TOPLINE SIX-MONTH RESULTS FROM RANDOMISED PHASE IIB STUDY OF AVICURSEN IN DMD

Melbourne, Australia – 18 December 2024: Percheron Therapeutics Limited (ASX: PER) ('the Company'), an international biotechnology company focused on the development of novel therapies for rare diseases, today announces topline six-month results from the Company's ongoing international phase IIb randomised, placebo-controlled trial of avicursen (ATL1102) in non-ambulant boys with Duchenne muscular dystrophy (DMD).

Key Points

- The trial did not meet its primary endpoint, which was Performance of the Upper Limb 2.0 (PUL2.0) score at week 25 compared to placebo. The least squares mean change in PUL2.0 score for patients receiving placebo was -1.4, for patients receiving 25mg of avicursen was -1.8 (p=0.695), and for patients receiving 50mg of avicursen was -1.6 (p=0.919). A p-value above 0.05 means that any numerical difference observed is not statistically significant.

- There were no statistically significant differences in efficacy on available secondary endpoints, nor was there a clear directional trend toward benefit associated with administration of avicursen.

- The drug was safe and well-tolerated, with injection site reactions the most common treatment-emergent adverse event. Injection site reactions were more common at the 50mg dose than at the 25mg dose, but all were considered mild or moderate by investigators.

- After careful consideration of these results, and in consultation with investigators, the Company has determined that it is not in the best interests of patients or shareholders for the study to continue and has therefore resolved to terminate it as soon as practicable.

- The Percheron team, working closely with investigators and advisors, will examine this data, along with further data expected in January 2025, to determine the best path forward for the avicursen program, noting in particular the drug's favourable safety profile. The Company expects to hold a strategic review of its pipeline in 1H CY2025 and will share further information with investors as it becomes available.

Percheron CEO and MD, Dr James Garner, said "We are of course disappointed by these results. In the more rigorous environment of a randomised, placebo-controlled, international study, avicursen has not performed in the way that we had been led to expect by earlier studies. The trial has not demonstrated therapeutic benefit in non-

ambulant DMD. We anticipate further data from this study in January 2025 and will be examining it closely to better understand this outcome and to assess future opportunities. The company is well-funded and so we can approach these discussions systematically and methodically. We expect to be able to share further information and plans with investors in the new year."

"In the meantime, I want to pay tribute to the investigators, healthcare professionals, and families that have placed their trust in us by participating in this study. Duchenne muscular dystrophy is an immensely challenging illness, and the need for new therapeutic options remains substantial. Although these results may not be as we had hoped, we are proud to have played a small role in advancing understanding of the disease, and in bringing hope to those affected by it."

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About Percheron Therapeutics Limited

Percheron Therapeutics Limited [ASX: PER | US OTCQB: PERCF] 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, which has been investigated in a range of inflammatory conditions, including multiple sclerosis and Duchenne muscular dystrophy.

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.

Clinical Trial Summary

Study Title	A Multicentre, Randomised, Double-blind, Placebo-controlled and Open Label Extension Study to Assess the Efficacy, Safety, and Pharmacokinetic Profile of ATL1102 in Non-ambulatory Participants With Duchenne Muscular Dystrophy.
Phase of Development	Phase IIb
Investigational Product	Avicursen (ATL1102)
Disease Area	Non-ambulant Duchenne muscular dystrophy
Registration	NCT05938023
Study Design	This is a randomised, double-blinded, placebo-controlled study to investigate the efficacy, safety, and pharmacokinetics of avicursen in boys with Duchenne muscular dystrophy. Patients are randomised 1:1:1 to receive placebo, 25mg of avicursen, or 50mg of avicursen for six months. After that period, patients in the placebo group are re-randomised 1:1 to receive either of the two doses of active drug, while patients originally randomised to active remain on their initially assigned therapy. After a further six months on study drug, all patients enter a four-month off-treatment follow-up period.
Number of Subjects	48 subjects.
Patient Population	All patients had genetically-confirmed Duchenne muscular dystrophy and were considered to be non-ambulant in the judgment of the investigator. Patients ranged in age from 10-17 years. If taking steroids, patients were required to have been on a stable dose for at least three months prior to study entry.
Endpoints	The primary endpoint was the performance in the upper limb module 2.0 (PUL2.0). Exploratory functional endpoints included PUL2.0 domain scores, Myopinch, and Myogrip, as well as safety, tolerability, and pharmacokinetics. Exploratory pharmacodynamic endpoints included lymphocyte counts over the course of the study.
Participating Centre(s)	13 sites in Australia, United Kingdom, Turkey, Serbia, and Bulgaria.