarily corticosteroids, but is evolving to include combination treatment with both dystrophin-restoration therapies and anti-inflammatory therapies, including novel, non-steroid anti-inflammatory therapies

2
Target the inflammation to reduce muscle damage from abnormal dystrophin

- Some side effects with older therapies such as prednisone
- Steroids are less effective in patients with high CD49d expression
- Applicable to most or all patients

PREDNISONE

Emflaza
(deflazacort)
6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

ATL1102

Corticosteroid therapies

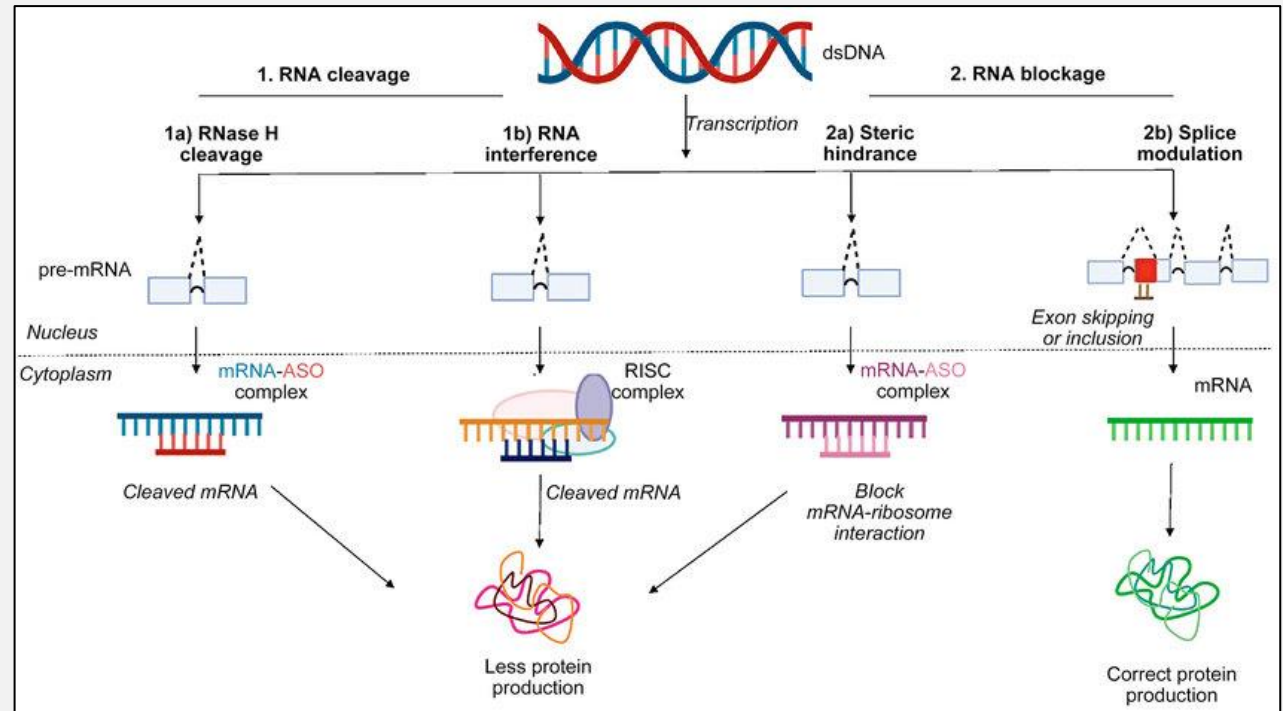
Novel non-steroid therapy

ATL1102's mechanism of action is well validated and clearly understood, with nine FDA-approved antisense oligonucleotide therapies already available to patients

1 Ordinarily, the genetic code in a patient's DNA is transcribed to RNA, which is used to direct synthesis of proteins. Aberrant proteins (e.g. dystrophin in DMD) cause disease.

2 Antisense oligonucleotides (ASOs) are short strands of genetic material (DNA or RNA) which interfere with transcription, or with the translation of RNA to protein, or work to correct protein production, via various mechanisms (shown right).

3 ATL1102 is an ASO which targets the production of CD49d, a protein involved in inflammation. By blocking synthesis of CD49d, ATL1102 has an anti-inflammatory action, which reduces chronic muscle tissue damage associated with DMD.









FDA-Approved Antisense Therapies



ATL1102 has shown compelling evidence of clinical efficacy across multiple validated endpoints in a phase IIa pilot study of 9 non-ambulant boys

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

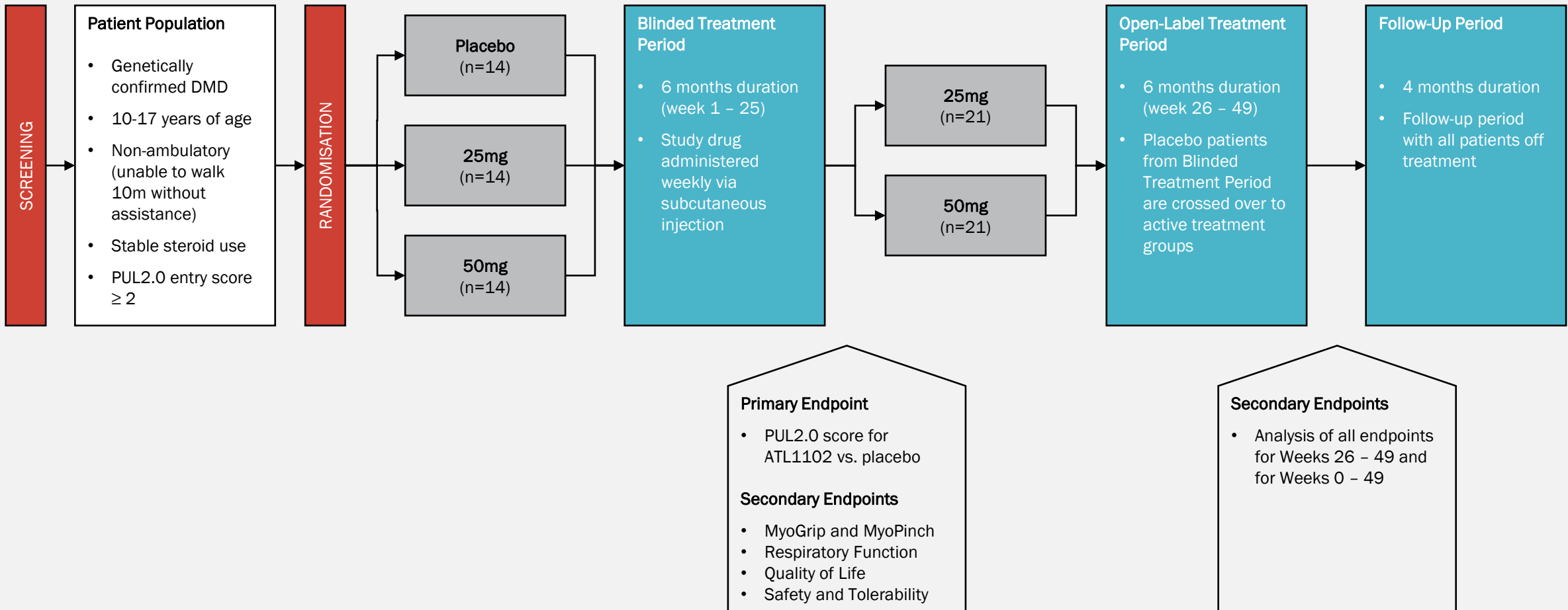
Study Results (Efficacy) [at 6 months]			
Endpoint	Description	ATL1102 Result	Historical Comparator
 PUL2.0	Performance of Upper Limb (PUL2.0) assesses the function of upper body muscles in 3 dimensions	↑ 0.9 (-1.33 - 3.11)	↓ 2.0 (-2.95 - -1.05)
 MyoGrip (dominant hand)	MyoGrip assesses the clamping force of the fingers	↑ 0.2 kg (-0.25 - 0.67)	↓ 0.5 kg (-1.01 - 0.00)
 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)
 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)
 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 mm² (-72.6 - 100.4)	↓ 32.1 mm² (-102.6 - 38.1)
 Lymphocyte Counts	Lymphocyte counts measure the ability of ATL1102 to modulate the immune system and reduce inflammation	↓ 0.28 x 10⁹ / L (-1.10 - 0.55)	↑ 0.47 x 10⁹ / L

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

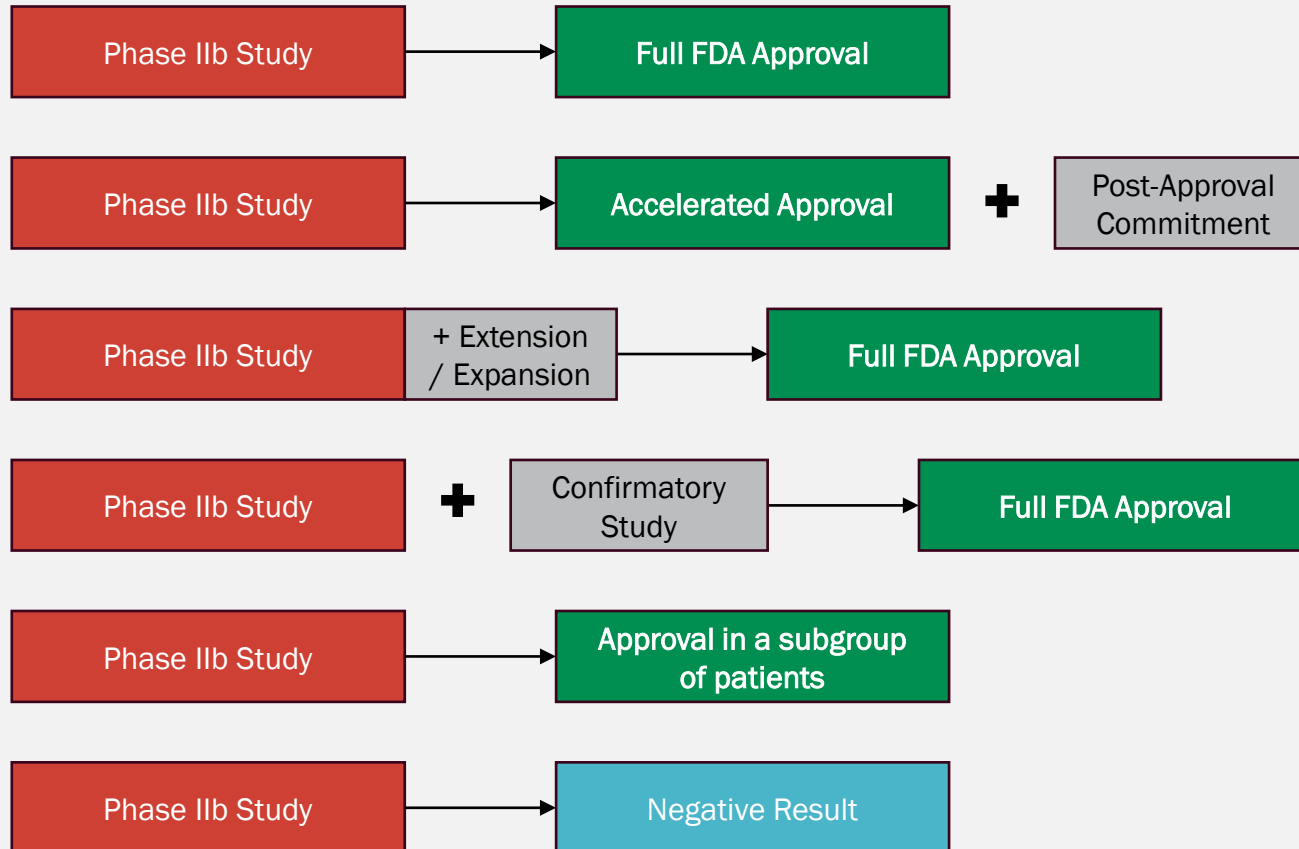
Source: [IR Woodcock et al. \(2022\) medRxiv 2022.01.16.22269029](#); [V Ricotti et al. \(2016\) PLoS ONE 11\(9\): e0162542](#); [G Tachas et al. \(2020\) Neuromuscul. Disord. 30\(S1\):S129-130](#)

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

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



Ongoing phase IIb study defines multiple potential paths to market for ATL1102, with possibility of earning a pediatric priority review voucher on approval



Pediatric Priority Review Voucher (pPRV)

- PRV system designed to incentivise private sector to develop new medicines for rare and underserved diseases
- pPRVs may be awarded by FDA on approval of a new medicine for a rare pediatric disease, providing it is the first approval for that medicine
- To be eligible for a pPRV, the drug must have been granted Rare Pediatric Disease Designation (RPDD) prior to filing for approval – **ATL1102 has been granted RPDD**
- A PRV allows the holder to accelerate FDA review of any new drug application from ~12 months to ~6 months. For a high-value product, this acceleration is very valuable. The holder does not have to use the voucher on the drug for which it was originally granted
- PRVs can be freely traded between companies. The current market price is in excess of **US\$ 100M**











The commercial opportunity in DMD is substantial, with a potential market size of ~US\$ 4 billion, reflecting favourable pricing dynamics

Comparator Revenues (2021-22)

Company	Product	2022 (US\$)	2021 (US\$)
 SAREPTA THERAPEUTICS	 EXONDYS 51 (eteplirsen) Injection	512M	454M
 SAREPTA THERAPEUTICS	 AMONDYS 45 (casimersen) Injection	215M	69M
 SAREPTA THERAPEUTICS	 VYONDYS 53 (golodirsen) Injection	117M	90M
 PTC THERAPEUTICS	 Emflaza [®] (deflazacort)	218M	187M
 PTC THERAPEUTICS	 transLarna [®] ataluren	289M	236M
 NS Pharma	 Viltepso (vitelarsen) injection	109M	56M

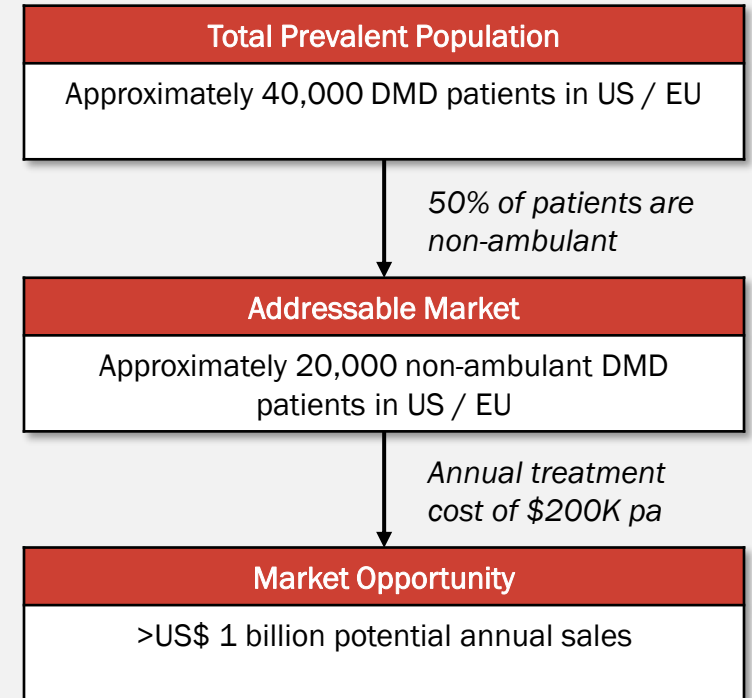
~\$1.5B in annual sales at 34% growth YoY

Comparator Pricing

Company	Product	Annual Cost (US\$)
 SAREPTA THERAPEUTICS	 EXONDYS 51 (eteplirsen) Injection	~\$750K
 SAREPTA THERAPEUTICS	 AMONDYS 45 (casimersen) Injection	~\$750K
 SAREPTA THERAPEUTICS	 VYONDYS 53 (golodirsen) Injection	~\$750K
 PTC THERAPEUTICS	 Emflaza [®] (deflazacort)	~\$100K
 PTC THERAPEUTICS	 transLarna [®] ataluren	~\$300K

Conservatively anticipate ATL1102 pricing at ~\$200K per patient per year

ATL1102 Commercial Opportunity



US\$1B potential, with additional upside in other territories and patient segments

Source: company SEC filings; news reports; Antisense Therapeutics analysis

The competitive landscape in DMD is not crowded, and most companies have focused on dystrophin-restoration therapies rather than anti-inflammatory approaches

	Early Development	Late Development	Approved
Exon-Skipping Therapies <i>ESTs use antisense oligonucleotides (ASOs) to help cells skip dysfunctional genetic code during transcription, yielding dystrophin which more closely resembles normal</i>			
Other Genetic Approaches <i>Gene therapies and other ASO approaches attempt to restore production of (approximately) normal dystrophin</i>			
Anti-Inflammatory and anti-Fibrotic Therapies <i>Anti-inflammatory therapies seek to reduce the damage caused to muscle tissue by dysfunctional dystrophin</i>			

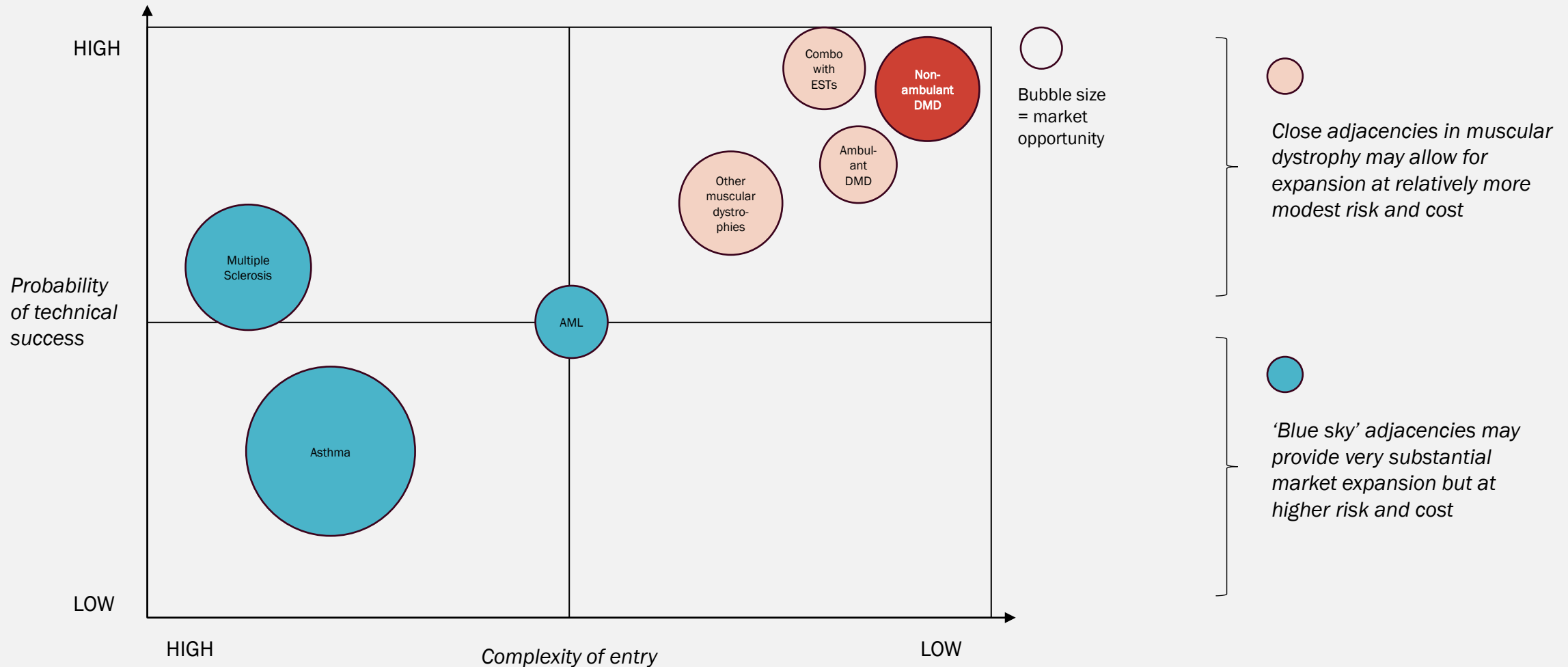
ESTs are only suitable for patients with specific genetic mutations, accounting for a small proportion of total:-

Exondys 51	14%
Amondys 45	8%
Vyondys 53	8%
Viltepso	8%




















Elevidys, the first gene therapy approved in DMD, is only indicated for boys 4-5 years of age, and costs US\$ 3.5 million

Following failure of Fibrogen's pamrevlumab in June 2023, ATL1102 is one of the only anti-inflammatory therapies in late-stage development for non-ambulant patients (pavrevlumab remains in development for ambulant)

Although focus is on non-ambulant DMD patients for now, there is rich opportunity to expand the use of ATL1102 beyond this patient population



Partnering opportunity for ATL1102 is substantial, with benchmark transactions suggesting opportunity for significant value realisation

Licensing Transactions						
Licensee	Licensor	Asset	Indication	Stage	Date	Deal Value (US\$)
 NS Pharma	 Capricor Therapeutics	CAP-1002 (United States)	Duchenne muscular dystrophy	Phase II	Jan 2022	\$735M
 VERTEX	 entrada THERAPEUTICS	ENTR-701	Myotonic dystrophy type I	Preclinical	Dec 2022	\$709M
 uniQure	 ApicBio	APB-102	Amyotrophic lateral sclerosis	Preclinical	Jan 2023	\$55M
 NOVARTIS	 AVROBIO	AVR-RD-04	Cystinosis	Phase I	May 2023	\$88M
 sanofi	MAZE THERAPEUTICS	MZE-001	Pompe disease	Phase I	May 2023	\$750M
 Catalyst pharmaceuticals	 santhera	Vamorolone (North America)	Duchenne muscular dystrophy	Pre-Approval	Jun 2023	\$231M+
M&A Transactions						
Acquirer	Target	Key Asset(s)	Key Indication(s)	Stage	Date	Deal Value (US\$)
 Pfizer	 globalblood THERAPEUTICS	Voxelotor	Sickle cell anaemia	Approved	Aug 2022	\$5.4B
 novo nordisk	 forma THERAPEUTICS	Etavopiat	Sickle cell anaemia	Phase III	Sep 2022	\$1.1B
 MERCK	 IMAGO BIO SCIENCES	Bomedemstat	Myeloproliferative disorders	Phase II	Nov 2022	\$1.4B
 NOVARTIS	 GYROSCOPE VISION FOR LIFE	GT005	Geographic atrophy	Phase II	Dec 2022	\$1.5B

Source: Company press releases and SEC filings

Note: list is non-exhaustive

New Antisense team brings extensive international experience in drug development, partnering, and commercialisation



Dr Charmaine Gittelson
Board Chair

25 years of experience, including 15-year tenure with CSL in international roles



Dr Gil Price
Non-Executive Director

Experienced biotech executive and entrepreneur with extensive experience in drug development



Dr James Garner
CEO & Managing Director

20-year track record of international drug development in multinational companies



Dr Anthony Filippis
Chief Operating Officer

25 years of life sciences leadership experience, with a focus on BD, corporate strategy, and operations



Phillip Hains
Chief Financial Officer

25 years of strategic financial experience with a diverse range of ASX-listed companies



Dr George Tachas
Principal Scientist

Immunologist and molecular biologist with substantial IP experience; inventor of ATL1102 in DMD

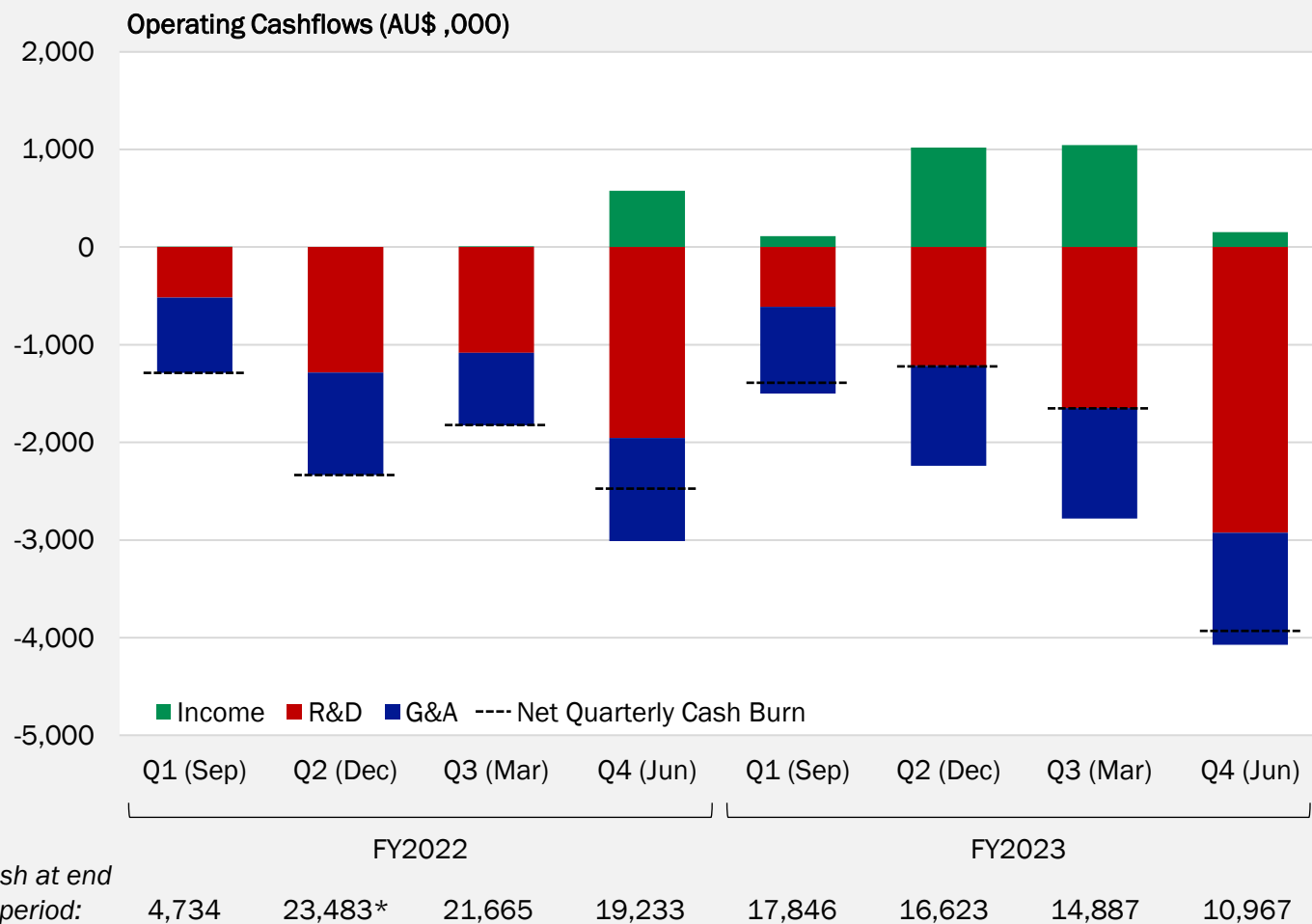


Dr Andrew McKenzie
Director, Clinical Development

23 years of international drug development experience



Antisense enjoys a strong financial position, with the ongoing phase IIb study of ATL1102 well funded



Corporate Fundamentals

Market Capitalisation:	~AU\$ 46M
Primary Listing:	ASX: ANP
Secondary Listings:	FSE: AWY; OTC: ATHJY
Shares on Issue:	~669 Million
Average Daily Trading (FY23):	~AU\$ 40K

Financial Position

Cash Balance (30 Jun 23):	AU\$ 11 million
Runway:	Q2 CY2024

Substantial Shareholders

Platinum Asset Management	13.3%
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as at 31 July 2023

Analyst Coverage



*Note: Financing in Q2 FY2022 provided ~\$21M in net proceeds

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	
Full recruitment to international phase IIb study of ATL1102 in Duchenne muscular dystrophy	2H CY2023	
CY2024		
Operational completion 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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