www.pharmaxis.com.au

ASX: PXS	
Share price (13/07/21)	$0.082
52-Week range	$0.071-$0.170
Market capitalisation	$38m
Shares outstanding	452m
Pro forma cash (31/3/21)	$22m
Enterprise value	$16m

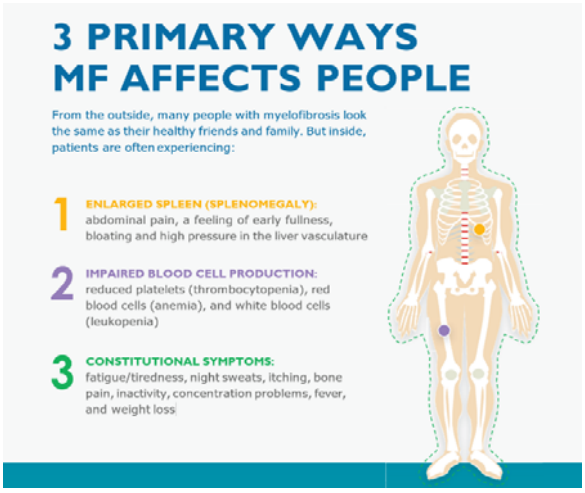
COMPANY SUMMARY

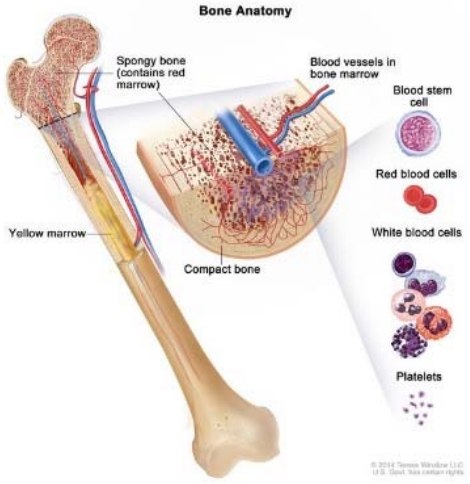
Pharmaxis Ltd is an Australian pharmaceutical research company developing a pipeline of oral drugs to treat inflammation and fibrosis; the underlying processes that cause many chronic diseases of the liver, kidney, lung, heart and several cancers. Pharmaxis also manufactures two respiratory products Aridol® and Bronchitol® which are approved in worldwide markets and from FY21 are expected to make a positive cash return to help fund the development pipeline.

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, Jakafi® and Inrebic® 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 2 trial of PXS-5505 commenced Q1 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. myelodysplastic syndrome and liver cancer
- 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 1 studies commenced 1Q 2021 with studies in patients with burns injuries or established scars due to commence in 2H 2021
  - 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
- FDA approval of Bronchitol for adult Cystic Fibrosis (CF) patients (Oct 2020) transformative for mannitol respiratory business
- - Mannitol respiratory business goes from cash burn of $4m FY20 to cash flow positive in FY21 growing to $10+m in FY26
- Cash positioned at 31/3/2021strengthened by $4 million placement and ~A$2 million sale of Russian distribution rights; proforma cash March 21: $22m. Additional $2m from sale of Australian distribution rights on 1 July 2021
  - Further opportunities to extend cash runway with business restructure

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; a dose escalation phase (up to 18 patients) to select the optimum dose followed by a six-month dose expansion phase (24 patients) to evaluate safety and efficacy. The dose escalation phase includes sites in Australia and South Korea and commenced recruitment in Q1 2021. Follow up studies are planned to look at safety and efficacy in combination with JAK inhibitors which are the current standard of care.

Dr Gabriela Hobbs, Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital said, "*JAK inhibition alone is insufficient in the treatment of patients with myelofibrosis; it is not associated with changes in underlying disease biology and it can worsen blood counts, leading to high drug discontinuation rates over time. The trial utilizing PX-5505 is supported by a sound scientific rationale, based on pre-clinical work demonstrating the importance of lysyl oxidase in the development of myelofibrosis. PXS-5505 has a unique mechanism of action that has the potential for disease modification. I am looking forward to seeing the effect of this drug in clinical trials*."

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 myelodysplastic syndrome and liver cancer. 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.

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 and Russia and for patients aged 18 years and over in the US and the European Union.

Bronchitol is distributed in the US by Chiesi Farmaceutici SpA (Chiesi) with Pharmaxis receiving a high teens % sales on top of product supply. In the EU Bronchitol is distributed by Chiesi with Pharmaxis receiving ~50% revenue share. Bronchitol is sold in Russia and Turkey by GEN, in Eastern Europe by specialist 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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