



FDA APPROVAL OF BRONCHITOL TRANSFORMS
CASH AND OPERATIONS

BREAKTHROUGH CLINICAL
PROGRAM IN MYELOFIBROSIS

pharmaxis

developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | November 2020
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

Transformative impact of FDA approval on Pharmaxis operations

FDA approves Bronchitol for adult Cystic Fibrosis patients triggering approval and launch **milestones totaling US\$10m**

Access to large US market **transformative** with mannitol business cash flow positive from FY 2021 onwards

Cash position strengthened* and increased sales provide **cash runway to H2 2022** with potential cost savings and further opportunities to extend



Breakthrough clinical program launched for PXS-5505 with **disease modifying potential in Myelofibrosis**. Phase 2 trial funded to conclusion, begins Q1 2021

Global scientific and clinical collaborations to **extend value** of PXS-5505 into other myeloproliferative diseases and cancer indications

Strong pipeline of assets supported by funding and collaborations with industry, government and academia until commercial opportunity is clear

Best in class LOXL2 inhibitor in partnering discussions for entry into phase 2 trials for chronic fibrotic disease

Experienced Scientific Leadership Team

Significant experience in drug development, commercialisation and partnering

In senior management



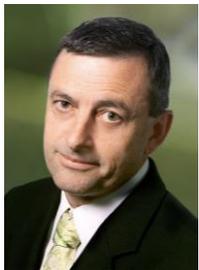
Wolfgang Jarolimek – Drug Discovery

- 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-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

- 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



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

On the board



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



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

FDA approves Bronchitol for Cystic Fibrosis

US Marketing Authorisation granted on 30 October, 2020

FDA approval as “add-on maintenance therapy to improve pulmonary function in adult patients 18 years of age and older with cystic fibrosis”

US CF market >65% of global market

- US market doubles global cystic fibrosis patient opportunity with attractive pricing

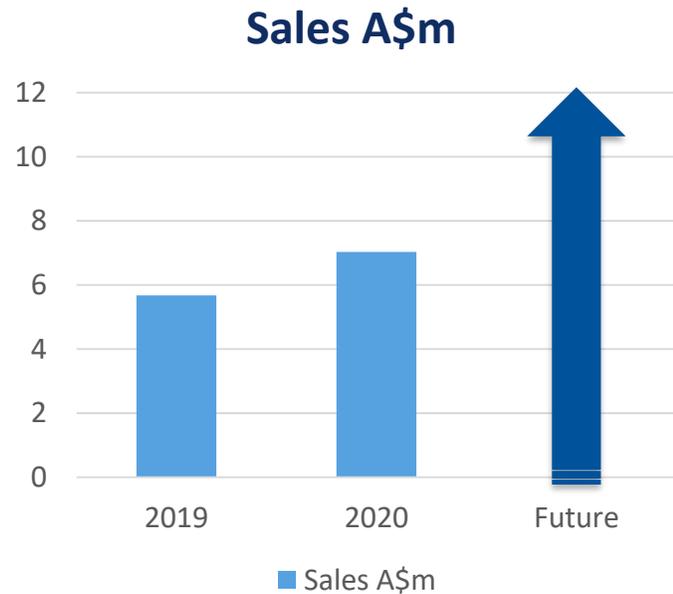


Chiesi US License

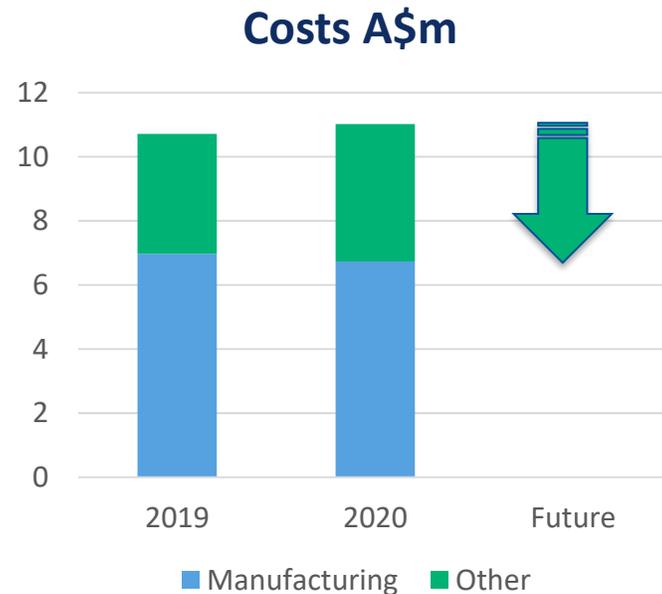
- Chiesi approval /launch milestone payments US\$10m
- US sales commence in H1 CY 2021
- High teens royalty + long term supply contract - ~20% of Chiesi US Bronchitol net sales flow directly to the mannitol business segment EBITDA
- Three sales milestones totaling US\$15m payable on achieving annual sales thresholds

Transformational impact of FDA Bronchitol approval

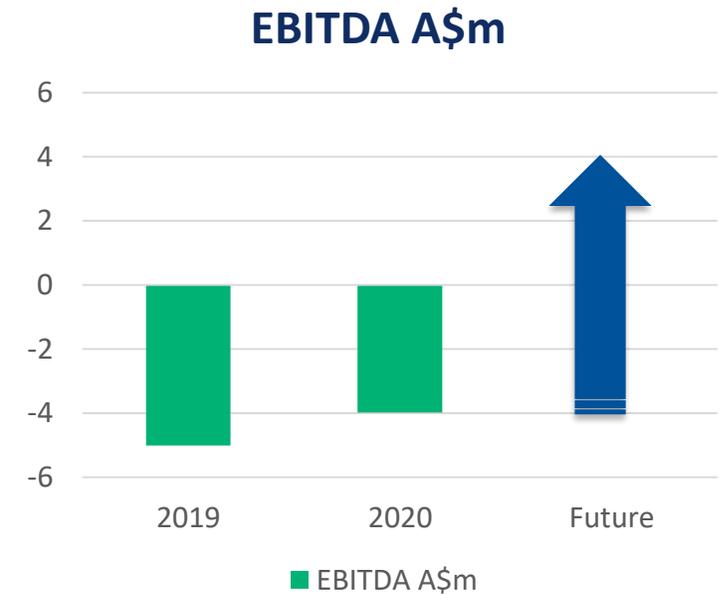
Mannitol business (Bronchitol® and Aridol®) cash flow positive from FY 2021 onwards*



- Mannitol 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
- New markets eg Brazil



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



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

Strengthened cash position

Further opportunities to extend cash runway ahead

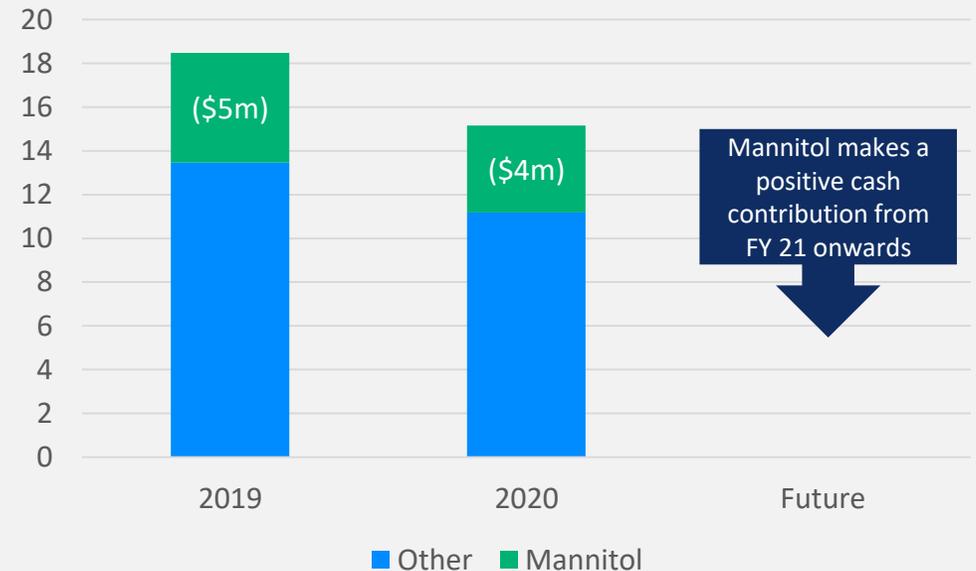
- **June 20 proforma cash balance of A\$34m**
 - Cash June 2020: A\$20m (includes \$5m R&D tax credit received Oct 2020)
 - Chiesi milestone payments ~A\$14m (US approval US\$7m and launch stock US\$3m)

- **Mannitol business to go from cash burn (FY 20: A\$4m) to cash flow positive from FY 21 onwards (FY 26: A\$10m+)**

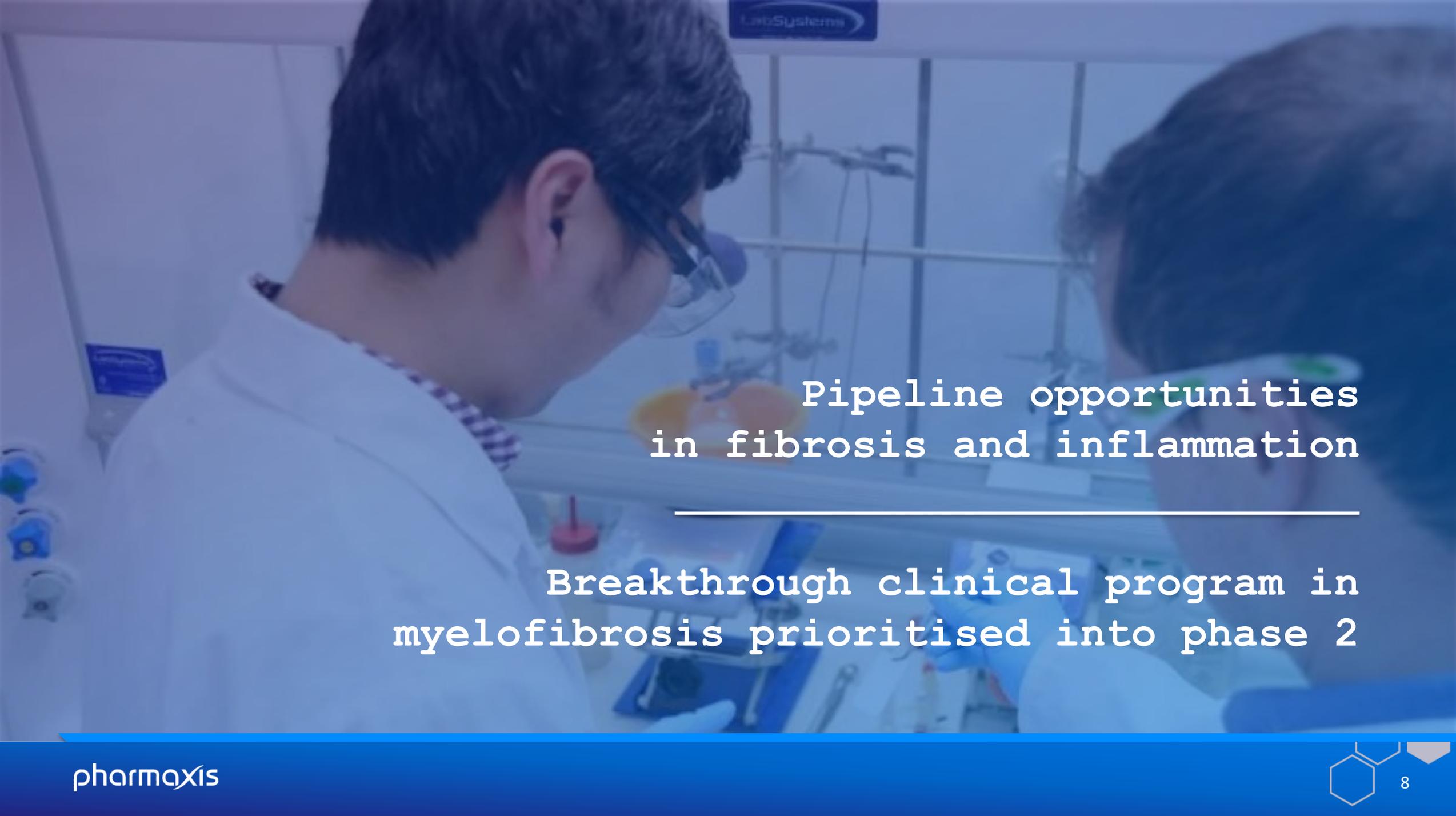
Year	2019	2020	From 2021 E
EBITDA (A\$m)*	(\$5.0)	(\$4.0)	<i>Cash Flow Positive</i>

- **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 (eg LOXL2)

Proforma Cash Usage¹ A\$m



¹ Proforma cash usage is the total of segment EBITDA (mannitol business, new drug discovery and corporate), finance lease payments, capex and financing agreement payments. Refer financial slides for further detail.



Pipeline opportunities
in fibrosis and inflammation

Breakthrough clinical program in
myelofibrosis prioritised into phase 2

First in class PXS-5505 fast tracked into 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
- Fully funded phase 1/2a proof of concept myelofibrosis study starts 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 to explore independent grant funded studies; eg Myeloproliferative disorders, liver carcinoma, pancreatic cancer, etc
- 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

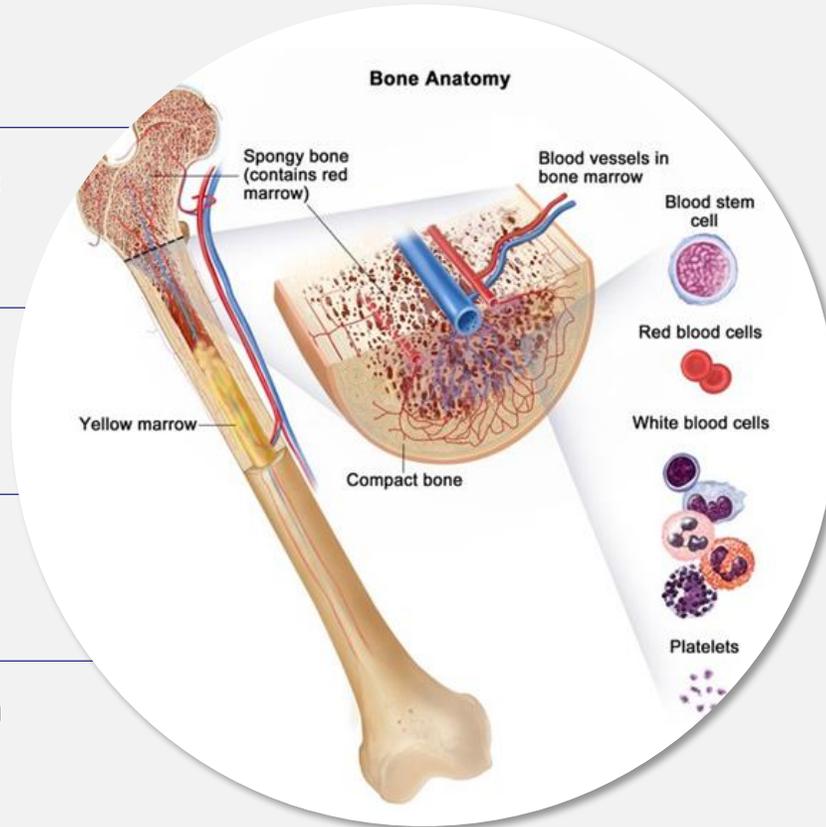
KEY FACTS

Affects 1 in 500,000 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

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

Myelofibrosis – unmet need

“JAK inhibition alone is insufficient for long-term remission and offers modest, if any, disease modifying effects”*

Jakafi / Ruxolitinib – (Incyte / Novartis)

- Tolerability: anemia, thrombocytopenia and neutropenia common.
- Long term discontinuation of 75% of patients after 5 years; adverse events (25%) and loss of response (25%)

Inrebic / Fedratinib – (Celgene/Impact)

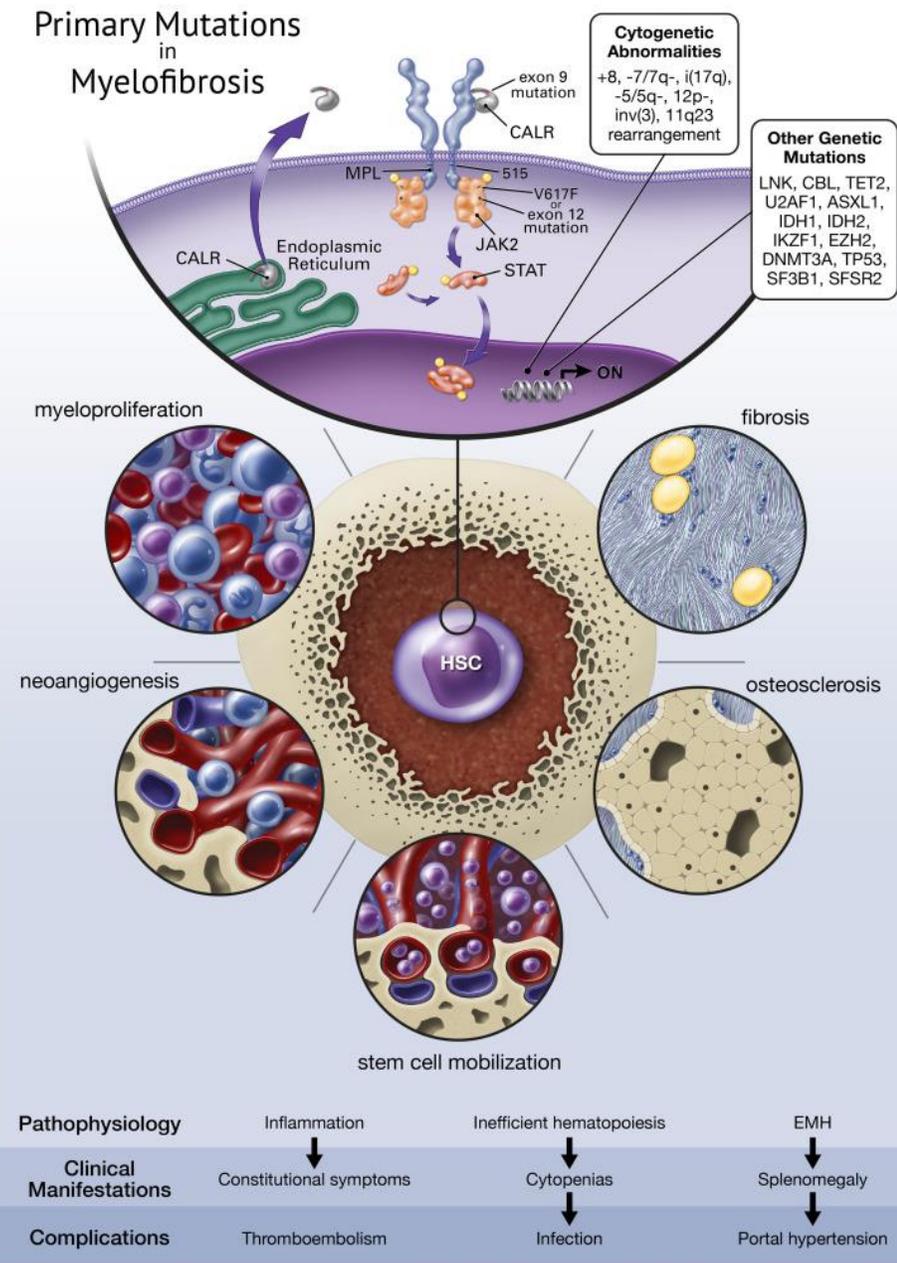
- second line after ruxolitinib failure or first line in ruxolitinib-ineligible
- Anemia and thrombocytopenia common
- Gastrointestinal toxicities very common

In development

- Momelotinib in phase 3 (Sierra/Gilead)
- Pacritinib in phase 2 (CTI BioPharma)

“...there is great interest in identifying mechanisms that cooperate with JAK-STAT signaling to predict disease progression and rationally guide the development of novel therapies”*

Primary Mutations in Myelofibrosis



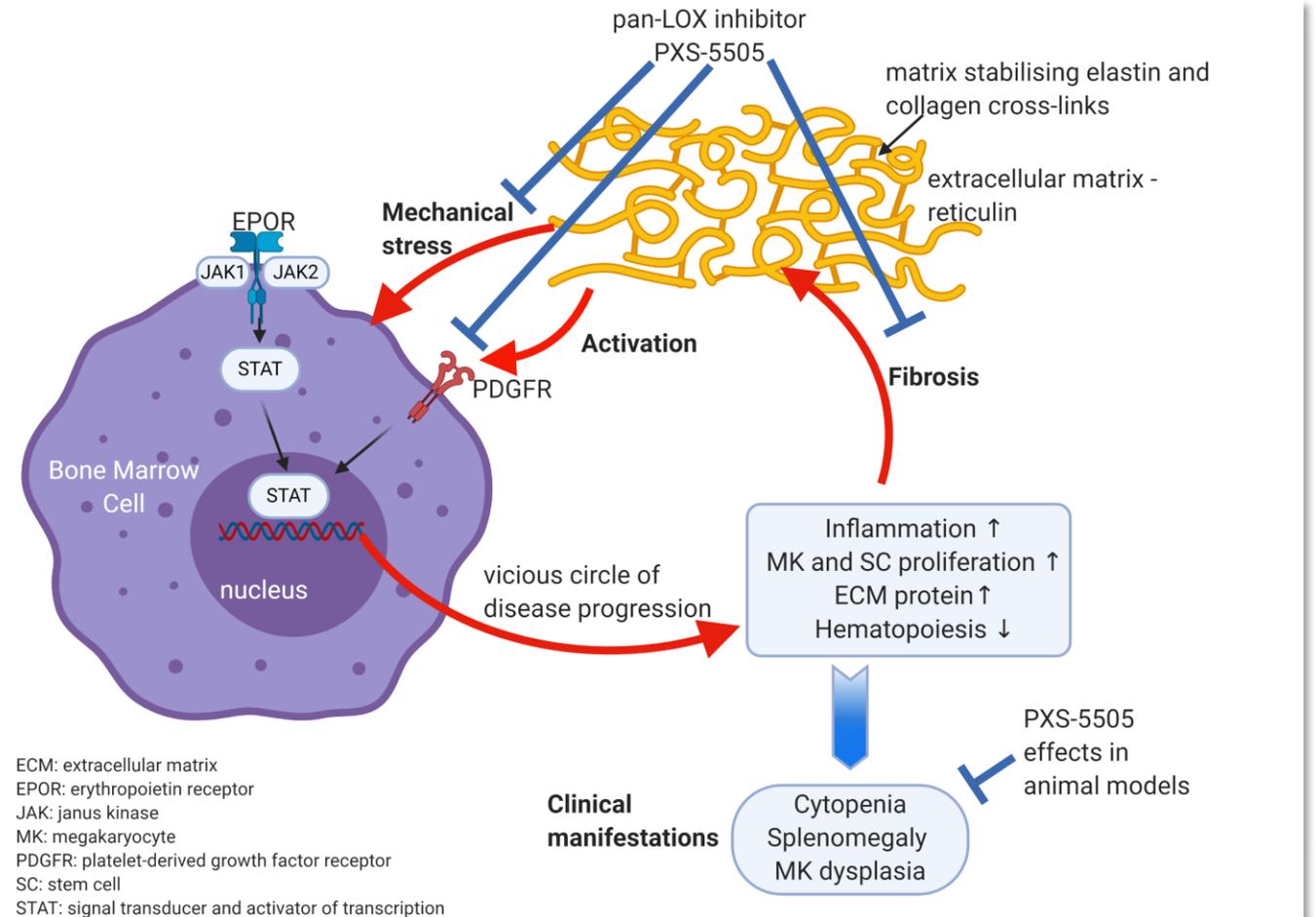
Mode of action in MF

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

- Unique mechanism of action targeting the extracellular matrix
- Disease modifying potential
- Efficacy on top of existing standard of care -
- AND development pipeline drugs

“Specific targeting of ECM dysregulation to prevent and diminish MF may prove the frontline of research and therapy development in PMF with the greatest promise of relieving symptoms and extending life expectancy of patients”

Blood Cancer Journal (2017) 7, e525; doi:10.1038/bcj.2017.6



Myelofibrosis – other programs

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

Company	Market cap ⁽¹⁾	Bourse	Asset	Description	Clinical phase
 Constellation PHARMACEUTICALS	\$1.1bn	Nasdaq	CPI-0610	BET inhibitor	Phase 2 data
 KARTOS THERAPEUTICS	\$0.7bn ⁽²⁾	n.a. – private	KRT-232	MDM2 antagonist	Phase 2
 geron	\$0.5bn	Nasdaq	Imetelstat	Telomerase inhibitor	Phase 2 data
 pharmaxis	\$23.6m (A\$32.9m)	ASX	PXS-5505	Pan-LOX inhibitor	Phase 2 ready

Existing pipeline in development all have challenging safety / side effect profiles

PXS-5505 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

PXS-5505 unique mechanism of action with expected good efficacy AND tolerability

Potential consolidators, licensors & collaborators

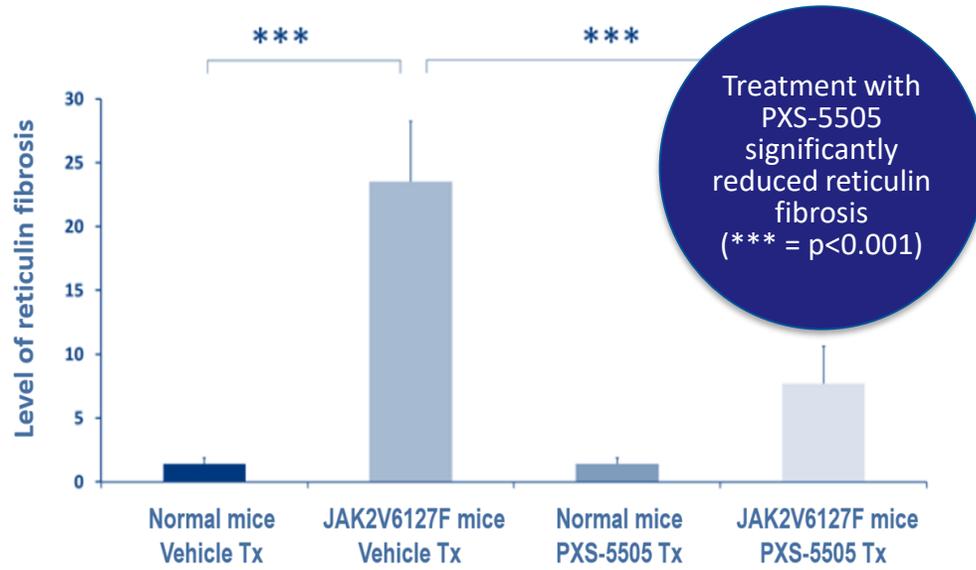
Attractive therapeutic area with multiple consolidators / potential licensors

Company	Bourse	Asset	Description	Clinical phase	Current revenue	Acquired / licensed / in-house
 Promedior	SIX Swiss	Pentraxin-2	Monocyte development inhibitor	Phase 2 complete	n.a.	Roche acquired Promedior's fibrosis portfolio ⁽¹⁾ \$390m upfront + additional contingent payments (up to \$1bn)
 	SIX Swiss	Jakavi	iAP	Marketed (Launched in 2012)	2019: \$1.1bn	Novartis acquired ex-US rights from Incyte Corp \$150m upfront + immediate \$60m development milestone, in addition to future potential milestones and royalties (up to \$1.1bn)
	NYSE	Navitoclax	BCLXL and BCR inhibitor	Phase 2	n.a.	In-house development
 FORBIUS	NYSE	AVID200	TGFb inhibition	Phase 1 complete	n.a.	BMS acquired Forbuis ⁽²⁾ Value not disclosed
  	Nasdaq	Luspatercept	TGFb inhibition	Phase 2	n.a.	Licensing agreement with Celgene (now BMS) Acceleron received upfront payment of \$25m + up to \$217m in development, regulatory and commercial milestones

PXS-5505; Pan-LOX inhibitor with promising profile

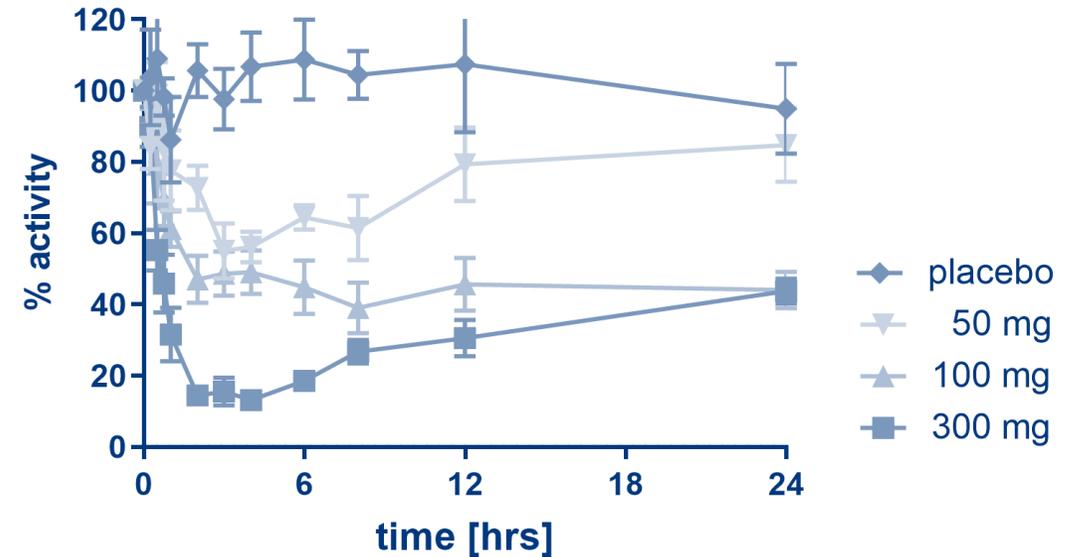
Pre clinical and clinical studies strongly support entry into patient studies

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

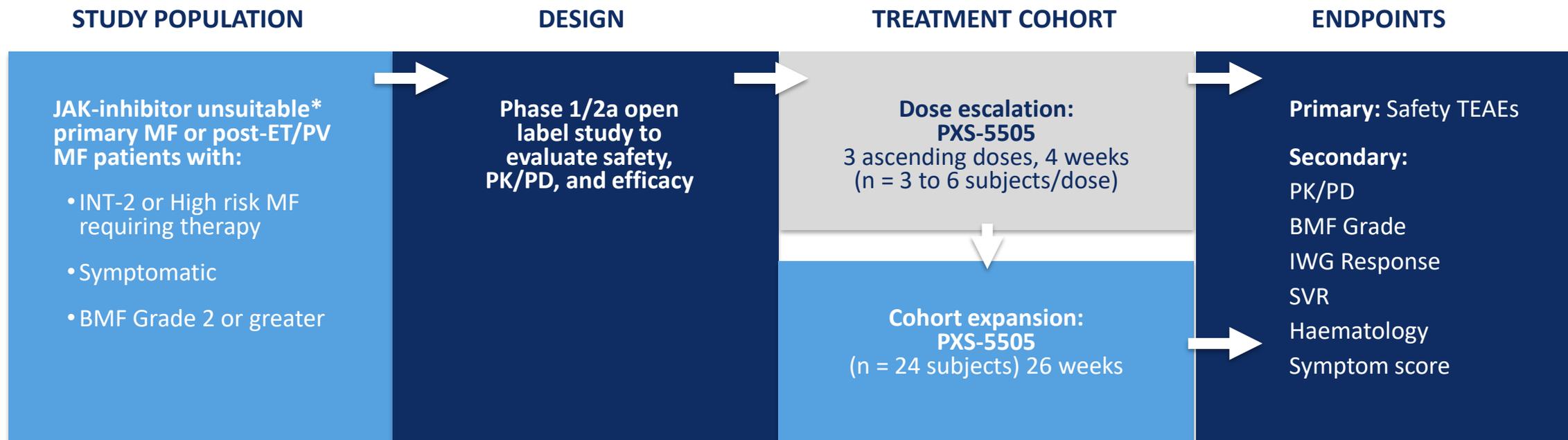
PXS-5505 – Phase 1 SAD



- 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 adverse events 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



FDA granted orphan drug designation July 20 and IND approved August 2020

Multiple sites across 3 countries to enhance trial recruitment (USA, Korea, Australia)

Study budget funded to conclusion (~US\$5m)

Study recruitment commences Q1 2021, study concludes H2 2022

*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 pan LOX: value enhancing indication extensions

LOX enzymes implicated in multiple cancers*

Cancer	LOX	LOXL1	LOXL2	LOXL3	LOXL4	# of patients
Breast invasive carcinoma	■	■	■	■		1,212
Esophageal carcinoma	■	■	■			196
Glioblastoma multiforme			■	■		171
Head and neck squamous cell carcinoma	■	■	■			566
Kidney renal clear carcinoma	■		■		■	606
Kidney renal papillary cell carcinoma		■			■	323
Lung squamous cell carcinoma	■	■	■		■	552
Mesothelioma	■	■	■	■	■	87
Ovarian serous cystadenocarcinoma	■	■		■	■	307
Pancreatic adenocarcinoma	■	■	■	■	■	183
Pheochromocytoma and Paraganglioma			■	■		187
Sarcoma	■	■	■	■		265
Skin cutaneous melanoma	■			■	■	473
Uterine carcinoma	■	■	■	■		57
Uterine corpus endometrial carcinoma		■				201

Numerous cancers have lysyl oxidases upregulated. PXS-5505 of significant interest globally to academics and clinicians

PXS-5505: Significant opportunity in other cancers

Global academic and clinical interest in LOX inhibition drives development plan

Pharmaxis Research Collaborations

Other Myeloproliferative Disorders; e.g.

Myelodysplastic syndrome

Germany

Liver Cancer

Rochester (NY)

Pancreatic Cancer

Sydney, Rochester (NY)

Melanoma and glioblastoma

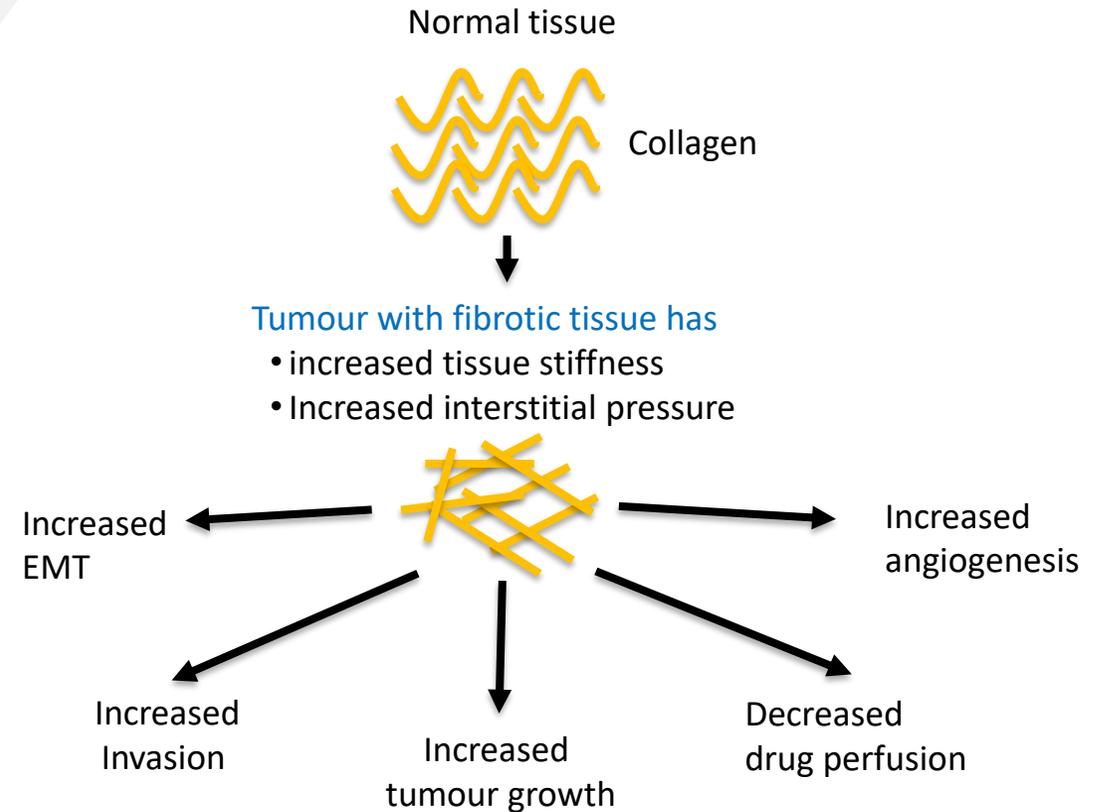
Houston

Head and Neck Cancer

Boston

Mesothelioma

Dublin



Multiple benefits from anti-fibrotic mechanism of action

Pipeline opportunities in fibrosis and inflammation

Funding of PXS-5505 prioritised

Product Candidate	Indications	Pre-clinical	Phase 1	Phase 2	Next Steps
Pan-LOX; PXS-5505	Myelofibrosis	MF-101			<ul style="list-style-type: none"> Phase 2 commencing Q1 2021
Pan-LOX; PXS-5505	Liver and pancreatic cancer				<ul style="list-style-type: none"> Protocol and funding discussions with independent investigators
LOXL-2; PXS-5382	Anti fibrotic CKD / IPF / NASH				<ul style="list-style-type: none"> Partnering discussions for phase 2 commencement
Pan-LOX; PXS-6302	Anti scarring; Burns, established scars				<ul style="list-style-type: none"> Phase 1 IIS* commencing Q4 2020 IIS patient studies in burns and established scars 2H 2021
SSAO/MAOB; PXS-4699	Anti inflammatory Muscular Dystrophy				<ul style="list-style-type: none"> \$1m matched funding grant DMD TACT committee Q2 2021 Advance to the clinic H1 2022
SSAO/MPO; PXS-5370	Anti inflammatory Multiple indications				<ul style="list-style-type: none"> Grant identification in process
SSAO; PXS-4728A	Anti inflammatory Neuro inflammation				<ul style="list-style-type: none"> Evaluate Boehringer data package and opportunity to repurpose

Anticipated news flow: 2020 – 2021

Transformative impact of FDA approval on Pharmaxis operations

Q4 2020

- FDA approval for Bronchitol to treat adult cystic fibrosis patients granted October 30, 2020
- Chiesi US\$7m milestone due November 2021

H1 2021

- Breakthrough drug PXS-5505 phase 1c/2a myelofibrosis study commences recruitment
- Chiesi pay US\$3m milestone on Pharmaxis shipment of US launch
- Cash receipts from sale of US Bronchitol launch stock
- Mannitol business simplification completed – realising annual cost savings
- Best in Class LOXL2 inhibitor partnering

H2 2021

- First collaborations to progress PXS-5505 into clinical trials in other myeloproliferative diseases and/or cancer indications
- Cash receipts from royalties on US Bronchitol sales commence
- LOX topical drug enters independent investigator patient studies
- Feedback from global advisory committee on development fast tracking for Duchenne muscular dystrophy clinical trials.



Appendices

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



Wolfgang Jarolimek – Drug Discovery

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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-Planck 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



Gary Phillips – Chief Executive Officer

- 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



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 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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- 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

Financials

Cash

Financial years ended 30 June (A\$'000)	2019	2020	Notes
Proforma cash 30 June			
Cash 30 June	31,124	14,764	
R&D tax credit	5,962	5,048	Received \$5,048 on 14 October 2020
Chiesi milestone payments	-	~14,000	US\$7m on approval (Q4 2020); US\$3m supply launch stock (Q1 21)
	\$37,086	~\$33,812	

Cash Flow Statement Highlights

Operations	(19,798)	(13,284)	2019 R&D tax credit received 2020
Investing (capex)	(981)	(574)	PP&E and patents
Finance lease payments ¹	(1,593)	(2,232)	Frenchs Forest facility lease liability
Financing agreement payments ²	(254)	(270)	Novaquest obligation - mid single digit % of Chiesi US sales for 7 years from launch
Share issue - net	22,677	-	
Net increase (decrease) in cash	\$51	(\$16,360)	

1. Lease over 20 Rodborough Rd (to 2024) – total liability at 30 Jun 2020: \$8.2 million
2. NovaQuest financing – not repayable other than as % of US & EU Bronchitol revenue – up to 7 years

Financials

Income statement highlights

Financial years ended 30 June (A\$'000)	2019	2020	Outlook
Segment Financials			
New drug development			
Oral Pan-LOX (external costs)	(3,833)	(3,124)	Phase 1c/2a costs ~US\$5m to Q4 2022
Other programs (external costs)	(5,108)	(2,380)	Limited spend; grant support
Employee & overhead - net of R&D tax credit (43.5%)	(2,138)	(2,694)	
R&D tax incentive – external costs	4,316	3,085	
	(\$6,764)	(\$5,113)	
Mannitol business			
Sales	5,703	7,047	Sales growth from US and other markets
Expenses	(10,716)	(11,024)	Potential cost savings. Manufacturing costs relatively fixed vs increased sales
	(\$5,013)	(\$3,977)	
Corporate	(\$3,874)	(\$2,990)	
Total Adjusted EBITDA	(\$15,651)	(\$12,080)	
Net Loss	(\$20,058)	(\$13,943)	

Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap ¹	A\$35m
Shares on Issue	397m
Employee Options	19m
Liquidity (turnover last 12 months) ¹	171m shares
Share price ¹	A\$0.088
Proforma cash balance (30 June 2020)	A\$34m

Institutional Ownership	30 Oct 20
BVF Partners (US)	20%
Arix Bioscience (UK)	8%
D&A Income Limited	7%
Other Institutions	9%
Total Institutional Ownership	44%



pharmaxis

developing breakthrough treatments for fibrosis and inflammation

www.pharmaxis.com.au



Contacts

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