PHARMAXIS LTD (ASX: PXS)

PHARMACEUTICAL RESEARCH

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Key Highlights

- **FDA approval to commence Phase 2 cancer treatment human trials of PXS-5505;**
- **Patient recruitment has commenced in Australia and South Korea;**
- **Bronchitol sales rising, following FDA approval in October 2020;**
- **Recurring need of Bronchitol as ongoing treatment for cystic fibrosis;**
- **Healthy bank balance with $16.1m as of 31 March 2021; increased by $4.4m placement and ~$2m payment from sale of Bronchitol distribution rights for Russia in April 2021**
- **Professor Fiona Wood to lead world-first trials to eliminate scar formation following burns and trauma, PXS-6602;**
- **Peer valuation of cancer treatment companies in Phase 2 trials suggests current market cap undervalues the development of PXS-5505;**

Outlook - Speculative Buy

Pharmaxis is in a rare microcap air for a pharmaceutical research company that has a commercialised product at market with sales nearing profitability that can fund transformational cancer research.

Addressing a substantial market opportunity through targeting of myelofibrosis where current treatment standards are limited, yet still sell upwards of USD $1 billion per year, Pharmaxis' market cap is not fully reflective of the progress they have made in their research compared to international peers. Recruiting of patients for the FDA-approved Phase 2 trial of their drug PXS-5505 (which has the potential to modify the disease and extend life expectancy rather than just treat symptoms like current therapies) has commenced.

Data from this human trial will be presented at the end of CY22.

Key to the development of PXS-5505 is the strong cash position of Pharmaxis. The Company reported $16.1m in the bank as of 31 March 2021 while trading with a market cap of just $34 million.

Outlook:	**SPECULATIVE BUY**
Date:	**18 May 2021**
Price (18 May):	$0.081
Ticker:	ASX: PXS
52-Week range:	$0.065-$0.17
Market cap (AUD):	$37m
Shares on issue	452m

	FY20	FY19	FY18
Revenue ($m)	12.6	12.1	50.2
EBITDA ($m)	-12.0	-15.6	11.5
NPAT ($m)	-13.9	-20.0	6.4

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Their healthy bank balance is supported by sales of cystic fibrosis treatment 'Bronchitol' which secured FDA-approval in October 2020 and is manufactured by Pharmaxis in their Australian facility for export around the world. The United States remains the most lucrative market for the product where pricing mechanisms enable higher margins on the treatment which costs around USD $35,000 for an annual supply of the recurring treatment.

Interest in Pharmaxis has increased notably over past months as the Company's sales rose alongside recruitment for their Phase 2 human trial of PXS-5505.

In April 2021, the Company secured $4.4 million via a private placement which included $3.2m for a 8.9% stake from Karst Peak Capital, who are well-known for some hugely successful investments in the biotech sector. The funds were raised at a higher Issue Price than PXS shares were trading at the time, whilst Pharmaxis' largest shareholder BVF Partners also participated to maintain its 19.5% holding with a range of growth levers for Pharmaxis on the horizon.

Company Summary - Pharmaxis (ASX: PXS)

Pharmaxis is an advanced pharmaceuticals company based in Sydney, New South Wales with a proven track record of bringing their research to commercialisation alongside their clinical-stage research into new cancer treatments. The Company operates two distinctly different divisions where their respiratory pharmaceutical business has two profitable products that functions alongside their drug development research division.

Pharmaxis' research into myelofibrosis is the Company's primary business focus where they are developing a drug to treat the rare form of bone cancer. The lead drug candidate PXS-5505 functions distinctly differently to the current standard of treatments which generate more than $1 billion in annual sales. In a Phase 1c/2 FDA-approved trial of PXS-5505, the commercial opportunity that comes with successful trials has the potential to drive Pharmaxis to significant value multiples of its current market cap.

Ongoing research is supported by their respiratory pharmaceutical manufacturing business which produces Bronchitol and Aridol from their Australian facility. Bronchitol is the primary product which is a treatment for cystic fibrosis, an inherited respiratory disease suffered by more than 70,000 people around the world. With no cure available, sufferers require ongoing treatment from products like Bronchitol where an annual supply in the United States can cost more than USD $35,000 per patient.

Alongside the development of PXS-5505, Pharmaxis is in active collaborations with researchers around the world for applications of their drug's enzyme inhibiting properties. Other medical applications being explored using Pharmaxis-owned research include liver cancer, pancreatic cancer, skin trauma, burn recovery and muscular dystrophy.

ANALYSTS CONSENSUS - BUY
Number of Analysts: **1**
Analysts Mean Price: **$0.14**

	SCORE OUT OF 100
Dividend Score	0
Quality	63
Price Momentum	70.8
Maturity Score	32.3
All Weather Score	65.7
Credit Score	20.6
Earning Score	82.4
Growth	71.9
Emerald Stock Score	73.8

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Cancer Research - Phase 2 Trials

Although it may not be getting as much attention as other areas of the business, pharmaceutical research for cancer treatment is where the greatest long-term value exists for Pharmaxis.

PXS-5505 is Pharmaxis' lead drug candidate which is being developed for the treatment of myelofibrosis - a type of bone cancer which affects 15 in every 1 million Americans. Although prevalence of the cancer is rare, current forms of treatment generate more than $1 billion annually.

Current approved therapies Jakafi® and Inrebic® are inhibitors of kinase proteins JAK-1 and JAK-2. Protein kinases are involved in various cellular functions, disruption of which can result in carcinogenesis. Combined, the two proprietary companies generated more than US$1 billion in revenue in 2019.

The opportunity for Pharmaxis exists to significantly disrupt that billion dollar industry due to the unique mechanism of action of PXS-5505. The compound functions differently to the current forms of treatment which can mask symptoms, but are insufficient for long-term remission. Unlike the aforementioned treatments, PXS-5505 blocks the enzymes that are directly responsible for the fibrosis of the bone marrow that drives the adverse symptoms and mortality associated with myelofibrosis.

WHAT IS MYELOFIBROSIS?

Myelofibrosis is a disorder in which normal bone marrow tissue is gradually replaced with a fibrous scar-like material, destroying the normal bone marrow and consequently preventing the production of adequate numbers of red and white blood cells and platelets. This results in anaemia, low platelet counts and the production of blood cells outside the bone marrow in the spleen and liver, which become enlarged and cause several debilitating effects. Once diagnosed, patients have an average life expectancy of just five years.

The mechanisms behind how PXS-5505 functions is where the compound has the potential to modify the disease rather than simply mask symptoms, as current treatments offer. PXS-5505 has now reached Phase 2 clinical trials meaning the Company has secured regulatory approval to commence dosing on patients.

The trial is open label which means that data from the study might be released earlier than the scheduled conclusion at the end of 2022. It is also the stage of drug development that draws the most attention from researchers and knowledgeable investors because successful clinical data reported from Phase 2 trials are the key indicator of eventual approval by the United States Food and Drug Administration for commercialisation.

Further highlighting the value to be capitalised upon is the company valuations of other drug developers targeting myelofibrosis. Companies that are developing treatments which have reported encouraging Phase 2 data and are in the process of moving to Phase 3 trials are valued at a significantly higher market cap than Pharmaxis (see table below). However, in the case of myelofibrosis, the rarity of the disease and its small patient numbers suggest there is minimal value-add for an investment case between successful data from ~50 patients in Phase 2, to ~250 patients in Phase 3. For this reason, the value multiplications are more likely to be realised upon reporting of Phase 2 data as per the companies in the table below which have all surpassed $500 million valuations.

In August 2020, Pharmaxis obtained approval from the FDA to commence its Phase 1c/2 trial of PXS-5505 and enrolled its first patients in February 2021. The trials will take place in the United States, Australia and South Korea. In the initial phase 1c stage, patients will undergo a one month dose escalation to determine the appropriate dose to be used in the subsequent Phase 2 stage where patients will be enrolled for 6 months of treatment. Results of the phase 1c are expected to be reported before the end of 2021. The phase 2 expansion stage will then be undertaken, with those results to be reported in 2022 to confirm efficacy.

Myelofibrosis – other programs

PXS-5505 unique mechanism of action promises disease modification and good tolerability

Company	Market cap[1]	Bourse	Asset	Description	Clinical phase
KEROS THERAPEUTICS	$1.2bn	Nasdaq	KER-050	TGF-β ligand trap	Phase 2
Constellation PHARMACEUTICALS	$1.1bn	Nasdaq	CPI-0610	BET inhibitor	Phase 3
KARTOS THERAPEUTICS	$0.7bn[2]	n.a. – private	KRT-232	MDM2 antagonist	Phase 3
geron	$0.5bn	Nasdaq	Imetelstat	Telomerase inhibitor	Phase 3
pharmaxis	$27m (A$35m)	ASX	PXS-5505	Pan-LOX inhibitor	Phase 1c/2 commenced

Other Research Programs

Fibrosis remains at the heart of Pharmaxis' research initiatives where the Company has multiple pipeline drugs aimed at treating other forms of cancer which stem from tumours that build fibrotic tissue.

Some of these other applications of PXS-5505 include Myelodysplastic syndrome, liver cancer, pancreatic cancer, melanoma, and glioblastoma. Rather than directly incurring the R&D expenses to undergo trials targeting these diseases, Pharmaxis is collaborating with research partners around the world that have shown enough interest in PXS-5505 and its enzyme inhibition properties to undergo the research at their expense. This research continues to progress led by academia with Pharmaxis retaining the IP linked to PXS-5505 where the Company would stand to benefit significantly from a collaboration partner's breakthrough where 5505 is involved.

These projects are still in early stages with the Myelodysplastic Syndrome study undergoing a pre-clinical proof of concept and a pre-clinical liver cancer study at combining PXS-5505 with existing treatments.

Beyond PXS-5505, Pharmaxis is also developing PXS-6302 which is linked to fibrous tissue but targeting external build up such as scarring developed following skin trauma or burns. Each year, more than 100 million people around the world develop scarring as a result of trauma of which many instances result in morbidity or disfigurement.

The current standard of care is largely unsatisfactory where corticosteroids and surgical revision can diminish the appearance of scars. PXS-6302 seeks to stop scars from developing all together, thus eliminating reliance on corrective surgeries following skin trauma.

In April 2021, Pharmaxis announced the commencement of human trials for PXS-6302 which would be led by Professor Fiona Wood AM and a group of researchers from the University of Western Australia (UWA) and Fiona Stanley Hospital.

PROFESSOR FIONA WOOD

Professor Wood is widely regarded internationally as one of the world's top surgeons for burns and skin trauma where she is a pioneer in the field of tissue engineering. Her rise to prominence in the field came via her invention of Spray-on Skin with laboratory partner Marie Stoner.

This product was then commercialised in the 90s by Clinical Cell Culture Pty Ltd which was re-named Avita Medical (ASX: AVH) in 2008 and has continued to develop Spray-on Skin as the RECELL® System while trading on the ASX with a market cap over $800 million.

Professor Wood was named Australian of the Year in 2005 for her contribution to medical research where her invention has saved the lives of thousands of Australians.

Still hugely active in medical circles, Wood has publicly stated on many occasions that the 'Holy Grail' of her research would be "scarless wound healing" which is precisely what PXS-6302 is engineered to achieve by inhibiting enzymes that play a critical role in the development of scar tissue.

The world-first human trials of PXS-6302 will determine the safety and tolerability of the drug in healthy volunteers, which will lead to further trials in burns patients. An exact timeline for these trials is still being mapped out by Professor Wood but it is likely further clinical trials will report by the end of 2022.

Cashflow Generation - Bronchitol and Aridol

Unlike other ASX-listed pharmaceutical research companies that fund their research entirely from Government grants and raising capital from investors, Pharmaxis has already commercialised two products that are actively generating revenue for the Company.

Both products - Bronchitol and Aridol - are part of Pharmaxis' mannitol respiratory business and showcase the Company's track record of monetising their research.

Bronchitol, which secured FDA approval in October 2020, is the lead product as a treatment for cystic fibrosis and was launched last quarter in the United States which accounts for 65% of the global cystic fibrosis market. The US market is particularly attractive for Pharmaxis courtesy of pricing mechanisms in place through the prevalence of private health insurance and recurring nature of cystic fibrosis treatments.

Cystic fibrosis is one of the world's most prevalent inherited diseases. People with this disease have limited ability to maintain a fully hydrated lung surface. This causes damage to the lungs, digestive system and other organs in the body. In the lungs, mucus build up clogs the airways and traps pathogens leading to recurrent lung infections. Clearing this mucus often requires medical intervention to physically drain it.

Bronchitol is a naturally occurring sugar formulated as an inhaled powder which offers a less invasive method to break up mucus. Part of the strategy to access the commercial opportunities from Bronchitol was partnering with Italian pharmaceuticals giant Chiesi Farmaceutici SpA (Chiesi). Through this partnership, Cheisi invested more than USD $22 million of their own money into the development of Bronchitol which included the FDA approvals process. In turn, Pharmaxis granted Chiesi with exclusive distribution rights for Bronchitol in the US with Pharmaxis paid approximately 30% of total sales revenue.

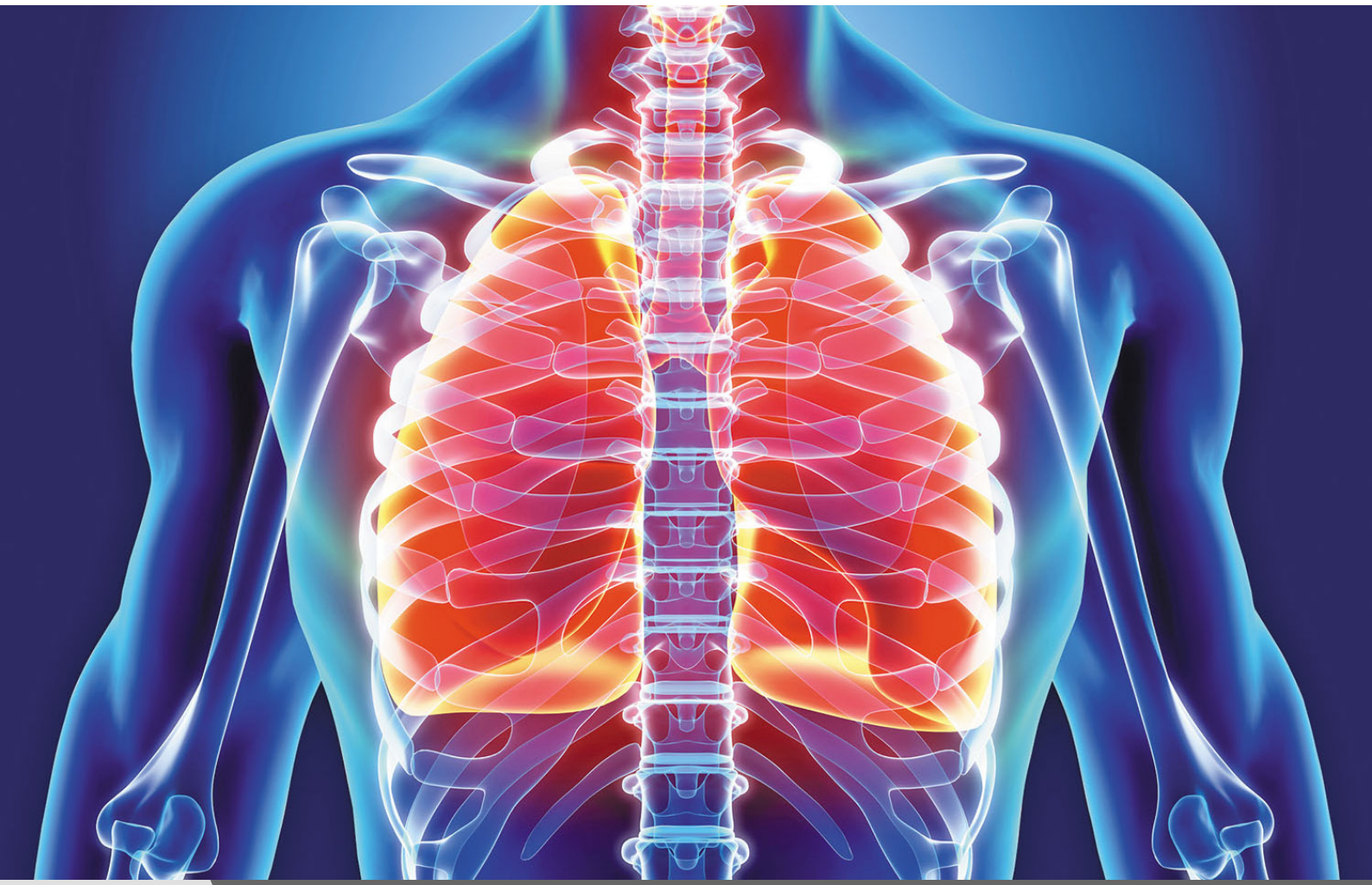
Since its commercial US approval launch in October 2020, Chiesi has paid USD $10m in milestone payments to Pharmaxis separate to the margin of sales revenue Pharmaxis will receive on ongoing Bronchitol sales.

Common feedback draws upon Bronchitol's ease of use from its pocket-sized inhaler as an alternative to bulky nebulisers. Beyond the US, Bronchitol is approved and marketed in Europe, Russia, Australia and several other countries. However, the US remains the highest value market due to the prevalence of cystic fibrosis and attractive pricing mechanics where 1 year's treatment can cost more than $35,000 per patient.

In April 2021, Pharmaxis sold exclusive distribution rights for Russia to GEN İlaç ve Sağlık Ürünleri San. ve Tic. A.Ş. (GEN). The sale also saves Pharmaxis more than $1 million per annum in operating expenses in Russia while still securing revenue from the market through manufacture and export of Bronchitol to Russia.

Factoring in their expected growth of Bronchitol globally, Pharmaxis management have stated on numerous occasions that they are confident that it, will drive the mannitol respiratory business segment to cash flow positivity from FY21 onwards to support ongoing R&D for its cancer treatment drugs.

Aridol is also FDA approved and is generating revenue for Pharmaxis around the world as a bronchial challenge test that can assist in identifying asthma.



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All Figures in this report were correct as of 18th May 2021.

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Speculative Buy*	Greater than +30%
Buy	Greater than +10%
Hold	Greater than 0%
Sell	Less than -10%

*A Speculative Buy is speculative in nature for young companies that do not have significant historical data