AGM PRESENTATION

Melbourne, Australia – 21 November 2024: In advance of this morning's Annual General Meeting of shareholders to be held at 10:00am, and in accordance with ASX Listing Rule 3.13.3, Percheron Therapeutics Limited (ASX: PER or 'the Company') is pleased to provide a copy of the presentation that will be made to shareholders at the Annual General Meeting.

About Percheron Therapeutics Limited

Percheron Therapeutics Limited [ASX: PER | US OTC: ATHJF] is a publicly listed biotechnology company focused on the development and commercialisation of novel therapies for rare diseases. The company's lead program is avicursen (ATL1102), an antisense oligonucleotide targeting the CD49d receptor. Avicursen 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 December 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 Company Secretary.

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2024 – A Transformative Year

Presentation to Annual General Meeting of Shareholders

Dr James Garner
Chief Executive Officer

Melbourne, VIC
21 November 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.

Agenda

- 2024 in Review
- December Data Read-Out
- Looking Ahead to 2025

Key Highlights
2024

	Full recruitment to international phase IIb randomised, placebo-controlled clinical trial of avicursen in DMD, with initial six-month data expected in December 2024
	Full completion of nine-month primate toxicology study, laying the path to regulatory discussion in CY2025
	Publication of positive phase IIa clinical trial of avicursen in a high-impact peer-reviewed scientific journal, providing exposure for our data to clinicians, investors, and partners
	Release of new data for avicursen in autoimmune epilepsy, suggesting a potential additional future indication for the drug
	Patent granted by US Patent & Trademark Office, providing exclusivity over avicursen in DMD until at least 2039, with potential for a patent term extension of up to five years thereafter
	Participation in three international scientific conferences, three patient advocacy meetings, and two industry partnering conferences
	Extensive investor relations campaign, including invited presentations at Wilsons and Bell Potter healthcare conferences, invited presentation to Cantor Fitzgerald rare diseases event, multiple Open House meetings, and non-deal roadshows
	Three Clinician Advisory Boards held to familiarise key opinion leaders with avicursen, understand commercial positioning of new therapies in DMD, and discuss potential strategies for clinical development and regulatory engagement

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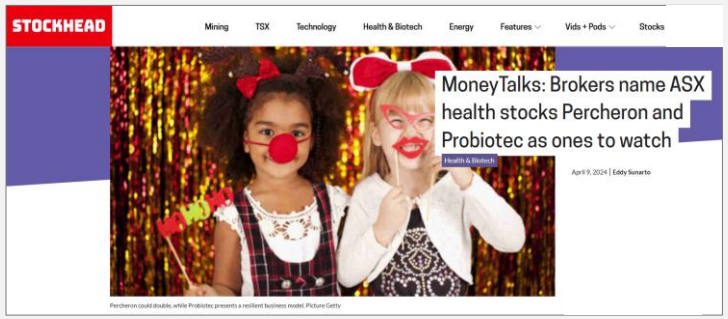
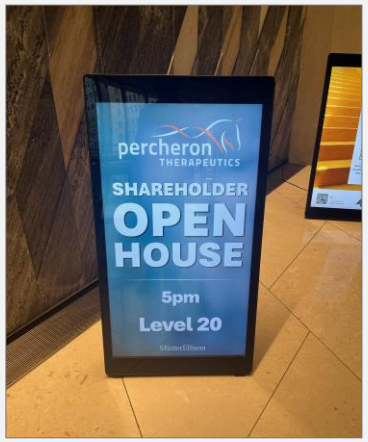
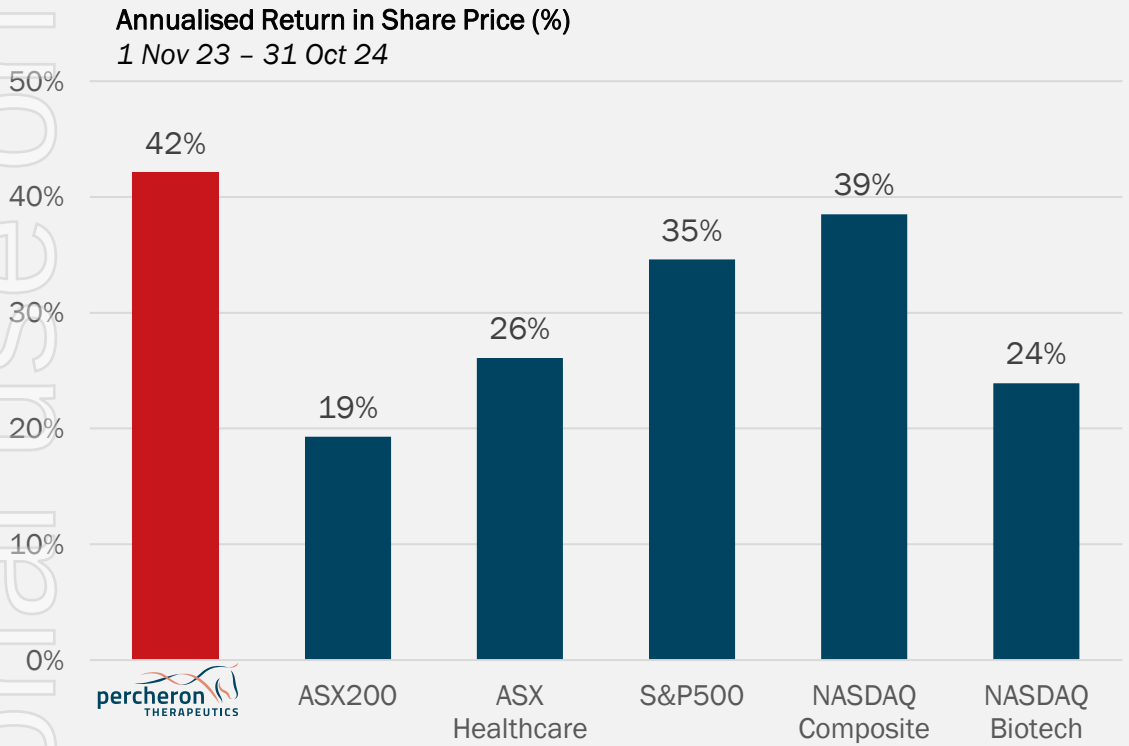
The year has been characterised by extensive engagement with clinicians, investors, and patient advocacy





The company's successes have been reflected in market performance

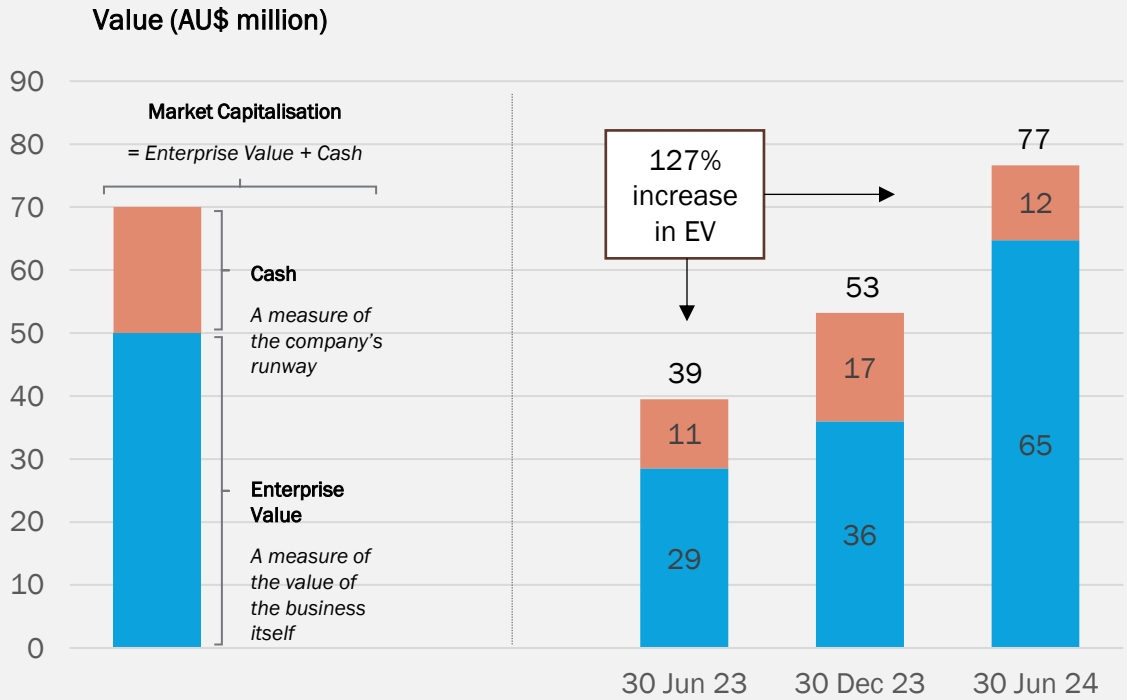
Year-to-Date Return Exceeds All Comparable Indices



1 Nov 23	0.057	6,838	35,254	4,238	13,061	3,754
31 Oct 24	0.081	8,155	44,464	5,703	18,086	4,649

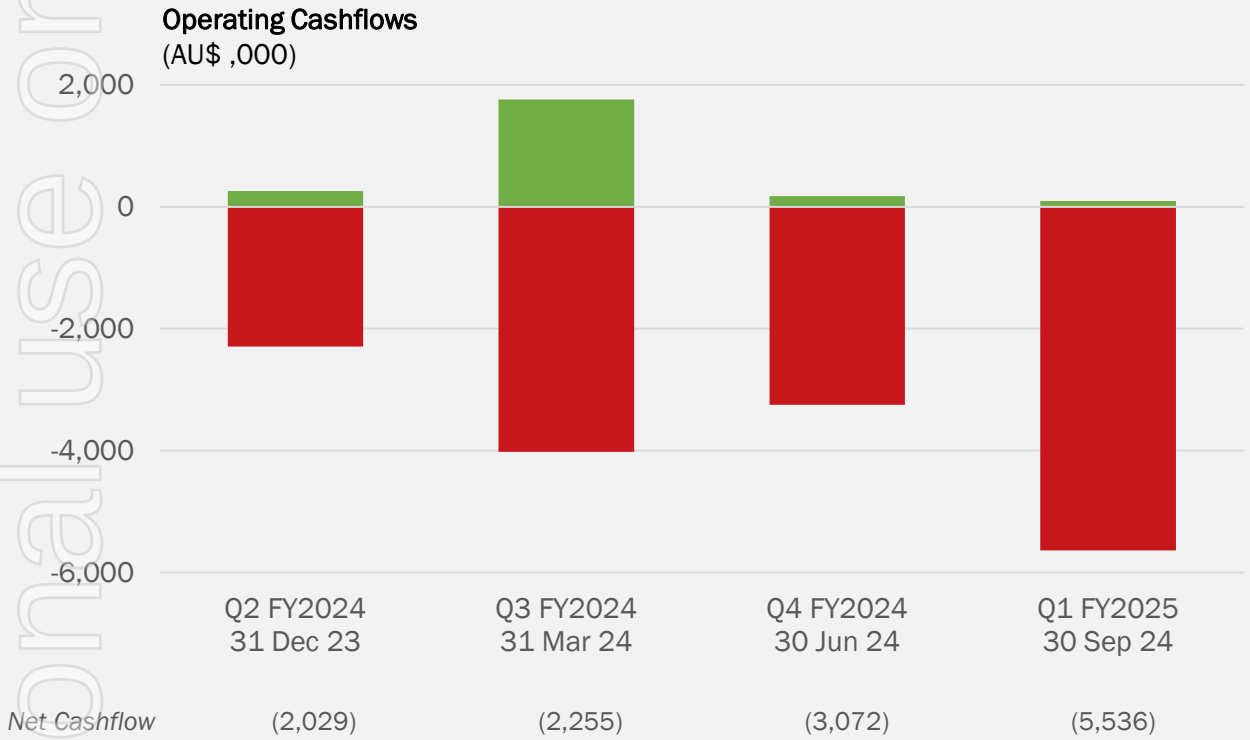
Source: Bloomberg; Company financial filings

Enterprise Value has More Than Doubled in FY2024



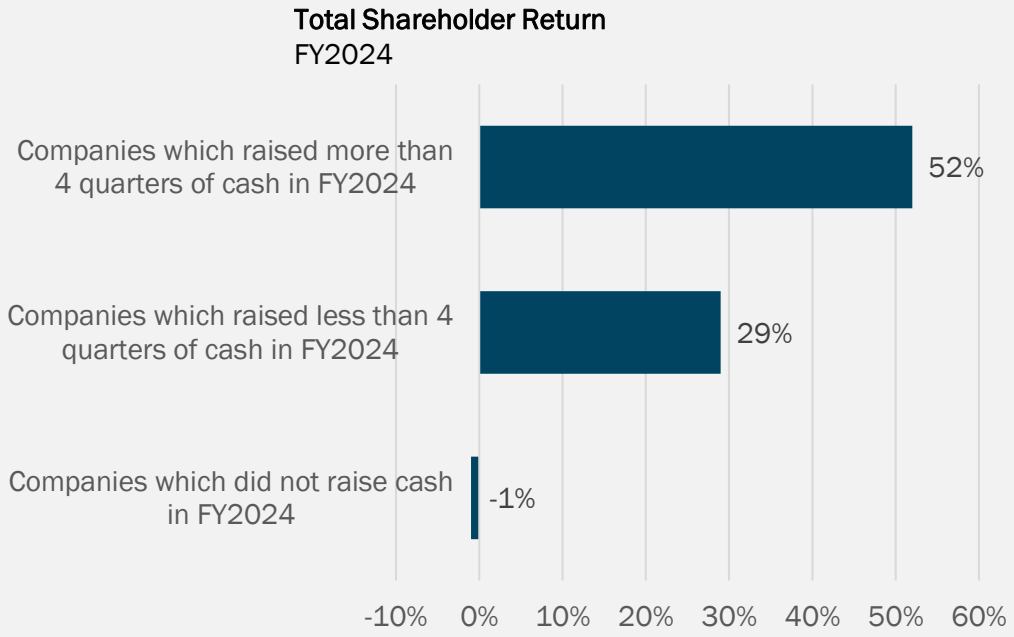
Percheron's capital raise in October 2024 leaves the company funded into CY2026

At average historical spend, funds on hand provide cash into CY2026



	Q2 FY2024 31 Dec 23	Q3 FY2024 31 Mar 24	Q4 FY2024 30 Jun 24	Q1 FY2025 30 Sep 24
Net Cashflow	(2,029)	(2,255)	(3,072)	(5,536)

Companies with good access to capital have outperformed in FY2024



Note: universe comprises ASX-listed healthcare companies with <$10M revenue and <$200M market capitalisation

Source: Percheron Appendix 4C filings; Percheron analysis

2024 has been an active year in the DMD field, with a number of developmental therapies seeing important milestones

January 2024

Solid Biosciences receive FDA orphan designation for SGT-003, an early-clinical stage gene therapy in DMD (link)

January 2024

SRP-5051 ('next generation Exondys'), shows favourable risk-benefit profile in phase II MOMENTUM study (link)

March 2024

FDA approves Italfarmaco's Duvyzat (givinostat) for patients six years and older (link)

April 2024

Long-term observational data suggests survival benefit for Exondys 51 (eteplirsen) in DMD (link)

May 2024

NS Pharma's Viltepso (viltolarsen), an exon-skipping therapy, fails to meet primary endpoint in phase III study in DMD (link)

June 2024

Pfizer terminates development of fordadistrogene movaparvovec, a gene therapy for DMD, after negative trial outcomes (link)

June 2024

First generic deflazacort product approved by FDA for DMD – an oral suspension form of the drug (link)

June 2024

FDA grants Sarepta's Elevidys (delandistrogene moxeparvovec) approval in ambulant DMD and accelerated approval in non-ambulant DMD (link)

September 2024

Capricor Therapeutics signs binding term sheet to license European rights to deramiocel (CAP-1002) to Nippon Shinyaku (link)

September 2024

Dyne Therapeutics reports positive data from DYNE-251 exon 51 exon-skipping therapy and initiates pivotal study recruitment (link)

October 2024

Capricor presents positive data from HOPE-2 OLE extension study of deramiocel in DMD at WMS annual meeting (link)

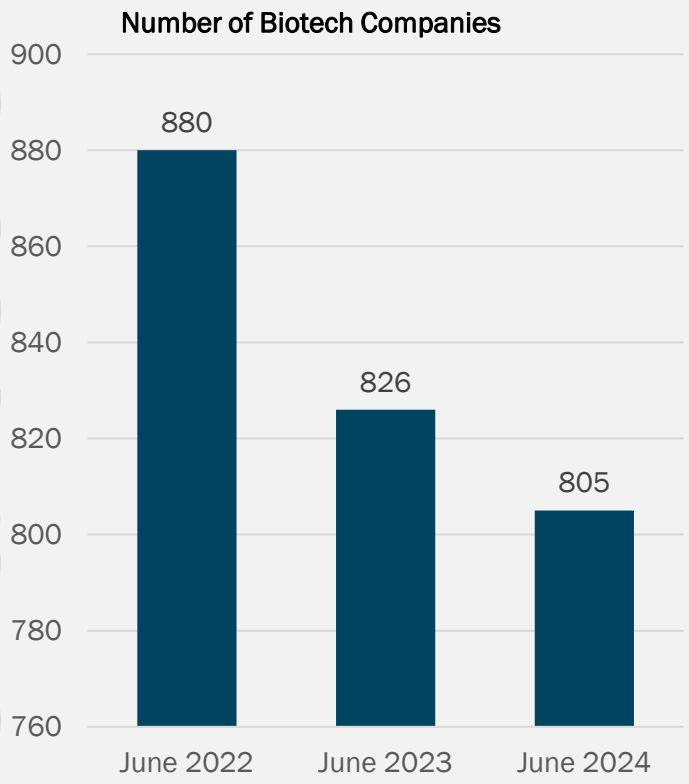
October 2024

EMA's CHMP reiterates recommendation not to renew approval of PTC Therapeutics' Translarna (ataluren) for DMD (link)

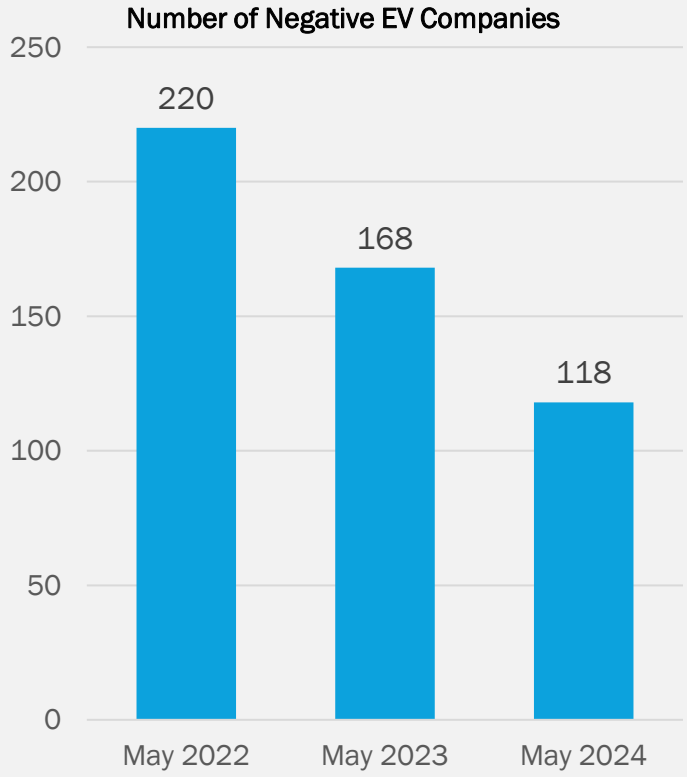
Source: Company news releases

The broader environment remains challenging for biotech, but there are green shoots of recovery among more substantial companies

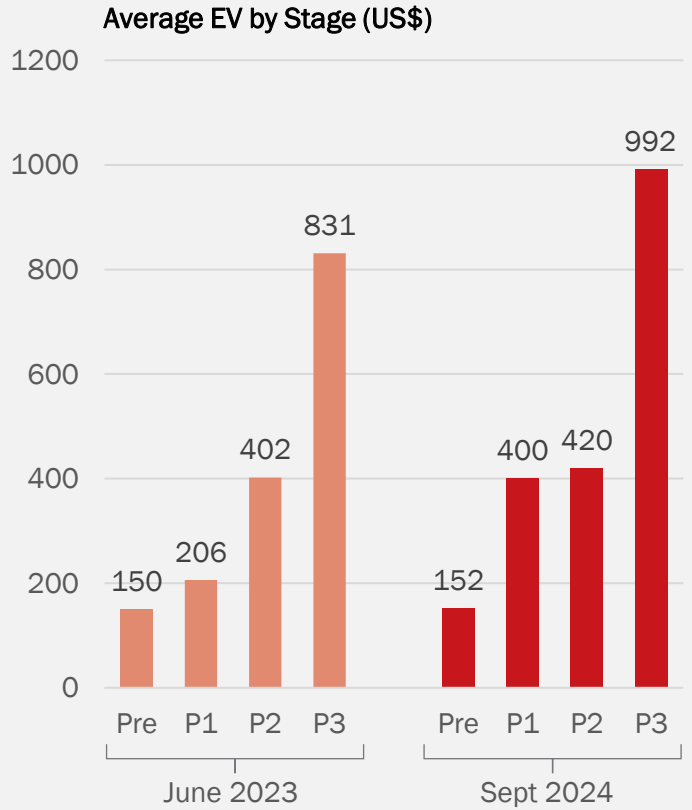
Total number of biotech companies is declining...



...but attrition is worst in companies with negative enterprise value...



...and late-stage companies are being well valued



Source: Stifel analysis; Note: figures shown are for NASDAQ-listed, development-stage biotech companies

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Agenda

- 2024 in Review
- December Data Read-Out
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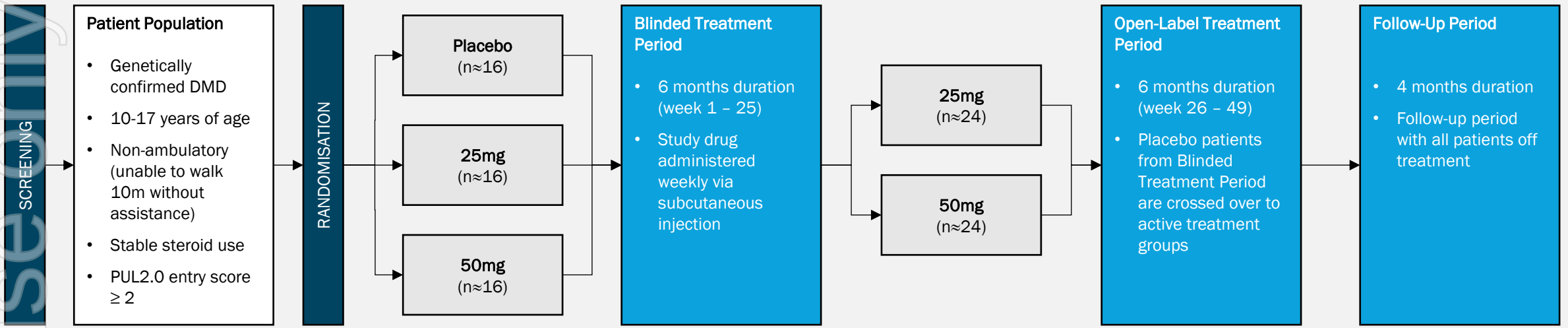
The ongoing phase IIb study of avicursen (ATL1102) in DMD will provide an initial six-month read-out in December 2024



Initial Readout December 2024

Primary Endpoint

- PUL2.0 score for avicursen vs. placebo

Secondary Endpoints

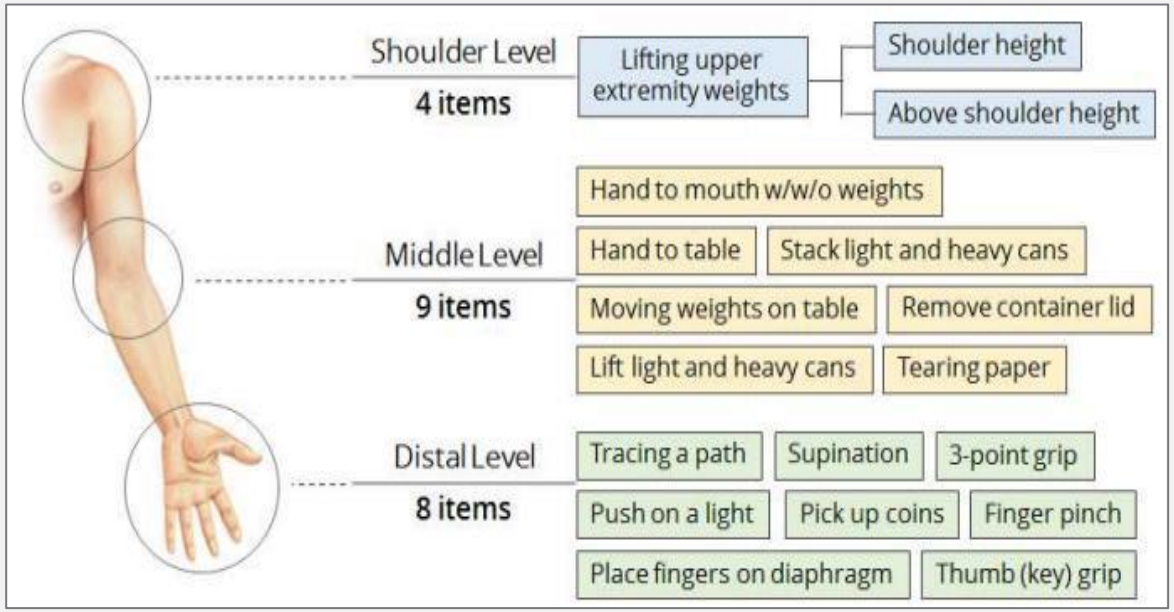
- PUL2.0 Component Domains
- MyoGrip and MyoPinch
- Respiratory Function
- Quality of Life
- Safety and Tolerability

Secondary Endpoints

- Analysis of all endpoints for Weeks 26 – 49 and for Weeks 0 – 49

The December 2024 read-out will be the first read-out from the study, but further data will follow over the course of CY2025

- December read-out will be ‘top-line’ data: a summary of efficacy, safety, and patient demographics
  - Detailed information, including secondary outcomes such as pharmacokinetic data, will follow in 1H CY2025
- Top-line data will capture the first six months of randomised, double-blind study treatment for all 48 patients
  - Even though some patients will have been in the study much longer, only the first six months will be reported at this read-out
- Percheron remains ‘blinded’ until the last moment
  - We do not have any visibility into the blinded study outcomes before we receive data from the contract research organisation
  - It may take time for us to fully interpret and contextualise the data, so there will be more to discuss during CY2025
- 12-month data will be reported in mid-CY2025, and final 16-month data will be reported in 2H CY2025
  - The December read-out is only the first of several key read-outs from the study
- Beneath the headline figures, the ‘totality of the evidence’, and the overall impact on the wellbeing of the child, is a key consideration for regulatory agencies

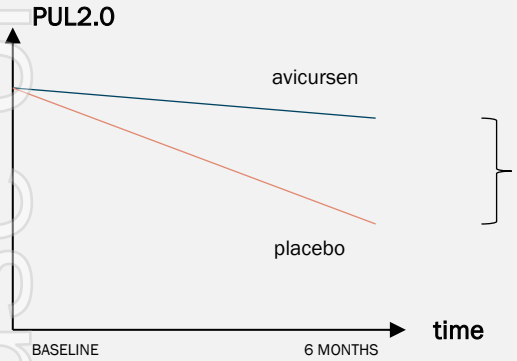


Components of PUL2.0 Assessment

The 6-month read-out will begin to answer a number of key questions for avicursen in DMD (1/3)

1

The study aims to detect a difference in PUL2.0 score between avicursen and placebo



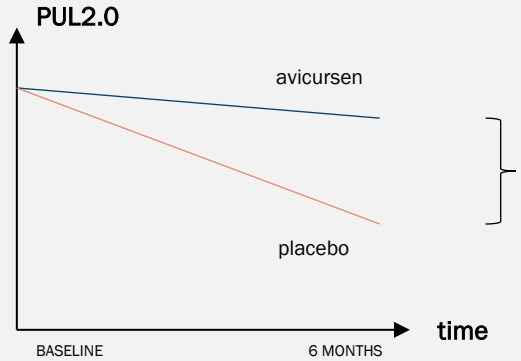
ILLUSTRATIVE ONLY

What is the difference in PUL2.0 score between avicursen and placebo at six months?

Select Comparison Points	
Percheron phase IIa study (n=9)[1] (*ATL1102 versus historical control – post hoc analysis*)	2.9
Capricor HOPE-2 study of deramiocel (CAP-1002) (n=20)[2] (*CAP-1002 versus placebo*)	1.8
US clinician feedback (*via Percheron October 2024 advisory boards*) on minimum threshold to be considered a positive result	1.5

2

Is the difference statistically significant?



ILLUSTRATIVE ONLY

What is the 'p-value' for the observed difference? A value of <0.05 is considered 'statistically significant'

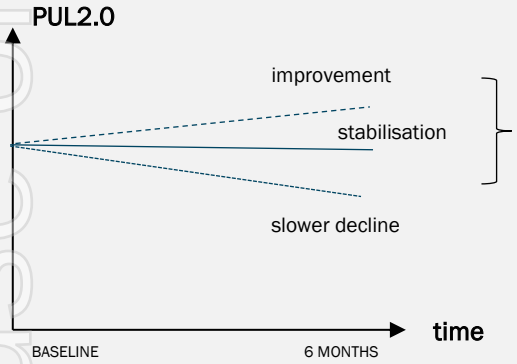
Factors That Can Influence the P-value

1. **Number of patients in the study** –sample size was based on the previous phase IIa study and was powered to detect a difference in PUL2.0 at 6-months of 2.7 or more.
2. **Time under observation** – clinical changes often become more significant over time and the 12-month data in mid-CY2025 may provide higher resolution.
3. **Reliability of the measurement system** – if there is a lot of variability in a clinical measurement, it can reduce the statistical significance of an observed effect.

Sources: [1]IR Woodcock et al. (2024) *PLoS ONE* 19(1): e0294847; [2]https://fintel.io/doc/sec-capr-capricor-therapeutics-ex991-2020-march-11-18397-361

The 6-month read-out will begin to answer a number of key questions for avicursen in DMD (2/3)

3 What is the trajectory of avicursen-treated patients?

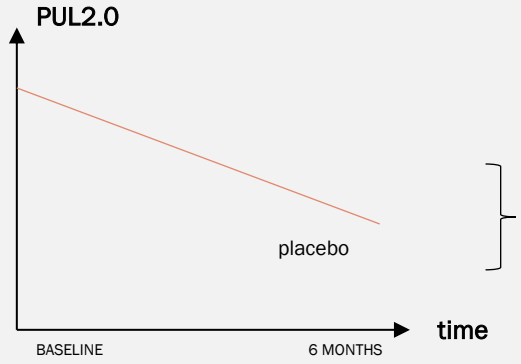


ILLUSTRATIVE ONLY

Irrespective of placebo trajectory, are the avicursen patients broadly stable over six months? The course of the disease is such that patients inevitably worsen over time.

Average PUL2.0 Loss Over 12 Months by Ambulation Status[1]		
< 12 months since loss of ambulation	Average age: 12.98 years	-4.20
12 – 24 months since loss of ambulation	Average age: 14.32 years	-2.82
2 – 5 years since loss of ambulation	Average age: 16.17 years	-1.92
> 5 years since loss of ambulation	Average age: 19.66 years	-0.93

4 Does the placebo group resemble placebo groups in comparable studies, and the historical control in the phase IIa study?



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Do the placebo treated patients follow a similar course to comparator groups in other studies of non-ambulant boys?

Select Comparison Points – Placebo Group PUL2.0 Loss Over 12 Months	
Fibrogen MISSION study of pavrelumab (n=21)[2] (*Historical control based on Mayhew dataset*)	-2.2
Capricor HOPE-2 study of deramiocel (CAP-1002) (n=10)[3] (*CAP-1002 versus placebo*)	-2.1
Percheron phase IIa study (n=20) (*Historical control based on Mercuri dataset*)	-2.0

Sources: [1]M Pane et al. (2023) *J Neuromuscul Dis.* 10(4):567–574; [2]AM Connolly et al. (2023) *J Neuromuscular Dis.* 10(4):685-699; [3]https://fintel.io/doc/sec-capr-capricor-therapeutics-ex991-2020-march-11-18397-361

The 6-month read-out will begin to answer a number of key questions for avicursen in DMD (3/3)

5 How does the drug perform in the secondary endpoints?

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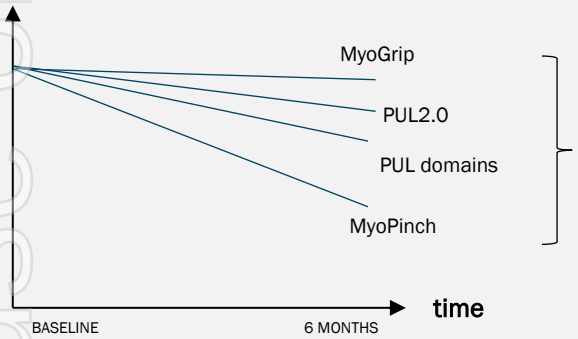

Does the 'balance of the evidence' point to a treatment effect, or are the endpoints directionally inconsistent?

Select Comparison Points – Basis of Approval		
EXONDYS 51 (eteplirsen) Injection	SAREPTA THERAPEUTICS	Approved on surrogate (secondary) endpoint of "dystrophin increase in skeletal muscle in some patients."
aGamree (vamorolone)	santhera	Approved on basis of "comparable efficacy [*to prednisolone*], with data suggesting a reduction in adverse events."
Elevidys delandistrogene moxeparvovec-rokl	SAREPTA THERAPEUTICS	Approved on endpoint of microdystrophin expression which is "reasonably likely to predict clinical benefit"

6 How does the safety profile of avicursen appear, in the context of approved DMD therapies?

Safety Considerations with Approved DMD Therapies	
Exon Skipping Therapies EXONDYS 51 (eteplirsen) Injection Viltepso (viltolarsen) injection VYONDYS 53 (golodirsen) Injection AMONDYS 45 (casimersen) Injection	Balance disorder, vomiting, contact dermatitis, joint pain, rash, and upper respiratory tract infection
Gene Therapies Elevidys delandistrogene moxeparvovec-rokl	Liver injury, thrombocytopaenia, as well as vomiting, nausea, fever, and anaphylactic reactions
Steroids Emflaza (deflazacort) aGamree (vamorolone)	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 Therapies Duvyzat (givinostat)	Thrombocytopaenia, myelosuppression, increased triglycerides, gastrointestinal disturbances (diarrhoea, nausea, vomiting, abdominal pain), and changes in heart rhythm

Source: US Food & Drug Administration; FDA-approved prescribing information

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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 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

Source: DealForma; Percheron 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 is just as important as the economic terms of a transaction

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We have five key objectives for FY25

1. **Complete phase IIb clinical trial of avicursen in DMD**
   - Key data readouts in December 2024 (6-month data), mid-CY2025 (12-month data), and 2H-CY2025 (16-month data)

2. **Lift clinical hold with FDA and define a path-to-market for avicursen in DMD**
   - Regulatory consultation in 1H CY2025

3. **Partner avicusen with one or more companies that are deeply committed to commercial success**
   - Outreach in 1Q CY2025, with 6-month data in hand, to build on discussions initiated during CY2024

4. **Optimise our pipeline to manage shareholder value**
   - Evaluate avicursen in other indications and pursue most value-adding opportunities

5. **Ensure company is well-financed**
   - Provide sufficient funds to complete phase IIb trial and to pursue above objectives

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 six-month 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	
Twelve-month data from international phase IIb study of avicursen in Duchenne muscular dystrophy	Mid-CY2025	
Strategic review of ex-DMD avicursen opportunities and atesidorsen	1H-2H CY2025	
Final data from international phase IIb study of avicursen in Duchenne muscular dystrophy	2H CY2025	

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