ATL1102 DMD PHASE IIA FINAL DATA PUBLISHED IN PEER-REVIEWED SCIENTIFIC JOURNAL

Melbourne, Australia – 29 January 2024: Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to announce that final data from a phase IIa study of its investigational drug, ATL1102, in Duchenne muscular dystrophy has been published in a peer-reviewed scientific journal.

The paper is entitled 'A Phase 2 open-label study of the safety and efficacy of weekly dosing of ATL1102 in patients with non-ambulatory Duchenne muscular dystrophy and pharmacology in mdx mice' and is available on an open access basis from the journal *PLoS ONE* via the following link:

https://journals.plos.org/plosone/article?id=10.1371/journal.pone.0294847

Key Points

- The study enrolled 9 boys with confirmed Duchenne muscular dystrophy (DMD), ranging from 12 – 18 years of age. All were non-ambulant, meaning that the disease has reached the stage where patients are unable to walk more than a few metres.

- The drug was generally safe and well-tolerated, with no patients withdrawing from the study for safety reasons and no serious unexpected adverse drug reactions reported. The most common adverse events were injection site reactions.

- The performance in the upper limb (PUL2.0) score of muscle function was essentially stable over the course of the study, with an increase of 0.9 points between baseline and week 24. While this was a single-arm study, previous comparisons against matched historical controls have suggested a typical deterioration of 2.0 points over the same period in boys treated with standard of care[1].

- Other measures of muscle function and strength generally showed stabilisation or a trend towards modest improvement. MRI scans showed a trend towards reduction in adiposity and an increase in lean muscle mass over the treatment period.

The study was run at the Royal Children's Hospital in Melbourne, Victoria, and the Principal Investigator was Dr Ian Woodcock, a paediatric neurologist with extensive research experience in Duchenne muscular dystrophy.

[1] G Tachas et al. (2020) *Neuromuscul Disord* 20, Supp1, S129-S130. (http://dx.doi.org/10.1016/j.nmd.2020.08.281)

"My colleagues and I are gratified to see this data now in publication," commented Dr Woodcock. "Despite recent progress, there remains a pressing need for new treatment options in muscular dystrophy. These data suggest that ATL1102 may in future have a role to play in the management of this devastating disease. We are now conducting an ongoing international phase IIb study to better understand the drug's effects."

"We are grateful to the investigators for their excellent work on this very encouraging study," commented Dr James Garner, CEO of Percheron Therapeutics. "The publication of this work reflects the company's commitment to making its scientific data available through peer-reviewed scientific journals. This trial has provided the basis on which the company has now taken ATL1102 into an international phase IIb randomised controlled trial, and we expect to see data from that study next year."

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 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[2].

Phase IIa Study of ATL1102

The phase IIa study that has been reported in this publication was designed as an exploratory study to investigate ATL1102 as a potential therapy for Duchenne muscular dystrophy. The primary endpoints were safety and tolerability. The study included a range of functional measures as exploratory endpoints but was not designed or powered to provide definitive evidence of efficacy. This was a single-arm study, with no in-study comparator group, so was not able to demonstrate statistically significant differences against standard of care.

Next Steps

ATL1102 is currently the subject of an ongoing international phase IIb clinical trial in Duchenne muscular dystrophy, with data anticipated in 2H CY2024.

~ ENDS ~

[2] 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 Phase 2 open label, study to determine the safety, efficacy and pharmacokinetic profile of weekly dosing of ATL1102 in patients with non-ambulatory Duchenne Muscular Dystrophy
Phase of Development	Phase II
Investigational Product	ATL1102
Disease Area	Non-ambulant Duchenne muscular dystrophy
Registration	ACTRN12618000970246
Study Design	This is an open-label, single-arm exploratory study to investigate the safety and tolerability of ATL1102 in boys with Duchenne muscular dystrophy. All patients receive the same dose of ATL1102, 25mg, for a period of 24 weeks.
Number of Subjects	9 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 10-18 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 of was safety and tolerability. Exploratory functional endpoints included PUL2.0, Moviplate, Myoset, Myopinch, and Myogrip. Exploratory pharmacodynamic endpoints included lymphocyte counts over the course of the study. Exploratory imaging endpoints included forearm adiposity and lean muscle mass, as ascertained by MRI.
Participating Centre(s)	**Royal Children's Hospital** Melbourne, VIC, Australia

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 drug has previously reported promising results from an exploratory phase IIa study 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.