# QUARTERLY ACTIVITIES REPORT AND APPENDIX 4C

**Melbourne, Australia – 31 July 2024:** Percheron Therapeutics Limited (ASX:PER, "Percheron" or "the Company"), an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to provide an update on the Company's continuing progress during the quarter ended 30 June 2024.

**Key Points**

- **Phase IIb clinical trial of ATL1102 in Duchenne muscular dystrophy (DMD) completes recruitment.** A total of 48 boys were randomised at 16 hospitals in five countries over a period of approximately 11 months. Initial data is expected in December 2024.

- **Preliminary results from ATL1102 nine-month animal toxicology study show no unexpected findings.** Certain analyses remain ongoing, with final data expected in 2H CY2024.

- **World Health Organization (WHO) awards provisional international non-proprietary name (pINN) of 'avicursen' to ATL1102.** Name expected to be definitively confirmed in 2Q CY2025.

- **Key patent granted for ATL1102 in DMD in United States.** Patent provides exclusivity until at least 2039.

- **Two senior executive appointments strengthen Percheron management team**. Dr Cathryn Clary appointed Chief Medical Advisor, and Deborah Ambrosini appointed Chief Financial Officer.

"We have made excellent progress this quarter," commented Percheron CEO, Dr James Garner. "In particular, completion of recruitment to the phase IIb clinical trial of ATL1102 in DMD now places the company on a direct path to an initial data readout in December 2024. Meanwhile, we have completed a substantial part of the analysis of the nine-month animal toxicology study and have seen results very much in line with previous studies. The team has been continuing to work in the background on optimising ATL1102 for a potential future marketing approval, and the award of a pINN by WHO is a meaningful milestone on that journey. Additionally, we have been active participants in several recent international conferences, including the BIO Annual Convention and the Parent Project Muscular Dystrophy Annual Meeting, and these events have provided excellent opportunities to broaden the company's international reach. It has also been a great pleasure to welcome two new colleagues to the team. Cathryn and Deborah are already making substantial contributions to the business."

## ATL1102 Phase IIb Clinical Trial Completes Recruitment

In May 2024, the Company announced that it had completed recruitment to an international phase IIb randomised controlled trial of ATL1102 in non-ambulant boys with DMD. In total, the study recruited 48 boys across 16 sites in five countries, over a period of approximately 11 months.

On enrolment, all boys are randomised to one of two doses of ATL1102 (25mg or 50mg) or placebo. After six months, they are assessed via the performance in the upper limb module, version 2.0 (PUL2.0), which is the primary endpoint of the study. Secondary endpoints include grip strength and safety and tolerability. After six months, patients in the placebo group are re-randomised to one of the two active arms, such that all patients receive treatment on the study. Patients are assessed again at 12 months, and then finally at 16 months, after a four-month period off-treatment.

The Company expects to report topline six-month data in December 2024. At this stage, 12-month data is expected in mid-CY2025, and final data by end of CY2025.

## Preliminary Results from ATL1102 Nine-Month Toxicology Study Are Broadly Consistent with Previous Toxicology Studies

In May 2024, the Company provided an update on an ongoing nine-month GLP toxicology study in non-human primates. The study had previously been identified by the US FDA as a prerequisite, under most circumstances, for clinical trial activity or marketing authorisation in the United States.

The study commenced dosing animals in March 2023 and completed on schedule in December 2023. The majority of animals were then submitted for detailed pathological analysis, while a proportion of 'recovery animals' continued in the study for a further six months without receiving additional doses of ATL1102. These recovery animals completed participation in June 2024 and are now also undergoing pathological analysis.

While some investigations remain ongoing, initial data is broadly consistent with previous animal toxicology studies, and no new toxicities attributable to ATL1102 have been identified. The Company expects to discuss the final data with its advisors as it becomes available, with a view to approaching FDA thereafter.

## World Health Organization Awards Provisional International Non-Proprietary Name of 'Avicursen' to ATL1102

In May 2024, the Company was notified by the WHO that 'avicursen' had been selected as the provisional international non-proprietary name (pINN) of ATL1102.
The name is expected to be published by WHO in the 132nd list of Proposed International Nonproprietary Names in January 2025. After publication, a proposed INN is subject to a four-month period for comment before it is confirmed.

In general, novel medicines begin their development with an internal code number that is chosen by the sponsor company. During development, and prior to an application for marketing authorisation, the company applies to WHO for an INN. The INN is chosen by WHO according to detailed guidelines and is thereafter used in reference to any pharmaceutical product containing that active ingredient. At the time of marketing authorisation, sponsor companies select a commercial brand name, which is typically trademarked.

The company intends to begin deploying the pINN in early CY2025 and will thereafter gradually phase out the ATL1102 code number.

## Key Patent Granted for ATL1102 in DMD in United States

The Company received notice in May 2024 from the US Patent and Trademark Office that the patent *Uses and Methods for Treating Duchenne Muscular Dystrophy* had proceeded to grant. This is the first approval received for this patent, which forms a critical component of the Company's strategy for protecting the use of ATL1102 in DMD. Similar patents remain under evaluation by patent authorities in other key territories.

## Partnership with Ionis Pharmaceuticals Inc

Post period, in July 2024, the Company entered into a revised agreement with Ionis, Inc (NASDAQ: IONS). The companies were previously parties to a Collaboration and License Agreement, which was entered into in 2001, and which formed the basis of a research partnership that led to the discovery and development of Percheron's current pipeline assets, ATL1102 and ATL1103. The parties have completed the collaborative research program and drug development program under the Collaboration and License Agreement and Percheron intends to pursue the further development and commercialisation of drug products deriving from this work. With the Company now contemplating potential future partnerships and commercialisation, it was considered appropriate to simplify and clarify the obligations of the parties to reflect the current status of Percheron's activities and the relationship between the parties. Consistent with the previous agreement Percheron remains obligated to use commercially reasonable efforts to bring products into commercial use as quickly as is reasonably possible. Percheron also retains access to relevant Ionis know-how and an obligation to pay certain royalties to Ionis based on commercialization proceeds received by Percheron. The revised agreement, now titled the 'Royalty Agreement', otherwise serves to retire certain research and development-focused provisions of the original which are no longer applicable.

## Participation in International Conferences

In June 2024, executives from the Company attended the BIO International Convention in San Diego, CA. The BIO Convention is hosted by the US-based Biotechnology Industry Organization and is the largest pharmaceutical partnering meeting in the annual calendar, typically attracting more than 20,000 delegates.

The Company conducted a broad range of meetings and discussions over the course of the conference and generated important new leads for a potential future partnership involving ATL1102. In addition, Percheron CEO, Dr James Garner, delivered a company presentation at the conference, which was well-attended.

Percheron was also grateful to sponsor the 30th Annual Meeting of Parent Project Muscular Dystrophy (PPMD), one of the key patient advocacy groups in the United States for families affected by DMD. The meeting was held in Orlando, FL.

Percheron Chief Medical Advisor, Dr Cathryn Clary, attended on behalf of the company and held meetings with clinicians and patient advocates. Dr Clary also conducted an Advisory Board with several physicians who specialise in the treatment of DMD, and this provided very valuable insights into the evolving treatment paradigm, emerging novel therapies, and any future studies of ATL1102 that may be conducted in the United States.

## Expansion of Senior Management Team

In June 2024, the Company announced the appointment of Dr Cathryn Clary as Chief Medical Advisor. A physician by training, Dr Clary has had a distinguished career as a senior executive with companies such as Pfizer, Inc (NYSE: PFE), Novartis AG (NYSE: NVS), and Ipsen SA. In recent years, she has worked primarily with emerging biotech companies, including a two-year engagement as Consulting Chief Medical Officer at Solid Biosciences (NASDAQ: SLDB), where she drove that company's gene therapy program in Duchenne muscular dystrophy. Dr Clary is based in New Jersey.

Also in June 2024, the Company announced the appointment of Deborah Ambrosini as Chief Financial Officer and Company Secretary. Ms Ambrosini is a chartered accountant who began her career with PricewaterhouseCoopers. She has served as CFO to a number of private and public growth-stage companies, including Cortical Dynamics Limited, Acrux Limited (ASX: ACR), and Cann Group (ASX: CAN). In addition to her accounting qualifications, Ms Ambrosini holds a Certificate in Governance Practice from the Governance Institute of Australia and is a highly experienced Company Secretary.

As part of a planned transition of responsibilities, Phillip Hains of Acclime Group, previously CFO and Company Secretary to Percheron, has stepped back from those roles, although the Company continues to work closely with Acclime Group for its general accounting activities.

## Financial Position

As noted in the accompanying unaudited quarterly cashflow report (Appendix 4C), the Company closed the quarter ending 30 June 2024 with a cash balance of $11.9 million, compared to $14.9 million at the end of the previous quarter.

Net cash outflows from operating activities for the quarter were $3.1 million including research and development expenditure of $2.3 million representing payments for the Company's phase IIb clinical trial of ATL1102 in non-ambulant boys.

During the quarter the Company made payments to related parties of the entity as disclosed in Item 6 of the Appendix 4C amounting to approximately $0.19 million. These payments represent salaries, directors' fees, and consulting fees on normal commercial terms.

Based on a forward-looking cashflow forecast, the Company continues to project cash runway into CY2025.

**~ 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 drug has previously reported promising results from an exploratory phase IIa study 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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# Appendix 4C

# Quarterly cash flow report for entities subject to Listing Rule 4.7B

| **Name of entity** | |
|---|---|
| Percheron Therapeutics Limited | |
| **ABN** | **Quarter ended ("current quarter")** |
| 41 095 060 745 | 30 June 2024 |

| **Consolidated statement of cash flows** | | **Current quarter $A'000** | **Year to date (12 months) $A'000** |
|---|---|---|---|
| **1.** | **Cash flows from operating activities** | | |
| 1.1 | Receipts from customers | - | - |
| 1.2 | Payments for | | |
| | (a) research and development | (2,288) | (8,222) |
| | (b) product manufacturing and operating costs | - | - |
| | (c) advertising and marketing | (23) | (109) |
| | (d) leased assets | (13) | (111) |
| | (e) staff costs | (467) | (1,965) |
| | (f) administration and corporate costs | (458) | (2,113) |
| 1.3 | Dividends received (see note 3) | - | - |
| 1.4 | Interest received | 124 | 587 |
| 1.5 | Interest and other costs of finance paid | - | - |
| 1.6 | Income taxes paid | - | - |
| 1.7 | Government grants and tax incentives | - | 1,577 |
| 1.8 | Other (provide details if material) | 53 | 192 |
| **1.9** | **Net cash from / (used in) operating activities** | **(3,072)** | **(10,164)** |

| Consolidated statement of cash flows | | Current quarter $A'000 | Year to date (12 months) $A'000 |
|---|---|---|---|
| **2.** | **Cash flows from investing activities** | | |
| 2.1 | Payments to acquire or for: | | |
| | (a) entities | - | - |
| | (b) businesses | - | - |
| | (c) property, plant and equipment | - | - |
| | (d) investments | - | - |
| | (e) intellectual property | - | - |
| | (f) other non-current assets | - | - |
| 2.2 | Proceeds from disposal of: | | |
| | (a) entities | - | - |
| | (b) businesses | - | - |
| | (c) property, plant and equipment | - | - |
| | (d) investments | - | - |
| | (e) intellectual property | - | - |
| | (f) other non-current assets | - | - |
| 2.3 | Cash flows from loans to other entities | - | - |
| 2.4 | Dividends received (see note 3) | - | - |
| 2.5 | Other (provide details if material) | - | - |
| **2.6** | **Net cash from / (used in) investing activities** | **-** | **-** |

| | | | |
|---|---|---|---|
| **3.** | **Cash flows from financing activities** | | |
| 3.1 | Proceeds from issues of equity securities (excluding convertible debt securities) | - | 11,612 |
| 3.2 | Proceeds from issue of convertible debt securities | - | - |
| 3.3 | Proceeds from exercise of options | - | - |
| 3.4 | Transaction costs related to issues of equity securities or convertible debt securities | - | (548) |
| 3.5 | Proceeds from borrowings | - | - |
| 3.6 | Repayment of borrowings | - | - |
| 3.7 | Transaction costs related to loans and borrowings | - | - |
| 3.8 | Dividends paid | - | - |
| 3.9 | Other (provide details if material) | - | - |
| **3.10** | **Net cash from / (used in) financing activities** | **-** | **11,064** |

**Appendix 4C**
**Quarterly cash flow report for entities subject to Listing Rule 4.7B**

| Consolidated statement of cash flows | | Current quarter $A'000 | Year to date (12 months) $A'000 |
|---|---|---|---|
| **4.** | **Net increase / (decrease) in cash and cash equivalents for the period** | | |
| 4.1 | Cash and cash equivalents at beginning of period | 14,939 | 10,967 |
| 4.2 | Net cash from / (used in) operating activities (item 1.9 above) | (3,072) | (10,164) |
| 4.3 | Net cash from / (used in) investing activities (item 2.6 above) | - | - |
| 4.4 | Net cash from / (used in) financing activities (item 3.10 above) | - | 11,064 |
| 4.5 | Effect of movement in exchange rates on cash held | - | - |
| **4.6** | **Cash and cash equivalents at end of period** | **11,867** | **11,867** |

| 5. | Reconciliation of cash and cash equivalents at the end of the quarter (as shown in the consolidated statement of cash flows) to the related items in the accounts | Current quarter $A'000 | Previous quarter $A'000 |
|---|---|---|---|
| 5.1 | Bank balances | 367 | 939 |
| 5.2 | Call deposits | 11,500 | 14,000 |
| 5.3 | Bank overdrafts | - | - |
| 5.4 | Other (provide details) | - | - |
| **5.5** | **Cash and cash equivalents at end of quarter (should equal item 4.6 above)** | **11,867** | **14,939** |

| 6. | Payments to related parties of the entity and their associates | Current quarter $A'000 |
|---|---|---|
| 6.1 | Aggregate amount of payments to related parties and their associates included in item 1 [1] | 187 |
| 6.2 | Aggregate amount of payments to related parties and their associates included in item 2 | - |

*Note: if any amounts are shown in items 6.1 or 6.2, your quarterly activity report must include a description of, and an explanation for, such payments.*

1. Director fees and salary payments made to Directors of the Company during 1 April 2024 and 30 June 2024.

**Appendix 4C**
**Quarterly cash flow report for entities subject to Listing Rule 4.7B**

| 7. | **Financing facilities**<br>*Note: the term "facility' includes all forms of financing arrangements available to the entity.*<br>*Add notes as necessary for an understanding of the sources of finance available to the entity.* | **Total facility amount at quarter end $A'000** | **Amount drawn at quarter end $A'000** |
|---|---|---|---|
| 7.1 | Loan facilities | - | - |
| 7.2 | Credit standby arrangements | - | - |
| 7.3 | Other (Corporate Credit Cards) | 40 | - |
| 7.4 | **Total financing facilities** | 40 | - |

| | | |
|---|---|---|
| 7.5 | **Unused financing facilities available at quarter end** | 40 |

7.6 Include in the box below a description of each facility above, including the lender, interest rate, maturity date and whether it is secured or unsecured. If any additional financing facilities have been entered into or are proposed to be entered into after quarter end, include a note providing details of those facilities as well.

Credit card facility – American Express

| 8. | **Estimated cash available for future operating activities** | **$A'000** |
|---|---|---|
| 8.1 | Net cash from / (used in) operating activities (item 1.9) | (3,072) |
| 8.2 | Cash and cash equivalents at quarter end (item 4.6) | 11,867 |
| 8.3 | Unused finance facilities available at quarter end (item 7.5) | 40 |
| 8.4 | Total available funding (item 8.2 + item 8.3) | **11,907** |
| 8.5 | **Estimated quarters of funding available (item 8.4 divided by item 8.1)** | 3.9 |

*Note: if the entity has reported positive net operating cash flows in item 1.9, answer item 8.5 as "N/A". Otherwise, a figure for the estimated quarters of funding available must be included in item 8.5.*

8.6 If item 8.5 is less than 2 quarters, please provide answers to the following questions:

8.6.1 Does the entity expect that it will continue to have the current level of net operating cash flows for the time being and, if not, why not?

Answer: N/A

8.6.2 Has the entity taken any steps, or does it propose to take any steps, to raise further cash to fund its operations and, if so, what are those steps and how likely does it believe that they will be successful?

Answer: N/A

8.6.3 Does the entity expect to be able to continue its operations and to meet its business objectives and, if so, on what basis?

Answer: N/A

*Note: where item 8.5 is less than 2 quarters, all of questions 8.6.1, 8.6.2 and 8.6.3 above must be answered.*

**Appendix 4C**
**Quarterly cash flow report for entities subject to Listing Rule 4.7B**

## Compliance statement

1 This statement has been prepared in accordance with accounting standards and policies which comply with Listing Rule 19.11A.

2 This statement gives a true and fair view of the matters disclosed.

Date: 31 July 2024

Authorised by: By the Board of Directors of Percheron Therapeutics Limited
(Name of body or officer authorising release – see note 4)

**Notes**

1. This quarterly cash flow report and the accompanying activity report provide a basis for informing the market about the entity's activities for the past quarter, how they have been financed and the effect this has had on its cash position. An entity that wishes to disclose additional information over and above the minimum required under the Listing Rules is encouraged to do so.

2. If this quarterly cash flow report has been prepared in accordance with Australian Accounting Standards, the definitions in, and provisions of, *AASB 107: Statement of Cash Flows* apply to this report. If this quarterly cash flow report has been prepared in accordance with other accounting standards agreed by ASX pursuant to Listing Rule 19.11A, the corresponding equivalent standard applies to this report.

3. Dividends received may be classified either as cash flows from operating activities or cash flows from investing activities, depending on the accounting policy of the entity.

4. If this report has been authorised for release to the market by your board of directors, you can insert here: "By the board". If it has been authorised for release to the market by a committee of your board of directors, you can insert here: "By the [*name of board committee – eg Audit and Risk Committee*]". If it has been authorised for release to the market by a disclosure committee, you can insert here: "By the Disclosure Committee".

5. If this report has been authorised for release to the market by your board of directors and you wish to hold yourself out as complying with recommendation 4.2 of the ASX Corporate Governance Council's *Corporate Governance Principles and Recommendations*, the board should have received a declaration from its CEO and CFO that, in their opinion, the financial records of the entity have been properly maintained, that this report complies with the appropriate accounting standards and gives a true and fair view of the cash flows of the entity, and that their opinion has been formed on the basis of a sound system of risk management and internal control which is operating effectively.