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Daily news on ASX-listed biotechnology companies

Dr Boreham's Crucible: Pharmaxis

By TIM BOREHAM

ASX Code: PXS

Market cap: $88 million

Share price: 27.5 cents

Shares on issue: 319.1 million

Chief executive officer: Gary Phillips

Board: Malcolm McComas (chairman), Gary Phillips, William Delaat, Dr Simon Buckingham

Financials (March quarter 2017): customer receipts $2.267m (year to date $3.957m), total revenue $4.146m ($11.056m), net loss $3.199m ($14.23m), net operating cash flow -$2.13m (-$11.02m), cash on hand $26.5m, estimated current quarter cash burn $9.8m

Major shareholders: BVF Partners LP 15.9%, Australian Ethical 10.2%

We love a tear-jerking redemption story and there is no more a heartening yarn than the revival of the once-tortured drug developer.

Four years ago Pharmaxis looked sicker than the patients it seeks to treat: a large trial of its Bronchitol treatment for respiratory ailments had flopped and the US Food and Drug Administration rejected approval for the use of the drug on cystic fibrosis (CF) patients.

Bronchitol works by reducing the mucus build up in the lungs that progressively restrict breathing.

In 2011 the European regulatory bigwigs said they would reject the company's marketing application, but then Pharmaxis won on appeal.

Fast forward to now and Pharmaxis continues its quest for FDA approval, this time as an adult-only cystic fibrosis treatment.

The difference is that Pharmaxis is more partnered-up than Elizabeth Taylor in her hubby-eating heyday, with its global buddies assuming most of the risk.

In crude terms, Pharmaxis can kick back and enjoy the proceeds from royalties and milestone payments – or at least focus on its busy slate of early-stage projects.

"We are quite unusual as a biotech,"' says chief executive officer Gary Phillips.

"We are not out capital-raising and we have a pipeline of more than one drug."

Previously the company's chief operating officer, Mr Phillips took the top job in March 2013. Apart from retrenching 100 of the company's 160 workers in an urgent cost cutting drive, management shifted focus from 'go to whoa' drug trials to only carrying out the early stages and then seeking partnerships for the expensive stuff.

"With the business model of taking a product to the US market not available, we were going to run ourselves into the ground," Mr Phillips says.

In 2015 Italian pharma house Chiesi acquired the US rights to Bronchitol, in return for milestone payments and royalties in the high teens. Chiesi is also the exclusive European distributor, working on a different margin arrangement.

Crucially Chiesi, which already has a cystic fibrosis portfolio in the US, has funded $US22 million of the $US25 million cost of the current 423-patient global clinical trial (CF303) to satisfy the FDA gatekeepers.

Top line results are due by July 2017.

On FDA approval, Pharmaxis would pocket a $US10 million milestone payment, with on-going annual US Bronchitol sales estimated at $US50-100 million.

Fatty liver busters:

Another major deal is with German drug company Boehringer Ingelheim to develop a treatment for the liver ailment, non-alcoholic steatohepatitis (NASH), more commonly known as fatty liver disease.

This relates to the compound and so-called SSAO inhibitor, PXS-4728A. For the technically-minded, that means it inhibits the semicarbazide-sensitive amine oxidase enzyme which is involved in inflammation processes, which can lead to scarring and fibrosis.

The start of a phase II trial will trigger a $25 million payment to Pharmaxis and this is also expected before July.

Given the milestone depends only on the phase II patient trial kicking off – and not on results – it's as good as money in the bank.

Not that management would say that, of course.

Should the program move to phase III, Pharmaxis receives another $55 million, with potentially another $200 million due along the way for drug and pricing approval milestones.

Mystery indication:

Then there's a mystery second indication targeted by Boehringer, which will generate a second milestone payment to Pharmaxis. In total these would be the same as the first indication, but "weighted more to the latter stage of development and approval".

Working out the mystery indication is like playing Guess Who with a daughter who cheats by giving the wrong clues. But here's a broad hint: as an anti-inflammatory, PXS-4728A has applications in chronic obstructive pulmonary disease, ocular disorders, Parkinson's disease and Alzheimer's disease.

All of these are under-treated conditions, while NASH (which affects obese people in particular) is forecast to be a $US35 billion a year market by 2025.

"We hope when we announce the $25 million payment we can give more information," Mr Phillips says.

The third arm:

Then there's a third arm to the partnership story.

With UK collaborator Synairgen, Pharmaxis plans to start a phase I clinical study on the lysyl oxidase type 2 enzyme (LOXL2) in the second half of 2017.

LOXL2 has anti-fibrotic qualities and its targets include NASH, cardiac fibrosis and the fatal lung disease idiopathic pulmonary fibrosis.

Synairgen is a drug discovery company linked to the University of Southampton.

Preclinical toxicology studies are being completed on two potential drugs, which are based on the same amine oxidase platform as PXS-4728A.

Dr Boreham's diagnosis:

With Bronchitol clinical results and news on the PXS-4728A milestone and second indication due by the end of June, it's a pivotal period for investors.

Meanwhile, March quarter sales included orders from Chiesi for the UK and German Bronchitol markets and the first sales of Bronchitol to its Russian distributor.

In the period, Pharmaxis received $1.8 million for European Bronchitol sales and $469,000 from sales of its asthma diagnosis tool Aridol.

The Pharmaxis $88 million market capitalization looks compelling given the $26 million cash backing and the dead-cert $25 million milestone.

Investors have discounted most or all of the vaunted NASH milestone payments and the royalties from a successful launch of Bronchitol in the US.

Pharmaxis expects a cash burn of $15-20 million a year as it dabbles in proof of concept and phase-one studies. At this rate, the company should be self-funding for some years.

On a risk-reward analysis, we've certainly seen worse roughies at Randwick and not necessarily the horses.

Disclosure: Dr Boreham is not a qualified medical practitioner and does not possess a doctorate of any sort. He is also partnered up for life but expects no imminent milestone payments.