SUCCESSFUL COMPLETION OF AVICURSEN (ATL1102) NINE-MONTH NON-HUMAN PRIMATE TOXICOLOGY STUDY

Melbourne, Australia – 30 September 2024: Percheron Therapeutics Limited (ASX: PER) ('the Company'), an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to announce full completion of a nine-month toxicology study in non-human primates for its lead program, avicursen.

Key Points

- The nine-month toxicology study is a key regulatory requirement to enable future clinical trials and commercialization in the United States.
- The study commenced in March 2023 and is now fully completed, with a final study report in hand.
- Results of the study remain broadly consistent with the earlier six-month study, as previously reported in the Company's announcement of 27 May 2024[1]. No new or unexpected toxicities were observed, and no animals died on study. Expected low-grade findings were fully reversible during the recovery period.
- The company expects to discuss these results with the FDA in early CY2025, with the goal of lifting clinical hold in the United States.

"We are very pleased to have this important piece of work completed on schedule," commented Percheron CEO, Dr James Garner. "The results seem to us, and to our consultants and advisors, to be consistent with earlier observations, which we expect to have positive implications for the future development of avicursen. Given the impending availability of clinical data from the phase IIb clinical trial of avicursen in December 2024, we will likely target our regulatory discussions with the FDA for early CY2025, so as to present the agency with the strongest possible package of data."

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 the FDA during previous regulatory discussions. Because avicursen is intended to be administered over an extended period, the study effectively represents a prerequisite for conducting clinical trials in the United States.

[1] https://per.live.irmau.com/pdf/d9c2b58f-c45c-4c07-b329-2f3c12168705/Preliminary-Results-of-9-Month-Toxicology-Study-of-ATL1102.pdf

The Company accordingly commenced a nine-month toxicology study in March 2023. The study is performed in accordance with Good Laboratory Practice (GLP) by a specialist contract research organization. Dosing concluded on schedule in December 2023. The majority of the animals then underwent pathological examination, while the remaining animals continued into a recovery phase, during which avicursen was not administered. The purpose of the recovery phase was to establish that any observations seen in the dosing phase of the study reversed on cessation of treatment. The six-month recovery phase concluded in June 2024, after which the recovery animals also underwent pathological examination.

Next Steps

The Company expects to discuss the outcomes of the study with the FDA in early CY2025, with a view to enabling the conduct of future clinical trials in the United States and supporting a potential product approval in that market. The exact timing of these interactions will be determined in consultation with the Company's regulatory advisors.

Avicursen is the subject of an ongoing international phase IIb randomized controlled trial in non-ambulant boys with Duchenne muscular dystrophy, with initial data expected in December 2024.

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About Percheron Therapeutics Limited

Percheron Therapeutics Limited [ASX: PER | US OTC: ATHJF] is a publicly listed biotechnology company focused on the development and commercialization 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 Board of Directors.
