PRELIMINARY RESULTS OF NINE-MONTH TOXICOLOGY STUDY OF ATL1102 SHOW NO UNEXPECTED TOXICITY FINDINGS

Melbourne, Australia – 27 May 2024: Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to provide an update on the company's preclinical toxicology assessment of its investigational new drug, ATL1102.

Key Points

- The nine-month toxicology study is a key regulatory requirement to enable future clinical trials and commercialisation in the United States.
- Preliminary results of the study indicate generally similar findings to an earlier six-month study[1]. No new or unexpected toxicities were identified. No animals died on study.
- The recovery phase of the study remains ongoing, with final data from all animals expected to be available in 4Q CY2024. Percheron will thereafter discuss results with FDA, with a view to lifting clinical hold in the United States.

"We are very encouraged by the results we have seen so far," commented Percheron CEO, Dr James Garner. "While some analyses remain ongoing, and definitive interpretation awaits completion of the recovery phase, the observations to date seem quite consistent with earlier data, and the study has not identified any new or unexpected toxicities associated with ATL1102. We await data from the recovery phase of the study before we can draw final conclusions, but the results appear to be broadly confirmatory so far."

The requirement for a nine-month non-rodent toxicology study prior to any clinical trial involving dosing beyond six months was communicated to the Company by FDA during previous regulatory discussions. Because ATL1102 is intended to be administered over an extended period, the study effectively represents a prerequisite for conducting clinical trials in the United States.

The Company accordingly commenced a nine-month toxicology study in March 2023[2]. The study is performed in accordance with Good Laboratory Practice (GLP) by a specialist contract research organisation. Dosing concluded on schedule in December

[1] https://per.live.irmau.com/pdf/5ced8610-b602-4b4c-a368-884332cc0c32/ATL1102-for-MS-Toxicology-Study-Main-Findings.pdf
[2] https://per.live.irmau.com/pdf/52c3cb6e-0b62-4086-9933-4c65423824c7/Dosing-commenced-in-the-ATL1102-toxicology-study.pdf

2023[3]. The majority of the animals then underwent pathological examination. A proportion of the animals continued into a recovery phase, during which ATL1102 is not administered, and the purpose of which is to establish the reversibility of any observations. This recovery phase is expected to conclude in June 2024, after which the recovery animals will also undergo pathological examination.

Next Steps

Final data from the nine-month toxicology study, together with specialist evaluation and interpretation of the results, are expected to be available in 4Q CY2024.

The Company expects to discuss the outcomes of the study with FDA once the final data is available, with a view to enabling the conduct of future studies in the United States, should they be needed, and as a necessary step toward any future NDA filing. The exact timing of these interactions will be determined in consultation with the Company's regulatory advisors.

ATL1102 is currently the subject of an ongoing international phase IIb clinical trial in non-ambulant boys with Duchenne muscular dystrophy. Data is expected in 2H CY2024.

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About Percheron Therapeutics Limited

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

For more information, please contact info@PercheronTx.com.

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

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[3] https://per.live.irmau.com/pdf/331ff29f-f123-40b6-a087-dac9de45028f/Quarterly-ActivitiesAppendix-4C-Cash-Flow-Report.pdf