Biotech Daily

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Daily news on ASX-listed biotechnology companies

Dr Boreham's Crucible: Prescient Therapeutics

By TIM BOREHAM

ASX code: PTX

Share price: 4.2 cents

Shares on issue: 1,051,514,543

Market cap: $44.2 million

Chief executive officer: James McDonnell

Board: Dr James Campbell (chair), Dr Ellen Feigal, Dr Allen Ebens, Dr Gavin Shepherd, Melanie Farris

Financials (year to June 30, 2025): interest income $225,611, loss of $7.3 million (previous $8.23 million deficit), cash of $6.9 million (down 34%)

Identifiable major shareholders: Anthony and Michelle Kittell 3.69%, David Kenley 1.96%, Dr Gavin and Catherine Shepherd 1.67%

As the Wise Man sayeth, one should giv-eth a task to a busy person if one want-eth it done well.

Sadly-eth, that doesn't translate too well into the corporate biotech sphere.

Many drug developers proclaim they have 'multiple shots at goals' - more than one clinical program, that is - and are busier than a one-armed bricklayer.

The trouble is, they don't have Sam Kerr - now recovered from injury and back in form - or the funding to get past the goalie.

In the words of Precision Funds Management's Dermot Woods, companies also need to present scientific information in "semi understandable" form.

"It helps if [investors] can pronounce the name of a disease, or it has an action we can remember."

Cancer drug developer Prescient has picked up the simplicity-is-godly vibe, having frozen several cell therapy pursuits to focus on a single blood cancer program.

Not too long ago, Prescient highlighted its acquired assets in the sexy CAR-T space.

Now, it's gone DeLorean and Back to the Future with its more advanced legacy program, PTX-100.

This is to treat the difficult blood cancer, cutaneous T-cell lymphoma (CTCL).

The CAR-T programs have been parked, so to speak.

"You only have so many resources and you need to manage them carefully," says Prescient's new CEO, James McDonnell.

"You need to fight for your cash and have a clear message about what you are going to do with it."

Clear path

While Prescient only has one shot at goal in biotech's penalty shoot-out, there's a clear trajectory into the back of the net.

Having reported "strong" phase Ib trial data, Prescient has embarked on a phase IIa/IIb effort for lead compound PTX-100 (see below).

Management hopes it can become a registration study pitched at regulatory approval - the World Cup of any drug development.

The trial dosed the first patients in June.

Mr McDonnell says it's still early days, but "it means a lot to dose the first patient because everything we have done to get to that point is enormous".

The US Food and Drug Administration has bestowed orphan and fast track drug designation on PTX-100.

The Hand of Hopper (with apologies to Diego Maradona)

Prescient evolved from oncology house Virax Holdings, acquiring the 'old' part of its current portfolio, via the acquisition of Aktivate Therapeutics in October 2014.

The $2.3 million scrip deal was engineered by legendary biotech entrepreneur Paul Hopper. For those new to the planet, Mr Hopper was also involved in founding ASX-listed cancer plays Imugene, Chimeric Therapeutics, Radiopharm Theranostics and the acquired Viralytics.

Virax undertook a one-for-20 share consolidation and changed its name to Prescient.

Prescient's foundation programs were PTX-100 and PTX-200, which are not Peruvian airline codes but different disciplines of targeted therapies.

A pathway inhibitor, PTX-100 has the ability to block oncogenic pathways called Ras and Rho (see below).

In May 2000, Prescient acquired its Omnicar CAR-T program, from the Ivy League institution University Pennsylvania.

This august establishment is reputed to be the home of CAR-T therapy – and who are we to argue?

Mr McDonnell took over from former analyst and molecular biologist Steve Yatomi-Clarke, who had run the company since 2016.

It's no accident that Mr McDonnell has a background in haematological disorders. A registered pharmacist, he held numerous senior leadership roles.

Mr McDonnell was head of global marketing at Pharmion, eventually acquired by Celgene Corporation for $2.9 billion.

More recently, he headed the Swiss mob Vifor Health's Australian operations, before and after the kidney specialist was taken over by CSL in 2022, for circa $17 billion.

Earlier he worked at the ASX-listed Chemgenex, which developed the chronic myeloid leukemia drug Omapro (omacetaxine), as did Prescient chair Dr James Campbell.

About CTCL

Cutaneous T-cell lymphoma (CTCL) is a rare type of cancer that begins in the white T-cells, which boost the immune system.

The T-cells become cancerous and accumulate in the skin, causing symptoms like red, scaly patches or bumps that can resemble eczema or psoriasis.

Unlike with most cancers, the treatment effects are evident with the naked eye. Common types include mycosis fungoides, a slow-growing form, and the more aggressive Sézary syndrome.

In the US CTCL affects about 3,000 new cases a year.

In the case of advanced patients, two-thirds will die within five years.

Mr McDonnell says many CTCL patients have undergone multiple unsuccessful therapies, with no more options.

“Only one third respond and only for an average six months, so there are a lot of relapsed and refractory patients out there.”

The prevalence of CTCL is only about twice the incidence (new cases), thus highlighting the short life expectancy.

About the trial

After the FDA cleared the company’s investigational new drug (IND) application in December, Prescient established the first clinical site at the Epworth Freemasons’ haematology clinical trial unit, overseen by principal investigator Prof Miles Prince.

In late May, the company dosed the first patient, at a Perth site and so far, the phase IIa trial has dosed four patients at three sites.

“This is a rare disease, so getting patient access is critical,” Mr McDonnell says. “These are significant cancer centres with a significant number of patients.”

The company expects to enrol up to 40 patients - 20 in the initial dose-ranging stanza.

The trial targets 15 clinical sites: three in Australia, six in the US, three in Italy and three in France.

The dose methodology is in keeping with the FDA’s desire for drug developers to find the most effective - rather than highest - dose.

The first 40 patients will be dosed either 500 or 1,000 milligrams per square metre.

After 20 patients, a dose optimization committee will scrutinize the data.

The study is open label, which means the company can announce the results immediately.

Naturally, the phase IIb study will go ahead with the optimal dose. The single-arm effort is expected to enrol about 75 patients who have failed two lines of previous therapy.

But this number could increase if the FDA requires a control group.

Ras-ing things up

What's all the fuss about?

In terms of mechanism of action, PTX-100 disrupts the so-called Ras family pathway, which consists of 170 proteins with different purposes.

The 'razzes' are switches that attach to the cell membrane.

In tumors, the switches stay on. They need to be turned off, like a dodgy power point that requires a $300 electrician's house call to fix.

As commissioned research house Pitt Street Research describes it, PTX-100 blocks a cancer growth enzyme known as geranyl-geranyl-transferase type 1 (GGT-I).

Yes, it does. Yes it does.

The process also disrupts oncogenic Ras pathways by inhibiting the activation of cancer cells known as Rho, Rac and Ral. (Not to be confused with 'shake, rattle and roll' - and apologies to Bill Haley and Elvis there.)

The key thing is this inhibition leads to apoptosis (death) of cancer cells.

"In some ways, PTX-100 isn't different from other cancer drugs in the sense that it is disrupting an aberrant cellular signalling pathway that prevents cells from dying," Pitt Street says.

"But PTX-100 is believed to be the only GGT-1 inhibitor in the world in clinical development. PTX-100 can block multiple pathways that other inhibitors cannot."

Recognising this, the FDA in April granted fast-track designation to PTX-100, for adults with relapsed or refractory mycosis fungoides (the most common subtype of CTCL).

While Prescient is convinced PTX-100 ultimately works, Mr McDonnell says there's still a lot under the bonnet to understand.

Okay, but where's the evidence?

An earlier phase Ia trial of PTX-100 showed a 45 percent objective response rate (ORR).

Mr McDonnell says this was "way better than the 30 percent we were looking for".

This response also compares with a 36 percent ORR for current therapies, which also have a 36 percent severe adverse event rate. Only four percent of the PTX-100 patients had such side effects.

ORR measures the percentage of patients in a clinical trial whose cancer shows a significant decrease in size, or completely disappears after a treatment.

One patient had a complete response - tumor disappearance - sustained for about two and a half years.

The patient duration on trial was 10.7 months, compared with 6.7 months for the standard-of-care.

The earlier trial covered two patient cohorts: those with CTCL and the harder peripheral T-cell lymphoma.

Of the 11 responders, seven had CTCL.

“I would hope and expect our duration of therapy [for this cohort] is around the 12-month mark,” Mr McDonnell says.

Finances and performance:

Prescient had $6.9 million of cash at June 30 and in July raised a further $9.8 million, in a $6.8 million share purchase plan and $3 million placement at 4.0 cents a share.

Mr McDonnell says management will keep an open mind on funding as the PTX-100 program progresses. Successfully completing phase IIa provides an opportunity to engage with potential funders and partners.

Over the last 12 months, Prescient shares have ranged between 3.7 cents (late November last year) and 5.9 cents (late January this year).

The stock peaked at 29 cents in September 2021.

Top marks to FDA

Mr McDonnell says the FDA was expeditious in accepting the company’s IND, while the fast-track designation was - well - fast track.

Fast-track status means the company doesn’t have to wait for the end of the phase IIb trial to request a ‘type B’ guidance meeting with the agency.

Mr McDonnell says: “I would give them a good scorecard at the moment, but we need more information to get down to the nitty gritty.”

This of course refers to the mayhem afflicting the agency in the early days of the President Donald Trump administration.

Prescient plans a US facility to make the drug, but the FDA had favoured this approach dating well before Mr Trump’s election.

Dr Boreham's diagnosis:

Not only do drug developers need to strive for more simplicity in their programs, they also need to communicate to investors more clearly as well.

In his initial weeks, Mr McDonnell has undergone a blitzkrieg of investor briefings (and media interviews).

"The feedback has been quite positive," he says.

"The central theme is that everything is clearer and directed. We have a clear destination, and we need to get there."

Mr McDonnell says beyond ascertaining that a drug candidate works, biotechnology management must consider commercial aspects such market access, insurance coverage and what clinicians think of the therapy.

He says the CAR-T program had no clear funding pathway and would have involved a clunky treatment pathway.

"Nothing was easy".

In contrast, PTX-100 offers a clear pathway to approval.

"We just need to deliver on the results," Mr McDonnell says.

Indeed! Could anything be so simple?

Disclosure: Dr Boreham is not a qualified medical practitioner and does not possess a doctorate of any sort. Is that simple enough?