

**LETTER TO PERCHERON THERAPEUTICS LIMITED SHAREHOLDERS**

**Melbourne, Australia – 6 January 2025:** Percheron Therapeutics Limited (ASX: PER) ('the Company'), an international biotechnology company focused on the development of novel therapies for rare diseases, is pleased to attach a letter addressed to all shareholders from the Board of Directors.

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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](mailto:info@PercheronTx.com).

*This announcement has been authorized for release to the Australian Securities Exchange by the Board of Directors.*

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6 January 2025

**From the Board of Directors of Percheron Therapeutics Limited**

Dear Shareholder,

On 18 December 2024, we announced negative topline results from our phase IIb trial of avicursen (ATL1102) in Duchenne muscular dystrophy (DMD).<sup>1</sup> This has understandably come as an enormous disappointment for all our shareholders, as indeed it has to all those of us who work in the company on your behalf. We write to you collectively as a Board to outline some of the implications for Percheron and to explain how we plan to rebuild and restore the company following this setback.

We proceed for now on three fronts. Our first priority is to better understand the results of the avicursen trial and to see if there are convincing reasons to continue development in DMD. Companies in our position must have the courage to recognize futility when it occurs, and our assumption at this stage is that the drug will not move forward in DMD. However, we owe it to all those who have invested their time, money, energy, and hope in avicursen for DMD to make sure that we do not abandon it prematurely. We expect to receive further data from the trial at various points throughout the month of January, and we plan to complete our analyses by the end of the first quarter of CY2025. We aim to share our conclusions with investors in approximately that timeframe. To manage expectations, we should be clear that not all trial results are entirely explicable – sometimes drugs simply don't work – but we think it is important to learn everything we can so that we are able to make rational and informed decisions about the program's future, and to benefit future research in DMD.

Second, and in parallel with our analysis of the study, we will undertake a broad strategic review of the company's pipeline to more comprehensively evaluate alternative sources of value within the current assets. Avicursen has shown evidence of activity in a range of other conditions, and we have always been clear that, while DMD was the lead indication for the drug, it was not the only use case. However, if we are to pursue an alternative indication, we will need to be convinced that the negative result in DMD does not prejudice the chances of success in another illness. Meanwhile, we had been planning in any case to make a definitive decision about the future of atesidorsen

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<sup>1</sup> [ToplineSixMonthResultsFromPhaseIIbStudyofAvicursen.pdf](#)

(ATL1103) in CY2025, and we will now accelerate those discussions. Once again, we expect to complete these deliberations over the first quarter of CY2025.

Finally, and regardless of what we conclude from these efforts, it is quite likely that the company will need to diversify its pipeline, and we will look to add one or more new programs to our portfolio. We have already begun exploring opportunities that may provide a faster and more secure path to restoring shareholder value and we have initiated discussions with several potential partners. By virtue of our recent capital raising activities we are in the fortunate position of having choices in respect of what Percheron may become. The Board is approaching this question with an entirely open mind, and our only guiding principles are to continue to seek impactful new therapies for diseases with high unmet need, and to strive to reconstitute the value of the company on behalf of our shareholders.

In the meantime, we have taken aggressive measures to conserve the company's cash. Our most substantial cost has been the clinical trial itself, and we are moving to finalise its termination as quickly as possible. We are also making substantial reductions in our operating expenditure.

Some very successful companies have been built with lesser ingredients, and we remain highly optimistic that Percheron can yet become the business that each of us joined the Board to create, and that our shareholders have so resolutely supported in recent years.

Regrettable though the news has been, this is unfortunately a common occurrence in drug development. Analysis by the Biotechnology Innovation Organization (BIO) calculates the probability that a drug entering phase I human trials will become a marketed product at 7.9%.<sup>2</sup> To put it another way, the task of developing impactful new medicines for a disease as complex as DMD is enormously challenging, and there is no success without commensurate risk. We are not the first company whose drug candidate has failed to meet hopes expectations and, like so many of our peers, it falls to us now to adapt and reorient our business so as to place it back on the path to success.

We will communicate with you regularly and transparently through this period of transformation, but we will need to ask for your patience, particularly over the next several months. The decisions that must be made are weighty and intricate and they will decide the future direction of the company. It is important that we make them carefully and conscientiously.

We recognize that moments such as this in the life of a biotech company place immense demands on the endurance and fortitude of shareholders. Some of our investors have already made the decision to crystallise a loss on their investment and divert funds elsewhere, and we entirely understand and respect their choice. Percheron's path forward will undoubtedly be more complex than originally expected. However, the Board remains wholly committed to fulfilling the company's potential, and

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<sup>2</sup> [Clinical Development Success Rates and Contributing Factors 2011-2020 | BIO](#)

we want to assure you that we have the capabilities, the resources, and the steadfastness to accomplish that task.

In closing, we want to recognize and pay tribute to the Duchenne community, many of whose members have passionately engaged with and generously supported the avicursen program during its development for DMD. It has been our privilege to work with the clinicians, researchers, patient advocates, and families who collaborate every day to seek new therapies for patients with this devastating illness, and we will never cease to be inspired by their devotion and perseverance. We greatly regret that avicursen has not yet rewarded the hopes that these many stakeholders have invested in it, but we do not regret that we tried.

Yours faithfully,



**Dr Charmaine Gittleston**  
Chair of the Board



**Dr Gil Price**  
Non-Executive Director  
and Chair of Audit



**Dr James Garner**  
Managing Director

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by the Board of Directors.*