



## Investor Presentation

**pharmaxis**

developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | 8 December 2021  
Gary Phillips CEO

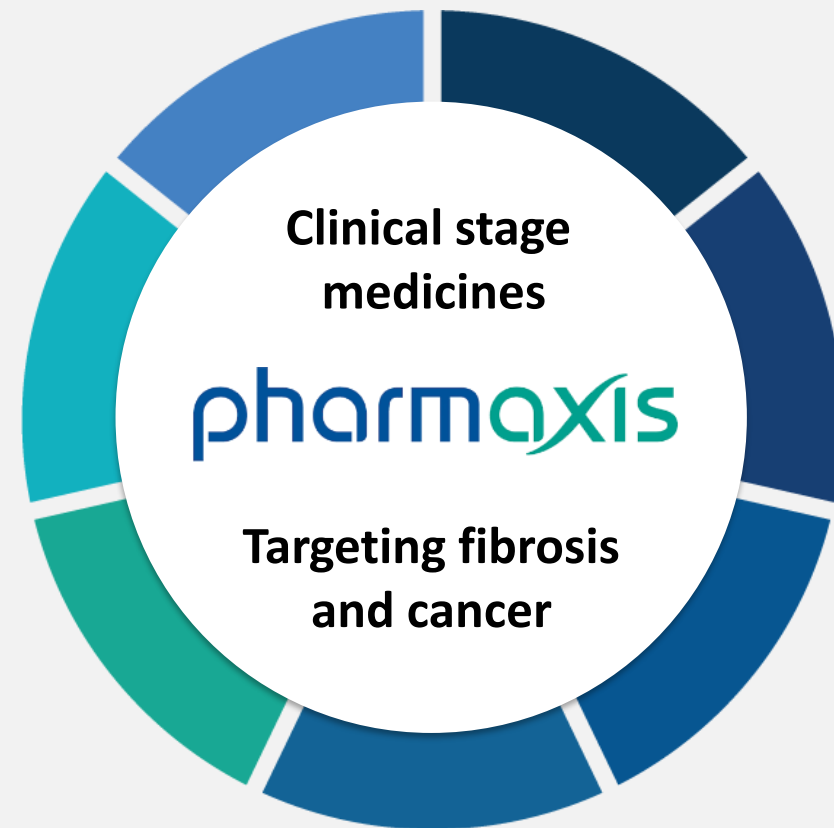
## Forward looking statement

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.

## Executive Summary

- Pharmaxis is a clinical stage drug development company targeting fibrosis and cancer indications with first in class or best in class small molecule drugs in markets of high value
- Lead asset PXS-5505 is in phase 2 trial – a breakthrough clinical program with disease modifying potential in Myelofibrosis
- IND approval to commence US investigator led phase 2 trial in liver cancer with PXS-5505 as first line treatment added to existing chemotherapy.
- Topical drug PXS-6302 progressing to phase 1c trial in patients with potential to improve function and appearance of scars
- Specific corporate strategy to deliver non-dilutive cash and cost savings from commercial stage mannitol business;
- Pharmaxis is in a strong position to fund its focused clinical program



## Capital Raising

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## Offer Summary

Placement	<ul style="list-style-type: none"><li>• Oversubscribed placement of \$7.2 million (15% of issued capital), to institutional and sophisticated investors completed on 24 November 2021</li><li>• Offer Price of \$0.105 per share represents a ~12.% discount to VWAP</li><li>• Strong support from existing substantial shareholders BVF Partners LP, Karst Peak Capital Limited and D&amp;A Income Ltd, together with a number of new institutional and sophisticated investors.</li></ul>
Share Purchase Plan	<ul style="list-style-type: none"><li>• Share Purchase Plan (<b>SPP</b>) to raise approximately \$2.0 million to eligible shareholders currently open</li><li>• Offer price same as placement - \$0.105</li><li>• Closes 15 December 2021</li></ul>
Use of Funds	<ul style="list-style-type: none"><li>• Proceeds raised will be used to fund trials and for working capital</li></ul>
Lead Manager	<ul style="list-style-type: none"><li>• Morgans Corporate Limited</li></ul>

# Shareholders & cash



Financial Information	7 Dec 21
ASX Code	PXS
Share price	\$0.102
Liquidity (turnover last 12 months)	239m shares
Market Cap	A\$53m
Pro-forma cash balance <sup>1</sup> (30 Sept 2021)	A\$23m
Enterprise value	A\$30m
Clinical development program supported by:	
<ul style="list-style-type: none"><li>• Mannitol business* forecast to provide ongoing positive EBITDA growing to \$10m in 5 - 6 years</li><li>• R&amp;D tax credits</li><li>• Strategy of partnering deals with pipeline assets</li></ul>	
1. Pro-forma cash after share placement. SPP targeting \$2m closes 15/12/21	

Institutional Ownership	7 Dec 21
BVF Partners LP	20%
Karst Peak Capital Limited	12%
D&A Income Limited	8%
<b>Total Institutional Ownership</b>	<b>41%</b>

## Share Price





# Myelofibrosis background

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

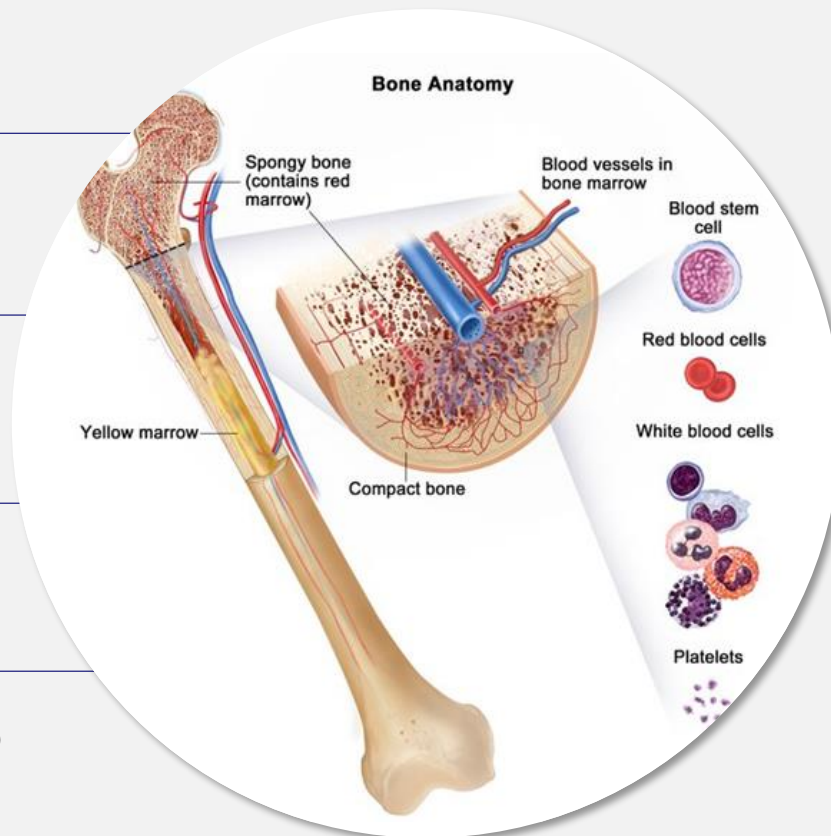
## KEY FACTS

- Affects 15 in 1m people worldwide

- 5 Years Median survival

- Age of onset 50 – 80

- 11% transformation to leukemia



**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

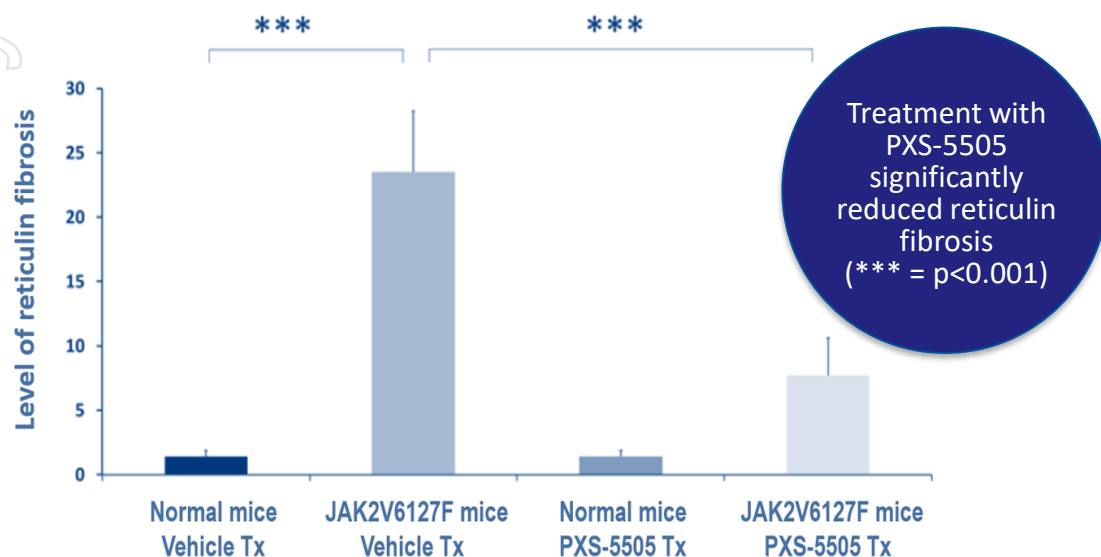
## Standard of Care; JAK inhibition

- Current standard of care; revenue ~US\$1b per annum
- Symptomatic relief plus some limited survival improvement. 75% discontinuation at 5 years
- Median overall survival is 14 – 16 months after discontinuation

# PXS-5505; An effective and safe inhibitor of LOX in myelofibrosis patients

Pre clinical and clinical studies strongly support entry into long term phase 2 patient studies

## PXS-5505 attenuates hallmarks of primary myelofibrosis in mice

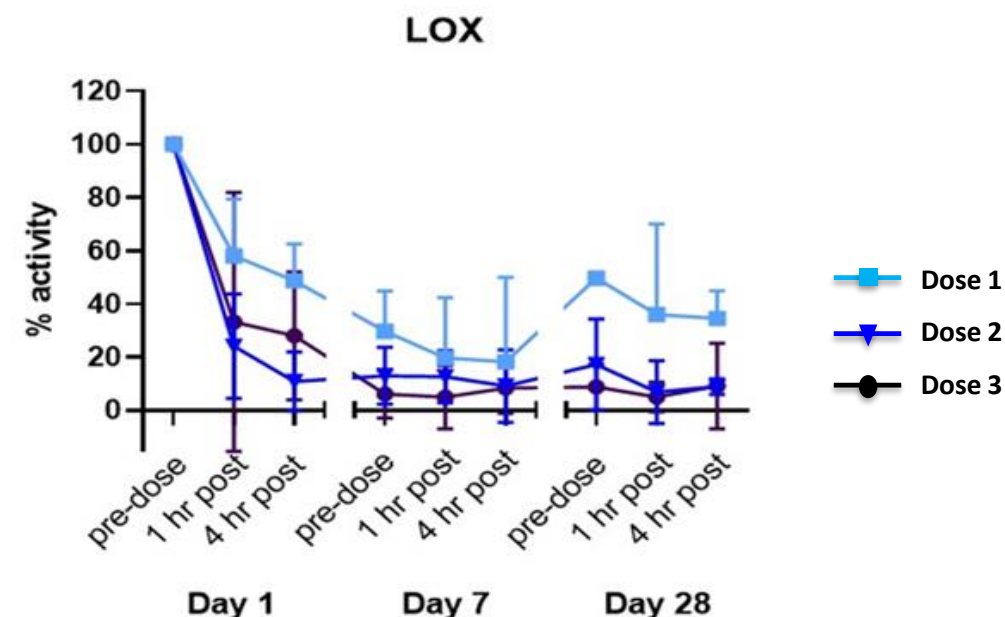


*"None of the drugs approved to date consistently or meaningfully alter the fibrosis that defines this disease. PXS-5505 has a novel mechanism of action by fully inhibiting all LOX enzymes.*

*Preliminary data thus far, demonstrate that PXS-5505 leads to a dramatic, >90% inhibition of LOX and LOXL2 at one week and 28 days. 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"*

*Dr Gabriela Hobbs<sup>1</sup>*

## PXS-5505 – Phase 1c dose escalation in MF patients



- Open label dose expansion in JAK-inhibitor unsuitable<sup>2</sup> primary MF or post-ET/PV MF patients
- Maximum of 3 patients on each dose for 28 days
- Good safety profile with no adverse events at highest dose
- >90% inhibition of LOX and LOXL2 at trough on highest dose at day 7 and 28

Ref Graph1: Leiva et al. Intl J Hemat 2019. <https://doi.org/10.1007/s12185-019-02751-6>

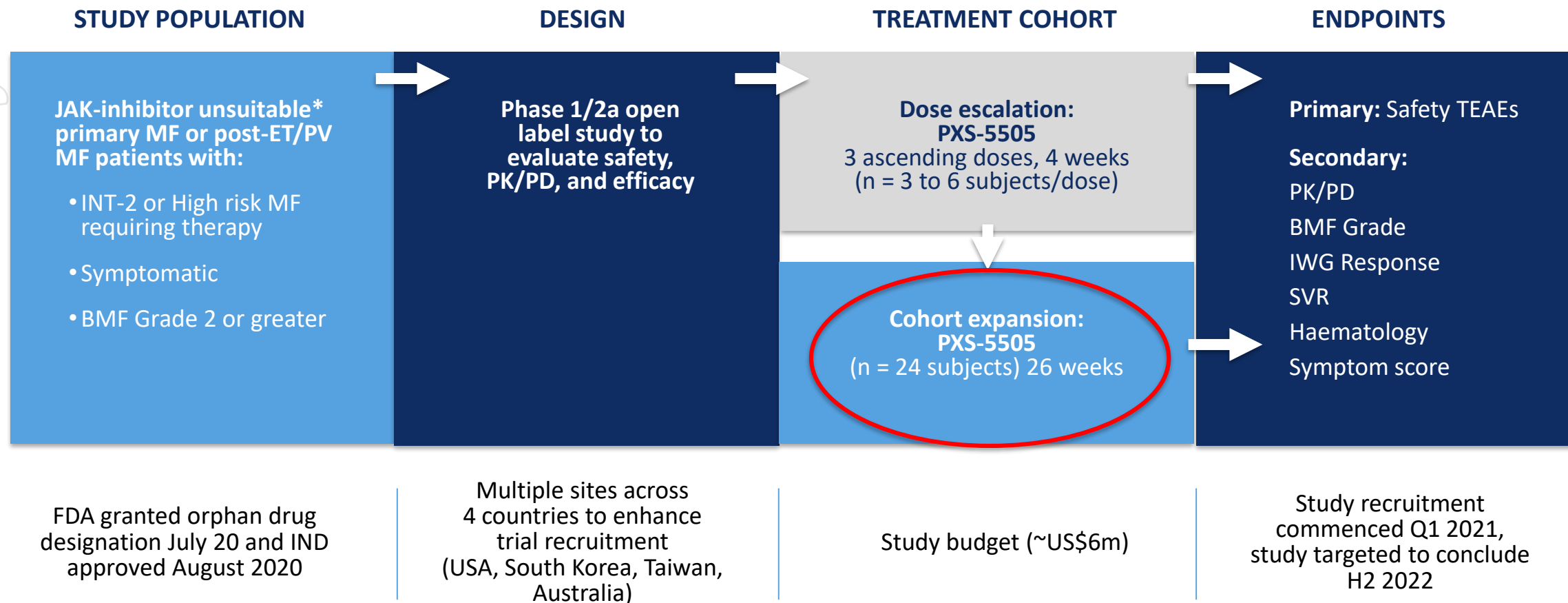
<sup>1</sup> Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital

<sup>2</sup> 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



# PXS-5505 Phase 1/2a Trial in myelofibrosis

6 month monotherapy study with meaningful safety and efficacy endpoints (phase 1c complete)



\*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

# PXS-5505: Significant opportunity in other cancers

Global academic and clinical interest in LOX inhibition drives development plan

## Pharmaxis Research Collaborations

### Myelodysplastic syndrome

Germany

### Liver Cancer

Rochester (NY)

### Pancreatic Cancer

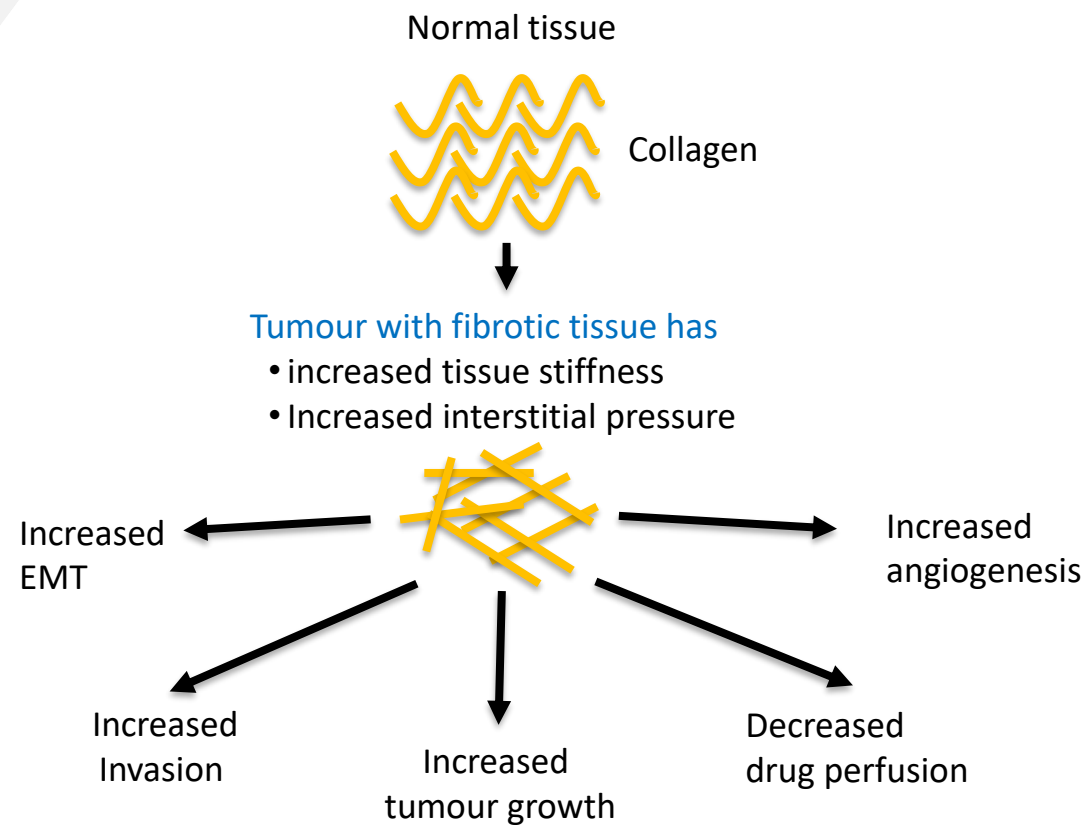
Sydney, Rochester (NY)

### Melanoma and glioblastoma

Houston

### Head and Neck Cancer

Boston, (MA)

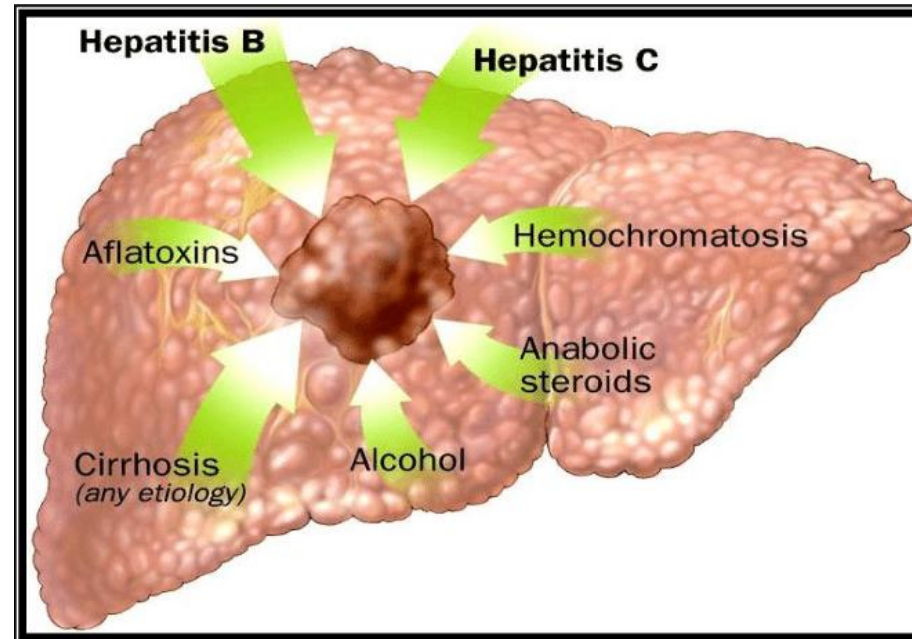


Multiple expected benefits from inhibition of LOX enzymes

# 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.
- 4th leading cause of cancer-related mortality worldwide with a 19.6% 5-year relative survival
- HCC is a stromal (fibrotic) tumour
  - Accumulation of collagen cross-links increases stromal stiffening and interstitial fluid pressure reducing delivery of chemotherapy and immunotherapy
- Current standard of care
  - 20-30% are resectable at presentation with many patients relying on systemic therapy:
  - Tyrosine kinase inhibitors
  - PD-L1 inhibitors + anti-VEGF



**Commercial Opportunity**  
Drugs market currently worth ~US\$2bn with rising incidence forecasted to drive growth to ~US\$7bn by 2027

- Pre-clinical data (Rochester Uni; Aug 2021)
  - Tumour tissue specimens show LOX enzymes are significantly elevated in human liver cancer and correlate with poor prognosis.
  - PXS-5505 with or without chemotherapy treatment in a pre-clinical model significantly **improves survival, delays tumor growth, and reduces intratumoral pressure.**
- Proposed clinical strategy
  - Enhance the intratumoral response to standard of care through the addition of LOX inhibition in human HCC
  - 6 month study combination PXS-5505 on top of standard of care in newly diagnosed unresectable or metastatic hepatocellular carcinoma
  - Investigator led trial – University of Rochester. Cost ~US\$2.5m

# Hypertrophic and keloid scarring

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

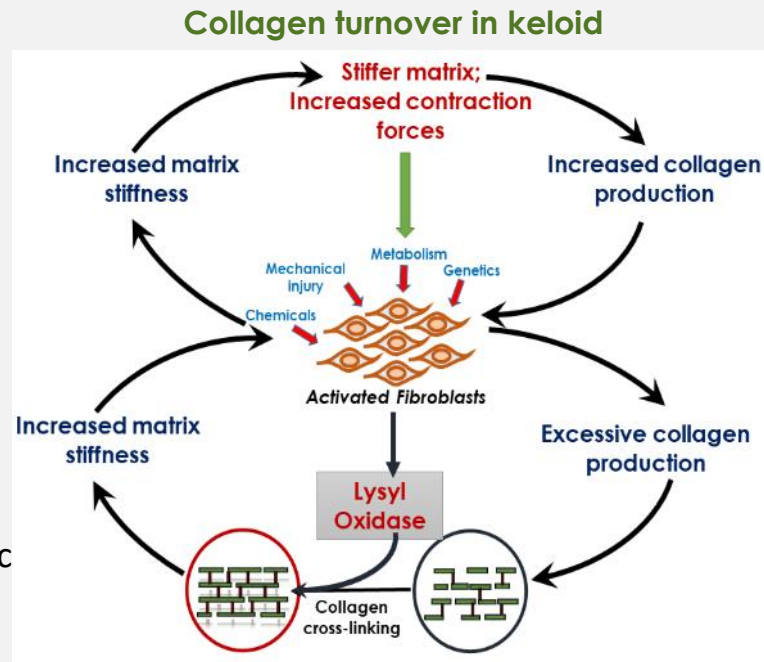
## KEY FACTS

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

Total scar treatment market in 2019 exceeded US\$19b. Keloid and hypertrophic scar segment ~US\$



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; prophylactic and treatment strategies remain unsatisfactory

- Current standard of care includes:

- Corticosteroids
- Surgical revision
- Cryotherapy
- Laser therapy
- 5-fluorouracil



- Pre clinical evidence

- Treatment with PXS-6302 monotherapy demonstrates cosmetic and functional improvements to scarring in pre clinical models (data on file)

- Clinical evidence

- 1 month phase 1a in healthy volunteers demonstrates good tolerability and full inhibition of LOX in skin.

- Next Steps

- 3 month study versus placebo in patients with established scars to commence Q4 2021
- Study to investigate scarring subsequent to burn injury to follow in 2022

# Multiple potential value inflection points over next two years

Pipeline creates multiple opportunities in high value markets

## Target timelines

Product	2021	2022	2023
PXS-5505 LOX Oncology	Myelofibrosis Phase 1c	Myelofibrosis Phase 2	
	Pre-clinical	Liver cancer (HCC) Phase 1c/2	
	Other indications - pre clinical		
PXS-6302 LOX topical scarring	Phase 1	Established scars Phase 1c	
		Post burns scarring Phase 1c	
PXS-4699 DMD Preclinical	DMD pre-clinical		
Phase 2 ready PXS-4728: SSAO PXS-5382: LOXL2	Evaluating grant and partnering options		

◆ Potential value inflection point

■ Negotiating Investigator led clinical trial with University of Rochester





developing breakthrough treatments for fibrosis and inflammation

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[www.pharmaxis.com.au](http://www.pharmaxis.com.au)



## Contacts

Gary Phillips

Chief Executive Officer

[gary.phillips@pharmaxis.com.au](mailto:gary.phillips@pharmaxis.com.au)

David McGarvey

Chief Financial Officer






[david.mcgarvey@pharmaxis.com.au](mailto:david.mcgarvey@pharmaxis.com.au)

## Additional Information

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# Myelofibrosis – examples of other programs

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

Company	Market cap <sup>(1)</sup>	Bourse	Asset	Description	Clinical phase
 Keros Therapeutics	\$0.9bn	Nasdaq	KER-050	TGF- $\beta$ ligand trap	Phase 2
 Constellation Pharmaceuticals	\$1.6bn	Nasdaq	CPI-0610	BET inhibitor	Phase 3
 Kartos Therapeutics	\$0.7bn <sup>(2)</sup>	n.a. – private	KRT-232	MDM2 antagonist	Phase 3
 geron	\$0.4bn	Nasdaq	Imetelstat	Telomerase inhibitor	Phase 3
 pharmaxis	\$43m (A\$57m)	ASX	PXS-5505	LOX inhibitor	Phase 1c/2 commenced

PXS-5505 unique mechanism of action expected to deliver additional efficacy on top of existing standard of care and/or known pipeline drugs without adding to tolerability issues

# Mannitol respiratory business (Bronchitol® and Aridol®)

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

## Sales

- Bronchitol > 75% of sales
- Strong short term growth from Russia
- Sales growth expected in approved markets as patients access hospitals again post COVID-19 restrictions
- Strong longer term growth contribution expected from US

## Expenses

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

## Segment EBITDA

- Forecast ongoing positive EBITDA
- US volumes contribute to mannitol segment generating profit



## Bronchitol in US

- US CF market >65% of global market in value
  - US market doubles global cystic fibrosis patient opportunity with attractive pricing
- Chiesi approval /launch milestone payments US\$10m received FY 2021
- US sales commenced in Q2 CY 2021 – delay in patient initiation due to COVID
- High teens % of Chiesi sales + 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



# Experienced Scientific Leadership Team

Significant global experience in drug development, commercialisation and partnering

## In senior management



### Wolfgang Jarolimek – Drug Discovery

- 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-Planck Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany



### Dieter Hamprecht – Head of Chemistry

- 20+ years' experience with small molecule and peptide drug discovery, 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



### Brett Charlton - Medical

- 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

## On the board



### Gary Phillips – CEO and Managing Director

- 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



### 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



### 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



# Anticipated news flow: 2021 – 2022

Multiple anticipated value inflection points

## PXS-5505 – anti cancer drug

- PXS-5505 phase 1c liver cancer (HCC) study – starts recruitment
- PXS-5505 phase 2a myelofibrosis study – fully recruited
- PXS-5505 phase 2a myelofibrosis study safety and efficacy data
- PXS-5505 publications by KOL's in other cancers
- Results of Charlie Teo Foundation funded research into PXS-5505 in glioblastoma

## PXS-6302 – scar treatment

- LOX topical drug PXS-6302 commences independent investigator patient studies - established scars
- LOX topical drug PXS-6302 commences independent investigator patient studies - burns scars
- LOX topical drug PXS-6302 patient studies fully recruited – established and burns scars
- PXS-6302 publications by KOL's in scarring

## Other

- Mannitol business simplification – realising annual cost savings
- Mannitol business – appointment of new distributors
- Decision by Aptar whether to exercise (by Aug 22) option to license high payload inhaler for US\$2.5m plus royalties

# 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

- 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



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# Financials

## Income statement highlights

Periods ended (A\$'000)	Sept 2021 Qtr	Sept 2020 Qtr	June 2021 FY	June 2020 FY
<b>Segment Financials</b>				
<b>New drug development</b>				
Oral LOX (external costs)	(1,467)	(777)	(2,521)	(3,124)
Other program external costs (net of grants)	(303)	(297)	(1,850)	(3,315)
Employee costs	(715)	(924)	(3,270)	(3,373)
Overhead	(102)	(93)	(395)	(460)
R&D tax credit	-	148	148	5,159
EBITDA	(2,587)	(1,943)	(7,888)	(5,113)
<b>Mannitol respiratory business</b>				
Sales	3,272	661	6,680	7,027
Other revenue and income	2,342	142	15,985	20
	5,614	803	22,665	7,047
Expenses – employee costs	(1,197)	(1,385)	(5,558)	(5,855)
Expenses – manufacturing purchases	(1,205)	(71)	(1,168)	(1,456)
Expenses – other	(1,103)	(1,212)	(4,483)	(3,713)
EBITDA	2,109	(1,865)	11,456	(3,977)
<b>Corporate – EBITDA</b>	(755)	(860)	(3,795)	(2,990)
<b>Total Adjusted EBITDA</b>	<b>(1,233)</b>	<b>(4,668)</b>	<b>(\$227)</b>	<b>(\$12,080)</b>
<b>Net profit (loss)</b>	<b>(3,179)</b>	<b>(4,981)</b>	<b>(\$2,970)</b>	<b>(\$13,943)</b>

# Financials

## Cash

Periods ended (A\$'000)	Sept 2021 Qtr	Sept 2020 Qtr	June 2021 FY	June 2020 FY
<b>Cash</b>				
Cash period end	16,131	9,656	18,712	14,764

## Cash Flow Statement Highlights

### Operations

Receipts from customers	1,156	1,934	7,242	7,775
R&D tax incentive	-	-	5,433	6,271
Chiesi milestone	-	-	13,845	-
Sale of distribution rights	2,342	-	1,365	-
Payments to suppliers, employees etc (net)	(5,443)	(6,300)	(24,813)	(27,330)
<b>Total operations</b>	<b>(1,945)</b>	<b>(4,366)</b>	<b>3,072</b>	<b>(13,284)</b>
Investing (capex & patents)	(40)	(100)	(644)	(574)
Finance lease payments <sup>1</sup>	(593)	(574)	(2,305)	(2,232)
Financing agreement payments <sup>2</sup>	(3)	(68)	(240)	(270)
Share issue - net	-	-	4,065	-
<b>Net increase (decrease) in cash</b>	<b>(\$2,581)</b>	<b>(\$5,108)</b>	<b>\$3,948</b>	<b>(\$16,360)</b>

1. Lease over 20 Rodborough Rd (to May 2024) – total liability at 30 June 2021: \$6.3 million
2. NovaQuest financing – not repayable other than as % of US & EU Bronchitol revenue – up to 7 years



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