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Daily news on ASX-listed biotechnology companies

Dr Boreham's Crucible: Percheron Therapeutics

By TIM BOREHAM

ASX code: PER

Share price: 0.8 cents

Shares on issue: 1,087,437,633

Market cap: $8.7 million

CEO: Dr James Garner

Board: Dr Charmaine Gittleson (chair), Dr Garner, Dr Gil Price

Financials (March quarter 2025): revenue nil, cash outflows $4.47 million, cash balance $12.9 million

Major identifiable shareholders: Dr Garner 4.8%. **Former substantial holders** Powerhouse Ventures (last filing 4.46%), Platinum Asset Management 0.0%.

On Friday the 13th in December last year, Percheron CEO James Garner awaited news of top-line results of the company's phase IIb Duchenne muscular dystrophy trial from the lounge room of his Brisbane home.

The company's clinical development head Andrew McKenzie heard the grim tidings first and conveyed it to Dr Garner that evening.

"I joked that I had been sitting on the couch, with a bottle of champagne on ice on one end and a bottle of whisky and revolver at the other," Dr Garner said.

“I said to Andrew ‘what I am drinking’ and he said: ‘I hope it’s good whisky’.”

Testing Percheron’s drug candidate ATL1102 (avicursen), the trial failed its primary endpoint based on upper limb performance of the non-ambulant subjects.

“My heart sunk,” Dr Garner said.

“We spent the weekend picking through the data and it was just really clear it was as negative a result as they come.”

The company immediately scrapped the program.

After a bout of soul-searching Percheron hunted for another asset - and last month settled on an acquired immune-oncology program (see below).

Along the way, the board stared down two spill attempts from aggrieved shareholders proposing alternative uses for Percheron’s remaining cash kitty.

Sarepta setback highlights the perils of treating DMD

Percheron is not the only company to run into trouble with DMD, a regressive, fatal and poorly treated genetic disease.

After this column was first published on Friday, the Nasdaq-listed Sarepta Therapeutics said it agreed with the US Food and Drug Administration to “voluntarily halt shipment” of Elevidys (delandistrogene moxeparvovec), the only gene therapy for DMD.

Other reports assert Sarepta was not such a Boy Scout, having earlier refused an FDA request to do so.

The issue applies to non-ambulant (wheelchair bound) patients and follows two reported deaths of teenage boys from liver failure.

Sarepta says a 51-year-old man who also died from liver failure was not treated with Elevidys, but an investigational gene therapy as part of a separate phase I trial.

Sarepta shares tumbled 38 percent, having surged 20 percent the previous day on the company’s news it was sacking 500 people (one third its workforce).

Chance of success was ‘50-50’

A regressive, fatal and poorly treated genetic disease, Duchenne muscular dystrophy (DMD) affects about one in 10,000 males (or 300,000 in all).

DMD affects production of the muscle protein dystrophin, causing movement-related muscle damage leading to chronic inflammation and progressive loss of function.

Dr Garner says realistically the trial only had a 50 percent chance of success - the standard odds for a mid-staged program of its ilk.

The nature of the endpoints also added another degree of difficulty, with a placebo effect creeping in. One example is measuring the patient's ability to drink a cup of water.

"If everyone is watching and wants you to succeed, you put that little bit more effort into it," Dr Garner says.

He adds that merely being on a study can make a patient feel better and improve behavior.

He says the patients were older boys who had been subject to other treatment regimens - evidently with minimal success. As a result, carers and patients were realistic about the prospect of failure "but it still hurts".

Dr Garner adds that ATL1102 did show an effect - but it just was not meaningful enough.

From Toorak toff to draught horse

Formerly known as Antisense Therapeutics, the company is an offshoot of Circadian Technologies and is one of the oldest ASX biotechnology companies.

Quirkily, the company was based in Melbourne's upmarket Toorak, having sprung from Circadian Technologies.

The company dabbled in multiple sclerosis, acromegaly and - later - Covid therapies.

Having served as CEO for a record-breaking 17 years, Mark Diamond departed in May 2023 (he remains chairman of ASX listed kidney drug developer Dimerix).

His replacement, Dr Garner is a qualified medical doctor.

Dr Garner worked at Biogen, Takeda and Sanofi, overseeing more than 30 product approvals and more than a dozen clinical trials; and was then appointed CEO of the then ASX-listed (now Nasdaq) brain cancer drug developer Kazia for seven years.

Antisense licenced its key asset, ATL1102, from the Nasdaq-listed Ionis Pharmaceuticals (then Isis, but that lost popularity). Early last year, Antisense also changed its name - to Percheron - which means 'draught horse' in French.

After the trial failure, a cabal of five shareholders requisitioned an extraordinary general meeting (EGM) to replace Dr Garner and chair Dr Charmaine Gittleson and install three of its own board candidates. The resolution failed by a comfortable margin at the March 3 showdown.

The ASX-listed Powerhouse Ventures had a crack along similar lines, but its proposal was defeated at an April 24 gunfight (by a wider margin).

Percheron's new purpose

On June 26, Percheron said it acquired the exclusive rights to the HMBD-002 monoclonal antibody with potential applications in several cancers, from Singapore's Hummingbird Bioscience.

Percheron will pay Hummingbird an upfront $US3 million ($A4.6 million), with contingent milestone payments of up to $US287 million ($A443 million), plus royalties.

Dr Garner said the company has looked at more than 100 opportunities "of every imaginable shape and size".

About half were oncology opportunities, with others including neurological and skin diseases including alopecia, irritable bowel disease and reverse takeovers.

"Some were cursory - we looked at them for an afternoon and decided this doesn't sound promising," he says. "With others we got into deep due diligence and spent weeks looking under the hood."

Familiar with Hummingbird's multi-faceted activities, Dr Garner asked the company if it had anything interesting that didn't fit its strategy. And - voila! - HMBD-002 emerged from the bottom drawer.

How it works

HMBD-002 targets an agent called Vista, not a city view but a "v-domain immunoglobulin suppressor of T-cell activation". A new target Vista could be a new mechanism to treat a range of tumors.

As with other immuno-oncology drugs, HMBD-002 targets the interaction between the tumor and the immune system. (For a tumor to establish, it needs to dampen down the immune system or it gets attacked).

Commercial treatments such as Yervoy and Keytruda are based on pathways such as programmed death-ligand 1 (PD-L1) inhibitors.

ASX peer Immutep is tackling the Lag-3 pathway with encouraging results.

"There are nuanced differences, but they all basically work the same way," Dr Garner says.

But Vista shows promise in overcoming strong patient resistance to the current drugs.

"For example, half to two-thirds of patients don't response to PD-L1 therapies," Dr Garner says. "It may be that Vista stops them from responding."

This raises the prospects of a combination drug.

Safety first

HMBD-002 passed muster in a US-based 48-patient, phase I study, with 28 patients treated in combination with Keytruda.

The trial showed the agent was “pharmacologically active” and generally safe and well-tolerated.

Another distinction is that most drugs to date have been based on the immunoglobulin 1 (IgG1) antibody that causes toxicity, cytokine release syndrome (CRS, inflammation from a hyperactive immune system).

HMBD-002 harnesses the IgG4 antibody.

“While this sounds like a nerdy technical nuance, IgG4s don’t cause CRS,” Dr Garner says.

Of the safety trial enrolees, 28 were also dosed with Keytruda with no apparent CRS.

“Normally no one is excited about clearing phase I, but in oncology that’s half the battle,” Dr Garner says.

“If this had been presented to us as a pre-clinical opportunity, we would have been more wary.”

Finances and performance:

In April, Percheron reported end of March quarter cash of $12.9 million.

Accounting for the DMD trial wind-up costs and the acquisition, we estimate the June quarter statement will show cash of $5million to $6 million - the remnants of last year’s $14.85 million capital raising, by way of an $8 million placement and a share purchase plan that bought in another $1.85 million, at eight cents a share, a 14 percent discount.

Dr Garner reckons these funds can go a long way, especially when combined with potential grants.

“Our priority is to get good convincing data, but often in oncology there are ways to do that economically.”

Should the company become profitable, it can avail of $82.4 million of accrued tax losses.

Not surprisingly, Percheron’s registry has evolved to largely retail holders, with most institutions “gracefully” exiting after the Friday the 13th horror show.

“The register has been remarkably stable since [December],” Dr Garner says. “For the most part, we have been left with a register of loyal shareholders who want the company to succeed.”

Powerhouse Ventures has gone below the 5% threshold, while the five backers of the first coup attempt have shed their holdings altogether.

“We have a little bit of scar tissue and keep an eye on the register,” Dr Garner says.

Wisely, in April the company announced bonus loyalty options on a one-for-10 basis, exercisable at 3.5 cents within three years.

Dr Garner has forfeited half his salary, but - board note - he’s never worked harder in his life to fix the snafu.

In the meantime, ATL1102 remains on the books awaiting a divestment or partnership.

“It’s probably not going to run off the shelves,” he says.

“We must find the right partner and the right circumstances.”

“But if we can give the drug a home and another chance then we would love to do that.”

Percheron shares tumbled from six cents to one cent on the day of the trial news.

The stock hit a 12-month peak of 12 cents in mid-October last year and an all-time high of 27 cents in October 2021. The company held a 10-for-one consolidation in 2013.

Percheron jumped 30 percent, or one-third of a cent, on news of the asset purchase.

Dr Boreham’s diagnosis:

Dr Garner says the company could have done little differently to ameliorate the risk of the DMD trial.

For example, the “imperfect” endpoints largely were determined by the regulators.

“The company had done all the reasonable things to take the risk out of it, but it was never a slam dunk,” Dr Garner says.

He says the acid test of any drug candidate is when it is applied in a large, randomized, multi-centre study.

“It’s a reminder - if one is ever needed - that drug development is a risky business,” he says.

“Many drugs fail in development, through no fault of anyone. We set difficult tasks and sometimes don’t succeed.”

This is a lesson keenly felt by Opthea, which in March reported the failure of both of its phase III eye disease trials.

While Opthea's fate is undecided, it is likely to be cruelled by the complex debt funding it had in place.

Percheron is fortunate to be living another day, courtesy of its well-timed capital raising ahead of the Friday the 13th black-cat event.

The Vista program is early stage, but promising.

If management can learn from the setback, hopefully it can banish the curse of avicursen.

The crucial factor is that Percheron has acquired a genuinely promising asset, rather than "this year's Christmas turkey" (as Dr Garner puts it).

Ultimately, he says, there's an element of gut feel in acquisitions.

So, let's hope Percheron's corporate belly compass is pointing in the right direction.

Dr Boreham is not a qualified medical practitioner and does not possess a doctorate of any sort. He is relieved that no more Friday the 13ths are scheduled until mid-February next year.