www.pharmaxis.com.au

ASX: PXS	
Share price (28/1/22)	$0.099
52-Week range	$0.071-$0.150
Market capitalisation	$54m
Shares outstanding	549m
Cash (31/12/21)	$21m
Enterprise value	$33m

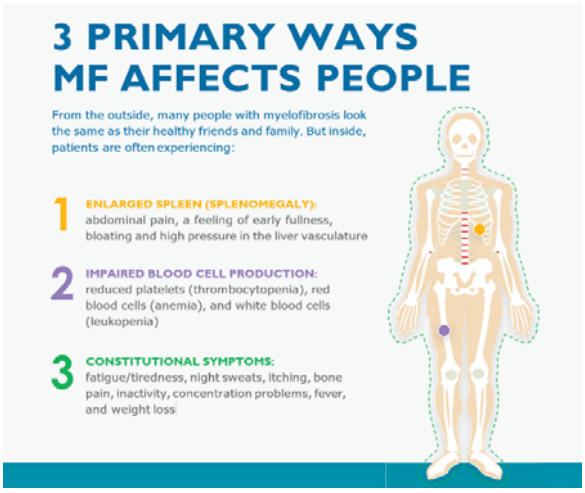
COMPANY SUMMARY

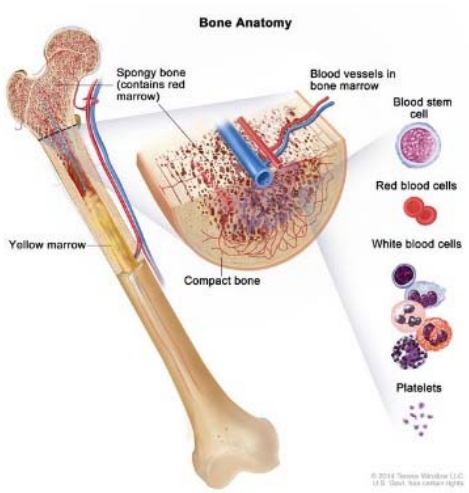
Pharmaxis Ltd is an Australian clinical stage drug development company developing drugs for inflammatory and fibrotic diseases, with a focus on myelofibrosis. The company has a highly productive drug discovery engine built on its expertise in the chemistry of amine oxidase inhibitors, with drug candidates in clinical trials. Pharmaxis has also developed two respiratory products which are approved and supplied in global markets, generating ongoing revenue

MYELOFIBROSIS – LEAD DRUG CANDIDATE

Its lead drug candidate (PXS-5505) is for the treatment of the rare bone cancer myelofibrosis, which affects 15 in 1 million people who have an average life expectancy of 5 years. Myelofibrosis is a disorder in which normal bone marrow tissue is gradually replaced with a fibrous scar-like material, destroying the normal bone marrow environment and preventing the production of adequate numbers of red and white bloods 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.

Current approved therapies Jakafi® and Inrebic® are inhibitors of kinase proteins JAK-1 and JAK-2. JAK inhibition alone, while impacting on myelofibrosis symptoms, is insufficient for long-term remission and offers modest, if any, disease modifying effects. PXS-5505 works via a completely different pathway, blocking the enzymes that are directly responsible for the fibrosis in the bone marrow that drives the adverse symptoms and mortality associated with myelofibrosis. Using PXS-5505 as a monotherapy or in combination with these current treatments holds the potential for better outcomes for patients. Combined, the use of Jakafi® and Inrebic® in myelofibrosis generated more than US$1 billion in revenue in 2019.





INVESTMENT HIGHLIGHTS

- Breakthrough FDA IND approved clinical program for disease modifying drug in orphan disease, myelofibrosis
  - High unmet need with current approved therapies offering mainly symptomatic relief & poor tolerability
  - Phase 2a trial of PXS-5505 commenced Q3 2021 to deliver safety & efficacy evidence in 2022
  - Potential to work as monotherapy or on top of current standard of care in market currently worth ~US$1 billion pa
- Unique & first in class mechanism of action attracts global scientific & clinical collaborations to extend value of PXS-5505 into other cancer indications e.g. liver cancer and myelodysplastic syndrome
- Pipeline of drugs well placed to deliver value in range of inflammatory and fibrotic diseases with high unmet need
  - Collaboration with Perth specialist skin centre to trial PXS-6302 topical anti-scarring medication. Phase 1c studies in patients with established scars commenced January 2022 (results 2H 2022) with burns patients expected to commence in 1H 2022 (results 1H 2023).
  - First in class SSAO inhibitor ready for entry into phase 2 trials for neuro degenerative indications; e.g. Parkinson's
  - Best in class LOXL2 inhibitor ready for entry into phase 2 trials for chronic fibrotic disease
  - Australian government backed program in Duchenne Muscular Dystrophy completing preclinical studies 1H 2022
- FDA approval of Bronchitol for adult Cystic Fibrosis (CF) patients (Oct 2020) transformative for mannitol respiratory business
- Cash at 31/12/2021 - $21 million

PXS-5505 BREAKTHROUGH TRIAL IN MYELOFIBROSIS COMMENCED Q1 2021

The open label Phase 1c/2a trial (MF-101) is recruiting patients who are intolerant, unresponsive or ineligible for treatment with a JAK inhibitor in two stages; the phase 1c dose escalation phase successfully completed in October 2021 demonstrating good tolerability and >90% inhibition of the target enzymes. The subsequent six-month phase 2 dose expansion phase (24 patients) to evaluate safety and efficacy commenced recruitment shortly thereafter and is due to report by end of 2022. Follow up studies are planned to look at safety and efficacy in combination with JAK inhibitors which are the current standard of care.

The use of PXS-5505 in myelofibrosis has been championed by Dr Gabriela Hobbs, Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital. In commenting on the progression of the phase 1c results Dr Hobbs stated, *"This confirms what's been shown in healthy controls as well as mouse models, that this drug can inhibit the LOX enzymes in patients. Inhibiting these enzymes is a novel approach to the treatment of myelofibrosis by preventing the deposition of fibrosis and ultimately reversing the fibrosis that characterizes this disease."*

PXS-5505 AND OTHER INDICATIONS

PXS-5505 is a small molecule drug designed to be taken orally. It is an irreversible inhibitor of the members of the lysyl oxidase enzyme family; responsible for the cross linking of collagen and elastin fibres to make fibrotic or scar tissue. PXS-5505 has been subjected to long term toxicity studies and trialed in phase 1 studies with healthy volunteers. Together with multiple pre-clinical studies in a raft of fibrotic disease models this data set was approved by the FDA as sufficient to grant orphan status and an IND to progress into the phase 2 myelofibrosis study. It has also convinced many independent scientific and clinical groups globally to collaborate with Pharmaxis and test PXS-5505 in several other myeloproliferative diseases and cancer indications such as liver cancer, pancreatic cancer and myelodysplastic syndrome. PXS-5505's ability to break down fibrotic tissue should reduce tissue stiffness and mechanical stress in these fibrotic tumours and lead to reduced tumour growth and metastases as well as enabling better access for existing chemotherapy drugs to beat these difficult to treat cancers. In August 2021 the University of Rochester (NY) released the first data from its collaboration with Pharmaxis showing that in a pre-clinical model of one type of liver cancer PXS-5505 significantly improved survival when added to existing chemotherapy drugs. In November 2021 the FDA granted an IND to the University of Rochester to conduct a phase 1c/2a study trial of PXS-5505 added to current standard of care in in newly diagnosed unresectable hepatocellular carcinoma patients (liver cancer)

BRONCHITOL

Bronchitol is a precision spray-dried form of mannitol, delivered to the lungs by a specially designed, portable inhaler. Bronchitol works by rehydrating the airway/lung surface and promoting a productive cough. The product is manufactured in the Pharmaxis FDA approved facility in Sydney and is approved for marketing for the treatment of cystic fibrosis patients aged over six years in Australia, Russia and Switzerland and for patients aged 18 years and over in the US and the European Union.

Bronchitol is distributed in the US and EU by Chiesi Farmaceutici SpA, in Russia and Turkey by GEN, in Eastern Europe by local distributors and in Australia by BTC Health.

EXPERIENCED SCIENTIFIC LEADERSHIP

- *Gary Phillips, BPharm MBA – CEO & Managing Director (2003)*
  Appointed to his current role in 2013, with 30+ years' experience in the pharmaceutical industry. Prior to joining Pharmaxis he was the Australian CEO at Novartis, one of the world's largest pharma companies and a leader in therapies for cancer and other diseases.
- *Dr Brett Charlton, Medical Director (Founding Scientist)*
  25 years+ experience in clinical trial design and management, previous positions include Stanford, the Walter and Eliza Hall Institute
- *Dr Wolfgang Jarolimek, Head of Drug Discovery (2010)*
  Joined in 2010 from Glaxo. 20+ years' experience in pharmaceutical drug discovery and published more than 30 peer reviewed articles
- *Dr Kathleen Metters, Non-Exec Director (2017)*
  Formerly Head of Worldwide Basic Research for Merck & Co. and President and CEO of biopharmaceutical company Lycera Corp
- *Dr Neil Graham, Non-Exec Director (2020)*
  Had roles overseeing pipeline development and clinical programs at Regeneron (REGN:US), Vertex, Trimeris and Tibotec

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