PERCHERON LAUNCHES POST-TRIAL ACCESS PROGRAM FOR PATIENTS COMPLETING PHASE IIB TRIAL IN DMD

Melbourne, Australia – 28 October 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 provide an update regarding post-trial access to drug for patients enrolled in the Company's ongoing international phase IIb clinical trial of avicursen (ATL1102) in Duchenne muscular dystrophy (DMD).

Key Points

- The ongoing randomised phase IIb trial (NCT05938023) was designed to evaluate the efficacy and safety of two doses of avicursen versus placebo in non-ambulant boys with DMD. The design of the study is such that all patients receive either six months or twelve months of avicursen treatment. Initial topline data is expected to be received in December 2024.

- Following requests from investigators in the trial, the Company has been exploring opportunities to make avicursen available to patients who successfully complete participation in the trial and who wish to remain on treatment. Such provision would be primarily on compassionate grounds rather than for the purpose of collecting additional data.

- Percheron now anticipates being able to provide avicursen on this basis to eligible patients in four of the five participating countries, with the first patients to begin treatment in Q4 CY2024. The Company is working with the relevant trial sites and regulatory agencies to complete the procedural and regulatory requirements associated with provision of a medicine that is not yet approved.

"We have heard a clear message from a number of the trial investigators that they would like to continue avicursen treatment for some of their patients beyond the formal completion of the trial," commented Percheron CEO, Dr James Garner. "The team has worked closely with the sites and with external advisors to make this possible, and we expect to be able to provide supply to patients who choose to continue, and whom investigators feel may benefit from doing so, free of charge, for a period of time after their participation in the trial concludes. We are grateful to the investigators, the patients and their families for their continuing interest in avicursen."

The design of the phase IIb study is such that, while the primary comparison between avicursen and placebo occurs after six months, all participants will then receive avicursen treatment for a further six months. The post-trial access program extends potential use of the drug beyond this period.

The notion of providing access to the study drug after a clinical trial, at the request of clinicians and patients, is part of the subject matter of the World Medical Association's Declaration of Helsinki (2000)[1]. The regulatory process and practical requirements for doing so are specific to each country, and Percheron has spent several months putting the necessary measures in place. The Company expects that the first patients will begin treatment under the post-trial access program in Q4 CY2024.

Next Steps

The Company expects to receive initial six-month efficacy and safety data from the trial 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 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 Board of Directors.

[1] https://www.wma.net/what-we-do/medical-ethics/declaration-of-helsinki/