WORLD HEALTH ORGANISATION SELECTS PROPOSED INTERNATIONAL NONPROPRIETARY NAME FOR ATL1102

Melbourne, Australia – 6 May 2024: Percheron Therapeutics Limited, an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to announce that the World Health Organization (WHO) has selected 'avicursen' as the proposed international nonproprietary name (INN) for Percheron's investigational drug candidate, ATL1102.

Key Points

- Prior to marketing approval, medicines generally receive an international nonproprietary name which describes the active chemical ingredient. The INN is selected by the World Health Organization, with input from the company developing the drug.

- Following an application by Percheron, WHO has selected 'avicursen' as the proposed INN (pINN) for ATL1102.

- The proposed INN is expected to be definitively confirmed around May 2025. The company will continue to use the ATL1102 designation through CY2024 and will begin adopting the avicursen name in CY2025.

"The selection of an INN is an important regulatory milestone on the journey to a commercial product," commented Percheron CEO, Dr James Garner. "Applying for an INN has been among a range of tasks that the team has been focusing on in recent months to begin preparing ATL1102 for a potential future marketing authorisation. We are grateful to WHO for their review of our submission and their selection of a proposed INN designation. We look forward to seeing this confirmed in due course."

The name is expected to be published by WHO in the 132nd list of Proposed International Nonproprietary Names in January 2025. After publication, a proposed INN is subject to a four-month period for comment before it is confirmed. The proposed name is consistent with other similar drugs in the antisense oligonucleotide class, which generally end in the suffix '-rsen'.

While responsibility for selection of pINNs rests with WHO, Percheron was able to make recommendations and suggestions. The name avicursen alludes to the Australian state of Victoria, which has been the company's home since it was founded.

Background

Investigational drug candidates typically begin their development with a code number or similar designation, which is used primarily for internal administrative purposes, and which is chosen entirely by the sponsor company. ATL1102 is an example of such a compound code number.

Typically, around the time of phase II clinical trials, an international nonproprietary name is selected. INNs are chosen by the World Health Organization or, for the United States, by the United States Adopted Names Council (USAN), based on recommendations and information provided by the sponsor company. There are strict guidelines regarding INNs, and they are not ultimately determined by the sponsor company. INNs cannot be trademarked and are not owned by the sponsor company. The INN is used to refer to any product that contains the same active ingredient, regardless of manufacturer. Avicursen is the proposed INN for ATL1102.

At the time of marketing authorisation, drugs receive a commercial brand name. This is selected by the sponsor company, although it must be approved by the regulatory agency for each country in which the product is marketed. The commercial brand name is trademarked and remains the property of the sponsor.

As an illustrative example, 'SRP-9001' is a company code number, 'delandistrogene moxeparvovec' is an INN, and 'Elevidys®' is a commercial brand name.

By convention, commercial brand names are usually capitalised, whereas INNs are not. When both are used, the INN is typically placed in brackets after the commercial brand name. Examples include Nexium® (esomeprazole), Prozac® (fluoxetine), Viagra® (sildenafil), Panadol® (paracetamol), etc.

The Company has not yet selected a commercial brand name for ATL1102 but expects to do so as the drug approaches commercial registration, in accordance with guidelines from FDA and other national regulatory agencies.

Next Steps

The Company will begin to deploy the INN in its communications in CY2025, subject to confirmation by WHO, and the ATL1102 code number will eventually be retired.

An international randomised controlled clinical trial of ATL1102 in non-ambulant boys with Duchenne muscular dystrophy remains ongoing, with data expected in 2H CY2024.

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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 authorized for release to the Australian Securities Exchange by the Board of Directors.