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*Delivering independent investment research to investors on Australian
biotech, pharma and healthcare companies*

Extract from Bioshares –

Pharmaxis Releases Initial Data in Myelofibrosis

Pharmaxis (PXS: \$0.066) has two drug candidates in clinical development, both based on its lysyl oxidase inhibition platform that seeks to reduce or stop collagen crosslinking that is crucial to the fibrosis process. The drug candidates are PXS-5505, being trialled in a Phase II study in myelofibrosis, and a second investigator-led study in liver cancer due to commence this year; as well as PXS-6302, which is being investigated as a treatment for existing and new scars. The trial in existing scars has reached the 75% recruitment mark.

Pharmaxis recently announced some initial data from the open label myelofibrosis study as well as an institutional capital raise for \$10 million at \$0.06 per share. Two new institutional investors participated (Platinum Asset Management and Regal Funds Management), with funds now owning around 50% of the company. Existing shareholder Karst Peak is also participating in the raise.

Myelofibrosis Data Released

So far 15 of the target 24 patients have been enrolled into the study with the balance expected to be recruited by year's end. Patients in the study are treated with PXS-5505 twice daily for 24 weeks. Six patients have completed the treatment and four patients stopped therapy due to a lack of clinical effect detected.

Myelofibrosis is a cancer of the bone marrow. The outcome is a reduction in red and white blood vessel production, and reduction of platelets that can promote bleeding and bruising. To try and counter these effects, the spleen becomes enlarged to increase blood cell production.

There are three JAK inhibitor drugs on the market that generate sales in excess of US\$1 billion a year. However, according to Pharmaxis CEO Gary Phillips, these drugs only treat the symptoms and not the underlying fibrosis. All but one patient in the Pharmaxis study had failed JAK inhibitor therapy. The life expectancy on these patients is on average between 11-14 months.

Pharmaxis has taken the highest dose explored in the Phase I studies into this Phase II study. It has been difficult to recruit the target patients with 18 sites across the US, Australia, South Korea and Taiwan.

Initial results show that PXS-5505 is very well tolerated by patients. With the patients being recruited having a life expectancy on just 12 months, Phillips said the expectation is that deterioration would continue in the first six months. However, in five of the first six patients, their bone marrow fibrosis, assessed from a biopsy of the bone marrow, was either stable or had improved and two of those patients had clinically improved symptoms. Five of the six patients had stable or improved platelet or haemoglobin levels.

Continued over

	Bioshares Portfolio
Year 1 (May '01 - May '02)	21.2%
Year 2 (May '02 - May '03)	-9.4%
Year 3 (May '03 - May '04)	70.6%
Year 4 (May '04 - May '05)	-16.3%
Year 5 (May '05 - May '06)	77.8%
Year 6 (May '06 - May '07)	17.4%
Year 7 (May '07 - May '08)	-35.8%
Year 8 (May '08 - May '09)	-7.4%
Year 9 (May '09 - May '10)	50.2%
Year 10 (May '10 - May '11)	45.4%
Year 11 (May '11 - May '12)	-18.0%
Year 12 (May '12 - May '13)	3.1%
Year 13 (May '13 - May '14)	26.6%
Year 14 (May '14 - May '15)	23.0%
Year 15 (May '15 - May '16)	33.0%
Year 16 (May '16 - May '17)	16.8%
Year 17 (May '17 - May '18)	-7.1%
Year 18 (May '18 - May '19)	-2.3%
Year 19 (May '19 - May '20)	39.5%
Year 20 (May '20 - May '21)	86.8%
Year 21 (May '21 - May '22)	-15.6%
Year 22 (May '22 - Current)	-5.6%
Cumulative Gain	1521%
Av. Annual gain (21 yrs)	19.0%

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The only measure that did not see a change was spleen volume, which Phillips says may occur if patients are treated for longer and possibly in combination with a JAK inhibitor. According to a clinician who reviewed the individual data, an important aspect was a correlation between improved clinical outcomes and the blood scores. Phillips said there has been a dramatic increase in platelet levels in some patients as well as improvements in symptom scores in the same patients.

One patient went from transfusion dependent to transfusion independent following treatment which Phillips said does not happen by accident. This development was highlighted by the investigator involved in the study.

It's likely that more interim data will be released from the myelofibrosis study. Pharmaxis will require one month safety data from at least 21 patients, with data already available from the first 15 patients who all passed the one-month treatment period.

Once final data is released (mid 2023) the company will seek to progress the program to a registration study, pending positive results. That study would require additional funding, most likely from a partner. A registration study would require at least 300 treatment naïve patients or 100 - 200 patients who are progressing on JAK inhibition therapy.

Competition in Myelofibrosis Treatment

Galecto Inc is also developing a LOX inhibitor for the treatment of myelofibrosis. In a Phase IIa study in a similar patient population, Galecto reported in September that four out of five patients experienced an improvement (reduction) in collagen fibrosis of the bone marrow at six months. Of the 16 patients treated, eight discontinued treatment due to adverse events or disease progression. The company stated that the trial was the first clinical validation of LOXL2 as a target for myelofibrosis.

Phillips said that PXS-5505 is a pan lox inhibitor (inhibiting LOX1, LOX2, LOX3 and LOX4) and has substantially better inhibition of LOXL2 than the Galecto compound (90% inhibition of the enzyme at its lowest point compared to 25%), with the Galecto compound achieving no improvements in symptom scores or blood counts.

Another more advanced competitor in the myelofibrosis field is Constellation Pharmaceuticals, which was acquired last year by MorphoSys for US\$1.7 billion. At the time of acquisition, Constellation had Phase II data with its drug compound pelabresib with a JAK inhibitor and had started a 400-patient combination study with a JAK inhibitor (ruxolitinib) in JAK treatment naïve patients.

Of the 84 patients enrolled in the Phase II study, 68% achieved more than a 35% reduction in spleen volume.

Funding

With the capital raise underway and the expected R&D tax rebate, Pharmaxis has just over \$26 million in funds. This is enough to get the company to the start of 2024. By then the company should have final data from its myelofibrosis study (mid 2023) and data from the current scarring study (1H 2023). In the first half of 2024 the company also expects data from the liver cancer study and the

second scarring study.

Pharmaxis is capitalised at \$42 million.

Bioshares recommendation: **Speculative Buy Class A**

Bioshares

How Bioshares Rates Stocks

For the purpose of valuation, Bioshares divides biotech stocks into two categories. The first group are stocks with existing positive cash flows or close to producing positive cash flows. The second group are stocks without near term positive cash flows, history of losses, or at early stages of commercialisation. In this second group, which are essentially speculative propositions, Bioshares grades them according to relative risk within that group, to better reflect the very large spread of risk within those stocks. For both groups, the rating “Take Some Profits” means that investors may re-weight their holding by selling between 25%-75% of a stock.

Group A

Stocks with existing positive cash flows or close to producing positive cash flows.

- Buy** CMP is 20% < Fair Value
- Accumulate** CMP is 10% < Fair Value
- Hold** Value = CMP
- Lighten** CMP is 10% > Fair Value
- Sell** CMP is 20% > Fair Value
(CMP–Current Market Price)

Group B

Stocks without near term positive cash flows, history of losses, or at early stages of commercialisation.

Speculative Buy – Class A

These stocks will have more than one technology, product or investment in development, with perhaps those same technologies offering multiple opportunities. These features, coupled to the presence of alliances, partnerships and scientific advisory boards, indicate the stock is relative less risky than other biotech stocks.

Speculative Buy – Class B

These stocks may have more than one product or opportunity, and may even be close to market. However, they are likely to be lacking in several key areas. For example, their cash position is weak, or management or board may need strengthening.

Speculative Buy – Class C

These stocks generally have one product in development and lack many external validation features.

Speculative Hold – Class A or B or C

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