



Investor Presentation | April 2021 Gary Phillips CEO

Forward looking statement

This document contains forward-looking statements, including statements concerning Pharmaxis' future financial position, plans, and the potential of its products and product candidates, which are based on information and assumptions available to Pharmaxis as of the date of this document. Actual results, performance or achievements could be significantly different from those expressed in, or implied by, these forward-looking statements. All statements, other than statements of historical facts, are forward-looking statements.

These forward-looking statements are not guarantees or predictions of future results, levels of performance, and involve known and unknown risks, uncertainties and other factors, many of which are beyond our control, and which may cause actual results to differ materially from those expressed in the statements contained in this document. For example, despite our efforts there is no certainty that we will be successful in developing or partnering any of the products in our pipeline on commercially acceptable terms, in a timely fashion or at all. Except as required by law we undertake no obligation to update these forward-looking statements as a result of new information, future events or otherwise.

pharmaxis

Novel, small molecule medicines focused on cancer and fibrotic disease

In House Discovery and Development capability

Experienced team delivering stream of novel drugs to the clinic

Platform technology drives pipeline of clinical assets

Multiple opportunities from global leadership in amine oxidase enzymes

Cash flow positive manufacturing business

FDA approval for Cystic Fibrosis drug transformative with Pharmaxis manufacturing business now cash flow positive

Clinical stage medicines pharmaxis **Targeting Fibrosis** and Cancer

Lead asset PXS-5505 in phase 2 trial

Breakthrough clinical program with disease modifying potential in Myelofibrosis

Broad potential for PXS-5505 in oncology

Global scientific and clinical collaborations to extend value of PXS-5505 in further oncology indications

Anti scarring drug in phase 2 trial in 2021

PXS-6302 to enter patient studies in commercially important dermatology indications

Experienced Scientific Leadership Team

Significant global experience in drug development, commercialisation and partnering

In senior management



Wolfgang Jarolimek - Drug Discovery

- more than 20 years' experience in pharmaceutical drug discovery and published more than 30 peer reviewed articles
- previously Director of Assay Development and Compound Profiling at the GlaxoSmithKline Centre of Excellence in Drug Discovery in Verona, Italy
- spent 8 years as post-doc at the Max-Plank Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany





Gary Phillips – CEO and Managing Director

- more than 30 years of operational management experience in the pharmaceutical and healthcare industry in Europe, Asia and Australia
- joined Pharmaxis in 2003 and was appointed Chief Executive Officer in March 2013 at which time he was Chief Operating Officer
- previously held country and regional management roles at Novartis Hungary, Asia Pacific and Australia



Dieter Hamprecht – Head of Chemistry

- more than 20 years experience with small molecule and peptide experience, contributed to greater than 10 drug candidates brought to development and co-inventor of 50 patent families, co-author of 30+ scientific publications
- previously Managing Director Boehringer Ingelheim's research group in Milan
- senior medicinal chemistry positions at GSK



Kathleen Metters - Non Executive Director

- former Senior Vice President and Head of Worldwide Basic Research for Merck
 & Co. with oversight of all the company's global research projects.
- in a subsequent role at Merck &Co she led work on External Discovery and Preclinical Sciences
- former CEO of biopharmaceutical company Lycera Corp



Brett Charlton - Medical

- more than 25 years experience in clinical trial design and management
- author of more than 80 scientific papers
- founding Medical Director of the National Health Sciences Centre
- previously held various positions with the Australian National University, Stanford University, the Baxter Centre for Medical Research, Royal Melbourne Hospital, and the Walter and Eliza Hall Institute



Neil Graham - Non Executive Director

- former VP of immunology and inflammation responsible for strategic program direction overseeing pipeline development and clinical programs at Regeneron (REGN:US)
- former SVP program and portfolio management at Vertex Pharmaceuticals
- former Chief Medical Officer at Trimeris Inc and Tibotec Pharmaceuticals

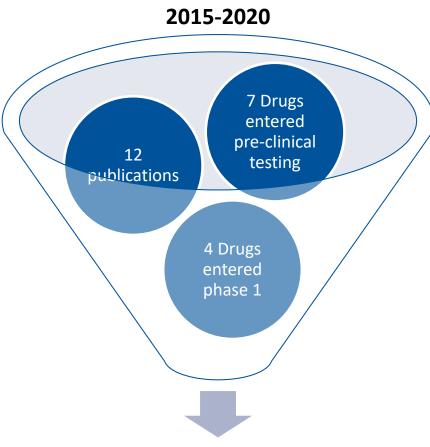
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Drug development capability

Established team in Drug Discovery and Clinical Trials with broad experience across multiple regulatory agencies

Organisation

- Leadership with extensive drug discovery/development experience from big pharma and biotech
- Full In house capabilities
 - On site laboratories
 - Leveraged with international network of external contract organisations
- Numerous collaborations with leading academic institutions in Australia and world-wide in pharmacology and medicinal chemistry
- High scientific reputation through peer-reviewed publications
- Direct management of Regulatory interaction with FDA, EMA, etc.



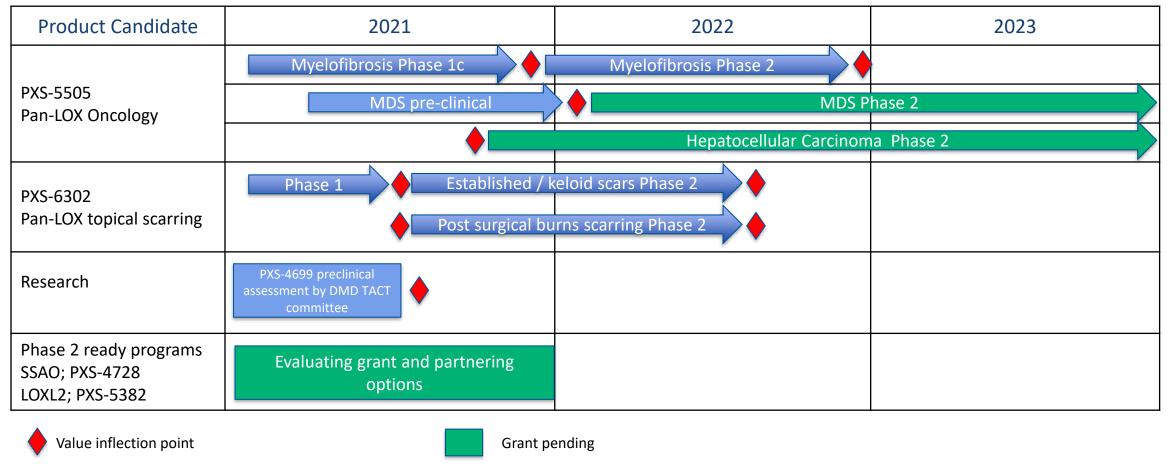
3 Drugs cleared phase 1 / IND ready
2 Drugs in phase 2

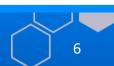
Strategy

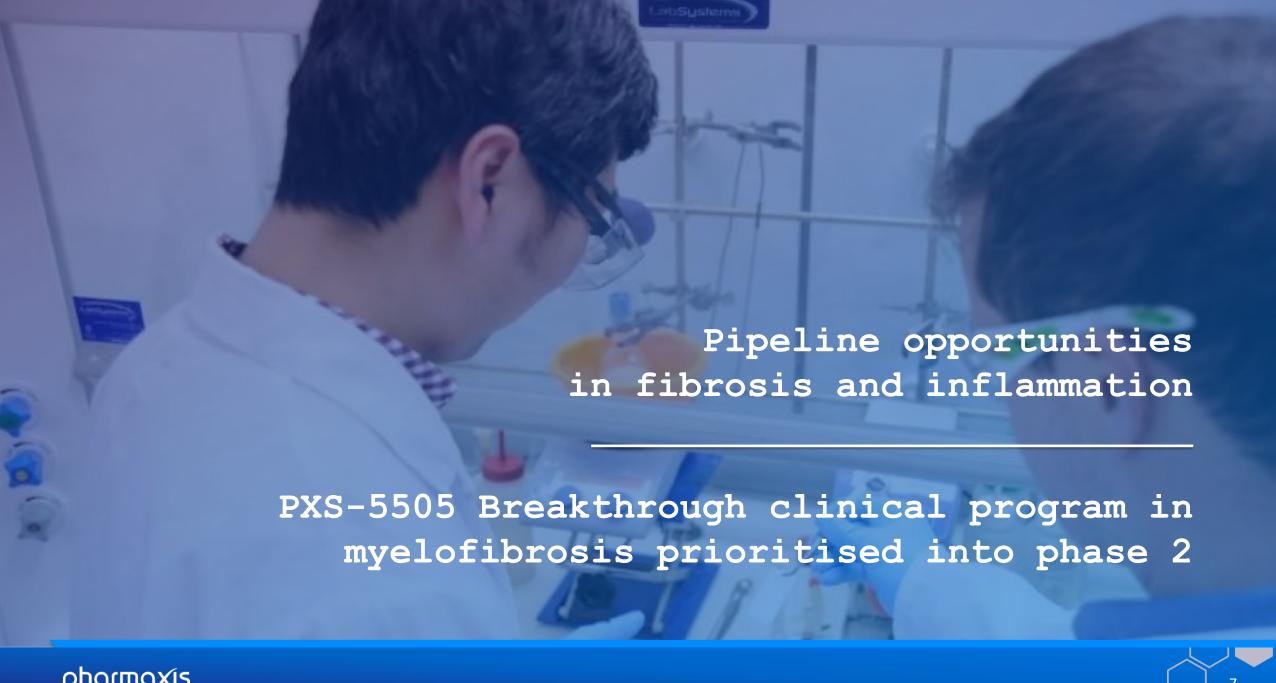
- Focus on inflammation and fibrosis/cancer driven diseases with high unmet medical need
- Leverage global leading position in amine oxidase chemistry and biology
- Create first / best in class small molecule inhibitors with biomarker assays for early validation of clinical hypothesis in phase 1 trials
- Protect intellectual property by focused chemical matter, use and biomarker patents
- Capture advantages of Australian location:
 - Accelerated (and lower cost) Phase 1 entry
 - Australian Government R&D tax credit system

Multiple potential value inflection points over next two years

Pipeline creates multiple opportunities







First in class PXS-5505 IND approved and in the clinic

Novel anti fibrotic approach with broad applications in difficult to treat cancers



Myelofibrosis: Orphan Disease with high unmet need forecast to exceed US\$1b

- Drug with disease modifying potential patented 2018
- Long term tox and phase 1 studies completed 1H 2020
- FDA orphan status granted July 2020
- IND approved August 2020
- Phase 1/2a proof of concept myelofibrosis study commenced recruitment Q1 21

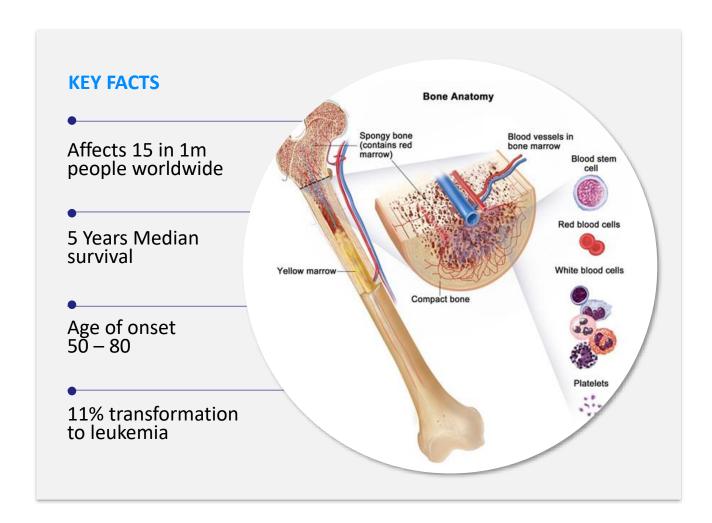


Adjunct to best standard of care in multiple cancers

- Pan-LOX inhibition synergistic with current standard of care and pharma development pipeline in many stromal cancers
- Academic and clinical interest in additional indications including;
 - Myeloproliferative disorders (e.g. MDS)
 - liver carcinoma (HCC)
 - o glioblastoma
- International studies facilitated by IND approval and availability of drug product

Myelofibrosis background

A rare type of bone marrow cancer that disrupts your body's normal production of blood cells



Primary Myelofibrosis is caused by a build up of scar tissue (fibrosis) in bone marrow reducing the production of blood cells:

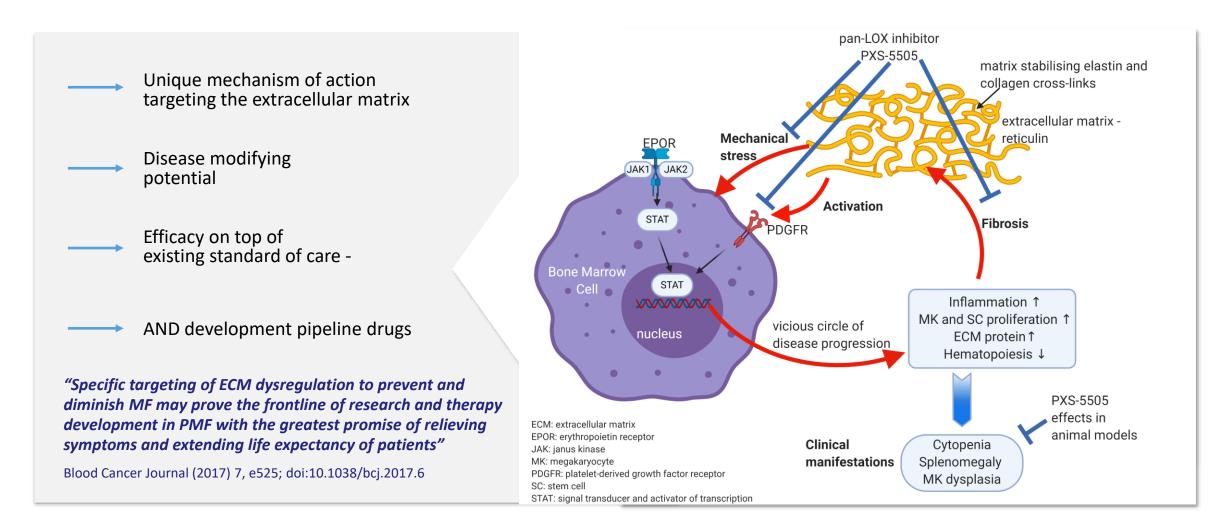
- Driven by clonal mutations of a hematopoietic stem cell (JAK, MPL, CALR genes)
- Reduced red blood cells can cause extreme tiredness (fatigue) or shortness of breath
- Reduced white blood cells can lead to an increased number of infections
- Reduced platelets can promote bleeding and/or bruising
- Spleen increases blood cell production and becomes enlarged
- Other common symptoms include fever, night sweats, and bone pain

Standard of Care; JAK inhibition

- Symptomatic relief plus some limited survival improvement. 75% discontinuation at 5 years
- Median overall survival is 14 16 months after discontinuation

Mode of action in MF

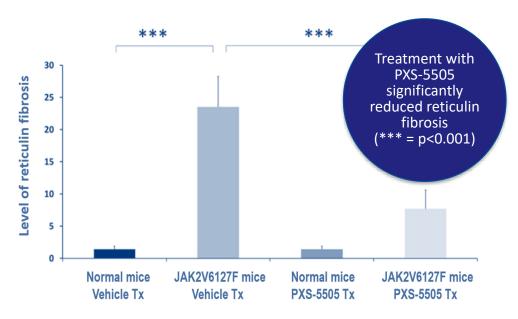
Disease modifying potential as monotherapy and on top of standard of care



PXS-5505; Pan-LOX inhibitor with promising profile

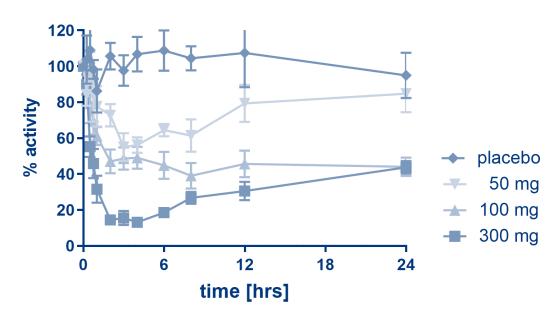
Pre clinical and clinical studies strongly support entry into patient studies

PXS-5505 attenuates hallmarks of primary myelofibrosis in mice.



"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." 1

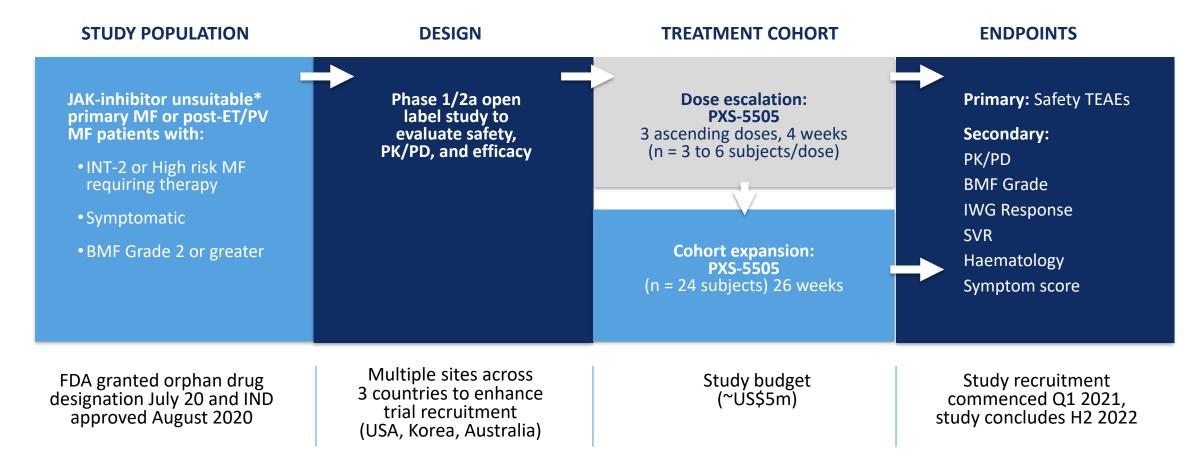
PXS-5505 - Phase 1 SAD



- Good safety profile with 6 month tox studies complete
- Dose dependant 24 hour inhibition of LOX enzymes from single once a day dose in humans
- No safety signal seen in phase 1 trials
- 2018 priority patent date

PXS-5505 Phase 1/2a Trial in myelofibrosis

6 month monotherapy study with meaningful safety and efficacy endpoints



^{*}Unsuitable = ineligible for JAKi treatment, intolerant of JAKi treatment, relapsed during JAKi treatment, or refractory to JAKi treatment. JAKi – Janus Kinase inhibitor, MF myelofibrosis, ET Essential Thrombocythaemia, PV polycythaemia vera, INT intermediate,

BMF bone marrow fibrosis, RP2D recommended phase 2 dose, TEAE treatment emergent adverse event, PK pharmacokinetics, PD pharmacodynamics, SVR spleen volume response, IWG International Working Group Myeloproliferative Neoplasms

Myelofibrosis - other programs

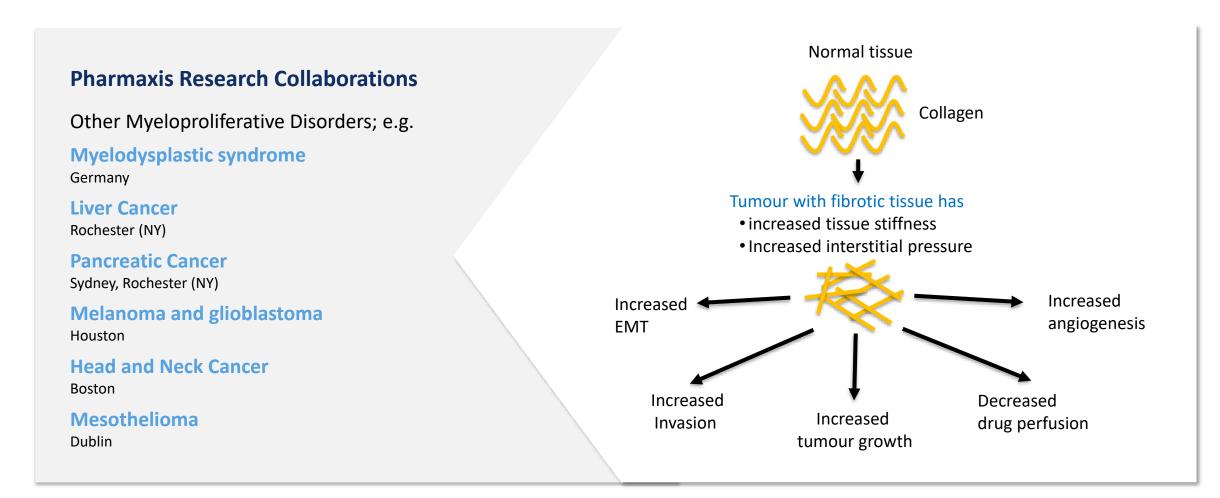
PXS-5505 unique mechanism of action promises disease modification and good tolerability

Company	Market cap ⁽¹⁾	Bourse	Asset	Description	Clinical phase
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
phormoxis	\$27m (A\$35m)	ASX	PXS-5505	Pan-LOX inhibitor	Phase 1c/2 commenced
Existing pipeline in development all have challenging safety / side effect profiles	de	KS-5505 mechanism of eliver additional efficat standard of care and/ rugs without adding t	cy on top of existing or known pipeline	l la companya di managanta di ma	PXS-5505 unique hanism of action with epected good efficacy AND tolerability



PXS-5505: Significant opportunity in other cancers

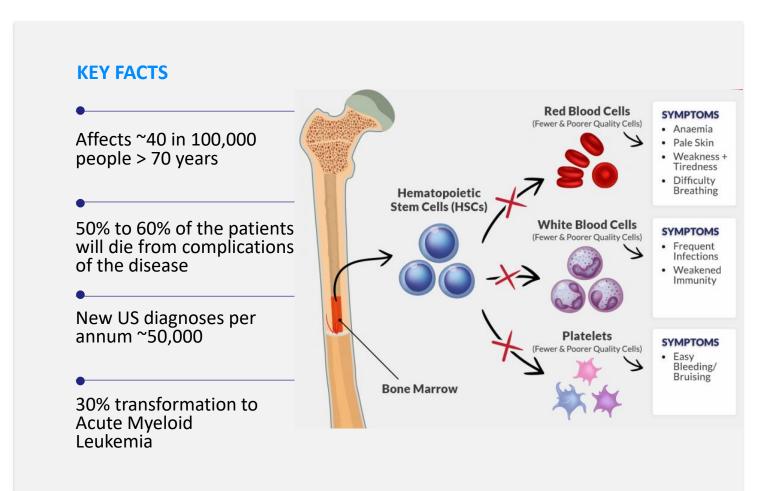
Global academic and clinical interest in LOX inhibition drives development plan



Multiple benefits from anti-fibrotic mechanism of action

Myelodysplastic Syndrome (MDS)

A rare type of bone marrow cancer that disrupts your body's normal production of blood cells



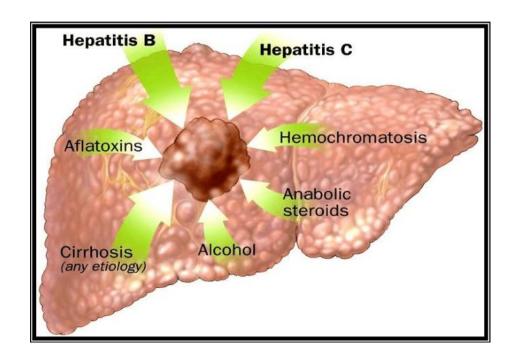
phormoxis

- A group of malignant hematopoietic neoplasms characterized by Bone marrow failure with resultant cytopenia and related complications
- Current standard of care
 - Allogeneic stem cell transplantation
 - Immunomodulatory drug lenalidomide,
 - Advanced disease: DNA hypomethylating agents (HMA), azacitidine (AZA), and decitabine
- Pre clinical evidence
 - Unpublished data from Pharmaxis scientific collaborations demonstrating strong proof of concept
 - Treatment with PXS-5505 and AZA significantly increases red cell count
- Clinical strategy
 - 6 month open label study in patients non responsive to AZA / current standard of care
 - Multi centre approach to follow on from myelofibrosis study

Hepatocellular Carcinoma (HCC)

4th leading cause of cancer-related mortality worldwide with a 19.6% 5-year relative survival

- Primary liver malignancies have doubled in incidence over the last two decades.
- HCC is a stromal (fibrotic) tumour
 - Accumulation of collagen crosslinks increases stromal stiffening and interstitial fluid pressure (IFP) reducing delivery of chemotherapy and immunotherapy.
- Etiology
 - Extrinsic factors e.g. Virus infections
 - Intrinsic factors e.g. auto immune diseases, fatty infiltration, genetics
- Current standard of care
 - Tyrosine kinase inhibitors
 - PD-L1 inhibitors + anti-VEGF

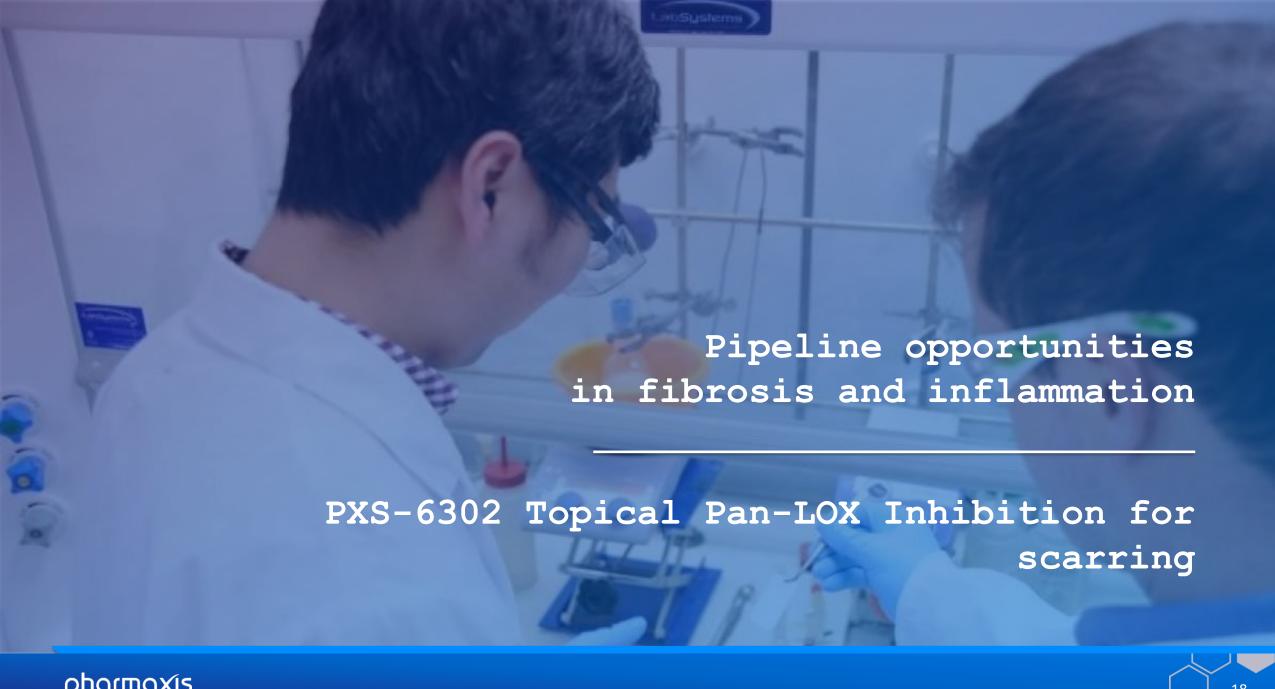


Pre-clinical data

- High LOX expression associated with reduced survival
- LOX is up-stream regulator of VEGF expression and inhibition of this enzyme could potentiate the intratumoral effects of anti-VEGF therapy
- Combination anti-PD-1 therapy with LOX inhibition has demonstrated synergistic decrease in tumor growth

Clinical strategy

- Enhance the intratumoral response to VEGF therapy and anti-PD-L1 blockade through the addition of LOX inhibition in human HCC
- Open label 6 month study combination PXS-5505 with Atezolizumab and Bevacizumab compared to historical standard of care in newly diagnosed unresectable or metastatic hepatocellular carcinoma



Hypertrophic and keloid scarring

Cutaneous scarring following skin trauma or a wound is a major cause of morbidity and disfigurement

KEY FACTS

100m patients develop scars in the developed world alone each year as a result of elective operations and operations after trauma

Hypertrophic scars and keloids are fibroproliferative disorders that may arise after any deep cutaneous injury caused by trauma, burns, surgery, etc.

Hypertrophic scars and keloids are cosmetically and functionally problematic significantly affecting patients' quality of life

Increased matrix stiffness

Increased contraction forces

Increased collagen production

Mechanical injury

Chemicals

Activated Fibroblasts

Excessive collagen production

Lysyl

Oxidase

Collagen cross-linking

Collagen turnover in keloid

The increase in extracellular matrix is a key factor and this depends on collagen and elastin cross-linking to make them less degradable.

- Mechanisms underlying scar formation are not well established, and prophylactic and treatment strategies remain unsatisfactory
- Current standard of care
 - Corticosteroids
 - Surgical revision
 - Cryotherapy
 - Laser therapy
 - 5-fluorouracil



- Pre clinical evidence
 - Unpublished data from Pharmaxis scientific collaborations demonstrating strong proof of concept
 - Treatment with PXS-6302 monotherapy and tool compound demonstrates cosmetic and functional improvements to the scar
- Clinical strategy
 - 3 month placebo controlled study in patients versus current standard of care.
 - Initial patient groups will either have established scars or scarring subsequent to burn injury.

Further non core pipeline opportunities in fibrosis and inflammation

Leveraging global leadership position in amine oxidase enzymes to deliver targeted drugs for cancer and fibrosis

Product Candidate	Indications	Pre- clinical	Phase 1	Phase 2		Next Steps
SSAO; PXS-4728	Repurposed for neuro inflammatory disease				•	Partnering discussions; phase 2 protocol and funding discussions with independent investigators
LOXL-2; PXS-5382	Chronic fibrotic disease e.g. chronic kidney disease, idiopathic pulmonary fibrosis				•	Partnering discussions; phase 2 protocol and funding discussions with independent investigators
SSAO/MAOB; PXS-4699	Anti inflammatory Muscular Dystrophy				•	\$1m matched funding grant DMD TACT committee Q2 2021 Advance to the clinic H1 2022
SSAO/MPO; PXS-5370	Anti inflammatory Multiple indications				•	Grant identification in process

Mannitol respiratory business (Bronchitol® and Aridol®)

Transformational impact of FDA Bronchitol approval (Oct 2020) - cash flow positive from FY 2021 onwards

Sales

- Mannitol respiratory sales forecast to double by FY 2022 with Bronchitol > 75% of sales
- Strong longer term growth contribution from US
- Growth in Ex-US markets including Russia

Expenses

- Relatively fixed production cost base
- Potential for simplified business model to reduce costs

EBITDA

- Positive EBITDA from FY 2021 onwards (before potential cost savings).
- US volumes enable mannitol segment to generate profit



Bronchitol in US

• US CF market >65% of global market

US market doubles global cystic fibrosis patient opportunity with attractive pricing

- Chiesi approval /launch milestone payments US\$10m received FY 2021
- US sales commence in Q2 CY 2021
- High teens % of Chiesi sales + long term supply contract - ~20% of Chiesi US Bronchitol net sales flow directly to the Pharmaxis bottom line
- Three sales milestones totaling US\$15m payable on achieving annual sales thresholds

Strengthened cash position

Further opportunities to extend cash runway ahead

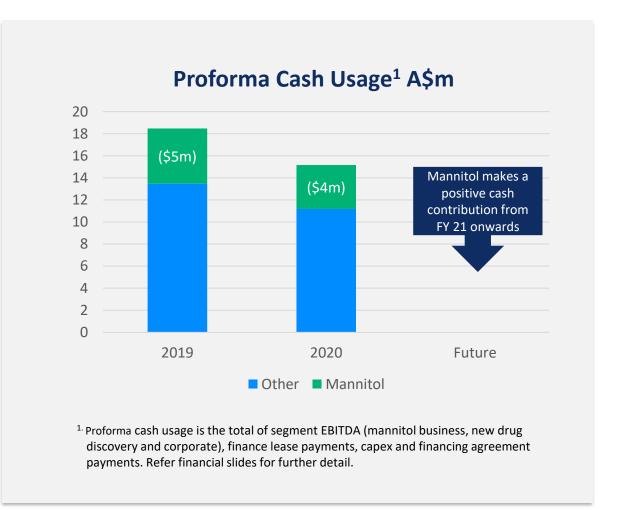
Dec 20 proforma cash balance of A\$22m

- Cash Dec 2020: A\$18m
 (includes \$4m Chiesi milestone received March 2021)
- US Bronchitol sales commence in Q2 CY 2021
- High teens % of Chiesi sales + long term supply contract ~20% of Chiesi US Bronchitol net sales flow directly to the Pharmaxis bottom line
- Mannitol business to go from cash burn (FY 20: A\$4m) to cash flow positive from FY 21 onwards (FY 26: A\$10m+)

Year	2019	2020	From 2021 E
EBITDA (A\$m)*	(\$5.0)	(\$4.0)	Cash Flow Positive

Further opportunities to extend cash runway

- Mannitol potential cost savings
- Distribution license fees from additional Aridol and Bronchitol territories
- Pipeline supported by grants and R&D tax credit (~A\$5m 2020)
- Partnering deals with pipeline assets



²²

Anticipated news flow: 2021 - 2022

Multiple value inflection points over next two years

H1 2021

- Feb 22: Breakthrough drug PXS-5505 phase 1c/2a myelofibrosis study commenced recruitment
- Mar 19: Chiesi pays US\$3m milestone on Pharmaxis shipment of US launch
- Mar 31: LOX topical drug PXS-6302 commenced independent investigator studies safety
- Mannitol business simplification completed realising annual cost savings

H₂ 2021

- PXS-5505 phase 1c/2a myelofibrosis study dose expansion stage commence
- First collaborations to progress PXS-5505 into clinical trials in other myeloproliferative diseases and/or cancer indications
- Ongoing cash receipts from supply of Bronchitol for US sales
- LOX topical drug progresses into independent investigator patient studies - burns and established scars
- Feedback from global advisory committee on development fast tracking for Duchenne muscular dystrophy clinical trials.

CY 2022

- PXS-5505 phase 2a myelofibrosis study safety and efficacy data
- PXS-5505 dose escalation data in myelodysplastic syndrome
- PXS-5505 dose escalation data in hepatocellular carcinoma
- LOX topical drug phase 2 studies burns and established scars safety and efficacy data





Experienced senior management team

Significant experience in drug development, commercialisation and partnering



Gary Phillips – CEO

- more than 30 years of operational management experience in the pharmaceutical and healthcare industry in Europe, Asia and Australia
- joined Pharmaxis in 2003 and was appointed Chief Executive Officer in March 2013 at which time he was Chief Operating Officer
- previously held country and regional management roles at Novartis Hungary, Asia Pacific and Australia



David McGarvey - CFO

- more than 30 years' experience building Australian based companies from inception to globally successful enterprises
- joined Pharmaxis as Chief Financial Officer and Company Secretary in December 2002
- previously Chief Financial Officer of the Filtration and Separations
 Division of US Filter (1998-2002), and Memtec Limited (1985-1998)
- commenced career at PricewaterhouseCoopers



Brett Charlton - Medical

- more than 25 years experience in clinical trial design and management
- author of more than 80 scientific papers
- founding Medical Director of the National Health Sciences Centre
- previously held various positions with the Australian National University, Stanford University, the Baxter Centre for Medical Research, Royal Melbourne Hospital, and the Walter and Eliza Hall Institute



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- previously Director of Assay Development and Compound Profiling at the GlaxoSmithKline Centre of Excellence in Drug Discovery in Verona, Italy
- spent 8 years as post-doc at the Max-Plank Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany



Kristen Morgan – Alliance Management

- more than 20 years' experience in the pharmaceutical industry having previously held a senior role in medical affairs at Sanofi-Aventis, and a commercial sales role at GlaxoSmithKline
- responsibility for alliance management and medical and regulatory affairs

Non Executive Directors

Malcolm McComas

- Chair, former investment banker; former MD Citi Group

Kathleen Metters

former head of worldwide basic research at Merck; former CEO of biopharmaceutical company Lycera Corp

Will Delaat

former CEO of Merck Australia; former chair of Medicines Australia

Neil Graham

former strategic program director at Regeneron Inc; extensive career in pipeline development and clinical development

Board

Significant international pharmaceutical experience



Malcolm McComas - Chair

- former investment banker and commercial lawyer
- former MD Citi Group
- has worked with many high growth companies across various industry sectors and has experience in equity and debt finance, acquisitions and divestments and privatisations.
- joined Pharmaxis Board in 2003
- chair since 2012



Will Delaat - Non-Executive Director

- more than 35 years' experience in the global pharmaceutical industry
- former CEO of Merck Australia
- former chair of Medicines Australia and Pharmaceuticals Industry Council
- joined Pharmaxis Board in 2008



Dr Kathleen Metters - Non-Executive Director

- former Senior Vice President and Head of Worldwide Basic Research for Merck & Co. with oversight of all the company's global research projects.
- in a subsequent role at Merck &Co she led work on External Discovery and Preclinical Sciences
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Dr Neil Graham - Non-Executive Director

- former VP of immunology and inflammation responsible for strategic program direction overseeing pipeline development and clinical programs at Regeneron (REGN:US)
- former SVP program and portfolio management at Vertex Pharmaceuticals
- former Chief Medical Officer at Trimeris Inc and Tibotec Pharmaceuticals

Financials

Cash

Periods ended (A\$'000)	Dec 2020 HY	Dec 2019 HY	Jun 2020 FY	Jun 2019 FY
Proforma cash				
Cash period end	18,249	25,864	14,764	31,124
R&D tax credit	-	-	5,048	5,962
Chiesi milestone payments	~4,000¹	-	~14,000	-
	~\$22,249	\$25,864	~\$33,812	\$37,086
Cash Flow Statement Highlights				
Operations				
Receipts from customers	3,602	3,973	7,775	6,893
R&D tax incentive	5,099	6,221	6,271	-
Chiesi milestone	9,949	-	-	-
Payments to suppliers, employees etc	(13,602)	(13,886)	(27,330)	(26,691)
Total operations	5,048	(3,692)	(13,284)	(19,798)
Investing (capex)	(281)	(328)	(574)	(981)
Finance lease payments ²	(1,147)	(1,111)	(2,232)	(1,593)
Financing agreement payments ³	(135)	(129)	(270)	(254)
Share issue - net	-	-	-	22,677
Net increase (decrease) in cash	\$3,485	(\$5,260)	(\$16,360)	\$51

- 1. US\$3m milestone earned February 2021
- 2. Lease over 20 Rodborough Rd (to 2024) total liability at 31 December 2020: \$7.1 million
- 3. NovaQuest financing not repayable other than as % of US & EU Bronchitol revenue up to 7 years

Financials

Income statement highlights

Periods ended (A\$'000)	Dec 2020 HY	Dec 2019 HY	Jun 2020 FY	Jun 2019 FY
Segment Financials				
New drug development				
Oral Pan-LOX (external costs)	(1,323)	(1,400)	(3,124)	(3,833)
Other program external costs (net of grants)	(775)	(1,078)	(3,315)	(5,108)
Employee costs	(1,799)	(1,529)	(3,373)	(2,837)
Overhead	(238)	(281)	(460)	(606)
R&D tax credit	148	259	5,159	5,962
EBITDA	(3,987)	(4,029)	(5,113)	(6,764)
Mannitol respiratory business				
Sales	3,086	3,259	7,027	5,676
Other income	10,098	10	20	27
	13,184	3,269	7,047	5,703
Expenses – employee costs	(2,914)	(3,037)	(5,855)	(6,083)
Expenses – manufacturing purchases	(1,172)	(746)	(1,456)	(1,689)
Expenses – other	(2,374)	(1,755)	(3,713)	(2,944)
EBITDA	6,724	(2,269)	(3,977)	(5,013)
Corporate – EBITDA	(2,024)	(1,701)	(2,990)	(3,874)
Total Adjusted EBITDA	713	(7,999)	(12,080)	(15,651)
Net profit(loss)	\$46	(\$10,319)	(\$13,943)	(\$20,058)

Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap ¹	A\$32m
Shares on Issue ¹	397m
Employee Options ¹	19m
Liquidity (turnover last 12 months) ¹	369m shares
Share price ¹	A\$0.081
Proforma cash balance (31 December 2020)	A\$22m

Institutional Ownership	31 Dec 20
BVF Partners (US)	19%
D&A Income Limited	7%
Other Institutions	8%
Total Institutional Ownership	34%



pharmaxis

developing breakthrough treatments for fibrosis and inflammation

www.pharmaxis.com.au





Contacts

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