PHASE IIB STUDY OF ATL1102 IN DMD COMPLETES RECRUITMENT

Melbourne, Australia – 29 May 2024: Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to announce the successful completion of recruitment to its ongoing international phase IIb study of ATL1102 in the treatment of Duchenne muscular dystrophy.

Key Points

- In June 2023, Percheron commenced recruitment to an international phase IIb randomised controlled trial of ATL1102 in the treatment of non-ambulant boys with Duchenne muscular dystrophy (DMD)[1].

- The study is now fully enrolled. In total, 48 boys were randomised to the study across sixteen hospitals in five countries. The last patient into the study was randomised on 28 May 2024.

- The primary endpoint of the study is the change in PUL2.0 score at six months, and so data is expected in December 2024, consistent with prior guidance.

"We are delighted to have this study fully recruited," commented Percheron CEO, Dr James Garner. "The need for new therapies in Duchenne muscular dystrophy has never been more acute, and we very much hope that ATL1102 will be able to make an important contribution to the treatment of this devastating disease. We are profoundly grateful to the participating investigators and their teams, and to the families who have agreed to participate. With the study now fully enrolled, we expect to have initial topline data in December 2024."

ATL1102 is an antisense oligonucleotide that targets CD49d. Administration of ATL1102 has been shown to reduce CD49d positive lymphocytes, thereby exerting an immunomodulatory effect which may be therapeutic in a range of inflammatory diseases. A completed phase IIa study of the drug in nine non-ambulant boys with DMD showed very promising signals of activity, and this work was recently published in a peer-reviewed journal[2].

The present study aims to provide a deeper and more robust evaluation of ATL1102's potential activity in DMD. The design comprises two doses of ATL1102 compared against placebo and enrolled approximately 15 subjects in each arm. The primary endpoint is

[1] https://per.live.irmau.com/pdf/c760f592-ea31-495e-9b97-7f66046157c2/First-Patient-Dosed-in-ATL1102-Phase-IIb-DMD-Trial.pdf
[2] https://per.live.irmau.com/pdf/468994c5-fcf6-4baa-8712-6ded86dfc9ed/ATL1102-DMD-Phase-IIA-Final-Data-Published.pdf

the change in the performance of the upper limb module (PUL2.0) at six months. Subjects thereafter transition to an open-label extension phase where those treated with ATL1102 receive a further six months of treatment and those originally randomised to placebo are reallocated to one of the two active arms. After twelve months, all subjects enter a four-month off-treatment period.

Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a genetic condition that is thought to affect around 1 in 10,000 males. The disease results from mutations in the DMD gene, which codes for dystrophin, a structural protein in muscle. Patients with DMD accumulate movement-related muscle damage, which leads to chronic inflammation and progressive loss of function. Symptoms typically manifest in early childhood, and patients are generally wheelchair-bound by their teens, with life expectancy between twenty and thirty years of age[3].

Next Steps

With enrollment now complete, the timelines for the remainder of the study can be confirmed as follows:

Topline Data (at 6 months)	December 2024
Topline Data (at 12 months)	Mid CY2025
Final Data (at 16 months)	4Q CY2025

The Company expects to discuss emerging results from the study with regulatory agencies such as FDA to determine the optimal path to registration for ATL1102.

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[3] D Duan et al. (2021) *Nat Rev Dis Primers* 7,13 (https://doi.org/10.1038/s41572-021-00248-3)

Clinical Trial Summary

Study Title	A Study of ATL1102 or Placebo in Participants With Non-ambulatory Duchenne Muscular Dystrophy
Phase of Development	Phase IIB
Investigational Product	ATL1102 (via weekly subcutaneous injection)
Disease Area	Non-ambulant Duchenne muscular dystrophy
Registration	NCT05938023
Study Design	This is a double-blind, randomised, prospective, multicentre clinical trial of ATL1102 versus placebo in non-ambulant boys with Duchenne muscular dystrophy. Subjects are randomised in equal proportions to receive one of two doses of ATL1102 (25mg or 50mg) or placebo, on top of standard of care corticosteroids. The primary endpoint is assessed after six months. All subjects then remain on study drug for a further six months, during which patients originally allocated to placebo will be re-randomised to one of the two active arms. Subjects will then remain on study for a further four months off-treatment.
Number of Subjects	45 subjects planned; 48 enrolled
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 10-18 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 is PUL2.0 at six months. Secondary functional endpoints include Myopinch and Myogrip. A range of exploratory biomarkers will also be assessed.
Participating Centre(s)	16 hospitals in Australia, United Kingdom, Turkey, Serbia, and Bulgaria

About Percheron Therapeutics Limited

Percheron Therapeutics Limited [ASX: PER | US OTC: ATHJY | FSE: AWY] is a publicly listed biotechnology company focused on the development and commercialisation of novel therapies for rare diseases. The company's lead program is ATL1102, an antisense oligonucleotide targeting the CD49d receptor. ATL1102 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 2H 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 authorised for release to the Australian Securities Exchange by the Board of Directors.