Biotech Daily

Friday March 14, 2025

Daily news on ASX-listed biotechnology companies

*** ASX, BIOTECH UP: PERCHERON UP 10%; PROTEOMICS DOWN 7%**

*** DR BOREHAM'S CRUCIBLE: SYNTARA (FORMERLY PHARMAXIS)**

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*** RACE PHASE I RC220 BISANTRENE COMBINATION TRIAL APPROVED**

*** MESOBLAST RYONCIL IN 4 US COMPENDIA**

*** PERCHERON POWERHOUSE BOARD SPILL EGM**

*** FIL (FIDELITY) TAKES 8% OF POLYNOVO**

*** PETERS TAKES 28% OF OPTISCAN**

*** MA FINANCIAL (MOELIS) TAKES 9.5% OF TRAJAN**

MARKET REPORT

The Australian stock market was up 0.52 percent on Friday March 14, 2025, with the ASX200 up 40.6 points to 7,789.7 points.

Twenty-one of the Biotech Daily Top 40 companies were up, 11 fell, seven traded unchanged and one was untraded. The four Big Caps were up.

Percheron was the best, up 0.1 cents or 10 percent to 1.1 cents, with 4.95 million shares traded. Dimerix was up seven percent; Imugene and Neuren climbed more than five percent; 4D Medical, Emvision and Polynovo were up more than four percent; Aroa, Cyclopharm, Genetic Signatures, Nova Eye, Paradigm, Starpharma and Universal Biosensors rose two percent or more; Amplia, EBR, Medical Developments, Micro-X and Pro Medicus were up more than one percent; with Clarity, Cochlear, CSL, Nanosonics, Resmed and Telix up by less than one percent.

Proteomics led the falls, down 3.75 cents or 7.25 percent to 48 cents, with 257,143 shares traded. Alcidion lost five percent; Avita and Curvebeam fell more than four percent; Orthocell was down 3.1 percent; Compumedics and Syntara shed two percent or more; Prescient was down 1.2 percent; with Clinuvel, Mesoblast and Opthea down by less than one percent.

DR BOREHAM'S CRUCIBLE: SYNTARA (FORMERLY PHARMAXIS)

By TIM BOREHAM

ASX code: SNT

Share price: 7.8 cents

Shares on issue: 1,623,327,805

Market cap: $126.6 million

Chief executive officer: Gary Phillips

Board: Dr Kathleen Metters (chair), Mr Phillips, Dr Simon Green, Hashan DeSilva

Financials (December half 2024): revenue nil, loss of $2.7 million (previous deficit $5.95 million), cash of $18.1 million (up 217%).

Major shareholders: D & A Income 18%, Platinum Investment Management 14%, BVF Partners 6%, HB Technology 3.5%

While the myelofibrosis drug landscape has become more crowded since Syntara (then Pharmaxis) decided to focus on that disease in 2020, CEO Gary Phillips has no qualms about the company's 'strategy about-face' that turned the rare blood disease into its lead program.

Syntara is all about amine oxidase chemistry, the basis of several enzymes involved in inflammation and fibrosis.

Pharmaxis's reason for being was its approved Bronchitol treatment for cystic fibrosis, but in 2023 the company sold the program and slashed its cost base.

"The ramifications of the change rumbles on," Mr Phillips says.

"We reduced our staff from 65 to 25 and saved $14 million in annual expenditure.

"Once we realized the underlying [Bronchitol] sales in the US weren't going to materially change, it was a no-brainer to get rid of it. It was a relief just to be able to focus on that lead program and it makes us easier to understand as well."

Now, new myelofibrosis drugs have entered the market, notably the home-grown Glaxosmithkline-Cytopia Ojjaara. Two more are at phase III.

But Mr Phillips says the Devil's in the detail and these 'rival' drugs may help Syntara's efforts to bring its lead drug to market.

Details below.

Bronchitol took its toll

In its Pharmaxis days, the company's business revolved around Bronchitol, an inhaled dry mannitol powder for cystic fibrosis.

While European, Australian, Brazilian and even Russian health regulators were quick to the party, the US Food and Drug Administration held out and did not approve the therapy until 2020.

By then, the pandemic was upon us and the treatment landscape had moved on anyway.

In October 2023, Pharmaxis sold its loss-making Bronchitol business to Arna Pharma for nil consideration (but with adjustments, see below).

Pharmaxis also closed its Frenchs Forest headquarters in Sydney and moved to smaller digs.

Luckily, Pharmaxis was partnered with the Italian-based Chiesi for Bronchitol, with Chiesi bearing all clinical - and most regulatory - costs of developing Bronchitol.

Pharmaxis listed on the ASX in 2006, raising $25 million at 50 cents a share. A secondary listing on the Nasdaq was abandoned in 2009 for cost reasons.

In 2019, long-time German partner Boehringer Ingelheim handed back the rights to a non-mannitol drug PXS4728A for the liver disease non-alcoholic steato-hepatitis (NASH or fatty liver disease), having generated $83 million of milestone payments for Pharmaxis along the way.

Syntara's 'gelati' flavors

The company's compound comes in three 'flavors': SNT-4728 (for Parkinson's disease), SNT-6302 (scarring) and SNT-5505 (myelofibrosis and hepatocellular carcinomas).

The target is pan-lysyl oxidase (LOX), an enzyme closely implicated in inflammation and fibrosis.

Investors are focused on Syntara's current phase I/IIa trial for the rare blood cancer, myelofibrosis, a scarring of the bone marrow that interrupts the normal production of white and red blood cells and platelets.

Globally there are about half a million sufferers, generally aged between 50 and 80 with an average five-year life expectancy.

Currently, myelofibrosis is treated by JAK (Janus kinase) inhibitors that provide symptomatic relief, but do not ameliorate the disease. They also cause unpleasant side effects.

Granted orphan drug status by the FDA in in 2020, SNT-5505 targets the matrix [inflammation] formation in the bone marrow and thus modifies the disease.

“Our drug does something about the underlying disease, so we have quite a different approach,” Mr Phillips says.

My oh my! Decent trial results

On December 12, 2024, Syntara reported the first interim results from its 16-patient, phase I/IIa myelofibrosis trial, showing “excellent” tolerability and improvements in symptoms, including spleen volume reduction (a good thing).

The open-label study, at 19 sites in Australia, South Korea, Taiwan and the US, combined SNT-5505 with ruxolitinib (branded Jakafi), and enrolled 16 patients with intermediate or high-risk myelofibrosis, with average ruxolitinib use of 3.2 years.

At 12 weeks of treatment, 46 percent of the evaluable patients (six of 13) had achieved a 50 percent improvement in their total symptom score, a standard primary endpoint for myelofibrosis trials.

This improved to 80 percent (four of five patients) who reached 38 weeks of treatment.

Also, 30 percent of patients achieved a “clinically meaningful” spleen volume reduction of 25 percent, with one-quarter of them having a reduction of at least 35 percent.

The company is now analyzing 52-week results, after which it plans to meet with the FDA to discuss a pivotal study aimed at US (and European) marketing approval.

Mr Philips hopes the study can be an adaptive phase IIc/III trial, which uses the current trial candidates as the base for expanded enrolment.

The company expects to announce nine-to-12-month results in the current half year.

No drug program is an island

John Donne said that no man is an island – and a drug development program also can’t be viewed in isolation.

As we said, there’s a lot going on in the myelofibrosis space. For a start, Jakafi - a $US17,000 per bottle drug - comes off-patent in 2028.

“When that is genericized, it will significantly impact the market for JAK inhibitors,” Mr Phillips says.

In September 2023, the FDA approved Glaxosmithkline’s Ojjaara, developed by local lads Prof Andrew Wilks and Dr Chris Burns (CEO of the listed Amplia).

Good job!

Meanwhile, two myelofibrosis drugs are in phase III, but Mr Phillips claims they are beset with problems.

Abbvie's Navitoclax did not show a significant difference in symptom score when combined with ruxolitinib.

"It didn't raise much hope within the myelofibrosis community it was going to offer much different."

The phase III trial of another candidate, pelabresib, showed improved symptoms - but maybe not enough.

Novartis acquired Morphosis, owner the program for $US2.9 billion in April 2024. But some patients on the treatment arm developed leukaemia - a salient case of the cure being worse than the disease.

Novartis' plans for an FDA approval application have been delayed.

Meanwhile, Kartos Therapeutics plans a phase III trial of Navtemadlin (KRT-232), which puts the program about a year ahead of Syntara's.

Mr Phillips says while Syntara's trial numbers were small, SNT-5505 had a superior symptom rating with similar spleen reduction stats.

Parkinson's program a good show

Syntara's secondary programs are for myelodysplastic syndrome (MDS), a Parkinson's disease precursor condition and a skin scarring program.

Another haematological malignancy, MDS is a high unmet need which Mr Phillips dubs a $US3 billion-plus market opportunity.

Pending a successful phase I effort, the company envisages a phase II dose escalation and expansion trial.

The program is backed by leukaemia groups locally and in Germany but ultimately funded by government grants.

SNT-4728 targets a precursor condition called idiopathic rapid eye movement sleep behavior disorder (IRBD).

IRBD sufferers thrash about and cry out in their sleep as they live out their dreams.

The disorder can precede motor cognition dysfunction by up to 20 years, with 70 percent of sufferers going on to develop neuro-degenerative diseases such as Parkinson's.

The problem with Parkinson's is by the time IRBD is diagnosed, about 80 percent of the dopaminergic neurons are gone.

The charity Parkinson's UK is funding a phase II trial, with clinical proof-of-concept data is expected in the December half year.

Scarring: a burning, unresolved issue

The company is also targeting burns-related scarring, in an investigator-led trial with Perth burns legend Prof Fiona Wood.

This one is all about using the company's pan-LOX inhibitors to prevent scarring after burns surgery.

In 2023, a 42-patient, phase Ic trial met its primary safety objective and two secondary biomarker endpoints in patients with established scars.

"We are still working with Prof Wood and her team on the next topic study, which is likely to be in keloid or hypertrophic scars," Mr Phillips says.

Some of the funds from the recent placement were earmarked for a follow-on company-sponsored study.

Finances and performances

In December 2024, the company launched a $15 million capital raising, in a two-tranche placement at six cents a share.

At the end of December, the company had $18.1 million of cash and no debt, before banking the second tranche $2.6 million.

This should last the company until mid-2026.

In the December 2024 quarter, the company reported $300,000 of net cash inflow, courtesy of a $4.6 million research and development tax incentive payment.

Still, the performance is a turnaround from the September quarter deficit of $4.2 million and average $3.4 million of outflows over four quarters.

Over the last 12 months, Syntara shares have JAK-knifed between 1.5 cents (May 2, 2024) and 8.7 cents (February 19, 2025).

The stock had a five-year peak of 14.5 cents in September 2021 – a long way from the $4.07 in 2007.

Arna you gonna pay up?

While the Pharmaxis/Bronchitol separation is complete, there is the wee matter of money owed to the company by Sydney's Arna as part of the deal.

So far, Arna has paid $3.5 million of the $7.5 million owed, while a "large chunk" of the remaining $4 million has been agreed to and is being recovered "steadily and surely".

The $4 million is not recognized as cash in the December quarter statement.

We take it the matter didn't get to court, but some stern correspondence has been entered into and Syntara is confident of full recovery.

"Slowly, slowly catchee monkey," Mr Phillips says.

Dr Boreham's diagnosis:

Despite the flurry of activity in the sector, Mr Phillips says the song remains the same for the myelofibrosis program.

The lyrics are that about 60 percent of myelofibrosis cases are not well controlled with JAK inhibitors, and the remaining patients deteriorate over time.

Mr Phillips is long in the tooth enough to know that taking the myelofibrosis drug to market - and even to next clinical trial phase - is a bridge too far for the company.

"We will talk to potential acquirors and investors in the first half of this year, as we wait for the FDA response to our application," he says.

"Our register is stuffed with specialist healthcare investors who understand the myelofibrosis space specifically," he says. "It's conceivable we could raise a significant amount of money in the second half of this year to do a phase III study."

Cutting through the noise from the myelofibrosis drug landscape, Mr Phillips says: "Our mechanism of action is very different from almost all the other drugs in the myelofibrosis pipeline."

He had better be right - and other challengers had best not emerge in the tortuous juncture between promising trials and approval.

In the words of Pat Benatar - sort of - the company should hit investors with its best shot with the myelofibrosis program.

The promise of the secondary programs aside, that's why they are shareholders.

Disclosure: Dr Boreham is not a qualified medical practitioner and does not possess a doctorate of any sort. But he will give it his best shot.

WALTER AND ELIZA HALL MEDICAL RESEARCH INSTITUTE

The Walter and Eliza Hall Medical Research Institute says its researchers have identified the structure of a protein, called Pink1, linked to Parkinson's disease.

The Walter and Eliza Hall Institute said the research determined the structure of human Pink1, or phosphatase and tensin homolog (Pten)-induced kinase 1, bound to mitochondria and hoped it could "accelerate the search for a drug to stop the condition".

The Institute said Pink1 was a protein that attached to the surface of damaged mitochondria, signaling for its removal, and when Pink1 was mutated in patients, broken mitochondria accumulated in cells, leaving toxins to accumulate and eventually killing the cell, with brain cells especially sensitive to the damage.

WEHI said that "no one had seen what human Pink1 looks like, how Pink1 attaches to the surface of damaged mitochondria, or how it is switched on".

The Institute said the idea of using Pink1 as a target for potential drug therapies had "long been touted but not yet achieved because the structure of Pink1 and how it attaches to damaged mitochondria were unknown".

WEHI said the study, titled 'Structure of human PINK1 at a mitochondrial TOM-VDAC array' was published in the journal Science, with the abstract available at: https://www.science.org/doi/10.1126/science.adu6445.

WEHI Parkinson's disease research centre laboratory head Prof David Komander said determining the structure of Pink1 was "a significant milestone for … Parkinson's".

"Our structure reveals many new ways to change Pink1, essentially switching it on, which will be life-changing for people with Parkinson's," Prof Komander said.

CYCLOPHARM

Cyclopharm says it has a five-year 'Federal supply schedule' agreement to provide its Technegas to the US Veterans Health Administration.

Last year, Cyclopharm said it had an interim agreement to supply 120 US Government Veterans Health Administration hospitals with Technegas for computed tomography-based lung ventilation imaging (BD: Oct 3, 2024).

At that time, the company said the deal would provide Veterans Affairs hospitals with nuclear medicine departments immediate access to the pharmaceutical and consumable components of its Technegas lung ventilation imaging device.

Today, Cyclopharm said the agreement eliminated the need for individual contracts across the Veterans Health Administration's 20 regional procurement offices, improved efficiency and ensured a consistent supply of Technegas patient consumables.

The company said "uncertainty around the evolving policy landscape in the US relating to healthcare funding may influence the timing of contract completion in the near-term".

Cyclopharm did not disclose the commercial terms of the agreement.

Cyclopharm managing-director James McBrayer previously told Biotech Daily that the company would receive $US7,000 ($A11,135) for Technegas generator installation, $US7,000 a year in licencing and $US225 per patient (BD: Dec 5, 2023).

Today, Mr McBrayer said the five-year supply agreement was "a major achievement for Cyclopharm's expansion in the US market".

"This contract strengthens our partnership with the [Veterans Health Administration], underscores the clinical value of Technegas for US veterans, and streamlines access to our technology across federal healthcare facilities," Mr McBrayer said. "This five-year agreement reinforces our position as a trusted provider of nuclear medicine solutions and sets the foundation for continued success in the US market."

Cyclopharm was up three cents or 2.1 percent to $1.46.

RACE ONCOLOGY

Race says it has ethics approval for an up-to 53 patient, phase I, safety, tolerability and pharmaco-kinetics trial of RC220 bisantrene alone and with doxorubicin for solid tumors.
Last year, Race said it had submitted a trial application to Adelaide's Bellberry ethics committee, for a trial at Sydney's Southside Cancer Care Centre (BD: Dec 5, 2024).
Today, Race said the study included a dose escalation stage one, dosing up-to 33 patients with an intra-venous infusion of RC2230 bisantrene alone on day-1, followed by a combination of RC220 and doxorubicin on a 21-day cycle.
The company said the final number of stage one patients was expected to most likely be in the range of 12 to 18 patients.
Race said patients in the trial would "continue to be treated until they reach either successful control of disease, disease progression, unacceptable toxicity, or withdrawal of consent".
Race said that once the maximum tolerated combined dose was determined, safety and pharmaco-kinetics data would be analyzed, followed by stage two of the study.
The company said in stage two it would recruit solid tumor patients not previously treated with doxorubicin or other anthracyclines, who would receive the optimal RC220 dose in combination with doxorubicin to "confirm the safety of the combination and study a range of exploratory endpoints".
Race said the exploratory endpoints included cardio-protection, anti-cancer, anti-aging and other clinical biomarkers, such as the effects of RC220 on the N6-methyl-adenosine M6A RNA regulatory system.
The company said the ethics approval allowed the lead clinical site Southside Cancer Care Centre to begin enrolling patients, subject to institutional approval and site activation this month, with up-to nine other sites to follow.
Race managing-director Dr Daniel Tillett said that ethics approval for the first RC220 trial was "a significant milestone for Race, in line with our vision to improve cancer patient treatment by developing new cardio-protective therapies with anti-cancer benefits".
Race was up 22 cents or 21.4 percent to $1.25.

MESOBLAST

Mesoblast says product information including pricing for Ryoncil for graft versus host disease has been published "in all four major drug pricing compendia in the US".
Last year, Mesoblast said the US Food and Drug Administration had approved Ryoncil, or remestemcel-L, for steroid-refractory acute graft versus host disease (GvHD) in children aged two months and older (BD: Dec 19, 2024).
Last month, the company said Ryoncil would cost $US194,000 ($A308,616) wholesale per intra-venous infusion (BD: Feb 27, 2025).
At the time, Mesoblast said that 375 US children were diagnosed with GvHD a year and that it cost about $US2.5 million to treat a child who died of GvHD within a year of transplant and a further $US1.8 million for those who remained alive.
Today, the company said the pricing compendia provided "the official source for all information required to order product, including product manufacturing details, National Drug Codes for kit purchase, and kit pricing".
Mesoblast said the compendia publishing Ryoncil pricing and product information were Merative Micromedex Red Book, First Databank FDB Medknowledge Drug Pricing, Elsevier Gold Standard Drug Database and Wolters Kluwer Medi-Span Price Rx.
Mesoblast fell one cent or 0.5 percent to $2.07 with 7.65 million shares traded.

PERCHERON THERAPEUTICS (FORMERLY ANTISENSE THERAPEUTICS)

Percheron says a second extraordinary general meeting will vote on a board spill requisitioned by Powerhouse Ventures Ltd.
Last year, Percheron fell as much as 91.5 percent after its phase IIb trial of avicursen for Duchenne muscular dystrophy did not meet its primary endpoint (BD: Dec 18, 2024).
In January, the company said investors had called a meeting to replace chair Dr Charmaine Gittleson and managing-director Dr James Garner with Gregory Peters and Gennadi Koutchin (BD: Jan 19, 2025).
Later, Percheron said it had submitted a non-binding proposal to a “pharmaceutical company” to licence a drug program for a “rare neurological disease” (BD: Feb 24, 2025).
The next day, the company said it had a separate notice from Powerhouse calling for another meeting to replace directors (BD: Feb 25, 2025).
Last week, the company said an extraordinary general meeting defeated the initial board spill, with up-to 56.55 percent against the removal Dr Gittleson and Dr Garner as well as more than 80.91 percent against Mr Peters and Mr Koutchin (BD: Mar 4, 2025).
Today, Percheron said the meeting would vote to replace Dr Gittleson, Dr Garner and Dr Ben Price with Doron Eldar, Dr Julian Chick and Richard Hamersley.
The company included a detailed statement and said its directors “unanimously recommend shareholders vote their shares against all six resolutions”.
The company said it had $17.6 million in cash reserves at December 31, 2024.
The meeting will be held at Level 40, 1 Farrer Place, Sydney on April 24, 2025 at 4pm (AEST).
Percheron was up 0.1 cents or 10 percent to 1.1 cents with 4.95 million shares traded.

POLYNOVO

The Sydney and Hong Kong-based FIL Limited (Fidelity) says it has increased its shareholding in Polynovo from 44,779,690 share (6.48%) to 52,252,136 shares (7.56%).
FIL said on February 20, 2025 it sold 5,795 shares for $1.9139 a share and bought shares between February 21 and March 12, at prices from $1.1570 a share to $1.9500 a share.
Polynovo was up five cents or 4.2 percent to $1.23 with 3.7 million shares traded.

OPTISCAN IMAGING

Peters Investments Pty Ltd says it has increased its substantial shareholding in Optiscan from 223,413,544 shares (26.745%) to 232,218,150 shares (27.799%).
The Perth-based Peters Investments said that between December 5, 2024 and March 12, 2025 it bought 8,804,606 shares for $1,333,248 or 15.1 cents a share.
Optiscan was unchanged at 13.5 cents.

TRAJAN GROUP HOLDINGS

Sydney’s MA Financial Group Ltd (formerly Moelis) says it has increased its substantial shareholding in Trajan from 12,980,266 shares (8.53%) to 14,520,963 shares (9.53%).
MA Financial said it bought and sold shares between September 20, 2024 and March 12, 2025, with the single largest purchase 300,000 shares for $382,048, or $1.27 a share.
Trajan fell 1.5 cents or 1.7 percent to 88.5 cents.