

## PERCHERON THERAPEUTICS CORPORATE PRESENTATION

**Melbourne, Australia – 26 February 2024:** Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to provide an updated non-confidential corporate presentation for the information of shareholders and investors.

This presentation supersedes the previous corporate presentation that was released on 28 August 2023.

This updated presentation covers the Company's pipeline and financial position and provides a summary of recent progress. It will be used in various upcoming investor roadshows and shareholder meetings.

~ ENDS ~

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### About Percheron Therapeutics Limited

Percheron Therapeutics Limited [ASX: PER | US OTC: ATHJY | FSE: AWY] is a publicly listed biotechnology company focused on the development and commercialisation of novel therapies for rare diseases. The company's lead program is ATL1102, an antisense oligonucleotide targeting the CD49d receptor. ATL1102 is currently the subject of an ongoing international phase IIb clinical trial for the treatment of non-ambulant patients with Duchenne Muscular Dystrophy (DMD), for which data is expected in 2H CY2024. The company previously reported promising results from an exploratory phase IIa study of in the same population and has been awarded orphan drug designation (ODD) and rare pediatric disease designation (RPDD) by the US FDA.

For more information, please contact [info@PercheronTx.com](mailto:info@PercheronTx.com).

*This announcement has been authorized for release to the Australian Securities Exchange by the Board of Directors.*

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Developing High Impact  
Therapies for Orphan Diseases

Corporate Overview

February 2024

[www.PercheronTx.com](http://www.PercheronTx.com)

ASX: PER | FSE: AWY | US OTC: ATHJY



# 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.

The Company does not warrant any of the forward-looking statements in this presentation, and investors are advised to interpret such statements in the context of other available sources of information and with the assistance of expert advisors as appropriate.

Many factors could cause the actual results of the Company to differ materially from the results expressed or implied herein, and you should not place undue reliance on the forward-looking statements. Drug development is inherently risky, and only a small proportion of research and development programs lead to a marketed product. Factors which could change the Company’s expected outcomes include, without limitation, our ability to: advance the development of our programs, and to do so within any timelines that may be indicated herein; the safety and efficacy of our drug development candidates; our ability to replicate experimental data; the ongoing validity of patents covering our drug development candidates, and our freedom to operate under third party intellectual property; our ability to obtain necessary regulatory approvals; our ability to enter into and maintain partnerships, collaborations, and other business relationships necessary to the progression of our drug development candidates; changes in the competitive landscape pertaining to our drug development candidates; the timely availability of necessary capital to pursue our business objectives; changes in the public policy environment in one or more countries in which we operate or may seek to operate which disfavour our business; our ability to attract and retain qualified personnel; changes from anticipated levels of customer acceptance of existing and new products and services; and other factors, including the COVID-19 pandemic and the conflict in Ukraine.

Although the Company believes that the expectations reflected in such forward-looking statements are reasonable, and although they reflect our current views as at the date of this presentation, there can therefore be no assurance that such expectations will prove to be correct. The Company has no obligation as a result of this presentation to pursue any specific strategy or plan outlined herein, or to deliver any specific outcome that may be implied or inferred.

Any forward-looking statements contained in this presentation speak only as of the date this presentation is made, and we expressly disclaim any obligation to update any forward-looking statements, whether because of new information, future events or otherwise, except as may be required by law.

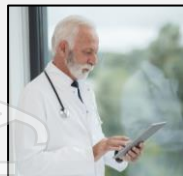
# Percheron Therapeutics (ASX: PER) is a late-clinical stage biotech company, focused on development of novel high-value therapies for orphan diseases

- 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
- **Percheron enjoys strong corporate fundamentals**
  - Highly-experienced Board and management team
  - Oversubscribed institutional financing of \$8.35M in July 2023, plus Share Purchase Plan proceeds of \$3.26M, leaves the company well funded for ongoing operations
  - Lean, virtual operating model





# Good progress continues to be made on ATL1102, with positive developments in recent months



## Deliver Phase IIb Clinical Trial in DMD

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

- All five countries open to recruitment
- **Recruitment on track for ~1Q completion**
- 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 period completed in Dec 23**
- **'Recovery period' and pathological analysis ongoing**

### Next Milestones

Final 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



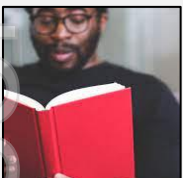
## Envisage Potential Expansion

*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*

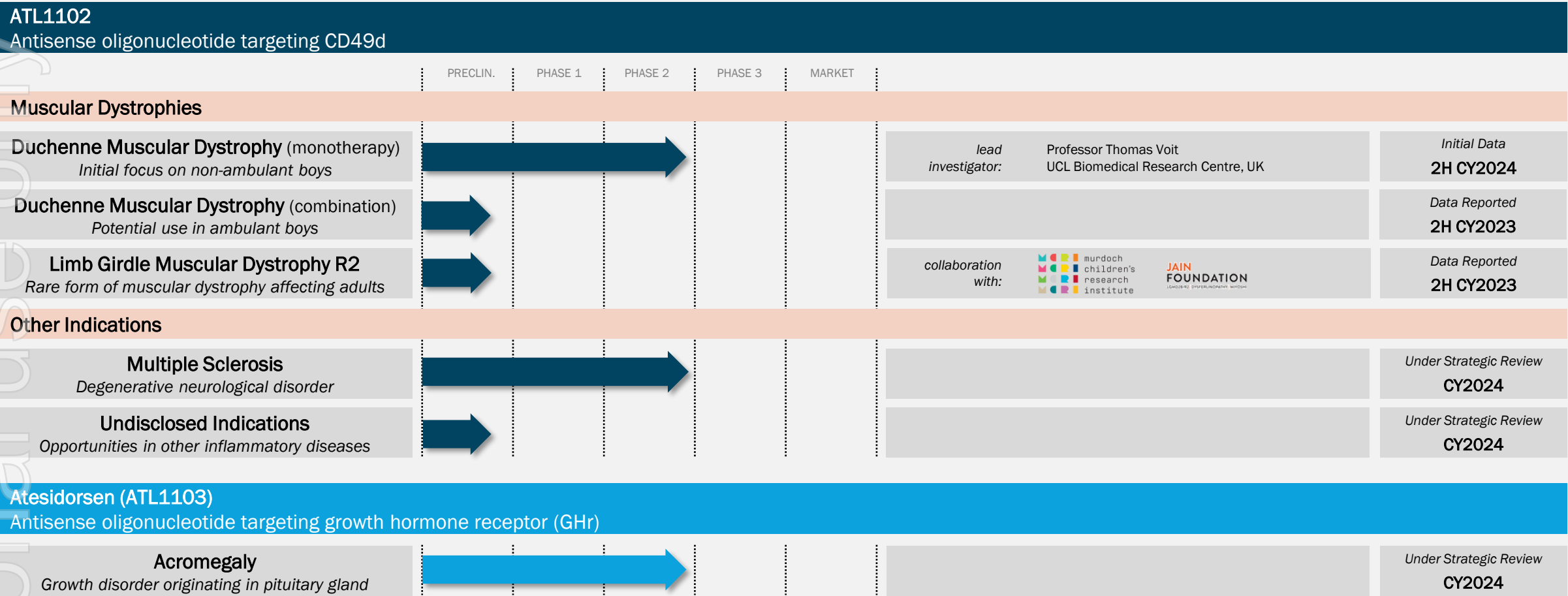
- **Phase IIa data published in PLoS ONE in Jan 24**
- **3x abstracts accepted for MDA Conference in Mar 24**

### Next Milestones

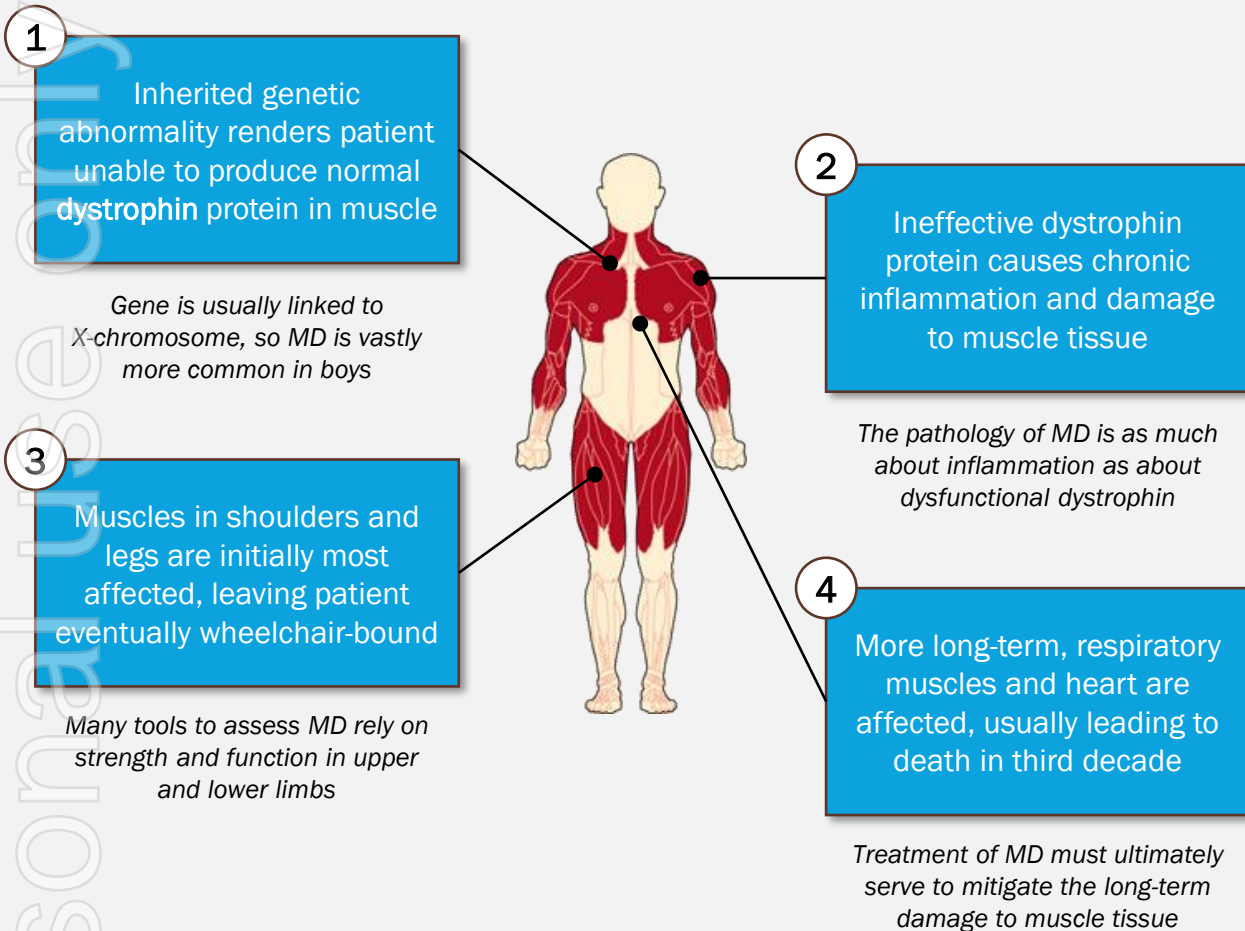
Conference presentations  
1Q CY2024

**bold** – new developments since November 2023

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



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



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

# 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

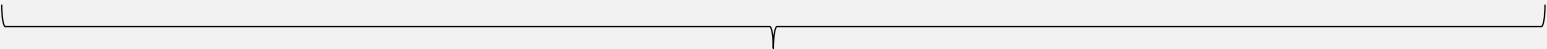
**AMONDYS 45**  
(casimersen) Injection

**Elevidys**  
delandistrogene  
moxeparvovec-rokl

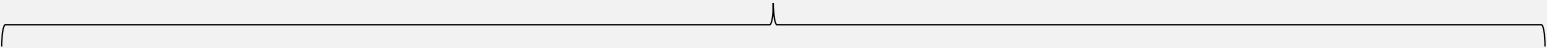
**Viltepso**  
(viltolarsen) injection

**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

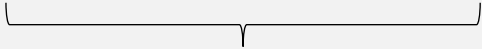
- Some side effects with older therapies such as prednisone
- Steroids are less effective in patients with high CD49d expression
- Applicable to most or all patients

**aGamree**  
(vamorolone) oral suspension  
40mg/mL

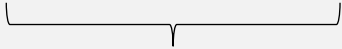
**PREDNISONE**

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

**ATL1102**



*Corticosteroid therapies*



*Novel non-steroid therapy*

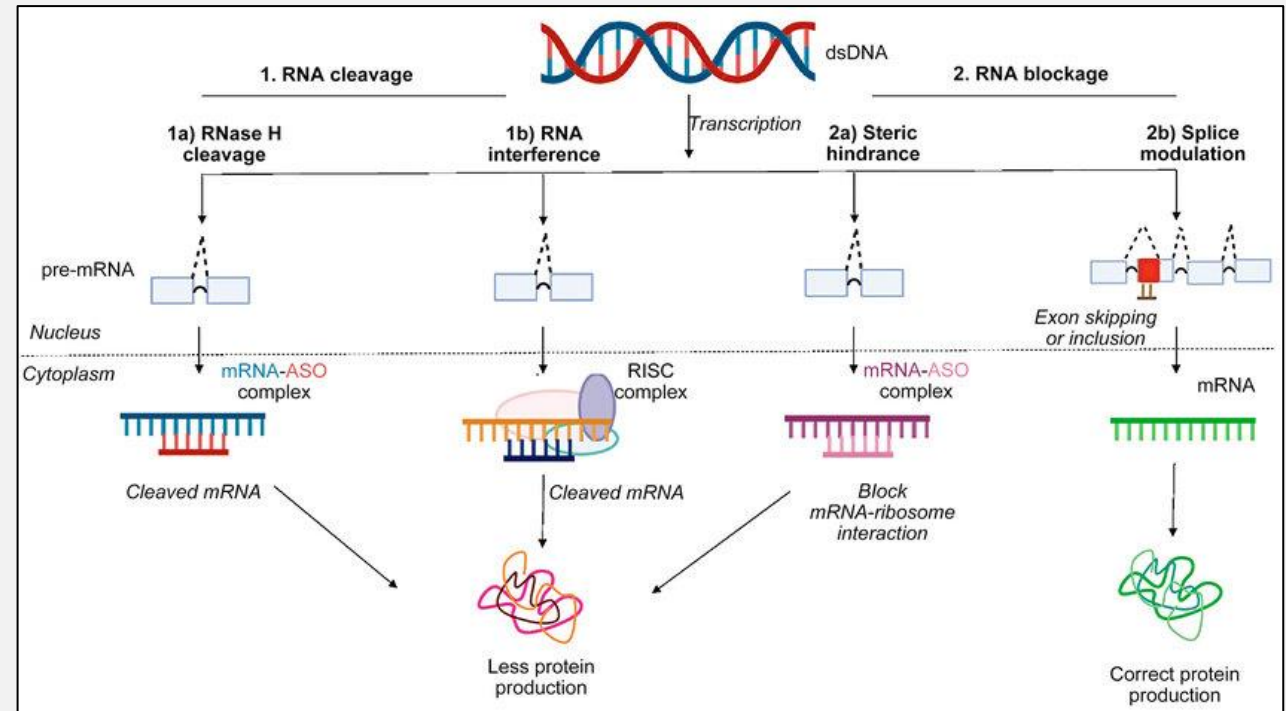


# ATL1102's mechanism of action is well validated and clearly understood, with nine FDA-approved antisense oligonucleotide therapies already available to patients

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

**EXONDYS 51**  
(eteplirsen) Injection

**VYONDYS 53**  
(golodirsen) Injection

**AMONDYS 45**  
(casimersen) Injection

**Viltepso**  
(viltolarsen) injection

**Tegsedi**  
(inotersen) injection  
200 mg/3 mL



















**QALSODY**  
(tofersen) injection  
100 mg/15 mL

**SPINRAZA**  
(nusinersen) injection  
12 mg/5 mL

**KYNAMRO**  
(mipomersen sodium) injection  
200 mg/mL

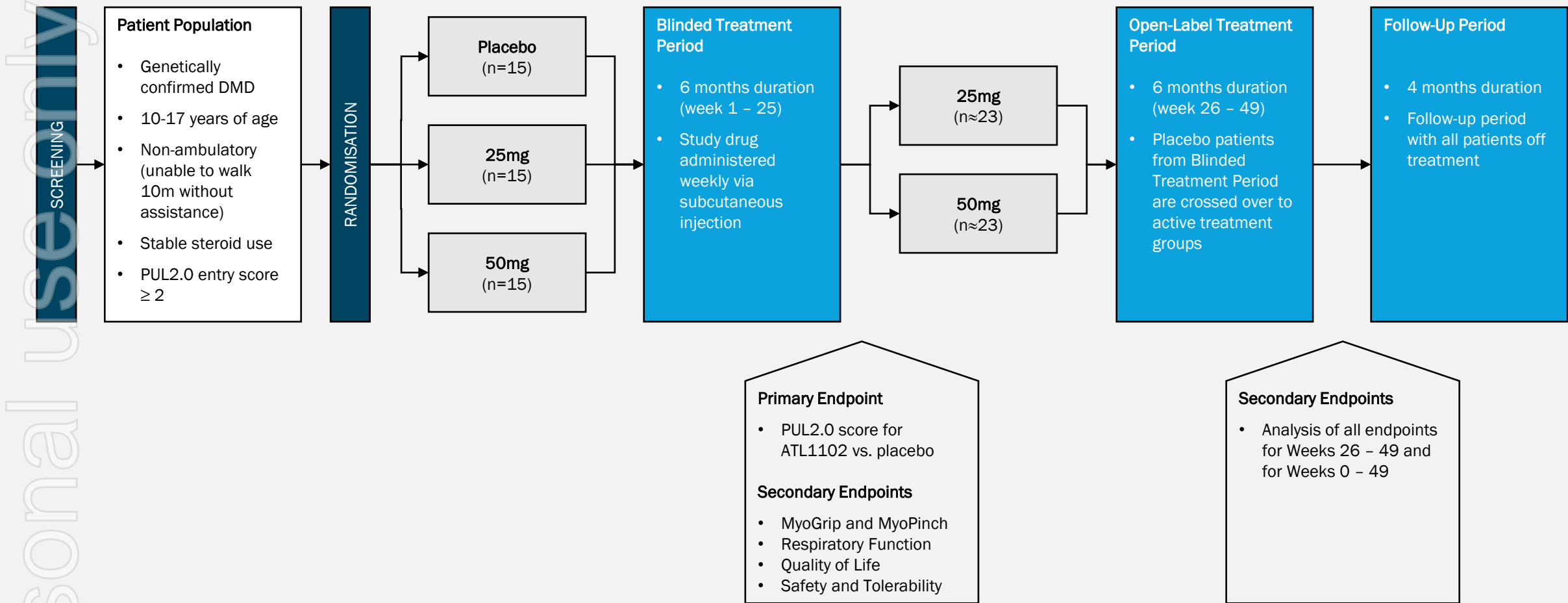
**waylivra**  
(volanesorsen) injection  
200 mg/3 mL

# 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		Study Results (Efficacy) [at 6 months]			
Population		Endpoint	Description	ATL1102 Result	Historical Comparator
Non-ambulant boys with confirmed Duchenne muscular dystrophy, aged 10-18		 <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)
Sample Size		 <b>MyoGrip</b> (dominant hand)	MyoGrip assesses the clamping force of the fingers	 <b>0.2</b> kg (-0.25 – 0.67)	 <b>0.5</b> kg (-1.01 – 0.00)
Intervention		 <b>MyoPinch</b> (dominant hand)	MyoPinch assesses the pinch strength between thumb and forefinger	 <b>0.0</b> kg (-0.18 – 0.19)	 <b>0.4</b> (-0.53 – -0.22)
Primary Endpoint		 <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)
Secondary Endpoints		 <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</b> mm <sup>2</sup> (-72.6 – 100.4)	 <b>32.1</b> mm <sup>2</sup> (-102.6 – 38.1)
Safety and tolerability		 <b>Lymphocyte Counts</b>	Lymphocyte counts measure the ability of ATL1102 to modulate the immune system and reduce inflammation	 <b>0.28</b> x 10 <sup>9</sup> / L (-1.10 – 0.55)	 <b>0.47</b> x10 <sup>9</sup> / L
n = 9					
ATL1102, 25mg weekly via sc injection for 24 weeks					
Location and Timing					
Melbourne, Australia 2018 - 2020					
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. \(2024\) PLoS ONE 19\(1\): e0294847](#); [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



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

Positive Data



Discussion with FDA and other regulatory agencies regarding potential marketing authorisation

Positive Signals



Discussion with regulatory agencies regarding potential need for additional work, or possibility to approve in select subgroups

Negative Data




Evaluation of ongoing potential in DMD; consider opportunities to pivot to other indications for ATL1102

## 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 <sup>®</sup> (deflazacort)	218M	187M
 PTC THERAPEUTICS	 translarna <sup>®</sup> ataluren	289M	236M
 NS Pharma	 Viltepso <sup>®</sup> (vitilorsen) injection	109M	56M

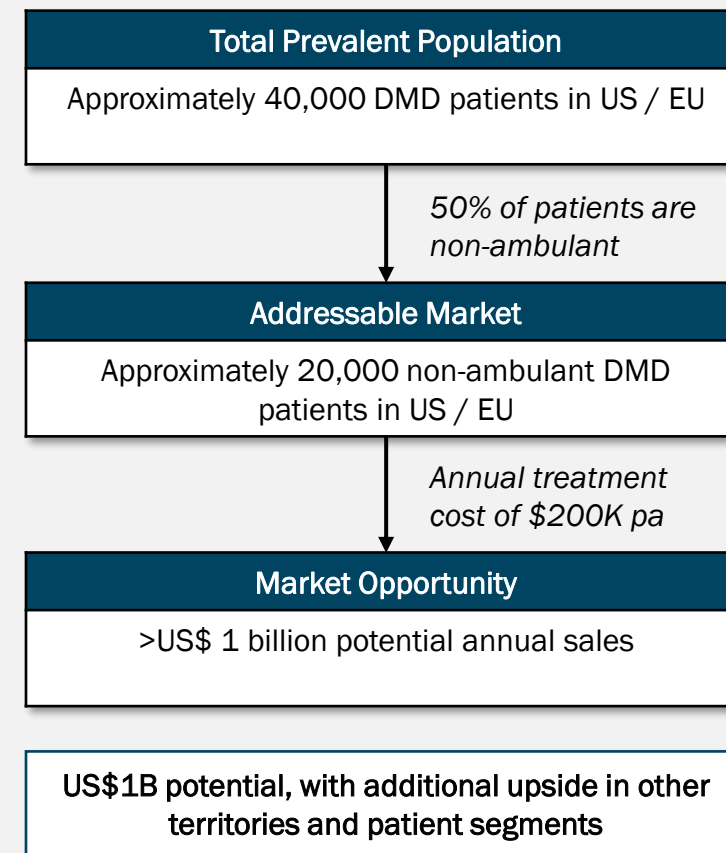
~\$1.5B in annual sales  
at 34% growth YoY

## 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 <sup>®</sup> (deflazacort)	~\$100K
 SAREPTA THERAPEUTICS	 Elevidys <sup>®</sup> delandistrogene moxeparovec-rokl	~\$3.2M

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


























## ATL1102 Commercial Opportunity



Source: company SEC filings; news reports; Percheron 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
<b>Exon-Skipping Therapies</b>  <i>ESTs use antisense oligonucleotides (ASOs) to help cells skip dysfunctional genetic code during transcription, yielding dystrophin which more closely resembles normal</i>	    	 SRP-5051	       
<b>Other Genetic Approaches</b>  <i>Gene therapies and other ASO approaches attempt to restore production of (approximately) normal dystrophin</i>		   PF-06939926	 ELEVIDYS
<b>Anti-Inflammatory and anti-Fibrotic Therapies</b>  <i>Anti-inflammatory therapies seek to reduce the damage caused to muscle tissue by dysfunctional dystrophin</i>		 ATL1102  CAP-1002	   

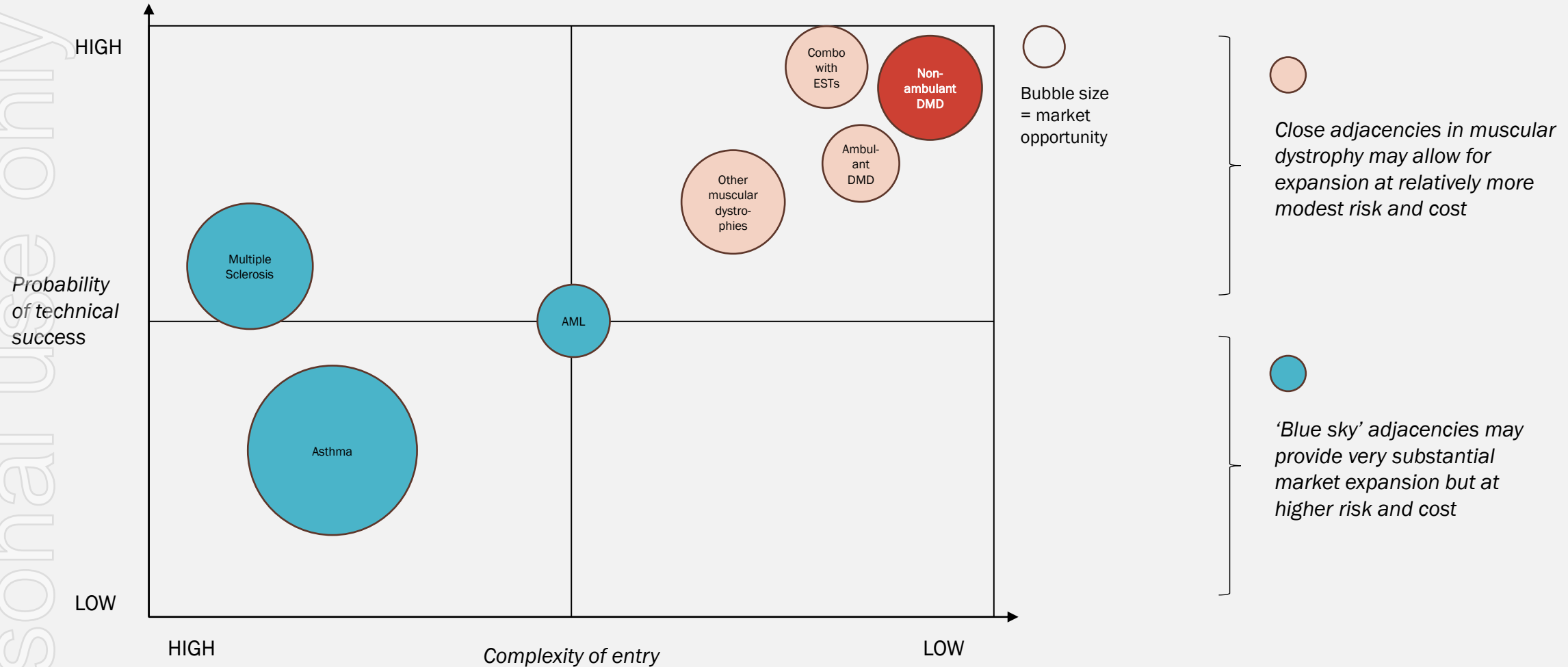
ESTs are only suitable for patients with specific genetic mutations, accounting for a small proportion of total:-

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\$ 3.2 million

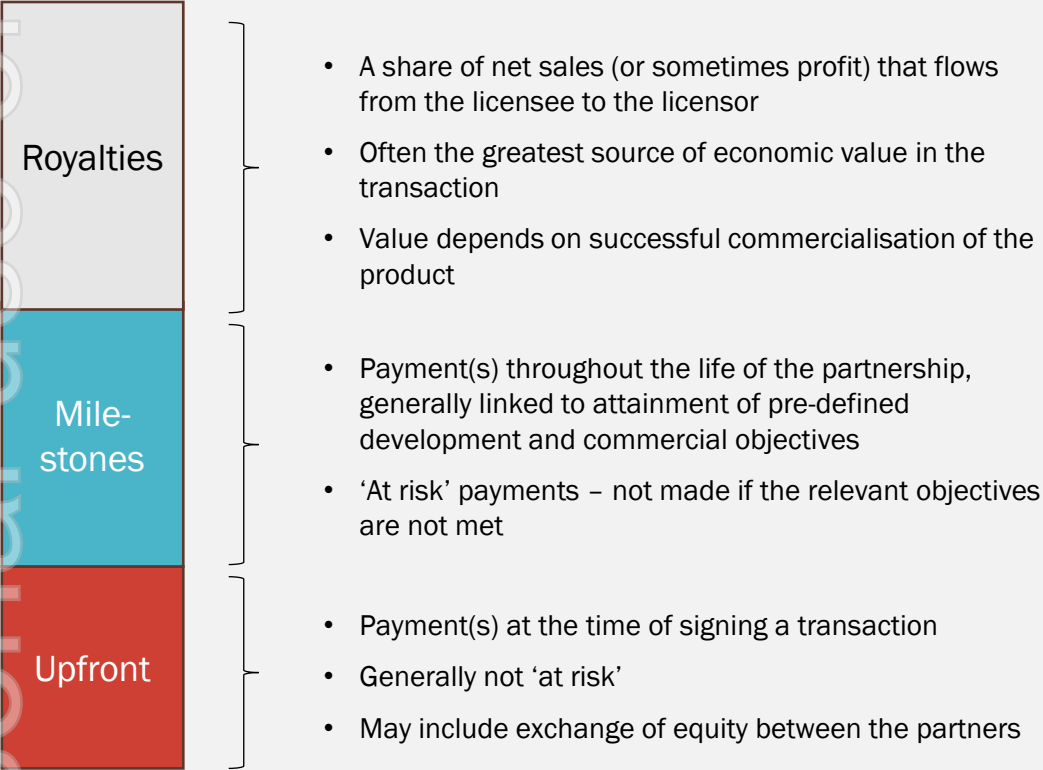
Following failure of Fibrogen's pamrevlumab in non-ambulant patients in June 2023, and in ambulant patients in September 2023, **ATL1102** is one of the only anti-inflammatory therapies in late-stage development for non-ambulant

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



# 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*

# 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 Percheron 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

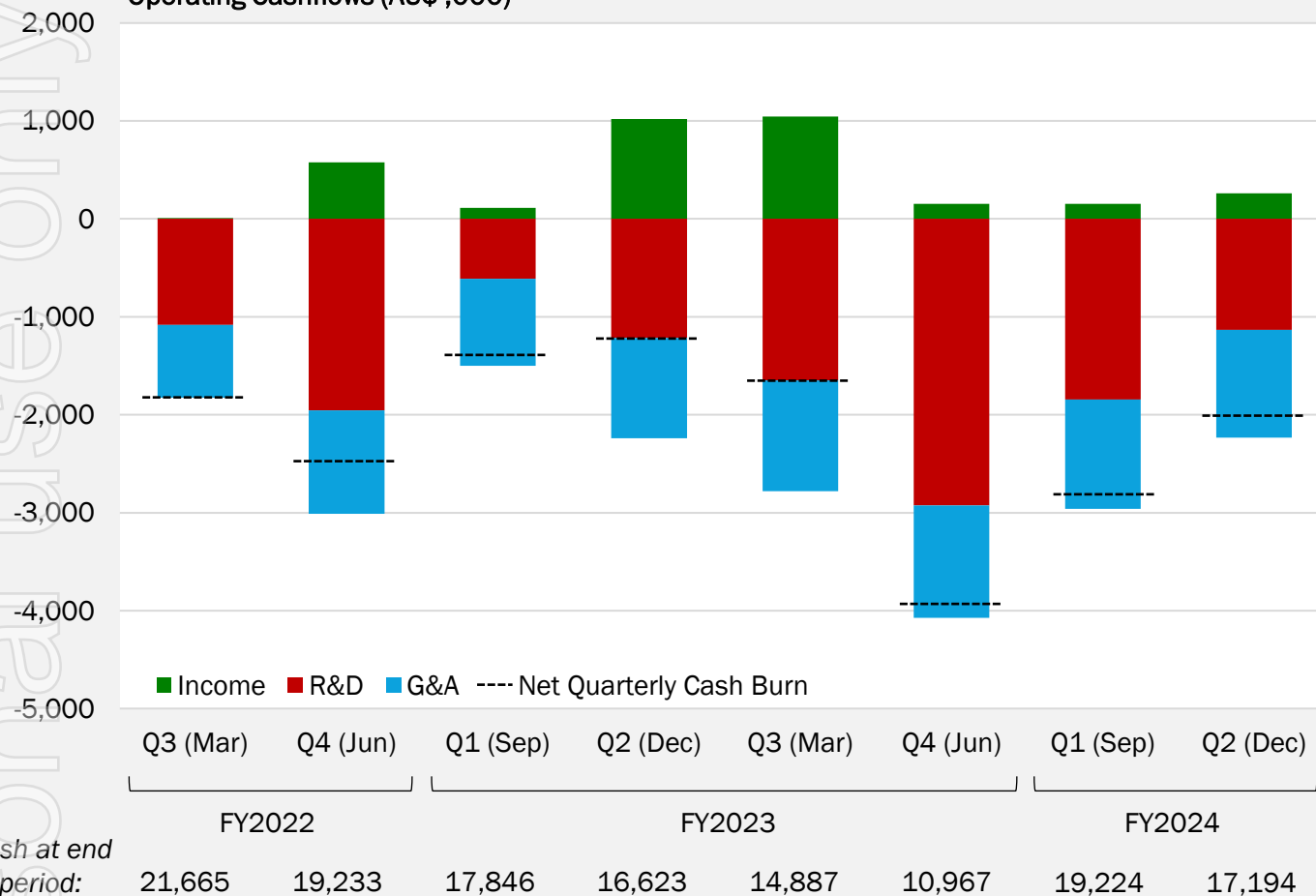
23 years of international drug development experience





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

Operating Cashflows (AU\$ ,000)



Cash at end of period:

FY2022		FY2023		FY2024	
Q3 (Mar)	Q4 (Jun)	Q1 (Sep)	Q2 (Dec)	Q1 (Sep)	Q2 (Dec)
21,665	19,233	17,846	16,623	19,224	17,194

## Corporate Fundamentals

Market Capitalisation:	~AU\$ 52M
Primary Listing:	ASX: PER
Secondary Listings:	FSE: AWY; OTC: ATHJY
Shares on Issue:	~900 Million
Average Daily Trading (FY23):	~AU\$ 40K

## Financial Position

Cash Balance (31 Dec 23):	AU\$ 17.2 million
Runway:	CY2025

## Substantial Shareholders

Platinum Asset Management	12.3%
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as at 31 Dec 2023

## Analyst Coverage



TAYLOR COLLISON

WILSONS

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

✓ - completed

CY2024		
Full recruitment to international phase IIb study of ATL1102 in Duchenne muscular dystrophy	1Q CY2024	
Operational completion of 9-month non-human primate toxicology study	1H CY2024	
Presentations at international muscular dystrophy conferences	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	
Strategic review of ex-DMD ATL1102 opportunities and ATL1103	2H CY2025	
Final data from international phase IIb study of ATL1102 in Duchenne muscular dystrophy	2H CY2025	



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