PERCHERON THERAPEUTICS CORPORATE PRESENTATION

Melbourne, Australia – 4 September 2024: Percheron Therapeutics Limited (ASX: PER or "the Company"), 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.

The attached presentation supersedes the previous corporate presentation that was released to the market on 26 February 2024.

The updated presentation covers the Company's pipeline and financial position and provides a summary of recent progress, as well as enumerating upcoming milestones. In general, the Company expects to use the presentation for investor discussions and shareholder meetings.

Upcoming Conference Presentations

Percheron has been invited to present at several upcoming investor conferences.

HealthInvest 2024 Summit
18 September 2024 – Sydney, NSW
Registration: www.healthinvestlive.com

Pitt Street Research Life Sciences Conference
19 September 2024 – Sydney, NSW

Parties interested in attending should contact the respective conference organisers directly.

~ 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 authorised 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

September 2024



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.

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 **avicursen (ATL1102)**, an antisense oligonucleotide (ASO) treatment for **Duchenne muscular dystrophy (DMD)** and other diseases
  - International double-blind, placebo-controlled phase IIb trial ongoing in non-ambulant boys with DMD
  - Positive clinical data from prior single-arm phase IIa study in this population
  - ASOs are a well-validated technology with multiple FDA approved therapies
- **Avicursen is a late-stage asset with substantial commercial opportunity**
  - Approximately 300,000 DMD patients worldwide
  - Existing therapies priced up to US$750K per treatment year; total market estimated at ~US$4B per annum; ~US$10B by 2030
  - Avicursen potentially applicable to almost all DMD patients, not just those with specific genetic mutations ('mutation agnostic')
  - Potential applications for avicursen in other disease areas
- **Percheron enjoys strong corporate fundamentals**
  - Cash position @ 30 June 2024 = AU$11.9M
  - Highly-experienced Board and management team
  - Track record of successful financing in Australia with significant institutional support
  - Lean, virtual operating model



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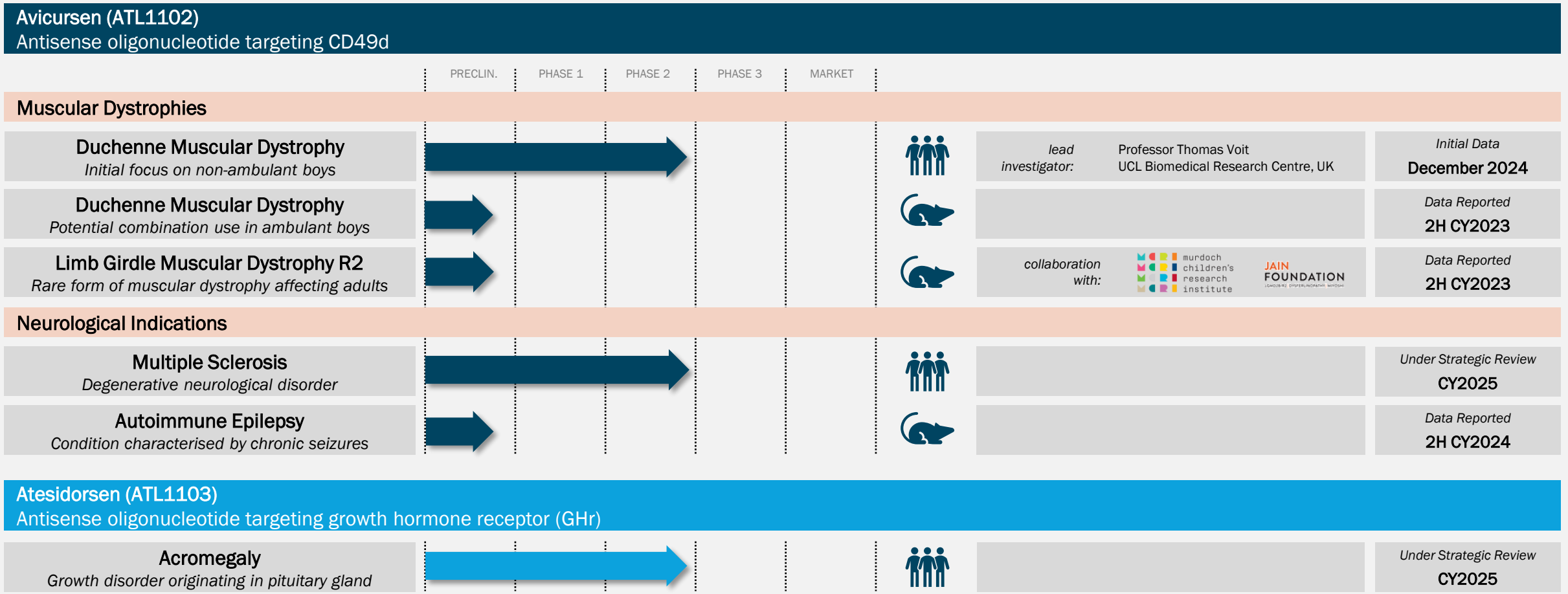
Percheron's pipeline comprises potential first-in-class assets for orphan diseases with high unmet clinical need



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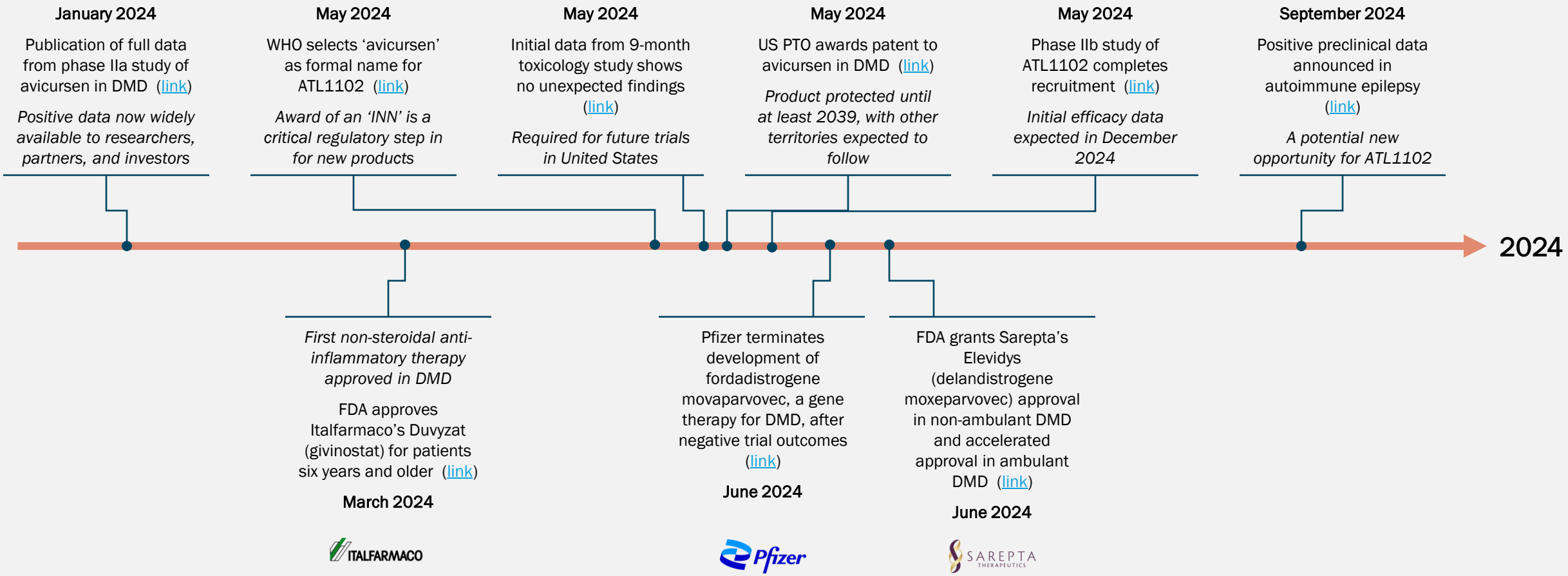
In FY2024, Percheron continued to make good progress with advancing its pipeline, against a backdrop of favourable market dynamics



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Avicursen (ATL1102)
in Duchenne Muscular Dystrophy

Phase IIb Clinical Program

Duchenne muscular dystrophy 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 linked to X-chromosome, so Muscular Dystrophy (MD) is vastly more common in boys

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

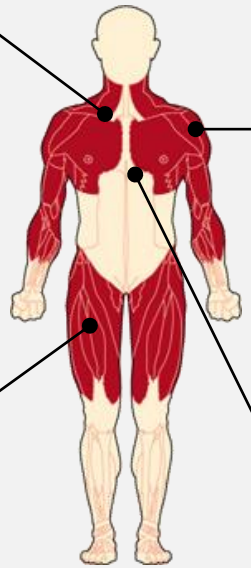
The pathology of MD is as much about inflammation as about dysfunctional dystrophin

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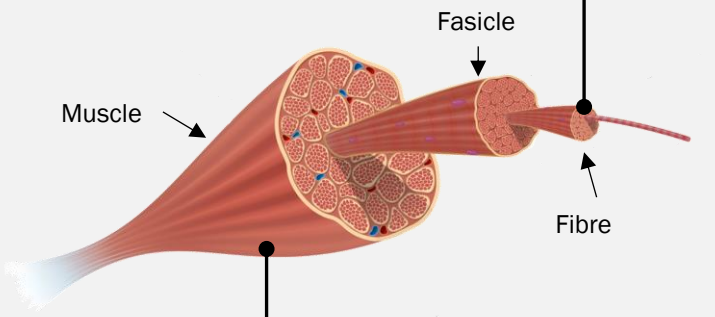
There are two fundamental approaches to the pharmacological treatment of DMD: (1) target the underlying genetic abnormality, and (2) target its effects

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

Corticosteroid therapies

Duvyzat (givinostat)

Avicursen (ATL1102)

Non-steroid therapies

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

a

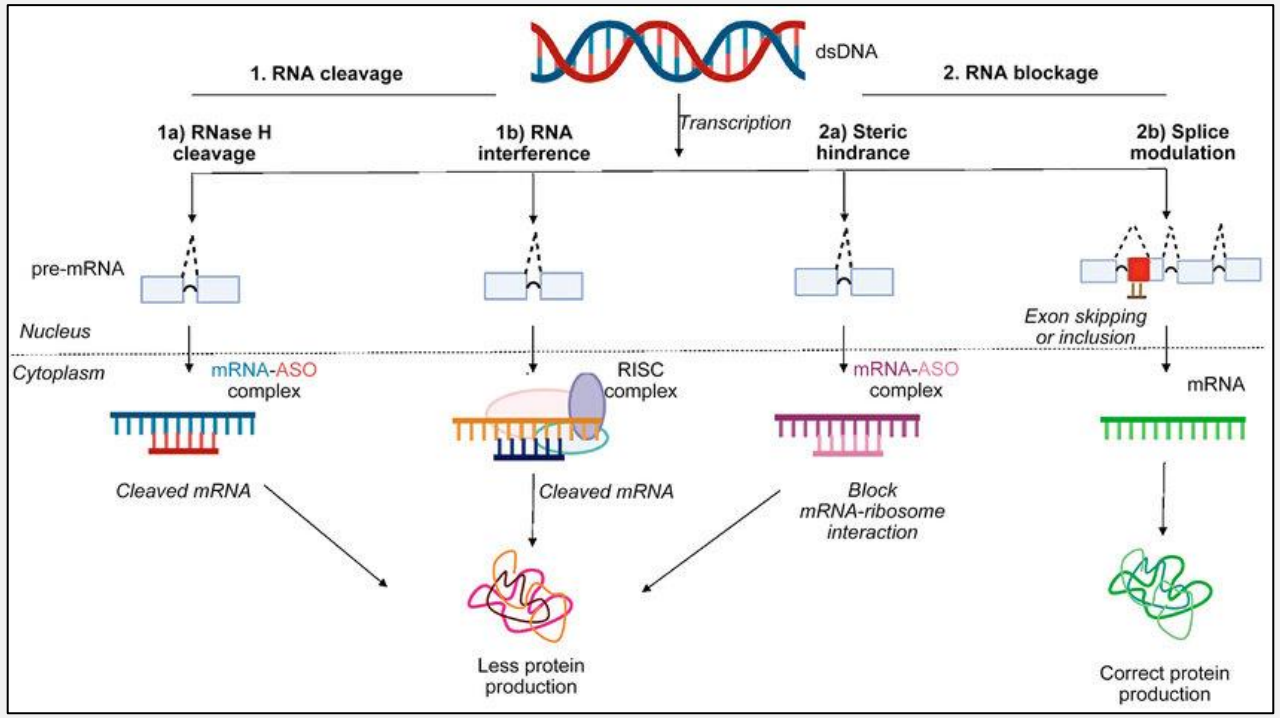
Ordinarily, the genetic code in a patient's DNA is transcribed to RNA, which is used to direct synthesis of proteins. Aberrant proteins (e.g. dystrophin in DMD) cause disease.

b

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).

c

Avicursen is an ASO which targets CD49d, a protein involved in inflammation. By cleaving mRNA and reducing synthesis of CD49d, avicursen has an anti-inflammatory action, which reduces chronic muscle tissue damage associated with DMD.



FDA-Approved Antisense Therapies (all indications)

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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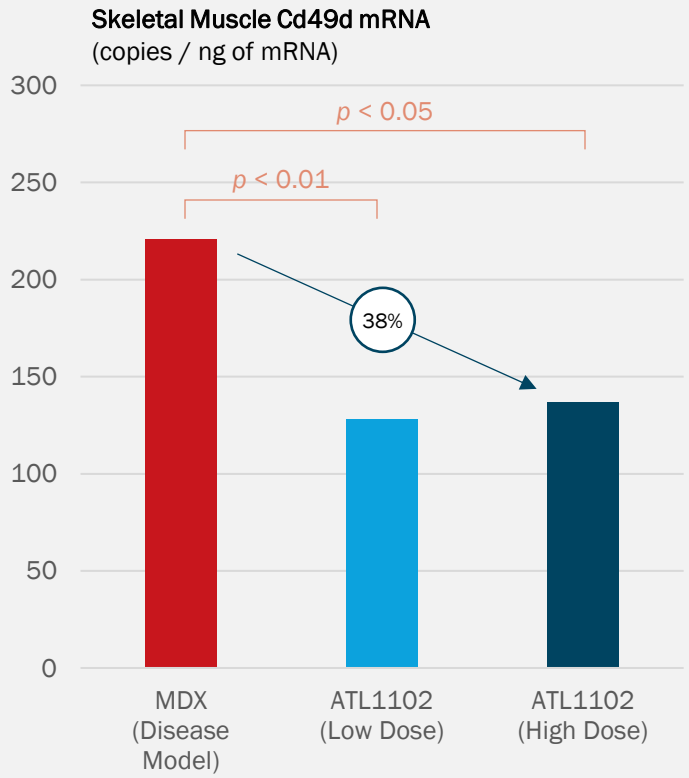
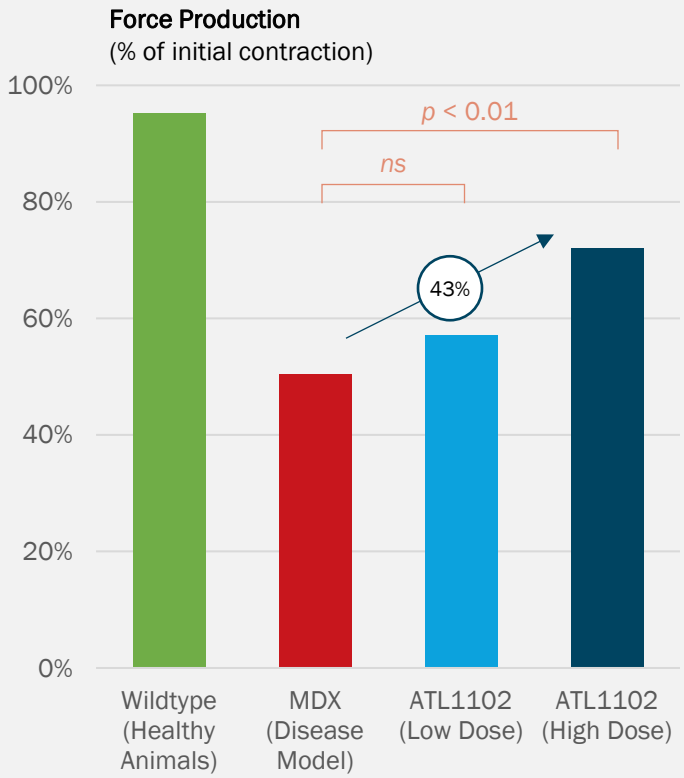
Preclinical investigation of avicursen (ATL1102) in DMD has shown it to be effective at reducing CD49d+ T-cells and improving muscle function

ATL1102 reduces CD49d in skeletal muscle...



...leading to an increase in muscle function



Key Takeaways

- Preclinical investigation of avicursen was conducted in the *mdx* mouse model – a standard research model of Duchenne muscular dystrophy – at the Murdoch Children's Research Institute in Melbourne.
- Experiments were performed using a murine analogue of avicursen, to reflect differences in the mouse immune system.
- Administration of avicursen caused a dose-dependent reduction in CD49d+ T-cells in spleen. These cells are considered a key driver of chronic muscle inflammation in DMD. The reduction was statistically significant.
- Administration of avicursen was also associated with improved muscle function, with those animals receiving the high dose of drug showing ~43% improvement in force production in the tibialis anterior muscle. These improvements were also statistically significant.

Source: adapted from IR Woodcock et al. (2024) *PLoS ONE* 19(1): e0294847

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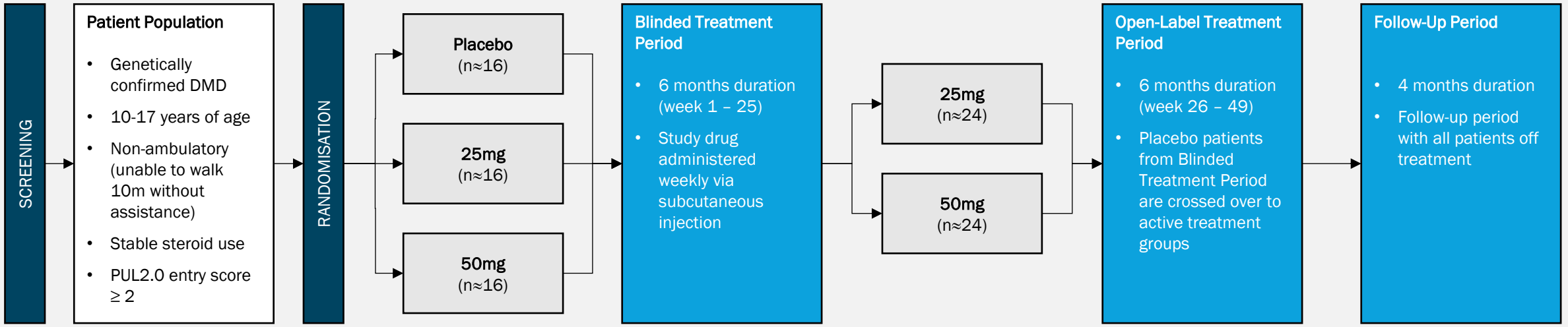
An ongoing, double-blind phase IIb clinical study has been designed to provide definitive evidence of efficacy for avicursen in non-ambulant boys with DMD



Six-month data from the ongoing phase IIb trial of avicursen 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 avicursen

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 – **avicursen 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 (2022-23)

Company	Product	2022 (US$)	2023 (US$)
Sarepta Therapeutics	EXONDYS 51 (eteplirsen) Injection	512M	541M
Sarepta Therapeutics	AMONDYS 45 (casimersen) Injection	215M	274M
Sarepta Therapeutics	VYONDYS 53 (golodirsen) Injection	117M	130M
PTC Therapeutics	Emflaza (deflazacort)	218M	255M
NS Pharma	Viltepso (viltolarsen) injection	106M	121M
Sarepta Therapeutics	Elevidys delandistrogene moxeparvovec-rokl	-	200M

~US$ 1.5B in annual sales with rapid growth

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 avicursen pricing at ~US$ 200-300K per patient per year

Avicursen Addressable Market

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: GlobalData; 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 Therapeutics 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 SOLID Biosciences	PTC Therapeutics — translarna (ataluren)	Sarepta Therapeutics — Elevidys (delandistrogene moxeparvovec-rokl)
Anti-Inflammatory and anti-Fibrotic Therapies *Anti-inflammatory therapies seek to reduce the damage caused to muscle tissue by dysfunctional dystrophin*		percheron Therapeutics — avicursen 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, costs US$3.2M per dose (single dose per patient) and does not provide curative benefit

Avicursen and CAP-1002 are the only late-stage assets in development for DMD, with the latter increasingly focused on the cardiac effects of the disease

Avicursen appears potentially competitive with existing therapies, which have generally shown limited evidence of functional benefit

Exon Skipping Therapies

Viltepso (viltolarsen) injection
EXONDYS 51 (eteplirsen) Injection
VYONDYS 53 (golodirsen) Injection
AMONDYS 45 (casimersen) Injection

- ESTs each only applicable to 8-12% of patients
- ESTs require weekly iv infusion (in a doctor's office)
- Have never demonstrated functional benefit in a randomised trial

Gene Therapies

Elevidys delandistrogene moxeparvovec-rokl

- Around 15% of patients are ineligible for GT due to anti-adenoviral antibodies
- Prohibitive cost (US$3.2 million)
- Have never demonstrated functional benefit in a randomised trial

Steroids

Emflaza (deflazacort)
aGamree (vamorolone)

- Potential significant side effects include alterations in hormonal function, increased risk of infection, alterations in heart and kidney function, behavioural and mood disturbances, osteoporosis, skin rashes, and growth retardation

Non-Steroidal Anti-Inflammatory Therapies

Duvyzat (givinostat)

- Potential significant side effects include thrombocytopaenia, myelosuppression, increased triglycerides, gastrointestinal disturbances (diarrhoea, nausea, vomiting, abdominal pain), and changes in heart rhythm

Emerging Avicursen Profile

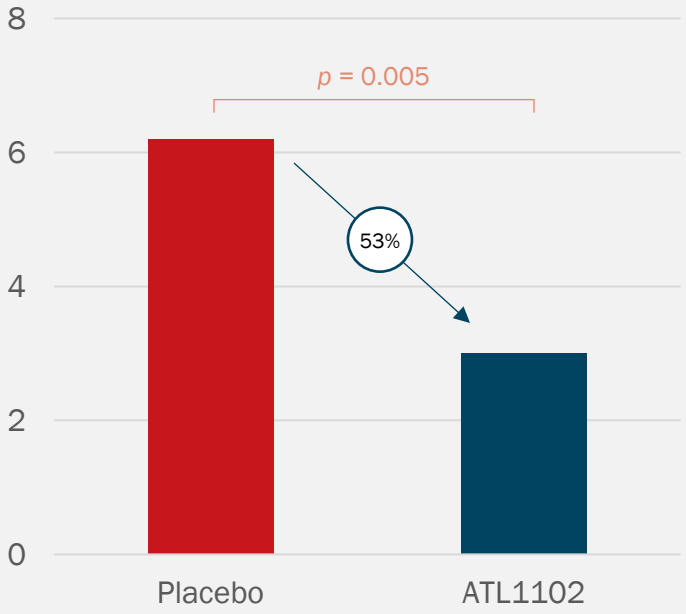
- 'Mutation agnostic' – likely applicable to almost all patients with DMD
- Positive signals of functional benefit from phase IIa study; awaiting data from ongoing phase IIb randomised trial
- Key toxicity observed to date is injection site reactions
- Weekly subcutaneous injection, which can be administered at home by a carer
- Cost likely to be highly competitive with existing therapies
- Likely amenable to combination with existing therapies, or accretive to gene therapies such as Elevidys

Source: FDA-approved prescribing information

Data in a broad range of other indications validates avicursen's mechanism and points to potential future expansion opportunities

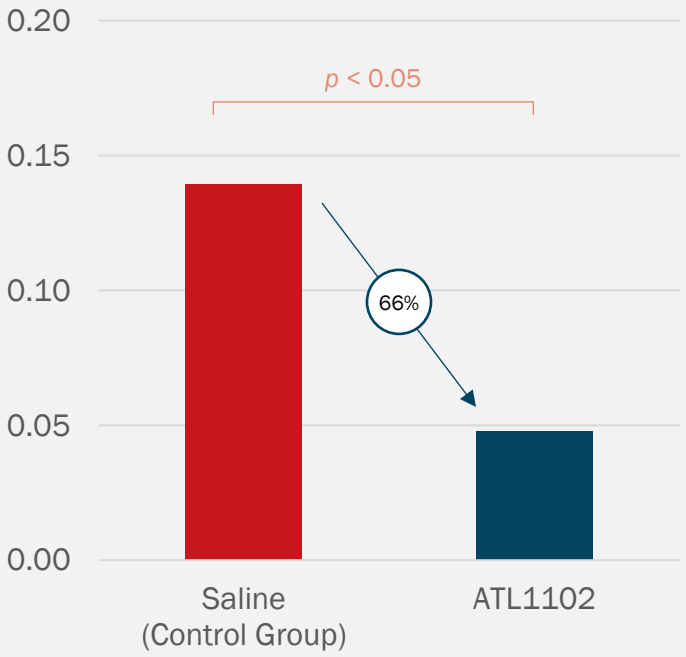
Multiple Sclerosis (clinical)

Cumulative Number of New Active Lesions
@ week 12



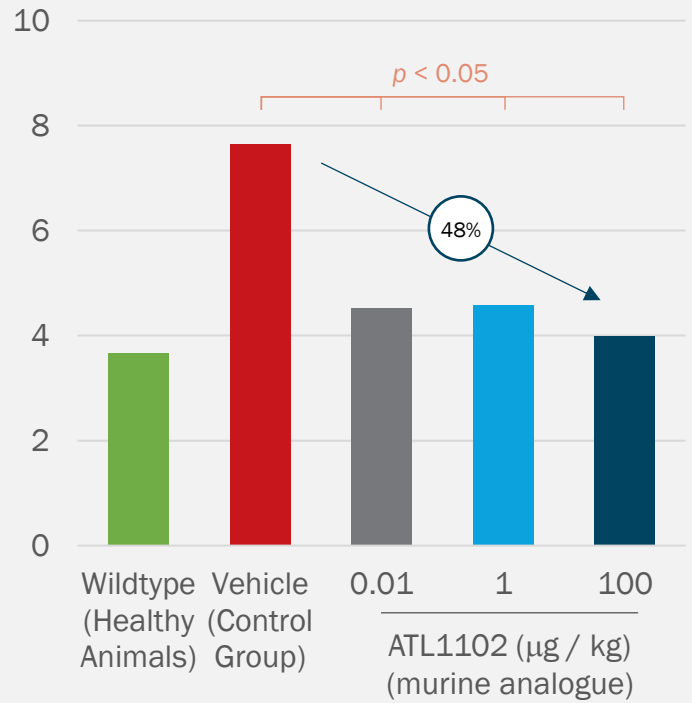
Autoimmune Epilepsy (preclinical)

Number of Seizures per hour
(day 31-43 in a pilocarpine-induced seizure model)



Asthma (preclinical)

Penh (measure of airway hyperresponsiveness)
(effect of aerosolised drug in OVA asthma model)



Source: V Limmroth et al. (2014) *Neurology* 83:1780–1788; company data on file
Note: references to ATL1102 in preclinical experiments are to a murine version of the drug, to reflect differences in sequences between humans and mice

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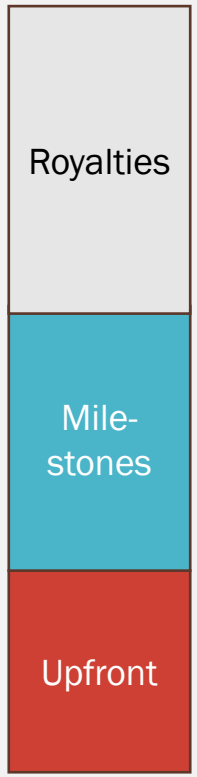
The company has initiated a broad outreach program to identify and cultivate potential future partners for avicursen

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 is just as important as the economic terms of a transaction

Source: DealForma; Percheron analysis

Partnering opportunity for avicursen 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

Corporate Profile

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



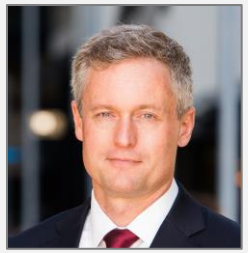
Dr Charmaine Gittelson
Board Chair

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



Deborah Ambrosini
Chief Financial Officer

Over 20 years of strategic financial experience with a diverse range of ASX-listed companies



Dr Cathryn Clary
Chief Medical Advisor

25 years of drug development experience in big pharma and small biotech



Dr Anthony Filippis
Chief Operating Officer

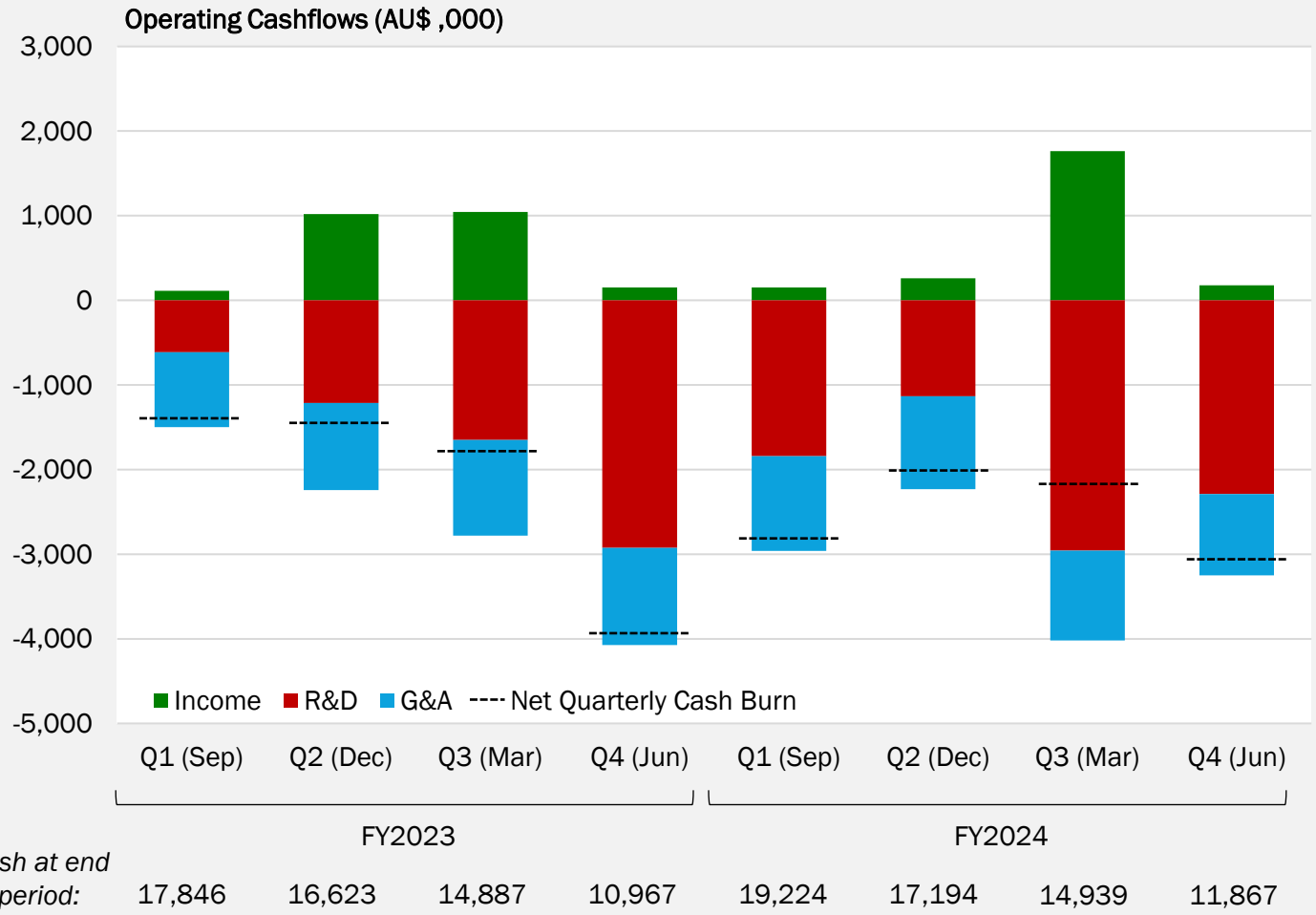
25 years of life sciences leadership experience, with a focus on BD, corporate strategy, and operations



Dr George Tachas
Principal Scientist

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

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



Corporate Fundamentals	
Market Capitalisation:	~AU$ 77M
Primary Listing:	ASX: PER
Secondary Listings:	FSE: AWY; OTC: ATHJY
Shares on Issue:	~900 Million
Average Daily Trading (FY24):	~AU$ 50K

Financial Position	
Cash Balance (30 Jun 24):	AU$ 11.9 million
Runway:	FY2025

Substantial Shareholders	
Platinum Asset Management	10.7%

Analyst Coverage

morgans
WILSONS
TAYLOR COLLISON
BELL POTTER

percheron THERAPEUTICS

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 avicursen in Duchenne muscular dystrophy	1H 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 avicursen in Duchenne muscular dystrophy	1H CY2024	✓
Initial data from international phase IIb study of avicursen in Duchenne muscular dystrophy	December 2024	
CY2025		
FDA consultation to discuss registration pathway for avicursen	1H CY2025	
Strategic review of ex-DMD avicursen opportunities and atesidorsen	2H CY2025	
Final data from international phase IIb study of avicursen in Duchenne muscular dystrophy	2H CY2025	



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