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Bioshares

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

Companies covered: FTT, PXS, VLA,
Visioneering IPO

	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)	-36%
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 - current)	19.5%
Cumulative Gain	781%
Av. Annual gain (14 yrs)	18.8%

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Extract from *Bioshares* –

Three Share Price Drivers for Pharmaxis

Pharmaxis (PXS: \$0.26) has a number milestones approaching in Q2 which could have a meaningful impact on the company's share price.

This first is a major milestone payment from Boehringer Ingelheim. Second, results are due in from the company's third Phase III in cystic fibrosis with its drug Bronchitol.

The company also expects to have completed its toxicology work on its next program with its LOXL-2 inhibitor, which will clear the way for a Phase I trial to start and finish in 2H 2017.

Boehringer Milestone Expected

Under the transaction terms with the SSAO inhibitor program sold to Boehringer Ingelheim in 2015, Pharmaxis stands to receive around \$25 million as a milestone payment when the program moves into a Phase II trial in the first indication, which is expected to be in the current quarter.

The lead indication is for NASH (non-alcoholic steatohepatitis). Pharmaxis CEO Gary Phillips said that Boehringer is placing its faith into the drug candidate discovered by Pharmaxis to be their NASH drug, which is an anti-inflammatory compound.

The compound inhibits the enzyme SSAO, the level of which is strongly proportional to the progression of NASH. What Pharmaxis was able to show in the Phase I trial prior to signing the deal with Boehringer was that the compound achieved target engagement (in healthy volunteers).

The early results from this program were so compelling that Boehringer selected the Pharmaxis compound over its internal NASH candidates on the same target.

Pharmaxis stands to receive additional milestone payments as well, which includes a major payment when the program moves into Phase III trials (around \$55 million), and an additional milestone when a Phase II trial commences in a second indication (which will be less than \$25 million). There are also payments for drug approval submissions, registration and pricing approvals which total around \$200 million.

For one of the leaders in the NASH filed, Genfit, it took three and a half years to move from the start of a Phase II trial in NASH (270 patients followed for 80 weeks) to commence its Phase III trial in NASH (in 2,000 patients). This included around one year to recruit into the study.

On this measure, the earliest time for the \$55 million Phase III milestone payment to Pharmaxis, pending positive Phase II results in NASH, would be towards the end of 2020.

Cont'd over

Pharmaxis is also entitled to receive high single digit royalties from sales under the Boehringer transaction.

Phase III Cystic Fibrosis Trial Results

Results are due in this quarter from a 423 patient Phase III trial with the company's drug Bronchitol in cystic fibrosis. The trial is being conducted in 21 countries and is mostly paid for by Chiesi Pharmaceutici, which has US rights.

Pharmaxis has conducted two other Phase III trials in CF. However, the trials failed to achieve consistent statistical significance on key endpoints. The structure of the current trial has been improved with learnings from the first two studies. The first learning is that using a small dose of the drug as a placebo very likely distorted results because the small level of drug in the placebo appeared to be showing benefit in children. This trial is in an adult population only.

The second improvement has been sought from better management of patients in the trial. In the first Phase III, the discontinuation rate was 33%. This was reduced to 15% in the second Phase III trial. The current Phase III trial is a very large study, with 423 adults enrolled, and is powered to deliver statistical significance.

Pharmaxis is entitled to receive a US\$10 million milestone payment upon FDA approval from Chiesi. US market sales could reach US\$50 - US\$100 million with Pharmaxis to receive a net royalty from sales estimated to around 15% after other royalty obligations.

Given the structure of the trial, we attribute a moderate-to-high chance of a positive outcome.

Next Cab off the Rank – LOXL-2 program

The next drug development program for Pharmaxis is its LOXL-2 program. The company has selected a lead candidate that is going through final toxicology steps. Results from that testing are due to be known by midyear. Important outcomes will be to assess for any off-target effects of the compound. Phase I trials are due to be underway in the second half of this year with results towards the end of this year or early 2018.

Pharmaxis will be seeking to measure target engagement once again (similar to the Boehringer acquired asset) in the Phase I trial. Phillips said showing target engagement will be more difficult because of the lower levels of the LOXL-2 enzyme. LOXL-2 is vital in cross linking of tissue to form fibrosis. The drug candidate will have potential applications in NASH as well as other fibrotic diseases of the kidney, lung and heart.

Pharmaxis will be seeking to transact the LOXL-2 program following the Phase I study, assuming success in that study.

Bronchitol Sales Progress

Chiesi took over Bronchitol sales in the UK and Germany in 2015. Bronchitol sales have been improving, both through Chiesi and also from entry into new markets, with the first sales having been made by Pharmaxis into Russia. Phillips expects the company's Bronchitol business to reach breakeven over the next 12 - 24 months, excluding US market entry. The main cost Pharmaxis has

for this program is maintaining its in-house manufacturing facility.

Bronchitol sales by Chiesi into the UK and Germany increased by 7% in 1H FY2017 over the previous corresponding period (pcp). However, overall sales fell by 46% to \$1.7 million (including \$0.98 million for Aridol) due to inventory stocking by Chiesi in the pcp.

Summary

The key assets for Pharmaxis are its drug development programs that have been transacted (with Boehringer Ingelheim) and follow up programs. Both have applications in the high interest area of drug development in NASH and have the potential to deliver a durable effect on the Pharmaxis share price. However, a positive Phase III trial result in cystic fibrosis has the potential to be a short-term (but not necessarily durable) driver for this stock.

Pharmaxis is capitalised at \$83 million with \$29 million in cash at the end of last year. The company's net loss in FY2016 was \$16.5 million and its half year net loss for FY2017 of \$11 million.

Bioshares recommendation: **Speculative Buy Class B**

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 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 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

Sell

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