

....Save the Date...
2023 Bioshares Biotech Summit
 24-25 July
 Hobart, Tasmania

BIOSHARES

Australia's Independent Biotech Investment Resource, est. 1999

24 February 2023
Edition 934

Companies covered: AVR, CGS, EBR, NEU, OPT, PXS, VHT

Extract from *Bioshares* –

Pharmaxis: A Re-Rating Approaching?

The coming months ahead should be a significant period for Pharmaxis (PXS: \$0.05) with the readout expected from two Phase II studies. Whilst market conditions currently pose challenges for investors, positive outcomes in one or both studies have the potential for a significant re-rating in a stock that is arguably well undervalued.

At an investment briefing last week, CEO Gary Phillips said that the results should have a fundamental impact on the company's valuation. Of more importance to the company is likely the result in its myelofibrosis study, with an established market and strong licensing interest. Pharmaxis is conducting a Phase IIa monotherapy study in up to 24 patients with myelofibrosis with its drug candidate PXS-5505.

Significant Acquisition in Myelofibrosis Drug Development

In June 2021 Morphosys announced that it would acquire Constellation Pharmaceuticals for US\$1.7 billion in cash. Constellation's lead asset was pelabresib, which six months earlier started a 400 patient Phase III study with this drug candidate. This was in combination with the JAK inhibitor ruxolitinib, in patients with myelofibrosis who were treatment naïve to ruxolitinib therapy.

It was an unusual acquisition whereby Morphosys financed much of the deal by selling off its royalty streams to existing products to Royalty Pharma.

Results of the study are summarised in the table on next page.

In the monotherapy arm, there were Grade 3 but no Grade 4 adverse events, with nine of the 46 patients discontinuing treatment. The most significant negative effect was thrombocytopenia (low platelet levels) which occurred in 15% of patients.

Treatments for myelofibrosis are measured in effectiveness in combination with a JAK inhibitor, ruxolitinib. Data from the Phase II Constellation study presented was mainly from the combination use, with 67% achieving greater than a 35% reduction in spleen volume. With respect to bone marrow fibrosis, 33% achieved at least a one grade improvement, 59% achieved stabilisation of BMF and only 4% showed a worsening of this measure at 24 weeks.

Pharmaxis Initial Data

In the Pharmaxis study, 18 patients have been enrolled with six of those stopping treatment due to lack of clinical response. These patients were either too far advanced or treatment without the combination of ruxolitinib did not show benefit.

Results from six patients have been released. Five of the six patients (83%) showed improvement or stability in BMF. One third of patients achieved a clinical improvement in symptoms and 83% achieved stable or improved platelet/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 - Dec '22)	-2.2%
Year 23 (CY2023)	-9.3%
Cumulative Gain	1423%
Av. Annual gain (22 yrs)	18.1%

2023 Top Six Picks: -10.8%



Bioshares is published by Blake Industry & Market Analysis Pty Ltd. ACN 085 334 292
 PO Box 447
 Flinders Lane Vic 8009
 AFS Licence No. 258032

Mark Pachacz - Editor/Analyst
 Email: mark[at]bioshares.com.au
 Ph: 0403 850 425

Edition Number 934 (24 February 2023)
 Copyright 2023 Blake Industry and Market Analysis Pty Ltd. ALL RIGHTS RESERVED.
 Secondary electronic transmission, photocopying, reproduction or quotation is strictly prohibited without written consent of the publisher.

A particularly important aspect of this trial result so far is the benign safety profile of the PXS-5505, with no serious adverse events reported in the monotherapy. This compares to the Constellation drug candidate in which a 15% rate of thrombocytopenia (low platelet levels) was observed. By comparison, PXS-5505 has achieved an improvement in blood platelet levels in some patients, with two patients more than doubling their platelet levels.

When the Constellation drug candidate was combined with ruxolitinib, the thrombocytopenia incidence increased to 26%. The appeal of the Pharmaxis drug candidate at this point is the potential to maintain treatment with ruxolitinib for longer in combination use, whilst addressing the core fibrotic process in myelofibrosis.

Encouraging results from this study should allow the company to progress the program into a Phase II combination study with ruxolitinib, potentially also in ruxolitinib treatment naïve patients.

Significantly more data from Pharmaxis' Phase IIa study in myelofibrosis is expected around mid-year, with results from the 50 patients scar treatment study expected this half.

Pharmaxis is capitalised at just \$36 million. Its proforma cash balance at the end of last year, including funds received last month from the R&D tax rebate, was \$21.4 million.

Bioshares recommendation: **Speculative Buy Class A**

Phase II Data from Constellation Pharma in Myelofibrosis

Trial size	Result	Treatment	Patients
Improvement in at least 1 grade in bone marrow fibrosis at 24 weeks	30%	Monotherapy and combination therapy with JAK inhibitor (ruxolitinib)	63
Patients with at least 35% reduction in spleen volume at 24 weeks	67%	In combination with ruxolitinib	63
Patients with at least 50% reduction in total symptom scores at 24 weeks	57%	In combination with ruxolitinib	63
Worsening of bone marrow fibrosis	4%	In combination with ruxolitinib	27

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

Sell

Corporate Subscribers: Cogstate, Opthea, Pharmaxis, Dimerix, Patrys, Antisense Therapeutics, Imugene, Chimeric Therapeutics, Neuren Pharmaceuticals, Aroa Biosurgery, Radiopharm Theranostics, Imricor Medical Systems, Anteris Technologies, Bio-Gene Technology, EBR Systems

Disclaimer:

Information contained in this newsletter is not a complete analysis of every material fact respecting any company, industry or security. The opinions and estimates herein expressed represent the current judgement of the publisher and are subject to change. Blake Industry and Market Analysis Pty Ltd (BIMA) and any of their associates, officers or staff may have interests in securities referred to herein (Corporations Law s.849). Details contained herein have been prepared for general circulation and do not have regard to any person’s or company’s investment objectives, financial situation and particular needs. Accordingly, no recipients should rely on any recommendation (whether express or implied) contained in this document without consulting their investment adviser (Corporations Law s.851). The persons involved in or responsible for the preparation and publication of this report believe the information herein is accurate but no warranty of accuracy is given and persons seeking to rely on information provided herein should make their own independent enquiries. Details contained herein have been issued on the basis they are only for the particular person or company to whom they have been provided by Blake Industry and Market Analysis Pty Ltd. The Directors and/or associates declare interests in the following ASX Healthcare and Biotechnology sector securities: Analyst MP: ACR,CGS, CYC, DXB, IMM, OPT,CUV,MX1, NEU, PAB, PXS,RNO,SOM. These interests can change at any time and are not additional recommendations. Holdings in stocks valued at less than \$100 are not disclosed.

Subscription Rates (inc. GST)

24 issues per year (electronic distribution): **\$550**

For multiple email distributions within \$900 2-3 email addresses
 the same business cost centre, our \$1200 4-5 email addresses
 pricing structure is as follows: \$1500 6-10 email addresses

To subscribe, post/email this subscription form to:

Bioshares
PO Box 193 Richmond VIC 3121
editor@bioshares.com.au

I enclose a cheque for \$ _____ made payable to **Blake Industry & Market Analysis Pty Ltd**, or

Please charge my credit card \$ _____ MasterCard Visa

Card Number

Signature _____ Expiry date _____

Subscriber details

Name _____

Organisation _____

Ph () _____

Emails _____

The information provided in Bioshares, including general investment advice, is provided only for receipt and use in Australia and New Zealand, for subscribers to Bioshares, who are Australian or New Zealand citizens or commercial entities.