Investor Presentation

pharmaxis

developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | 4 June 2021

Gary Phillips CEO

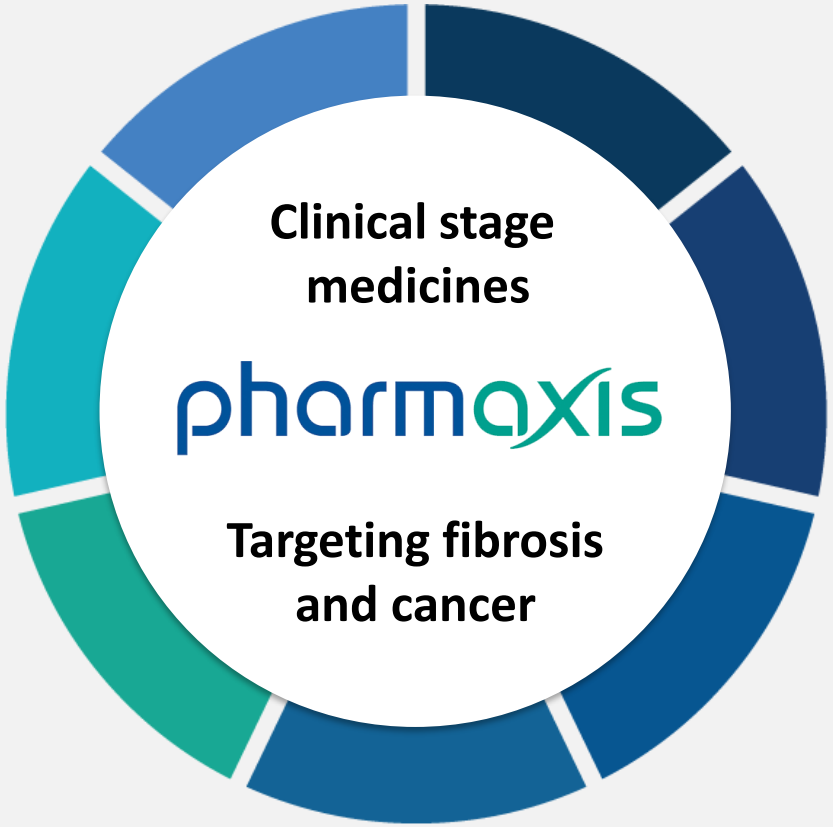
Forward looking statement

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
- Lead asset PXS-5505 is in phase 1c /2a trial – a breakthrough clinical program with disease modifying potential in Myelofibrosis
- PXS-5505 has further potential in oncology as an adjunct to standard of care
- Additional asset PXS-6302 is an anti-skin scarring drug in phase 1a/1c trial in 2021 – PXS-6302 to enter patient studies in commercially important dermatology indications with potential to improve function and appearance
- Specific corporate strategy to deliver non-dilutive cash and cost savings from other parts of our business
  - Distribution license fees from currently un-partnered mannitol territories
  - Simplification and rationalisation across business
- Post capital raising (April 2021) Pharmaxis is in a strong position to fund its focused clinical program



Cash and capital structure

Extended cash runway

Cash

- Cash at March A$16m
- Proceeds of placement A$4m
- Proforma cash balance as at March A$20m

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

Sale of Russian Bronchitol distribution rights effective 1 May

- €1.25m (~A$2m)** – 70% paid on signing, 30% in Q2 2021
- Cost reductions of ~A$1m per annum from 1 July 2021

Further opportunities to extend cash runway

- Potential cost savings from rationalization across business
- Distribution license fees from currently un-partnered Aridol and Bronchitol territories
- Pipeline supported by grants and R&D tax credit (~A$5m 2020)
- Partnering deals with pipeline assets

Share capital

- Current shares on issue 452m

Enterprise value

- Market capitalisation at $0.08 per share $36m
- Less: proforma net cash at March ($20m)
- Enterprise value $16m

Lead institutional shareholders

- BVF Partners LP 19.5%
- Karst Peak Capital Limited 8.9%

*Mannitol segment EBITDA only

**not included in proforma cash balance as at March

Experienced Scientific Leadership Team

Significant global experience in drug development, commercialisation and partnering

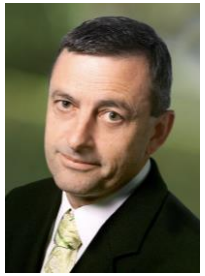
In senior management

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

Dieter Hamprecht – Head of Chemistry

- more than 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

- 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

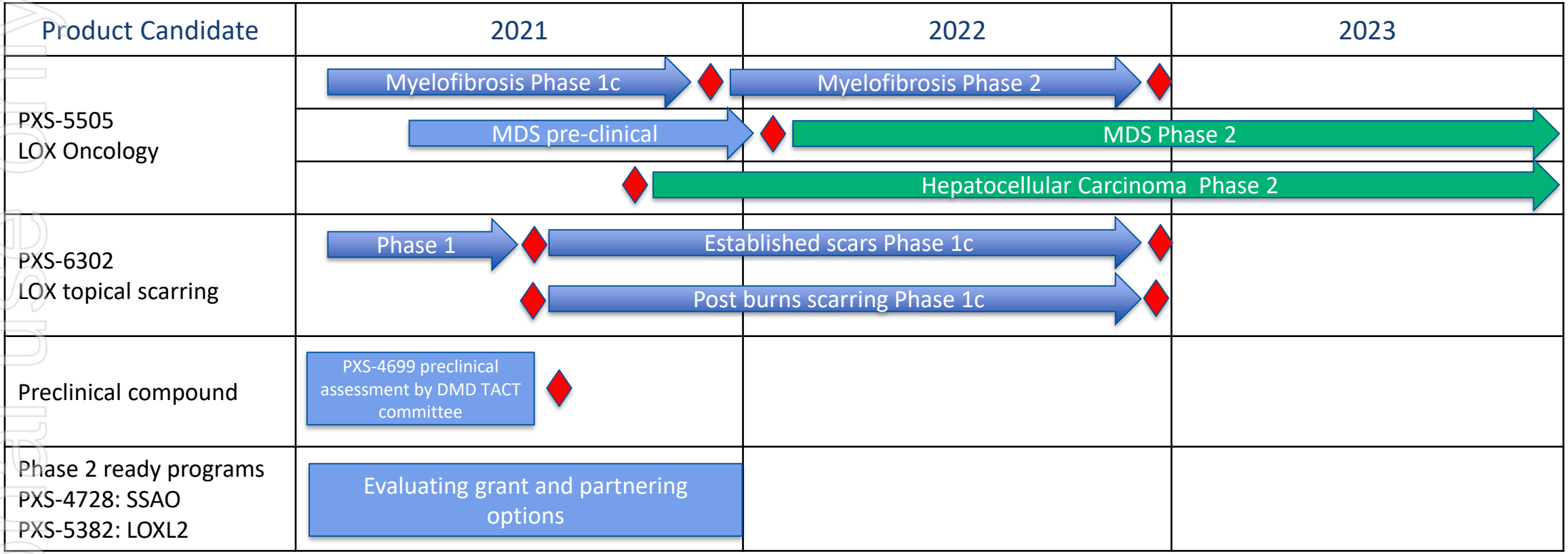
Neil Graham – Non Executive Director

- 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

Multiple potential value inflection points over next two years

Pipeline creates multiple opportunities

Target timelines



DMD: Duchenne Muscular Dystrophy
TACT: TREAT-NMD Advisory Committee for Therapeutics

MDS: Myelodysplastic Syndrome
LOX: Lysyl Oxidases

Myelofibrosis background

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

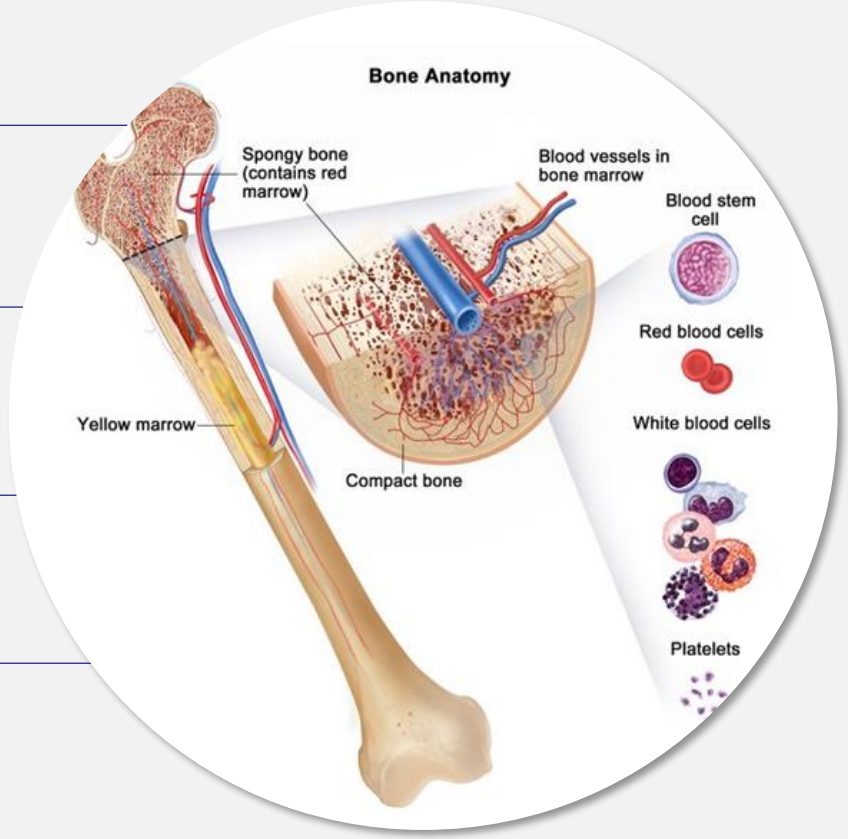
KEY FACTS

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:

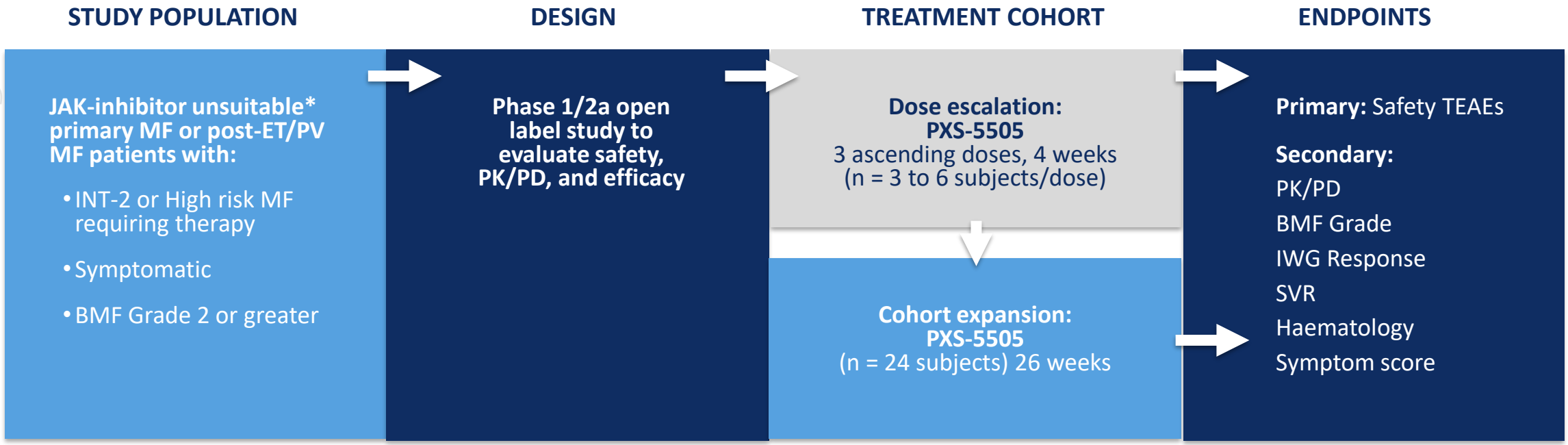
- Driven by clonal mutations of a hematopoietic stem cell (JAK, MPL, CALR genes)
- Reduced red blood cells can cause extreme tiredness (fatigue) or shortness of breath
- Reduced white blood cells can lead to an increased number of infections
- Reduced platelets can promote bleeding and/or bruising
-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

Standard of Care; JAK inhibition

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

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 (~US$5m)

Study recruitment commenced Q1 2021, study targeted to conclude 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

Myelofibrosis – examples of other programs

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

Company	Market cap[1]	Bourse	Asset	Description	Clinical phase
KEROS THERAPEUTICS	$1.2bn	Nasdaq	KER-050	TGF-β ligand trap	Phase 2
Constellation PHARMACEUTICALS	$1.1bn	Nasdaq	CPI-0610	BET inhibitor	Phase 3
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geron	$0.5bn	Nasdaq	Imetelstat	Telomerase inhibitor	Phase 3
pharmaxis	$24m (A$31m)	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

Notes: (1) Approximate market cap as at 22 March 2021
(2) Last round valuation

Hypertrophic and keloid scarring

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

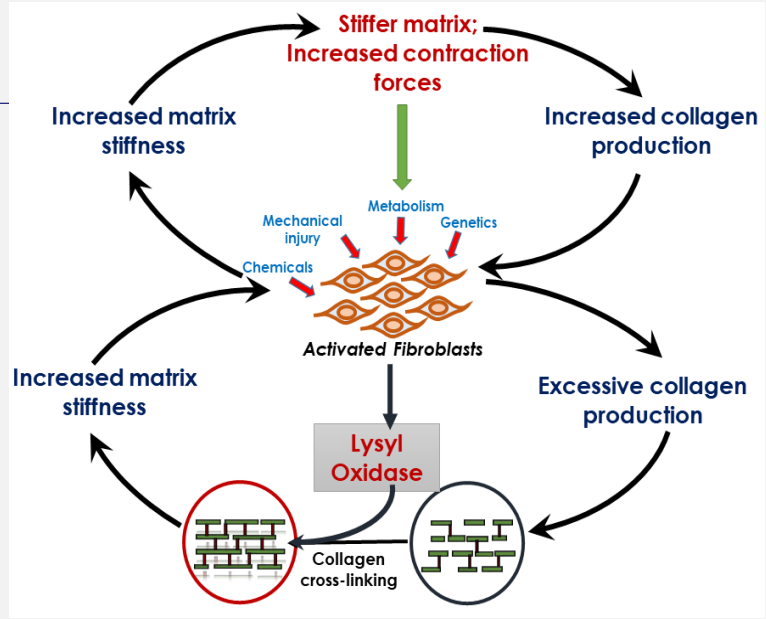
KEY FACTS

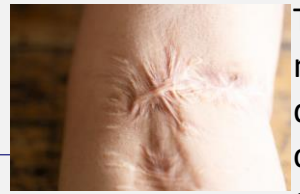
100m patients develop scars in the developed world alone each year as a result of elective operations and operations after trauma

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

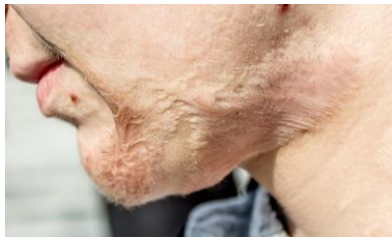
Collagen turnover in keloid





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; prophylactic and treatment strategies remain unsatisfactory
- Current standard of care includes:
  - Corticosteroids
  - Surgical revision
  - Cryotherapy
  - Laser therapy
  - 5-fluorouracil



- Pre clinical evidence
  - Unpublished data from Pharmaxis scientific collaboration demonstrating strong proof of concept
  - Treatment with PXS-6302 monotherapy 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 include those with established scars and those with scarring subsequent to burn injury

Anticipated news flow: 2021 – 2022

Multiple anticipated value inflection points over next two years

H1 2021

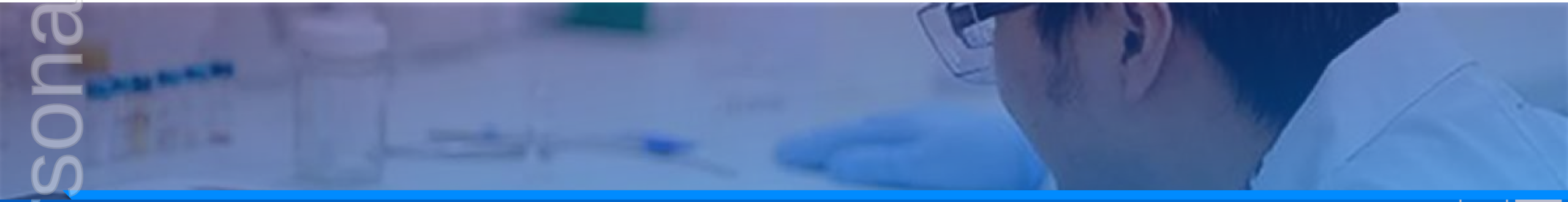
- Feb 22: Breakthrough drug PXS-5505 phase 1c/2a myelofibrosis study commenced recruitment
- Mar 19: Chiesi pays US$3m milestone on Pharmaxis shipment of US launch
- Mar 31: LOX topical drug PXS-6302 commenced independent investigator studies - safety
- April 14: Sale of Russian Bronchitol distribution rights
- May 3: Grant from Charlie Teo Foundation to test PXS-5505 in glioblastoma
- Mannitol business simplification – realising annual cost savings

H2 2021

- PXS-5505 phase 2a myelofibrosis study dose expansion stage commence
- First collaborations to progress PXS-5505 into clinical trials in other cancer indications
- Ongoing cash receipts from supply of Bronchitol for US sales
- LOX topical drug PXS-6302 progresses into independent investigator patient studies - burns and established scars
- Feedback from global advisory committee (TACT) on development fast tracking for Duchenne muscular dystrophy clinical trials.

CY 2022

- PXS-5505 phase 2a myelofibrosis study safety and efficacy data
- LOX topical drug phase 1c studies burns and established scars safety and efficacy data



pharmaxis

developing breakthrough treatments for fibrosis and inflammation

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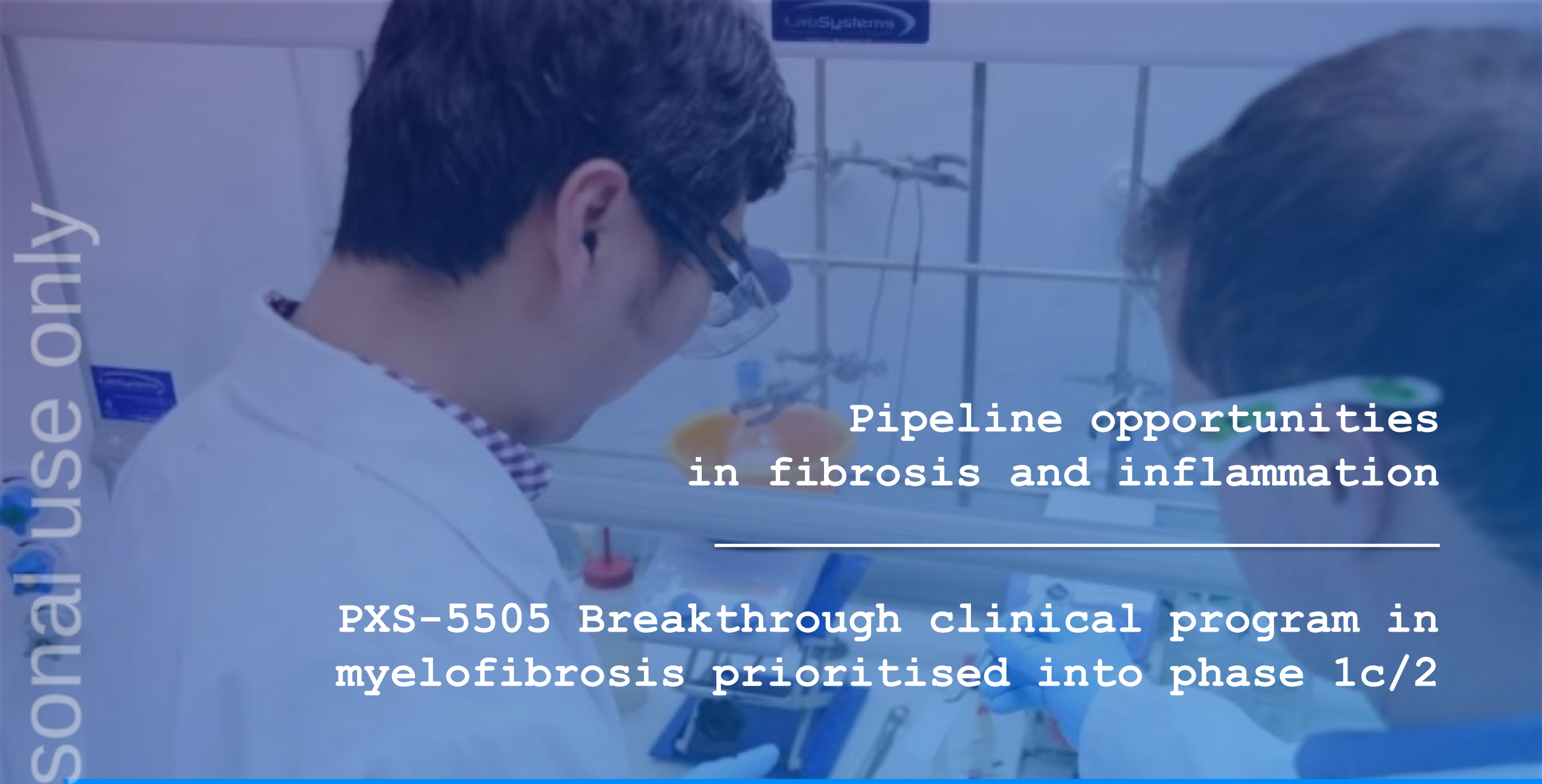
Additional Information

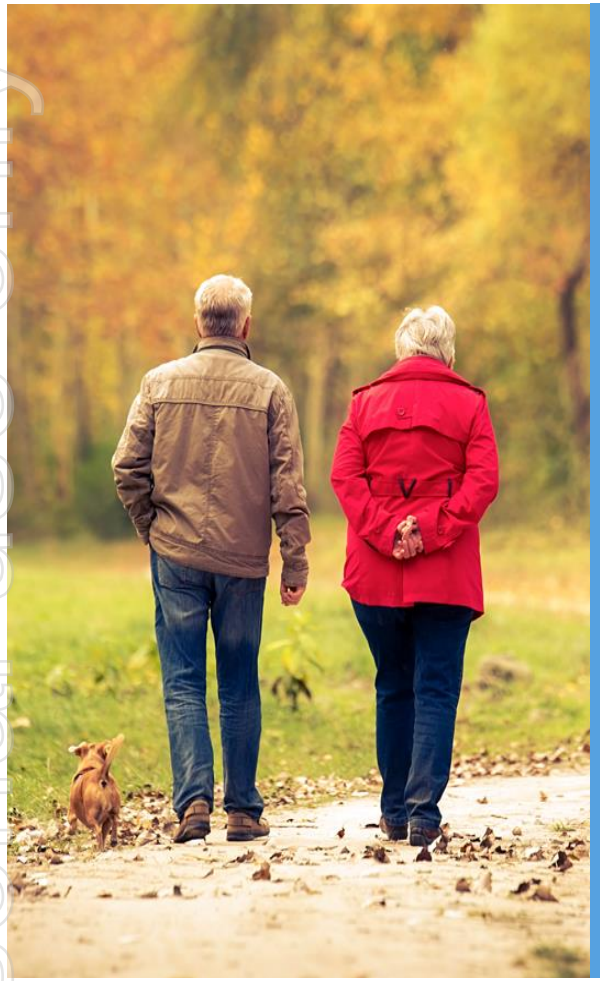


Pipeline opportunities in fibrosis and inflammation

PXS-5505 Breakthrough clinical program in myelofibrosis prioritised into phase 1c/2

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 patent application filed 2018
- Six month 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

- LOX inhibition synergistic with current standard of care and potentially pharma development pipeline in many stromal cancers
- Academic and clinical interest in additional indications including;
  - Myelodysplastic syndrome (MDS) ; liver carcinoma (Hepatocellular carcinoma); pancreatic cancer; 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

KEY FACTS

Affects 15 in 1m people worldwide

5 Years Median survival

Age of onset 50 – 80

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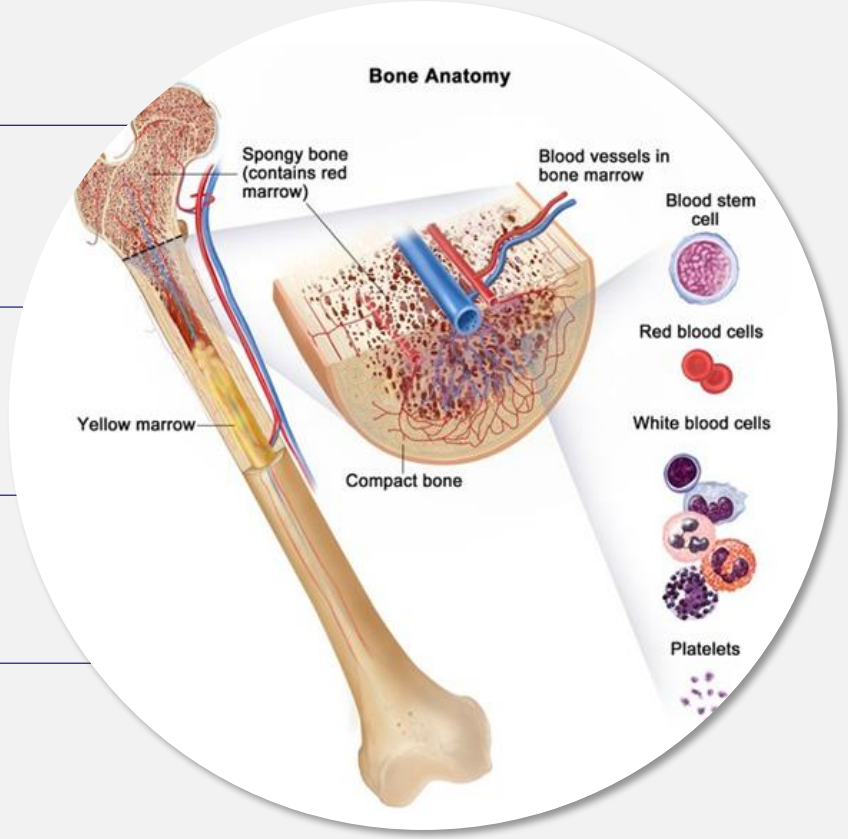

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Standard of Care; JAK inhibition

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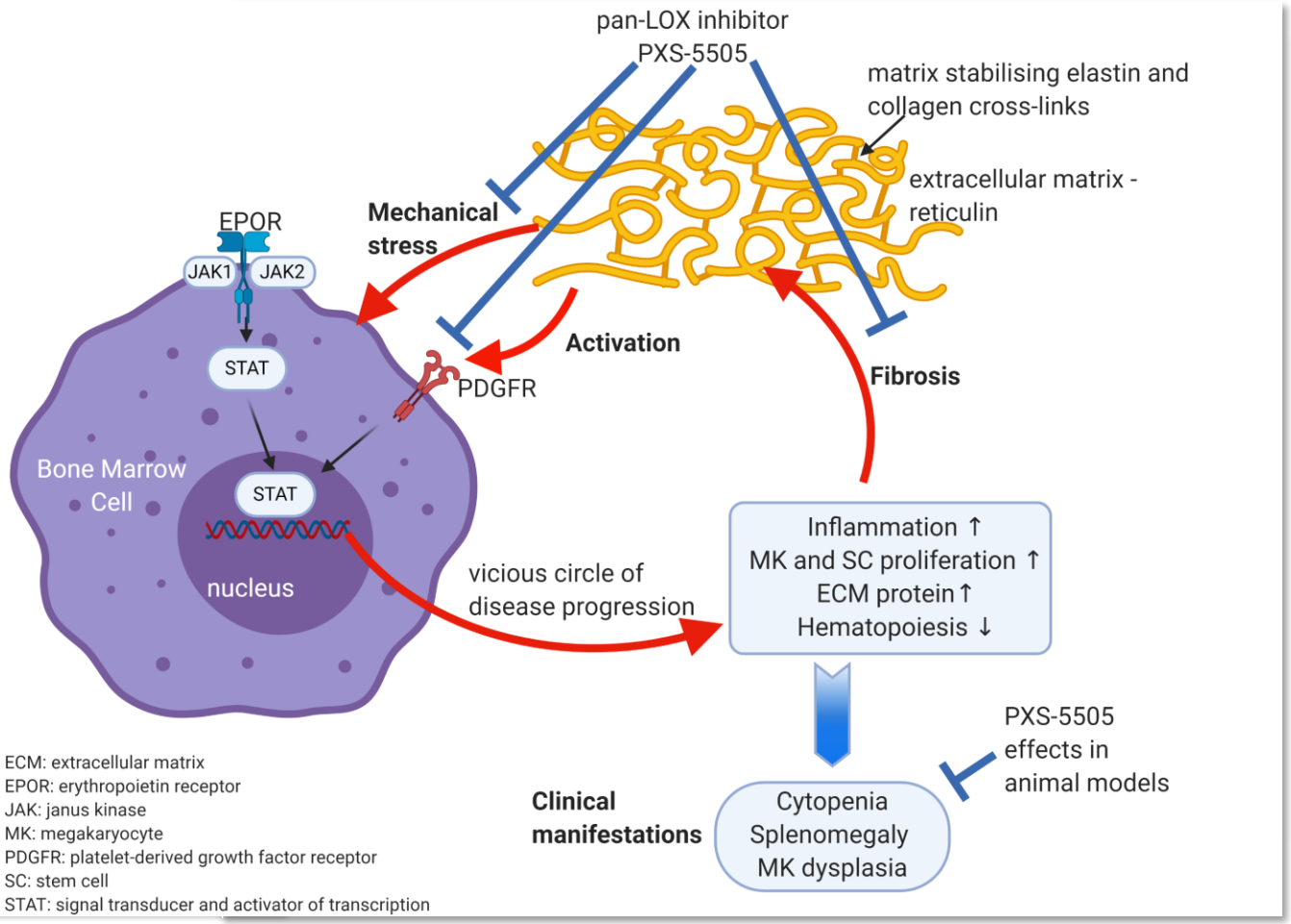
Mode of action in myelofibrosis

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

- Unique mechanism of action targeting the extracellular matrix
- Disease modifying potential
- Designed to provided efficacy on top of existing standard of care
- AND potentially 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"

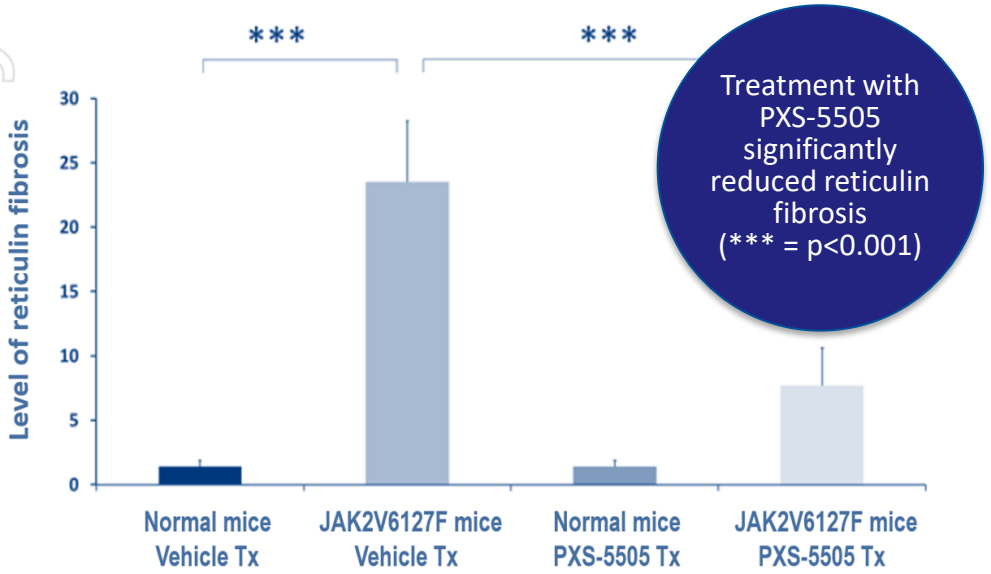
Blood Cancer Journal (2017) 7, e525; doi:10.1038/bcj.2017.6



PXS-5505; LOX inhibitor with promising profile

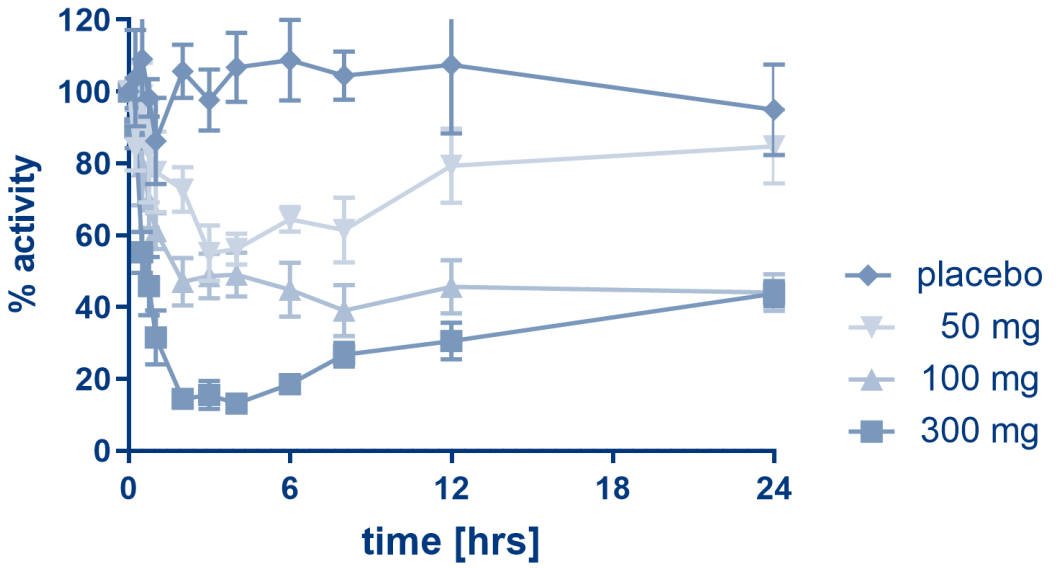
Pre clinical and clinical studies strongly support entry into patient studies

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



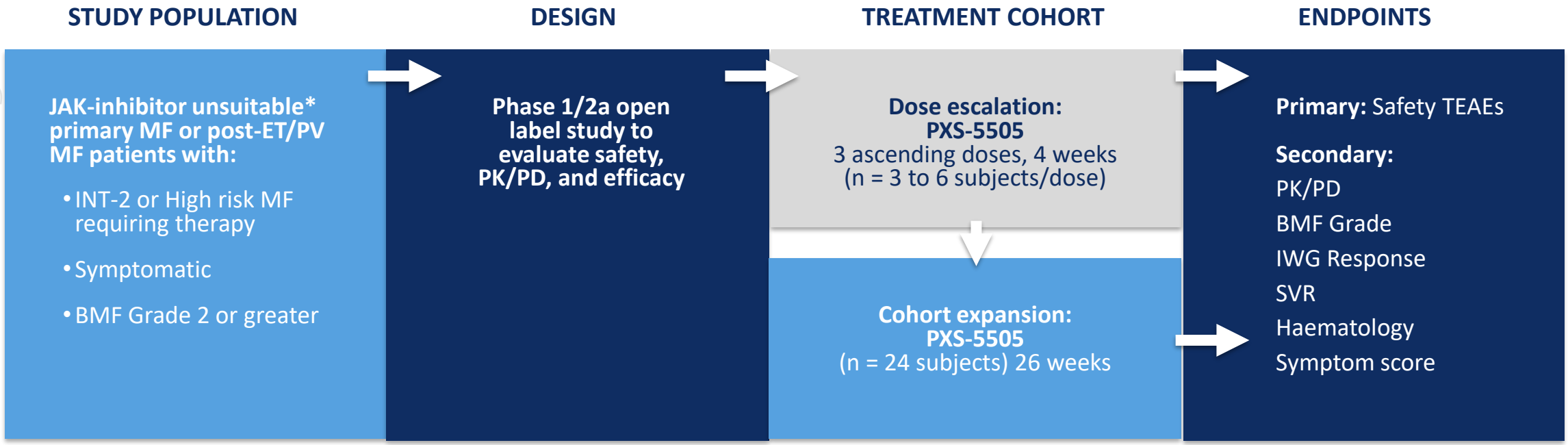
- Good safety profile with 6 month tox studies complete
- Dose dependant 24 hour inhibition of LOX enzymes from single once a day dose in humans
- No safety signal seen in phase 1 trials
- 2018 patent application filing date

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

[1]Dr Gabriela Hobbs, Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital

PXS-5505 Phase 1/2a Trial in myelofibrosis

6 month monotherapy study with meaningful safety and efficacy endpoints



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Myelofibrosis – examples of other programs

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Pipeline opportunities in fibrosis and inflammation

PXS-5505 Significant opportunity in other cancers;

- Myelodysplastic Syndrome
- Hepatocellular Carcinoma

PXS-5505: Significant opportunity in other cancers

Global academic and clinical interest in LOX inhibition drives development plan

Pharmaxis Research Collaborations

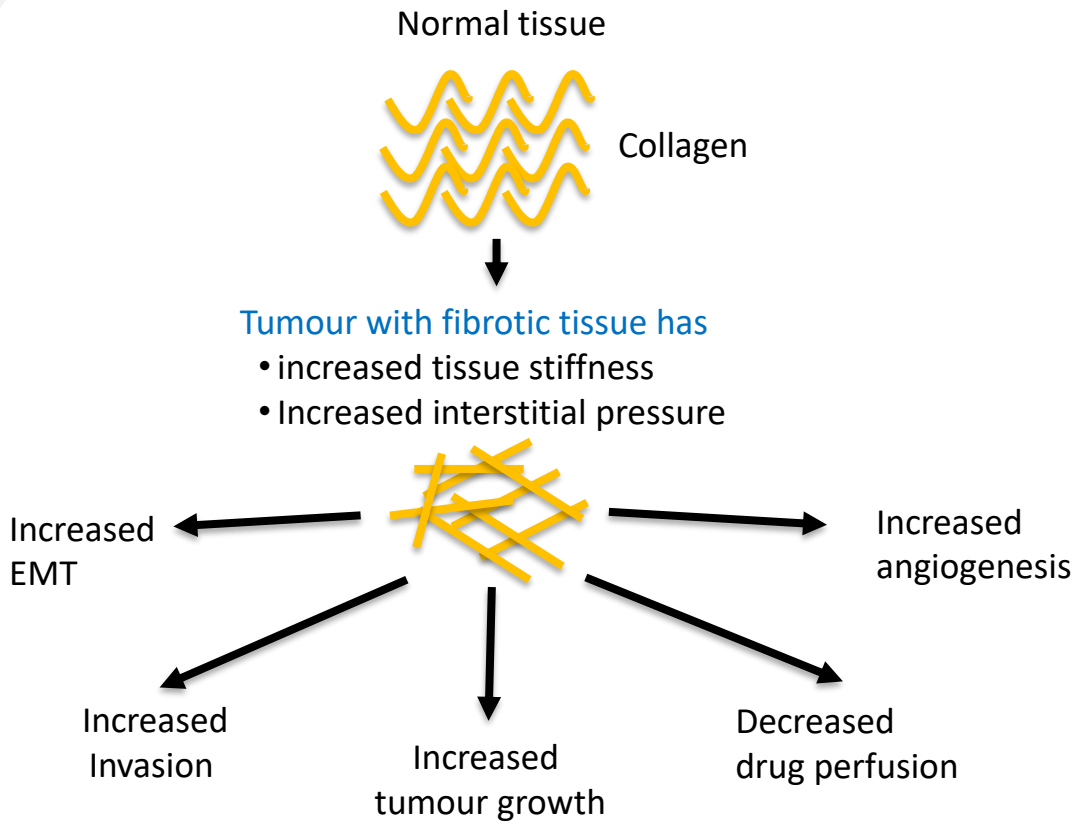
Myelodysplastic syndrome
Germany

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

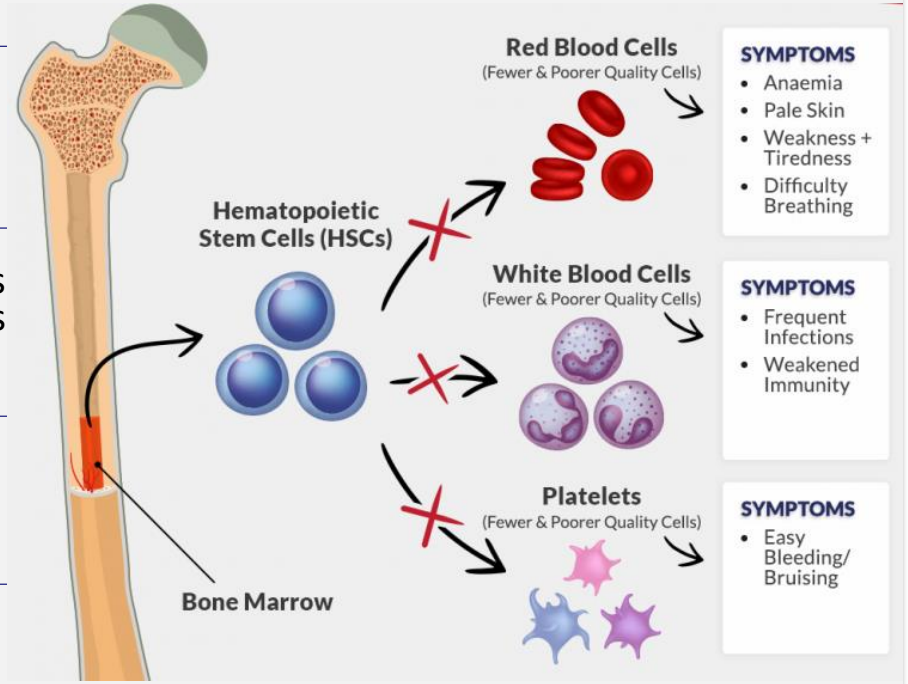
EMT: epithelial to mesenchymal transition

Myelodysplastic Syndrome (MDS)

A group of bone marrow cancers that disrupt normal production of blood cells

KEY FACTS

- Affects ~40 in 100,000 people > 70 years
- 50% to 60% of the patients will die from complications of the disease
- New US diagnoses per annum ~50,000
- 30% transformation to Acute Myeloid Leukemia

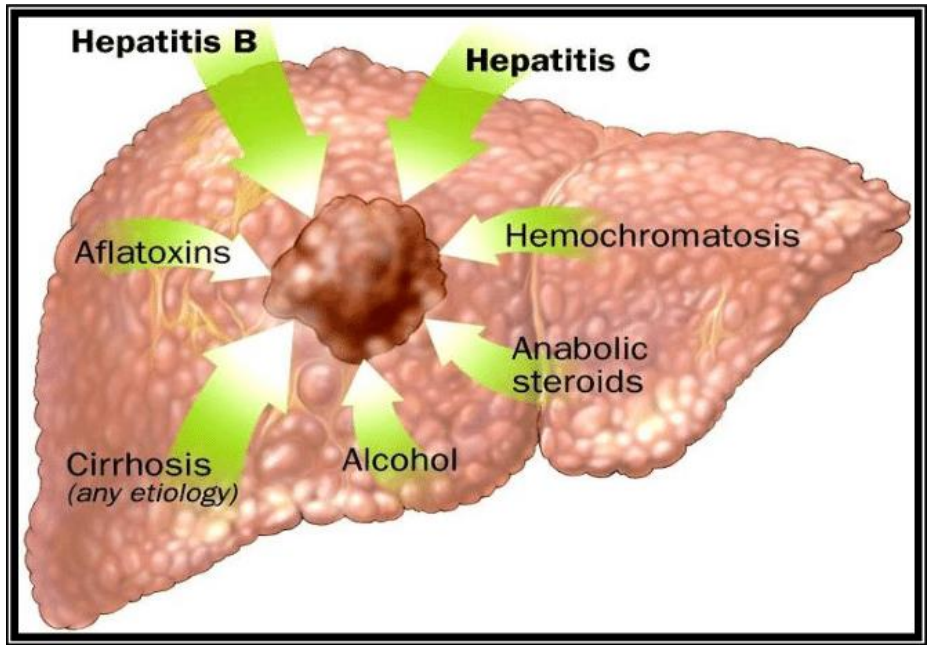


- A group of malignant hematopoietic neoplasms characterized by Bone marrow failure with resultant cytopenia and related complications
- Current standard of care
  - Allogeneic stem cell transplantation
  - Immunomodulatory drug lenalidomide,
  - Advanced disease: DNA hypomethylating agents (HMA), azacitidine (AZA), and decitabine
- Pre clinical evidence
  - Unpublished data from Pharmaxis scientific collaboration demonstrating strong proof of concept
- Proposed clinical strategy
  - Build on myelofibrosis strategy in hematological diseases
  - 6 month proof of concept study on top of standard of care

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 cross-links 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
- 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

Further non core pipeline opportunities in fibrosis and inflammation

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

Product Candidate	Indications	Pre-clinical / Phase 1 / Phase 2	Next Steps
SSAO; PXS-4728	Repurposing for neuro inflammatory disease		• Partnering discussions; phase 2 protocol and funding discussions with independent investigators
LOXL2; 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 • Explore funding opportunities to advance to the clinic H1 2022
SSAO/MPO; PXS-5370	Anti inflammatory Multiple indications		• Investigating funding opportunities including grants

Mannitol respiratory business

Mannitol respiratory business (Bronchitol® and Aridol®)

Transformational impact of FDA Bronchitol approval (Oct 2020) – business segment 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

Segment EBITDA

- Forecast positive EBITDA from FY 2021 onwards (before potential cost savings).
- 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
- 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

Corporate

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
- former CEO of biopharmaceutical company Lycera Corp

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

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)	March 2021 Nine months	March 2020 Nine months	June 2020 FY	June 2019 FY
Proforma cash				
Cash period end	16,165	20,251	14,764	31,124
R&D tax credit	-	-	5,048	6,221
Placement – net proceeds	~4,100	-	-	-
	~$20,265	**$20,251**	**~$19,812**	**$37,345**
Cash Flow Statement Highlights				
Operations				
Receipts from customers	4,786	5,624	7,775	6,893
R&D tax incentive	5,247	6,221	6,271	-
Chiesi milestone	13,844	-	-	-
Payments to suppliers, employees etc	(20,125)	(20,400)	(27,330)	(26,691)
Total operations	3,752	(8,555)	(13,284)	(19,798)
Investing (capex)	(433)	(458)	(574)	(981)
Finance lease payments[1]	(1,721)	(1,667)	(2,232)	(1,593)
Financing agreement payments[2]	(197)	(193)	(270)	(254)
Share issue - net	-	-	-	22,677
Net increase (decrease) in cash	**$1,401**	**($10,873)**	**($16,360)**	**$51**

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

Financials

Income statement highlights

Periods ended (A$'000)	March 2021 Nine months	March 2020 Nine months	June 2020 FY	June 2019 FY
Segment Financials				
New drug development				
Oral LOX (external costs)	(1,905)	(2,348)	(3,124)	(3,833)
Other program external costs (net of grants)	(1,254)	(2,212)	(3,315)	(5,108)
Employee costs	(2,540)	(2,472)	(3,373)	(2,837)
Overhead	(252)	(351)	(460)	(606)
R&D tax credit	148	259	5,159	5,962
EBITDA	(5,803)	(7,124)	(5,113)	(6,764)
Mannitol respiratory business				
Sales	4,810	4,346	7,027	5,676
Other revenue and income	13,997	15	20	27
	18,807	4,361	7,047	5,703
Expenses – employee costs	(4,182)	(4,553)	(5,855)	(6,083)
Expenses – manufacturing purchases	(1,589)	(825)	(1,456)	(1,689)
Expenses – other	(3,353)	(2,716)	(3,713)	(2,944)
EBITDA	9,683	(3,733)	(3,977)	(5,013)
Corporate – EBITDA	(2,801)	(2,283)	(2,990)	(3,874)
Total Adjusted EBITDA	**$1,079**	**($13,140)**	**($12,080)**	**($15,651)**
Net profit (loss)	**($905)**	**($19,858)**	**($13,943)**	**($20,058)**

pharmaxis

developing breakthrough treatments for fibrosis and inflammation

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