CLINICAL TRIAL UPDATE

Melbourne, Australia – 2 December 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 on its ongoing international placebo-controlled phase IIb clinical trial of avicursen (ATL1102) in Duchenne muscular dystrophy (NCT05938023).

Key Points

- All 48 patients enrolled in the trial have now completed their week 25 visit, which is the visit at which the primary endpoint of the study is assessed. Work is underway to confirm and validate data, and to perform the requisite statistical analyses.

- Barring unforeseen operational delays, the Company expects to be in a position to disclose initial topline data, representing the first six months on study for all patients, during the week beginning 16 December 2024.

- Twelve-month data is expected to be available in mid-CY2025, and final sixteen-month data in 2H CY2025, as previously indicated.

"We eagerly await the six-month data," commented Percheron CEO, Dr James Garner. "This readout will provide the first indication of avicursen's activity in Duchenne and will help us to better chart its path forward toward registration and commercialisation. This is only the first of three major readouts from the study, and we will learn much more as we progress through CY2025, but we nevertheless see this as a major milestone in the development of the drug. The team has worked hard to generate results as quickly as possible, and this is reflected in the fact that the data is currently anticipated just a few weeks after the final patient attended the relevant hospital visit. A significant amount of work remains to be done, and we will update the market if our expectations change, but we currently expect to be able to discuss data during the week beginning 16 December 2024."

The ongoing international, randomised, placebo-controlled phase IIb study of avicursen in Duchenne muscular dystrophy began recruitment in June 2023[1]. The study follows an earlier phase IIa pilot study, which reported very encouraging results, and which was published in a peer-reviewed scientific journal in January 2024[2]. In common with the earlier study, this phase IIb study is focused on non-ambulant boys, whose disease has progressed to the stage where they are substantially unable to walk, and who represent approximately half of the total Duchenne population. By contrast with the earlier study,

[1] https://per.live.irmau.com/pdf/c760f592-ea31-495e-9b97-7f66046157c2/First-Patient-Dosed-in-ATL1102-Phase-IIb-DMD-Trial.pdf
[2] https://journals.plos.org/plosone/article?id=10.1371/journal.pone.0294847

this phase IIb study adopts a 'gold-standard' randomised, placebo-controlled design, and has greater statistical power by virtue of having enrolled 48 patients. The study has been conducted at 13 sites including Australia, the United Kingdom, Turkey, Bulgaria, and Serbia.

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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.
