Biotech Daily

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Daily news on ASX-listed biotechnology companies

Dr Boreham's Crucible: Cynata Therapeutics

By TIM BOREHAM

ASX code: CYP

Share price: 1.2 cents

Shares on issue: 243,454,369

Market cap: $2.9 million

Chief executive officer: Dr Kilian Kelly

Board: Dr Geoff Brooke (chair), Dr Kelly, Dr Paul Wotton, Dr Darryl Maher, Janine Rolfe

Financials (March quarter 2026): revenue nil, net cash outflows $2.2 million, end of quarter cash of $1.6 million (before $1.5 million placement)

Identifiable major holders: Fidelity Investment Management 8.8%, Acuity Capital 4.8%, Fujifilm 3%, Phillips Asset Management (Bioscience Managers) 1.6%

The curse of the late-stage clinical trial has struck again, with the biotech gods delivering a double whammy blow to stem cell drug developer Cynata.

Last Friday, Cynata shares tumbled up to 94 percent after the company revealed its third-party funded knee osteoarthritis trial had flopped.

Two days earlier, the company announced a similar fate for its adult graft-versus-host disease (GvHD) study, which it has now abandoned.

Both trials did not meet their primary efficacy endpoints, as well as most secondary aims.

In both cases, the active patient groups did show efficacy – but so too did the placebo cohorts.

“This has been a really disappointing past week for the company and for all of our shareholders,” CEO Dr Kilian Kelly said. “This is clearly not where we wanted to be”.

But it is what it is.

The Cynata board is now “actively reviewing” alternative uses for the company’s Cymerus platform technology and promises an update “very soon”.

About Cymerus … or pixenstrocel

Cynata is based on its “revolutionary” stem-cell manufacturing platform, Cymerus.

Cymerus stems from the University of Wisconsin-Madison, the epicentre of US stem cell research.

Cynata strives to overcome the enduring problem of obtaining enough of the cells from donors to service patients cost-effectively.

Cynata has been trialing induced pluripotent stem cells (IPSCs), from which the healing agent - mesenchymal stem cells (MSCs) - are derived. IPSCs promise to produce a limitless number of high quality and high potency doses from a single donor.

“It’s a real game changer if these therapies can be produced at scale,” Dr Kelly told Crucible in 2024.

“The major obstacle to commercialization has been that standard manufacturing methods require ongoing new donors.”

Early last month the World Health Organization bestowed Cymerus with an international nonproprietary name: pixenstrocel.

About Cynata

University of Wisconsin-Madison Prof Igor Slukvin co-founded Cynata, to licence the technology from the Wisconsin Alumni Research Foundation.

That duly happened and Cynata back-door listed in October 2013, via the shell of green nappy maker Eco Quest.

In June 2023, Dr Kelly became CEO, with incumbent Dr Ross Macdonald retiring after 10 years in the job. Dr Kelly had been Cynata’s chief operating officer. Before then had senior roles at influenza drug house Biota and stem-cell peer Mesoblast.

In a 2019 ‘sliding door’ moment, Sumitomo made a provisional $2 a share cash bid for the Cynata, which was not progressed.

In late 2019, Cynata forged an alliance with Fujifilm, which involved Fujifilm funding the development of CYP-001 (the GvHD therapy) in return for the global selling rights.

But in another Gwyneth Paltrow moment, Fujifilm decided to emphasize its own cell manufacturing and the rights reverted to Cynata in 2021.

Graft versus host disease

GvHD occurs when transplanted bone marrow material rejects the body (this is the opposite to usual organ transplants, when the body writes the ‘Dear John’ letter).

It was thought that Mesoblast winning US Food and Drug Administration approval for its paediatric GvHD stem cell treatment might bode well for Cynata.

Evidently, there are stem cells and there are stem cells.

Cynata’s primary evaluation at 100 days showed “no significant differences” between active and control groups.

The data covered 57 patients, with 26 receiving the active drug and 31 the placebo.

Eight enrolled patients withdrew without receiving treatment.

The results showed an overall response rate (ORR, the primary endpoint) of 57.7 percent in the active group (15 patients) and 54.8 percent for the control group (17 subjects).

At day 100, the ORR was 23.1 percent for the ‘actives’ and 29 percent for the control.

The overall survival rate at 100 days was 88.1 percent for the CYP-001 group and 82.3 percent for the control.

Statistically significant? Nah.

The c(rux) of the problem

Dr Kelly said the GvHD results were “especially unexpected”, given earlier robust phase I data.

But the company could not replicate the structure of this study, which recruited patients who had not responded to steroids.

Phase II trial design was complicated by the emergence of a new drug, the Janus kinase inhibitor ruxolitinib (‘rux’), for these patients.

The study enrolled high-risk, newly diagnosed patients with an acute form of the disease.

None of the patients was on rux at the start, given it's only approved for steroid-resistant GvHD.

However, patients can be considered steroid-resistant quite quickly - within five to seven days.

"At that time, investigators were able to initiate rux if the patients were not improving or deteriorating, and that's exactly what happened in many patients," Dr Kelly says.

As per standard protocol, patients receiving such second line treatment were deemed trial 'failures'. That's because it's impossible to attribute any effect to either CYP-001 or rux.

"I fear that in at least some cases, rux may have been initiated before the [stem cells] had a chance to work," Dr Kelly says.

Dr Kelly adds that clinicians needed to have the best interests of the patients at heart - and if that meant prescribing rux, then so be it.

No PASS for knee OA study

Dubbed Sculptor, Cynata's 320-patient, phase III trial of its knee osteo candidate CYP-004 fell short of the co-primary endpoints of reduced pain and lower cartilage loss.

CYP-004 is formulated for intra-articular injections.

The study used the patient-acceptable symptom state – PASS – the threshold at which the pain becomes bearable.

"The pain reduction in the control group was much better than expected," Dr Kelly laments.

"The study design assumed that 35 percent of control patients would reach the PASS threshold, but … almost 50 percent have done so."

"The pain reduction in the active group was in line with our expectations, but unfortunately there is no evidence of a reduction in cartilage loss."

The data shows 51.7 percent of the active group reached PASS level, compared with 48.1 percent in the control group.

So, this PASS was a fail.

The trial also reported a mean loss of cartilage thickness of 0.27mm in the active group, versus 0.21mm for the controls.

Initiated and funded by the University of Sydney, the trial was aided by a $2 million grant from Australia's National Health and Medical Research Council (NHMRC).

The University is assessing the data on more "holistic" measures, such as quality of life.

Dr Kelly says given pain evaluation is subjective, the dreaded Placebo Effect may have been at work.

"There's huge expectation around stem cell-based treatments and people really want to get treated with these products," he says.

"If people believe that they have been given an effective treatment, it can really change their own perception of pain."

In the pipeline

What does the future hold for Cynata?

We can't be sure before the board reports back on its musings, but the company has two ongoing clinical trials - in kidney transplantation and diabetic foot ulcers (DFUs).

The phase I kidney study is being conducted – and funded – by The Netherland's Leiden University Medical Hospital.

The trial examines CYP-001's ability to reduce reliance on immune-suppressants to prevent organ rejection.

Current anti-rejection drugs are extremely toxic and - ironically - cause kidney damage and increase the risk of cancer.

In December last year, an independent safety review allowed the trial to proceed from the first to second cohort, having not identified any rejection or other issues.

Diabetic foot ulcers

Eighteen months ago, Cynata completed a phase I diabetic foot ulcer (DFU) trial "with encouraging results".

The DFU trial deploys a novel agent, CYP-006TK combining Cynata's stem-cell therapy with a wound dressing.

The company acquired the rights to the latter from Tekcyte's Cytopatch in 2021.

In late 2024, Cynata released data from the first eight patients after 10 weeks' follow up.

The results showed an “encouraging” 83.6 percent reduction in wound size, versus 47.8 percent for the standard-of-care.

While the company intends to partner the program, Dr Kelly says it likely will require another trial and further Cynata funding - money the company currently does not have.

It’s also a case of ‘never say never’.

“We need to evaluate our options on that and [the DFU program] will certainly be part of our upcoming announcements,” Dr Kelly says.

Finances and performance

At the end of March, Cynata had $1.6 million of cash in the bank, having reported March quarter outflows of $2.2 million.

The company then raised $1.5 million in a placement, at 25 cents apiece, a then 22 percent discount. It also drew down $1.2 million from a $7.5 million standby equity capital facility with Acuity Capital.

Management expects a Federal Research and Development Tax Incentive (RDTI) around October this year.

The company has pocketed $17.9 million in such payments over the last decade.

There’s not much to show for taxpayers. But if drug development were a sure thing, biotechs would be able to access conventional funding.

At December 2025, the company reported accumulated losses of $94.1 million.

Dr Kelly says that without the need to fund the GvHD trial, future outflows should be smaller. “We will seek to cut as many costs as we can, in order to prolong that runway in order for the next steps to be worked out.”

Over the last 12 months Cynata shares have traded between 40 cents, in early January this year, to last Wednesday’s 1.4 cents nadir.

The stock hit an all-time peak of $1.73 in July 2019.

Dr Boreham’s diagnosis:

Strictly speaking, the trials didn’t fail.

A clinical study is meant to prove that a drug works or otherwise – and they definitively, successfully proved the latter [Hat-tip: Dr Roland Scollay].

But that's cold comfort for Cynata investors, who have seen more than 90 percent of the value of their investment obliterated.

As for patients with little else available to them ...

The ASX biotech clinical trial casualty list now includes Opthea with its two phase III eye disease trials, Immutep with its lung cancer study, Argenica with its stroke trial and Percheron with its Duchenne muscular dystrophy effort.

Bear in mind that as with Cynata, Opthea is pursuing a new direction with a rare lung disease, while Argenica believes it has found a patient cohort for which its drug could be effective.

Immutep has other programs in cancers and autoimmune diseases.

In coming months, major trial results include Recce Pharmaceuticals (diabetic foot infections), Paradigm Biopharmaceuticals (knee osteo-arthritis) and Actinogen (Alzheimer's disease).

The sector is due for a lucky break.

And given Avecho on Wednesday announced positive interim signs for its phase III synthetic cannabis study, maybe there's a biotech god after all.

Dr Boreham is not a qualified medical practitioner and does not possess a doctorate of any sort. He was hoping for a lucky break but fractured his shoulder instead.