PERCHERON TO PRESENT AT 2024 BIO INTERNATIONAL CONVENTION

Melbourne, Australia – 31 May 2024: Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to advise that the company has been selected for an oral presentation at the 2024 BIO International Convention, to be held in San Diego, CA, from 3 – 6 June 2024.

Key Points

- The BIO International Convention is the largest annual partnering event for pharmaceutical and biotech companies, typically attracting in excess of 20,000 attendees.

- Percheron CEO, Dr James Garner, will give an overview corporate presentation at 12pm, PT, on Tuesday, 4 June.

- Several Percheron executives will attend BIO and the company anticipates an extensive schedule of meetings and discussions with potential partners and investors over the course of the conference.

"With recruitment to our phase IIb clinical trial now complete, BIO provides a very timely opportunity to introduce the company to a much broader audience," commented Percheron CEO, Dr James Garner. "We expect to have topline data from the study in December 2024, making this an excellent time for interested partners to familiarise themselves with the drug and with the company. We look forward to a busy and productive week in San Diego."

The company's presentation is appended to this announcement for ease of reference.

~ ENDS ~

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.

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

June 2024



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.

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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.

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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 phase IIb clinical trial in DMD is fully-recruited, with data expected in December 2024
  - 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



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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

- **Study is fully recruited (May 2024)**
- **Primary endpoint topline expected in December 2024**
- No safety concerns identified by DSMB; good engagement from patients and investigators

Next Milestones

Data: Dec CY2024



Complete 9-Month Toxicology Study

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

- **Initial data suggests no new toxicities; highly consistent with earlier 6-month study**
- **'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
- Systematic NDA-preparedness evaluation ongoing

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

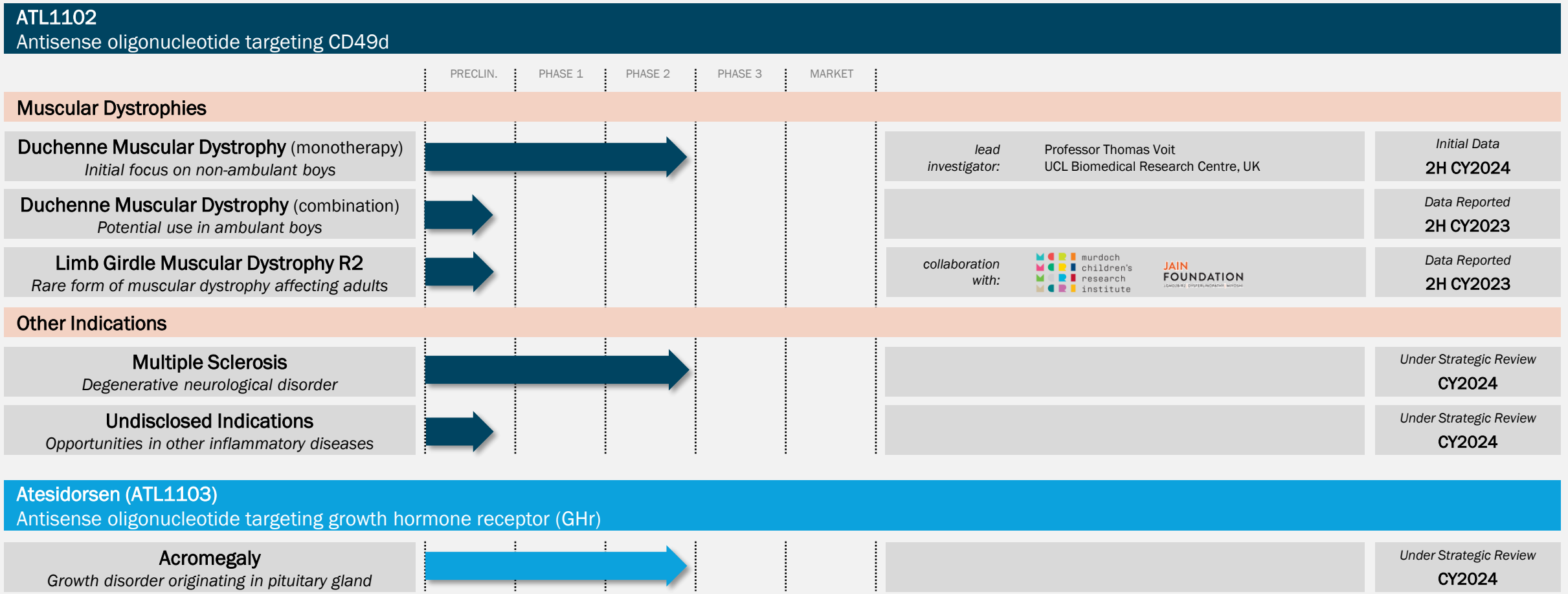
Share ATL1102's impressive dataset with the world via peer-reviewed journals and scientific conferences

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

Next Milestones

Further publications throughout CY2024

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



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

1. Inherited genetic abnormality renders patient unable to produce normal **dystrophin** protein in muscle

Gene is usually linked to X-chromosome, so MD is vastly more common in boys

2. Ineffective dystrophin protein causes chronic inflammation and damage to muscle tissue

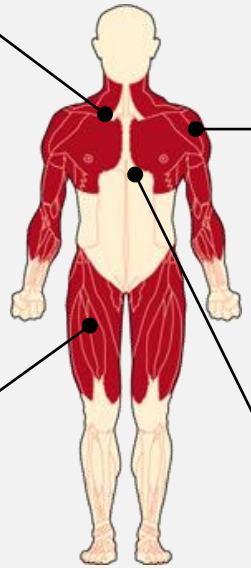
The pathology of MD is as much about inflammation as about dysfunctional dystrophin

3. Muscles in shoulders and legs are initially most affected, leaving patient eventually wheelchair-bound

Many tools to assess MD rely on strength and function in upper and lower limbs

4. More long-term, respiratory muscles and heart are affected, usually leading to death in third decade

Treatment of MD must ultimately serve to mitigate the long-term damage to muscle tissue



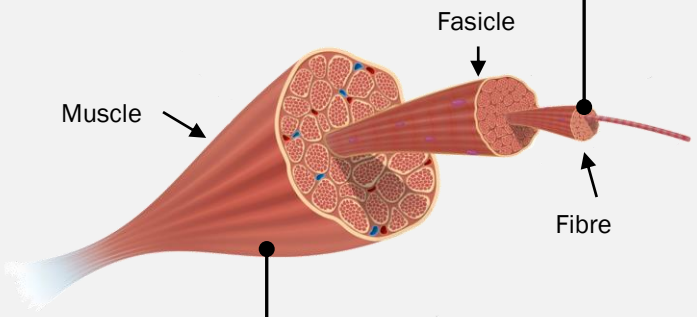
Duchenne muscular dystrophy (DMD) represents **~50%** of MD cases	Incidence is approximately **6 in 100,000 births**	DMD also associated with cognitive dysfunction, brittle bones, and other degenerative effects
Usually diagnosed by **Age 5**	Typically wheelchair-bound by **Age 12**	Life expectancy **20s**

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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



2

Target the inflammation to reduce muscle damage from abnormal dystrophin

- 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

- 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

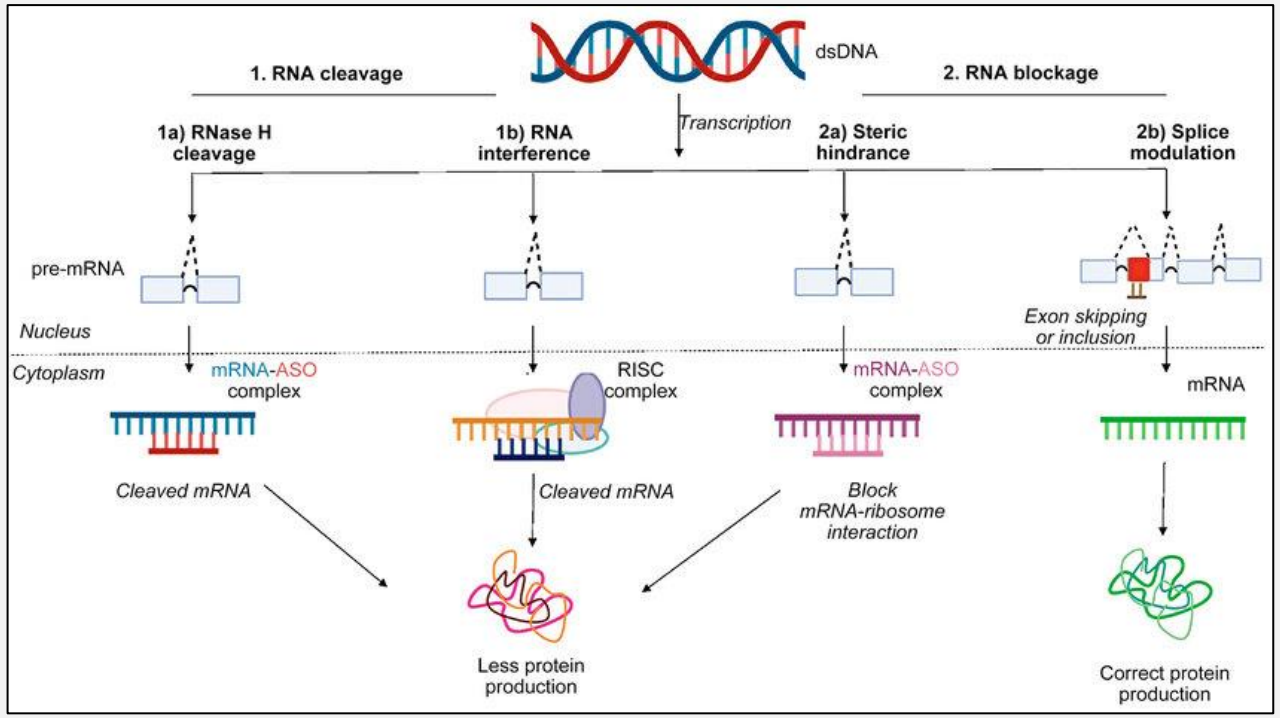
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ATL1102's mechanism of action is well validated and clearly understood, with nine FDA-approved antisense oligonucleotide therapies already available to patients

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)
QALSODY (tofersen)
SPINRAZA (nusinersen)
KYNAMRO (mipomersen sodium) injection
waylivra (volanesorsen)

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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 mm^2 (-72.6 – 100.4)	↓ 32.1 mm^2 (-102.6 – 38.1)
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

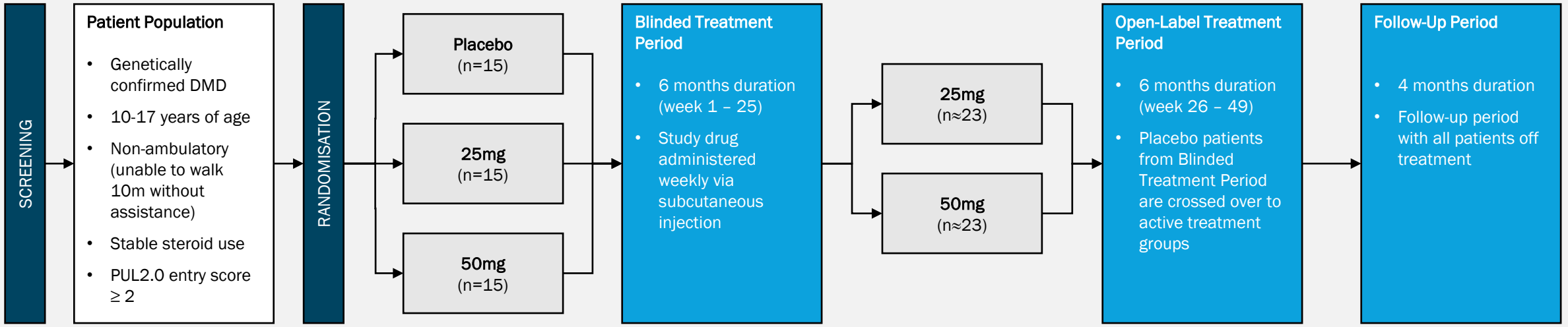
Study Results (Safety)

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

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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 marking 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 (deflazacort)	218M	187M
PTC Therapeutics	translarna ataluren	289M	236M
NS Pharma	Viltepso (viltolarsen) 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
Sarepta Therapeutics	Elevidys delandistrogene moxeparvovec-rokl	~$3.2M

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

ATL1102 Commercial Opportunity

Total Prevalent Population

Approximately 40,000 DMD patients in US / EU

50% of patients are non-ambulant

Addressable Market

Approximately 20,000 non-ambulant DMD patients in US / EU

Annual treatment cost of $200K pa

Market Opportunity

>US$ 1 billion potential annual sales

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

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
Exon-Skipping Therapies *ESTs use antisense oligonucleotides (ASOs) to help cells skip dysfunctional genetic code during transcription, yielding dystrophin which more closely resembles normal*	DYne, WAVE LIFE SCIENCES, AVIDITY BIOSCIENCES, PepGen, entrada THERAPEUTICS	SAREPTA THERAPEUTICS SRP-5051	SAREPTA THERAPEUTICS EXONDYS 51 (eteplirsen) Injection SAREPTA THERAPEUTICS AMONDYS 45 (casimersen) Injection SAREPTA THERAPEUTICS VYONDYS 53 (golodirsen) Injection NS Pharma Viltepso (viltolarsen) injection
Other Genetic Approaches *Gene therapies and other ASO approaches attempt to restore production of (approximately) normal dystrophin*	REGENXBIO	PTC THERAPEUTICS translarna ataluren Pfizer PF-06939926	SAREPTA THERAPEUTICS ELEVIDYS
Anti-Inflammatory and anti-Fibrotic Therapies *Anti-inflammatory therapies seek to reduce the damage caused to muscle tissue by dysfunctional dystrophin*		percheron THERAPEUTICS ATL1102 Capricor Therapeutics CAP-1002	PTC THERAPEUTICS Emflaza (deflazacort) santhera aGamree (vamorolone) ITALFARMACO Duvyzat (givinostat)

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

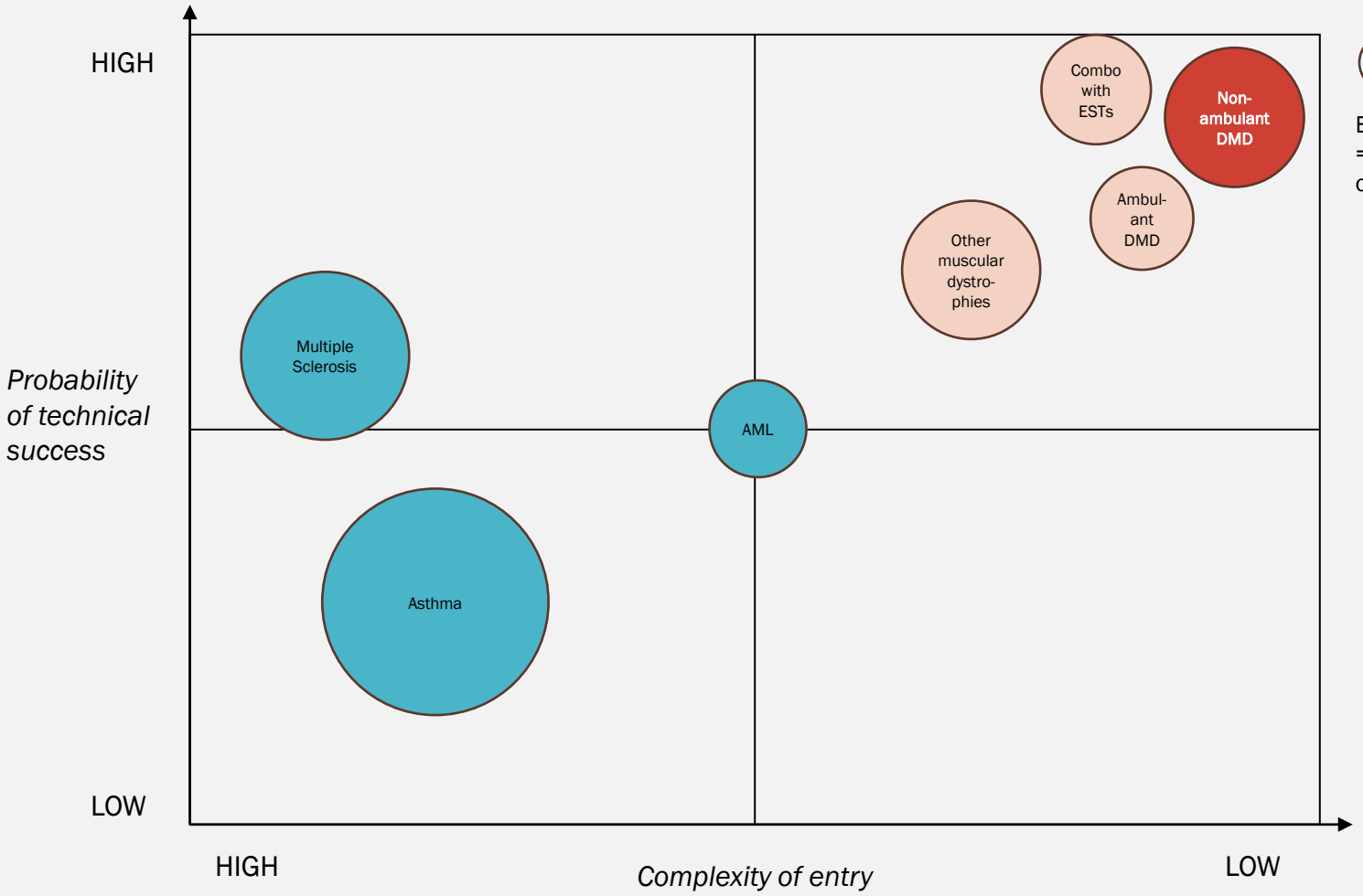
Exondys 51	14%
Amondys 45	8%
Vyondys 53	8%
Viltepso	8%

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

FibroGen

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

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



Bubble size = market opportunity

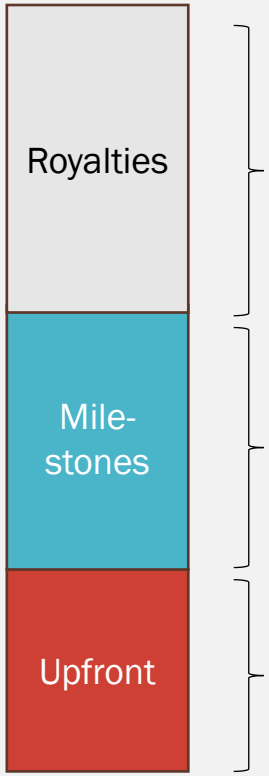
Close adjacencies in muscular dystrophy may allow for expansion at relatively more modest risk and cost

'Blue sky' adjacencies may provide very substantial market expansion but at higher risk and cost

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

Illustrative Composition of Typical Pharma Partnering Transactions



- A share of net sales (or sometimes profit) that flows from the licensee to the licensor
- Often the greatest source of economic value in the transaction
- Value depends on successful commercialisation of the product

- Payment(s) throughout the life of the partnership, generally linked to attainment of pre-defined development and commercial objectives
- 'At risk' payments – not made if the relevant objectives are not met

- Payment(s) at the time of signing a transaction
- Generally not 'at risk'
- May include exchange of equity between the partners

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

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 BIOSCIENCES	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



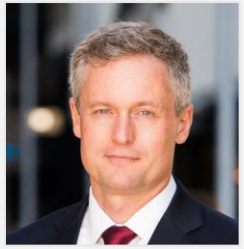
Dr Charmaine Gittelson
Board Chair

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

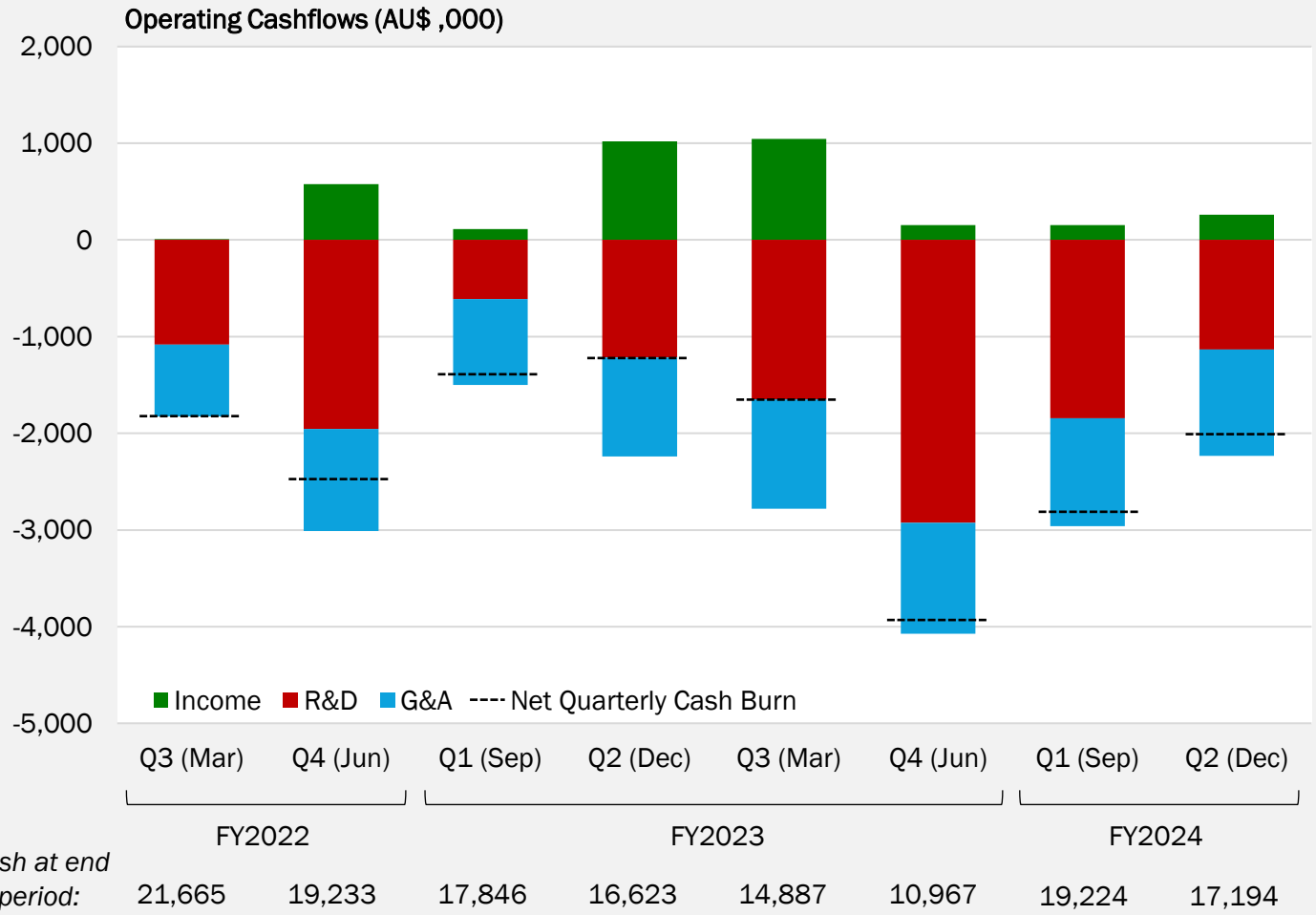
Immunologist and molecular biologist with substantial IP experience; inventor of ATL1102 in DMD



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



*Note: Financing in Q1 FY2024 provided ~$11M in net proceeds

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%

as at 31 Dec 2023

Analyst Coverage

morgans | TAYLOR COLLISON | WILSONS

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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 CY2024	
Final data from international phase IIb study of ATL1102 in Duchenne muscular dystrophy	2H CY2025	



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