ISSN 1443-850X



Companies covered: **PXS, TLX, Orphan Drugs Post Scriptum, Cash Analysis**

| | 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 - Current) | 51.2% |
| Cumulative Gain | 1546% |
| Av. Annual gain (20 yrs) | 19.0% |

*Bioshares* is published by Blake Industry & Market Analysis Pty Ltd.

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Individual Subscriptions (48 issues/year)
**$500** (Inc.GST)
Edition Number 867-007 (2 November 2020)



# Bioshares

**2 November 2020**
**Edition 867-007**

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

*Extract from Bioshares –*

## *Bronchitol FDA Approval a Turning Point for Pharmaxis*

After three Phase III studies, the first of which was started 13 years ago, Pharmaxis (PXS: $0.11) has finally gained approval for Bronchitol in the world's largest market pharmaceutical market, the USA, as an"add-on maintenance therapy to improve pulmonary function in adult patients 18 years of age and older with cystic fibrosis."

Pharmaxis is now prioritising its myelofibrosis drug candidate for mid-stage clinical studies, while the Bronchitol and Aridol assets are set to generate positive cashflows for the company.

Bronchitol will be marketed and distributed by Chiesi Farmaceutici in the US. Chiesi has an existing sales force that sells products to respiratory doctors in the US, including antibiotics for the treatment of lung infections in patients with cystic fibrosis.

There are around 30,000 people with cystic fibrosis in the US, more than half of whom are adults.

Bronchitol sells for between $11,000 - $14,000 per patient per year outside of the US, although the price for the main mucous clearing agent, Pulmozyme, is about three times that in the US according to Pharmaxis CEO Gary Phillips.

Pharmaxis will manufacture the drug as well as receive a high teens royalty. The company expects "~ 20% of Chiesi US Bronchitol net sales to flow directly to the mannitol business segment EBITDA." Importantly, the increased production volumes should improve margins for Pharmaxis and make the business immediately cash flow positive, due also to a US$10 million milestone payment that Pharmaxis will now receive from Chiesi, and into FY2022 from higher sales of Bronchitol.

Pharmaxis expects revenue from Bronchitol (and Aridol, the lung function test that also comprises of mannitol) to increase from $7 million in FY2020 to $14 million in FY2022. In FY2026, it expects the mannitol business (Bronchiol and Aridol) to contribute more than $10 million in earnings (EBITDA).

### Focus on Phase I/II Myelofibrosis Study with Lead Candidate PXS-5505

However, the focus for Pharmaxis now will be on its small molecule programs, with the drug candidate PXS-5505 becoming the priority program for the company. A 42 person study will commence in the first half of next year in patients with myelofibrosis, initially in Australia and South Korea, and then expand into the US. Myelofibrosis is a billion dollar market and a condition that is poorly served by current therapies.

In a myelofibrosis preclinical model, PXS-5505 produced statistically significant reductions in fibrosis and in a Phase I study in healthy volunteers, the drug candidate was able to reduce the LOX enzyme levels by around 80% in just six hours from a single dose.

*Continued over*

The Phase Ic/II study will be an open label trial, with the trial to complete at the end of 2022. Myelofibrosis treatment effect can be measured objectively and accurately with various tests, including increases in spleen volume and through a bone marrow biopsy to assess the level of fibrosis. This aspect (accurate and objective measurement) makes it attractive for drug development. The company has received Orphan Drug designation for its program.

Initially the company will assess the drug candidate as a monotherapy, however it should have synergistic outcomes with the JAK inhibitors on the market and in development.

Pharmaxis is developing PXS-5505 under an IND which has already been accepted by the FDA. This will allow the company to provide the compound for physician-sponsored studies in other fibrotic diseases such as pancreatic and ovarian cancers.

## Comparator Company

One comparator company for Pharmaxis is **Constellation Pharmaceuticals**. Its main program is with a small molecule for the treatment of myelofibrosis that inhibits what are called BET proteins.

In a completed Phase II study in 124 patients, its compound achieved a 35% reduction in spleen volume (SVR35) in 73% of first line patients and in 24% of second line patients with a tolerable safety profile. Constellation is capitalised at US$933 million and holds US$490 million in cash. The company intends to start a Phase III study this year.

## Next Milestones – Licensing of LOXL2 Program

Pharmaxis is still seeking to execute a licensing deal for its LOXL2 program. Phillips said that discussions were progressing and that he remained optimistic of concluding a deal.

Pharmaxis also has a topical fibrosis treatment program that is expected to start treating patients in early 2021 in Australia. The aim is to treat new and existing scars with its topical antifibrotic drug candidate (PXS-6302).

## Summary

The US approval of Bronchitol represents a significant turning point for Pharmaxis. Last year the mannitol business recorded a loss of $4 million. That business is expected to generate a positive cash inflow to the business immediately, commencing with US$10 million in milestone payments, and a contribution to earnings of over $10 million a year by 2026.

The approval also allows the company to prioritise and fund its Phase II myelofibrosis program to the end of 2022, when a positive clinical outcome could provide significant value creation for the company. With the trial being open label, any clear results may become available earlier.

Including the tax rebate received this month and the milestone payments due from Chiesi, Pharmaxis has $29 million in funds at the end of September on a proforma basis.

*Bioshares* recommendation: **Speculative Buy Class B**

## 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***

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