PERCHERON HOLDS INTERNATIONAL ADVISORY BOARDS WITH LEADING SPECIALISTS IN DUCHENNE MUSCULAR DYSTROPHY

Melbourne, Australia – 21 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, for the information of shareholders, an update on several recent international Clinical Advisory Boards held by the Company to discuss the development of avicursen (ATL1102) in Duchenne muscular dystrophy (DMD).

Key Points

- The Company has held two virtual Advisory Boards, in the United States and Europe respectively, each comprising leading specialists in the DMD field.

- The key objectives of the meetings included:
    - Familiarising clinicians with avicursen's pharmacology and clinical data;
    - Understanding potential use of the drug in clinical practice, and its competitive position in relation to other therapies, across a broad range of geographies; and
    - Discussing future development of the drug in a range of scenarios, including novel trial outcome measures and study designs.

- Parent Project Muscular Dystrophy (PPMD), a leading US-based patient advocacy organisation, was invited to attend the meetings and a representative participated to provide a patient perspective.

"Avicursen has a novel mechanism in comparison to other DMD therapies," commented Professor Thomas Voit, Director of the NIHR Great Ormond Street Hospital (GOSH) Biomedical Research Centre in London, UK, Chair of the European Advisory Board, and Principal Investigator for the ongoing international phase IIb clinical trial of avicursen in non-ambulant DMD. "Should the drug receive approval from regulatory agencies, we believe it may have a valuable role to play in the treatment of this very challenging disease."

"We are grateful to the attendees for their time, and for their generous sharing of insights," commented Percheron CEO, Dr James Garner. "The feedback we have received from these meetings will be of great value as we interpret emerging data from the avicursen clinical trial program, discuss the path forward with regulatory agencies, and consider how best to maximise the drug's benefit for patients."

Drug development companies regularly hold Advisory Boards such as these to consult with specialist clinicians and expert advisors on the development of novel medicines.

The feedback from participants has provided the Company with a much deeper understanding of the treatment landscape for DMD, expert guidance as to the interpretation of data from previous and ongoing studies, and valuable insights into potential future development paths for avicursen. The Company expects to hold further similar Advisory Boards in the future, as new data for avicursen becomes available, and as the regulatory strategy progresses. In the meantime, Percheron expects that this feedback from recent discussions will be of great utility as it considers potential discussions with the FDA and other regulatory agencies in CY2025.

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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.