



# Investor Presentation

Gary Phillips, CEO

October 2023

# 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 evolves to Syntara<sup>1</sup>: Cost savings and clear focus energise Syntara clinical programs

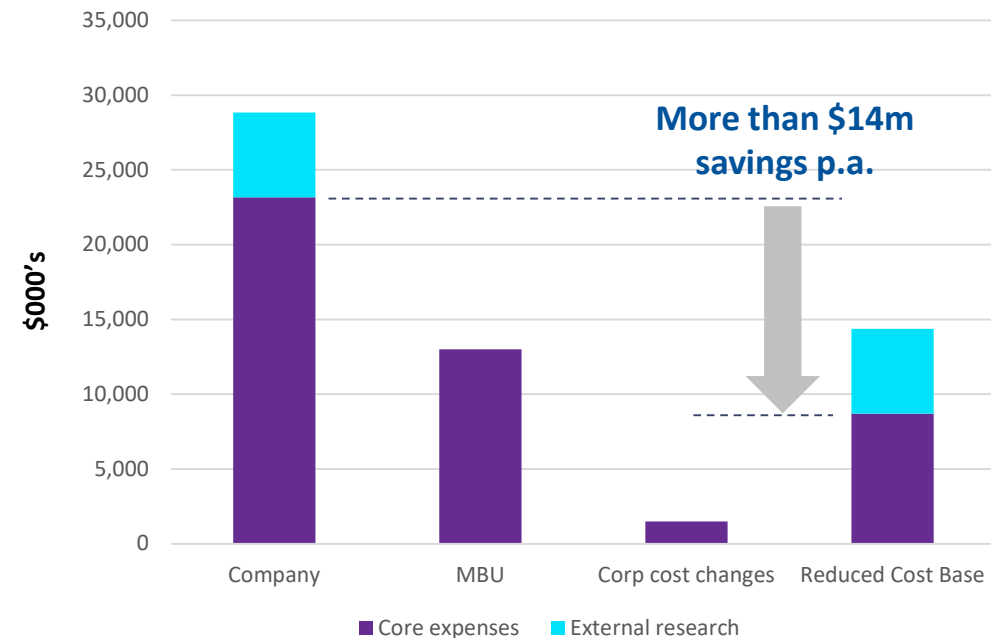
## The main factors impacting cash from separation of the mannitol business unit are:

- Employee numbers dropping from ~70 to ~25
- Much reduced new lease for research labs and a small corporate office
- Downsized Corporate and Administration requirements
- Removal of all direct and indirect costs associated with operating a manufacturing and global pharma distribution business

## Core expenses (excluding external clinical trial and drug discovery costs) cut by more than 60%<sup>2</sup>

- Cash expenses excluding clinical trials drops from ~\$23m to \$9m
- More corporate & admin savings to be realized after the separation is complete

## Proforma Cash Expenses 2023



**Core expenses include employee costs, rent, utilities, manufacturing, regulatory and admin expenses**

1. Change of name from Pharmaxis Ltd to Syntara Limited subject to shareholder approval at the Pharmaxis annual general meeting to be held in Sydney on Tuesday 28 November 2023.

2. Based on proforma FY 2023



A clinical stage drug development company



Focused on first and best in class disease modifying drugs to improve quality of life and extend life expectancy



Prioritising haematological malignancies with high unmet need:

Myelofibrosis (MF)

- Market opportunities in excess of US\$1b per annum
- Recent history of biotech exits in excess of US\$1.7b
- Monotherapy study reported positive data Q3 23
- Follow on headline generating Phase 2 MF trial starts Q4 2023

Myelodysplastic Syndrome (MDS)

- Preclinical data excites clinical and industry thought leaders
- Clinical opportunity being evaluated



Three further phase 1c/2 investigator initiated or externally funded clinical studies to deliver results in high unmet need diseases by mid-2025.



Pipeline of pre-clinical and clinical stage assets in fibrosis and inflammation

# Syntara Board under new leadership and downsized

Significant international pharmaceutical experience



**Dr Kathleen Metters**  
Chair

- 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



**Dr Simon Green**  
Non-Executive Director

- Experienced senior global pharma executive with 30 years' of experience in the biotechnology industry.
- Actively involved in CSL's global expansion over a 17-year period where he held roles as Senior Vice President, Global Plasma R&D and General Manager of CSL's manufacturing plants in Germany and Australia.
- Prior to joining CSL he worked in the USA at leading biotechnology companies Genentech Inc and Chiron Corporation.



**Gary Phillips**  
Chief Executive Officer

- 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



**Hashan De Silva**  
Non-Executive Director

- Experienced life sciences investment professional with extensive knowledge of the biotech, pharmaceutical and medical technology sectors.
- Worked as associate healthcare analyst at Macquarie Group and lead healthcare analyst at CLSA Australia before joining Karst Peak Capital in February 2021 as head of healthcare research.
- Prior to moving into life science investment Hashan worked at Eli Lilly in various roles focused on the commercialisation of new and existing pharmaceuticals.

# Experienced senior management team

Significant global experience in drug development, commercialisation and partnering



**Gary Phillips**  
Chief Executive Officer

- 30+ years' of operational management experience in the pharmaceutical and healthcare industry in Europe, Asia and Australia
- Previously held country and regional management roles at Novartis – Hungary, Asia Pacific and Australia



**Jana Baskar**  
Chief Medical Officer

- 20+ years' experience both in clinical medicine and the biopharmaceutical industry
- Former Medical Director at Novartis Oncology in Australia; former Medical Director for IQVIA in Australia and New Zealand



**Wolfgang Jarolimek**  
Head of 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



**David McGarvey**  
Chief Financial Officer

- More than 30 years' experience building Australian based companies from inception to globally successful enterprises
- Previously Chief Financial Officer of the Filtration and Separations Division of US Filter (1998-2002), and Memtec Limited (1985-1998)



**Kristen Morgan**  
Head of Medical & Regulatory Affairs

- More than 20 years' experience in the pharmaceutical industry
- Previously held a senior role in medical affairs at Sanofi-Aventis, and a commercial sales role at GlaxoSmithKline



**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

## Scientific excellence

- Global leaders in amine oxidase chemistry and biology – key to inflammatory and fibrotic diseases
- 3 Nature publications with collaborators in last 2 years

## Drug development expertise

- 7 drugs through preclinical and phase 1 / IND eligible since 2015
- 5 drugs successfully cleared phase 1
- 3 drugs completed Phase 1c/2 patient clinical proof of concept studies with acceptable safety and signs of efficacy

## Commercial acumen

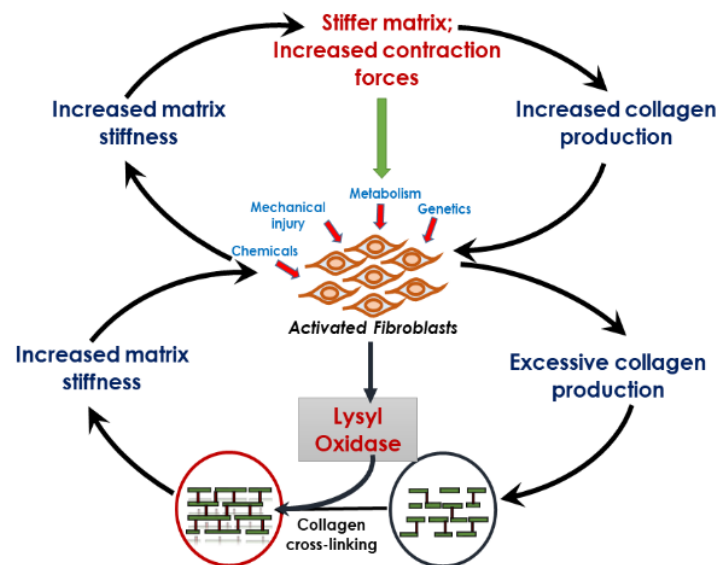
- Three licensing / asset sale deals worth >\$100m in cash receipts
- Extensive Pharma industry networks



# Syntara is the global leader in lysyl oxidase chemistry and biology

Multi year research program leveraged with extensive scientific collaborations worldwide has delivered 2 drugs now in phase 1c/2 studies

## Lysyl oxidases mediate the final stage in fibrosis



Lysyl oxidase inhibition provides a true anti-fibrotic therapy, directly addressing the tissue stiffening that occurs due to increases in collagen and number of cross-links.

## PXS-5505 in Oncology

- Clinical PoC: reduction of bone marrow fibrosis grade in 60% of evaluable myelofibrosis patients in 6-month Phase 2 study
- Excellent clinical safety and tolerability with a complementary mode of action to current standard of care
- INDs approved for myelofibrosis and hepatocellular carcinoma
- Potential in haematological indications such as MDS as well as solid tumours; two Nature publications
- Patent priority date of 2018 provides extended IP coverage

## Oral and topical pan-LOX inhibitors in Skin Scarring

- Clinical PoC: significant reduction of collagen and good safety in 3-month placebo-controlled Phase 1c study in patients with established scars
- Lead and back up compounds to support studies in multiple scar types (prevention of scar formation and modification of existing scars) in topical and oral dosage form
- Strong preclinical evidence in models of skin fibrosis and scarring; Nature publication
- Patent priority date of 2019 provides extended IP coverage

# Preclinical science and collaborations validated in high impact publications

## Myelofibrosis

- Treatment with lysyl oxidase inhibitor *significantly reduced reticulin fibrosis and megakaryocyte cell number in GATA-1<sup>low</sup> mice*

International Journal of Hematology  
<https://doi.org/10.1007/s12185-019-02751-6>

ORIGINAL ARTICLE

Novel lysyl oxidase inhibitors attenuate hallmarks of primary myelofibrosis in mice

nature communications



Article

<https://doi.org/10.1038/s41467-023-37175-8>

**Inhibition of lysyl oxidases synergizes with 5-azacytidine to restore erythropoiesis in myelodysplastic and myeloid malignancies**

nature cancer



Article

<https://doi.org/10.1038/s43018-023-00614-y>

**A first-in-class pan-lysyl oxidase inhibitor impairs stromal remodeling and enhances gemcitabine response and survival in pancreatic cancer**

nature communications



Article

<https://doi.org/10.1038/s41467-022-33148-5>

**Topical application of an irreversible small molecule inhibitor of lysyl oxidases ameliorates skin scarring and fibrosis**

## Pancreatic Cancer

- PXS-5505 anti-fibrotic effects *normalise the stroma*, providing increased gemcitabine penetration and *increased overall survival in pancreatic cancer*

## Myelodysplastic Syndrome

- In xenograft mouse model that closely resembles human disease, PXS-5505 on top of 5-Azacytidine *increased erythroid differentiation and reduced spleen size*

## Skin Scarring

- Topical application of PXS-6302 *improves scar appearance with no reduction in tissue strength* in porcine models of excision and burn injury



# Program Update

A blurred background image of a laboratory setting. A person in a white lab coat is visible on the right, and another person in a blue lab coat is leaning over a counter in the center. The scene is brightly lit, suggesting a clean, professional environment.

# Myelofibrosis (MF)

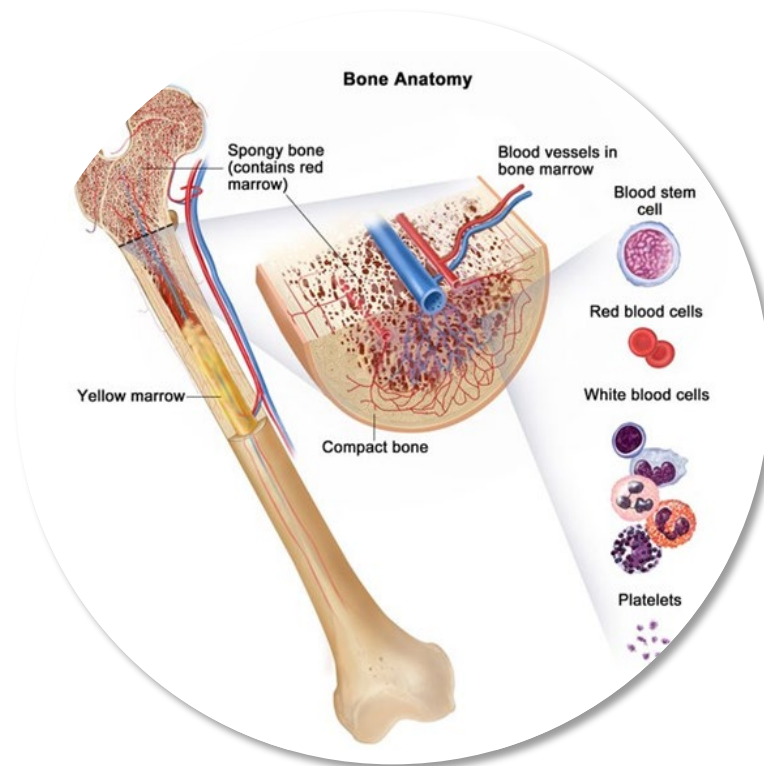
# Myelofibrosis

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

## Key Facts

- Affects ~15 in 1m people worldwide
- Age of onset typically from age 50; 5 years median survival
- 11% transformation to leukemia
- 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.

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



## Current standard of care (SoC): 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

In contrast to SoC PXS-5505 intervenes at the source, clearing fibrosis from the bone marrow and enabling the production of healthy blood cells to resume

## Clinical positioning

- Distinct mode of action, improved tolerability and a profile suitable for combination with SoC
- In addition to symptomatic relief, potential for disease modification.

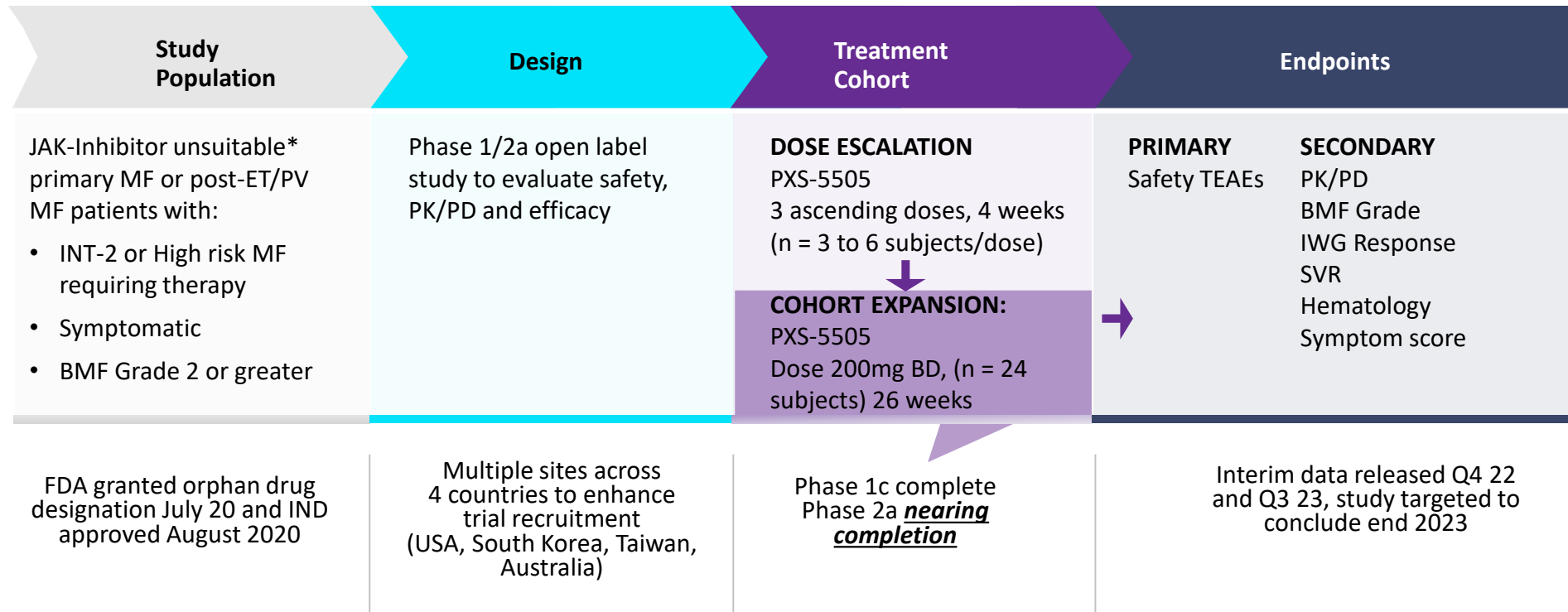
## Commercial Opportunity

- Current SoC; revenue ~US\$1b per annum
- Recent history of biotech exits in excess of US\$1.7b

# Myelofibrosis - PXS-5505 phase 1/2a trial

6 month monotherapy study with meaningful safety and efficacy endpoints

- Phase 1c: dose escalation in patients (complete)
- Phase 2a: cohort expansion (currently ongoing)



# PXS-5505 Phase 2a trial (INTERIM results)

## Study status

- 23 out of a targeted 24 patients have been enrolled
- 11 patients having completed 24 weeks of treatment

## Safety

- PXS-5505 has been well tolerated with no serious treatment related adverse events reported
- Majority of AEs were mild and not related to treatment
- 10 patients have dropped out of the study, none treatment related

## Efficacy

- 5/9 evaluable patients\* had improved bone marrow fibrosis scores of  $\geq 1$  grade
  - 4 out of 5 fibrosis responders demonstrating stable haematological parameters
  - 3 out of 5 patients reporting symptomatic improvement
- 4 had an improvement in symptom score of  $>20\%$
- 7 had stable/improved hemoglobin (Hb) counts
- 8 had stable/improved platelet counts;  
3 of these 8 patients entered the study with Grade 4 (potentially life-threatening) thrombocytopenia
- No spleen volume response (SVR35) was identified
  - Patients had a relatively smaller spleen size# at baseline
  - Majority of patients stopped JAKi treatment less than 1 month before commencing treatment

\*One of the 10 patients who completed the 6 months treatment could not be evaluated for bone marrow fibrosis grade due to an insufficient sample at baseline

#Median spleen volume of 1495ml versus COMFORT-1 (ruxolitinib) of 2598ml

# PXS-5505 Phase 2 trial (MF-101) expert review



**Dr. Lucia Masarova**

Assistant Professor, Department  
of Leukemia at MD Anderson  
Cancer Center, Houston

*“PXS-5505 continues to show not only an excellent safety profile but also promising clinical activity. The effect on bone marrow fibrosis is particularly exciting for a disease like myelofibrosis, where despite numerous years of research, we do not have any effective anti-fibrotic drugs.”*

*“It is encouraging to see that majority of 10 patients who completed 24 weeks of therapy also had improvements of symptoms and more importantly, stable or improved blood counts; including in those patients with severe thrombocytopenia.”*

*“These results support plans to continue clinical investigation of the agent, including combinations with JAK inhibitors where the lack of overlapping hematological toxicity would make PXS-5505 an ideal add-on candidate.”*



# Clinical development plan: FDA feedback

**Fastest route to meaningful data**

**FDA reviewed all safety and efficacy data available at that time**

- Provided guidance on the number of patients, treatment dosage, study duration and endpoints

**Protocol subsequently submitted to FDA and accepted without change Q3 23**

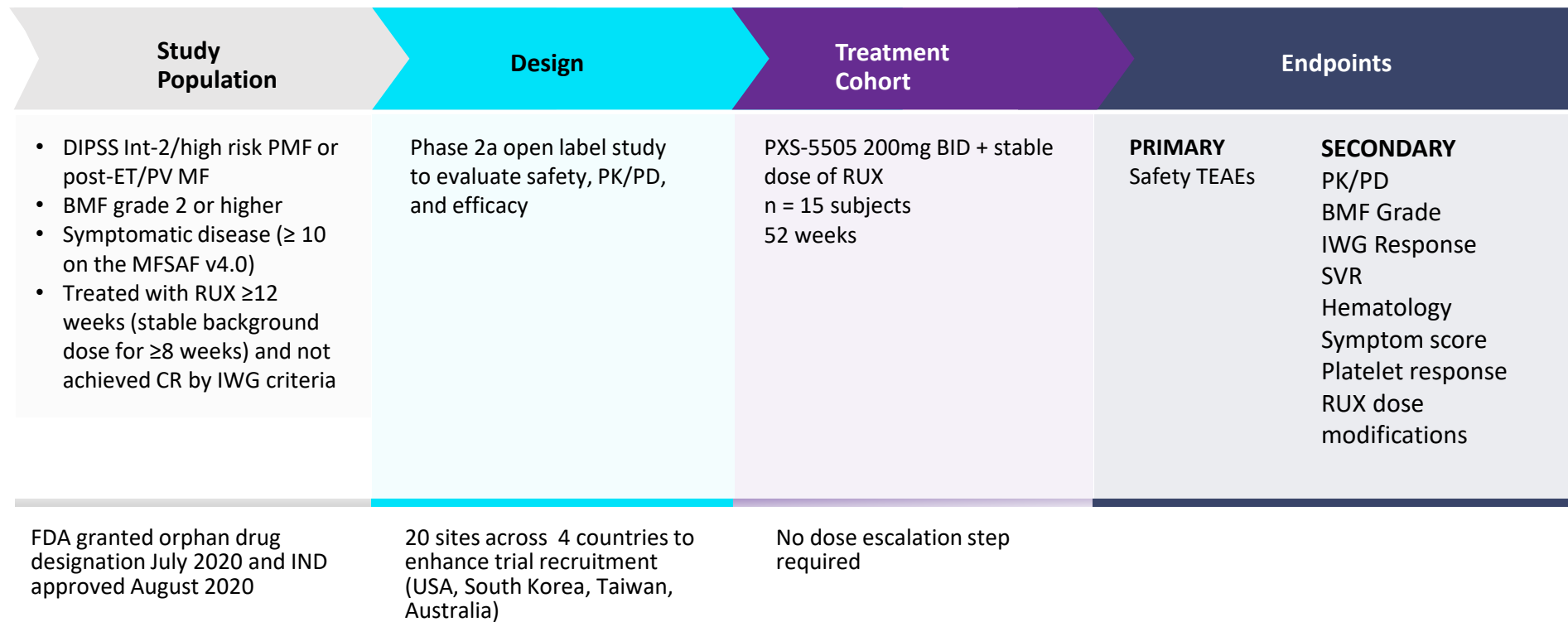
- In recognition of acceptable safety profile demonstrated by PXS-5505 in monotherapy phase 2a study, no dose escalation step required by FDA
- Approval to include elderly patients on standard of care and concomitant meds
- Can use existing trial sites, ensuring a fast start up with minimal initiation costs

**FDA Type C Meeting held in Q2 2023**

**FDA supported progression into a combination study with a JAK inhibitor**

# Phase 2a study cohort added to trial PXS-5505 in patients on a stable dose of JAK inhibitor

Fastest route to meaningful data with no dose escalation and utilizing existing trial infrastructure



[ClinicalTrials.gov ID NCT04676529](https://clinicaltrials.gov/ct2/show/study/NCT04676529)

\*Unsuitable = ineligible for JAKi treatment, intolerant of JAKi treatment, relapsed during JAKi treatment, or refractory to JAKi treatment. JAKi – Janus Kinase inhibitor, RUX – Ruxolitinib, 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

## Study Plan

- 20 clinical trial sites scheduled to be open for recruitment by end Q4 2023
- FPFV scheduled for Q4 2023
- Full recruitment scheduled for Q2 2024
- Interim data for 15 patients with 6 months data scheduled for Q4 2024
- Full data set by mid 2025

Interim data to drive FDA discussion on pivotal study design and partnering interest

# Strong interest in myelofibrosis assets from strategic

Target / Acquiror



Date of Announcement	June-2023	July-2022	December-2020	November-2022	September-2020	January-2018
Drug Name	Pacritinib	Momelotinib	Combination of Ruxolitinib & CK0804	Bomedemstat	AVID200	Fedratinib
Lead Indication / Phase (at transaction)	qas	Myelofibrosis (FDA Filed – June)	Myelofibrosis (Phase 1b)	Haematology (Phase 2)	Myelofibrosis (Phase 1)	Myelofibrosis & Polycythemia vera (Successful Phase 3 Trials)
Deal Type	Acquisition	Acquisition	Licensing	Acquisition	Acquisition	Acquisition
Upfront / Milestones (USD)	US\$1.7B	US\$1.9B	If option exercised <b>US\$20m Licensing fee</b> Sales Milestone up to <b>US\$294.5m</b>	US\$1.35B	Undisclosed but present	US\$1.1B / US\$1.25B
Earnout Payments / Royalty Rate (%)	None	None	Tiered royalties Mid single to low double digits	None	Undisclosed	None

A background image of a man with a beard and glasses, wearing a white lab coat, looking down at a piece of equipment in a laboratory setting. The image is overlaid with a large, semi-transparent graphic consisting of two overlapping circles, one in a light blue color and one in a light purple color.

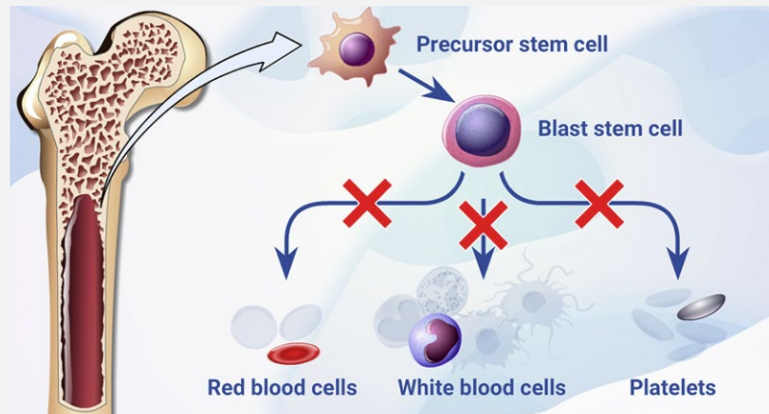
# Myelodysplastic Syndromes (MDS)

# Myelodysplastic syndrome (MDS) is a blood cancer

Diverse bone marrow disorders characterized by inadequate production of healthy blood cells

## Key Facts

12–20k new cases are reported every year in the US (87k p.a. worldwide)



- Prognosis and overall survival depend upon multiple factors including the severity of cytopenias (low blood counts)
- Low-risk MDS therapy is aimed at improving cytopenia(s) to prevent complications
- 25–30% have high-risk MDS with average survival of ~ 1 year
- 1 out of 3 MDS patients progress to acute myeloid leukemia (AML)

## Treatment of High Risk MDS

### Current standard of care (SoC): Hypomethylating agents (HMAs)

- First line therapy: agents such as azacytidine (5-AZA) or decitabine,
- Adverse effects of HMAs include low blood counts, risk of infections, nausea, vomiting, diarrhea or constipation, weakness and fatigue
- Only ~50% of patients respond to HMAs and most responders eventually progress; median overall survival 4–6 months

### Drugs in development

- Other investigational products in Phase 3 trials (eg. venetoclax, sapatolimab, magrolimab) have demonstrated encouraging response rates in combination with 5-AZA in Phase 1b studies<sup>1</sup>
- However, these results are offset by greater toxicity (e.g. neutropenia, thrombocytopenia, anemia) that are likely to result in frequent dose interruptions and treatment discontinuation.

### PXS-5505; potential for well tolerated combination therapy with HMAs

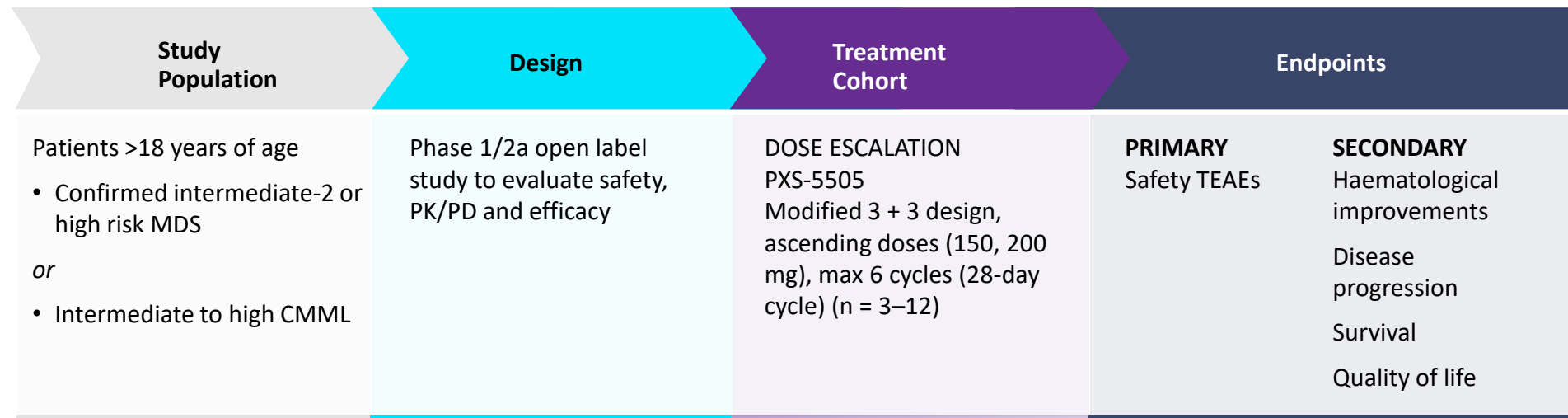
- Research reported in Nature communications demonstrated superior in vitro erythroid differentiation in cells of patients in 20/31 cases (65%) treated with PXS-5505 and 5-AZA versus 9/31 cases (29%) treated with 5-AZA alone.
- This response was confirmed in an in vivo xenograft model (closest mimic of human disease) that additionally demonstrated an enforced reduction of dominant clones as well as significant attenuation of disease expansion and normalization of spleen sizes.

Market Opportunity in high risk MDS ~US\$1bn p.a.<sup>2</sup>

1. Platzbecker U, 2021, Leukemia 2021  
 2. Total global MDS market US\$3.2b, with prevalence of high-risk MDS 25–30%

# PXS-5505 in high-risk MDS/CMML study

- Clinician designed multicenter open-label study in Germany evaluating the safety and efficacy of escalating doses of PXS-5505 for pan lysyl oxidase inhibition in combination with 5-AZA in patients diagnosed with MDS or CMML



- Clinical trial protocol near finalized after receiving a top rating from the German MDS clinical trial group led by International KOL, Prof. Uwe Platzbecker; <https://d-mds.de/>
- Competing Phase 3 trials are currently active but not recruiting.
- Safety and efficacy signals likely from 3 month dose escalation
- Timelines\*
  - FPFV: Q2 2024
  - LPLV: Q1 2025

\* CRO assumption based on 4-month start up period.  
 CMML: chronic myelomonocytic leukemia



A background image of a scientist with a beard and glasses, wearing a white lab coat, looking down at a piece of equipment in a laboratory setting. The image is overlaid with a large, semi-transparent graphic consisting of several overlapping circles in shades of blue and purple.

# Skin Scarring

# Hypertrophic and keloid scarring

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

## Key Facts

01

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

02

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

03

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



*"In (preclinical) models of scarring we found that topical application of PXS-6302 reduces collagen deposition and cross-linking and improves scar appearance without reducing tissue strength. This is a unique way of modulating a critical stage in scar formation and maintenance and holds out great promise for the treatment of scars."*

**Dr Mark Fear**  
UWA

- **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<sup>1</sup>
- **Clinical evidence**
  - 1 month phase 1a in healthy volunteers demonstrates good tolerability and full inhibition of LOX in skin.
  - 3 month phase 1c placebo controlled study in patients with established scars demonstrates unprecedented reduction in scar collagen content.

## Commercial Opportunity

- Total scar treatment market in 2019 exceeded US\$19b.
- Keloid and hypertrophic scar segment ~US\$3.5b

Note 1: Chaudhari et al, Topical application of an irreversible small molecule inhibitor of lysyl oxidases ameliorates skin scarring and fibrosis, Nature communications 2022  
<https://doi.org/10.1038/s41467-022-33148-5>

# PXS-6302 phase 1c Trial in established skin scars (Solaria 2); Top line results



PXS-6302 well tolerated and demonstrated a good safety profile.

- No serious adverse events reported
- Two patients withdrew from the study; reversible rash



**Mean inhibition of LOX activity 66% compared to baseline and placebo (p<0.001)**

- LOX measured 2 days post final dose
- LOX is responsible for the cross linking of collagen fibres implicated in adverse scarring.



**Meaningful changes in the composition of the scars**

- Patients in the active arm had a mean reduction in collagen<sup>1</sup> of 30% compared to placebo after three months treatment. (p<0.01).



**Longer study required to show appearance and physical improvements**

- No significant differences in the overall POSAS<sup>2</sup> score were seen between active and placebo groups after three months of treatment.

*“PXS-6302 leads directly to an unprecedented change to the scar composition that we have not seen with any other form of treatment.”*



**Professor Fiona Wood**  
Burns Service of Western Australia  
Director of the Burn Injury Research Unit University of Western Australia

1. Collagen content quantified via hydroxyproline assay  
2. POSAS: Patient and Observer Scar Assessment Scale

# Syntara skin scarring clinical development plan

Incidences of skin rash with PXS-6302 in Solaria trial suggest potential development limitations. IND-enabling studies with backup candidate addressing the issue are near completion.

**Unprecedented structural changes** effected by 3 months treatment with PXS-6302 open up a wide vista of potential skin fibrosis indications for clinical development. For example:

- Younger scars
- Scar prevention post surgery
- Keloids
- Dupuytren's
- Surgical adhesions

**Scar prevention** with oral pan-LOX inhibitor progressing in Q4 2023; PXS-5505 being used to establish fastest clinical proof of concept

**Update on plans** for topical treatment for scar modification 1Q 2024

**Collaboration** with Professor Fiona Wood and University of Western Australia extended.

# Upcoming News Flow

# Potential for five trials to deliver near term value

Pipeline creates multiple opportunities in high value markets

Drug Candidate	Indication	Phase	Trial design	Status	Upcoming Milestones	Addressable market (US\$)
PXS-5505	Myelofibrosis (MF)	Phase 2	<ul style="list-style-type: none"> <li>Open label 12 month study (n=15)</li> <li>MF patients receiving a stable dose of ruxolitinib (JAK inhibitor)</li> </ul>	First patient Q4 2023	2H 2024: Interim 6 month data	~\$1 billion
	Myelodysplastic Syndrome (MDS)	Phase 1c/2	<ul style="list-style-type: none"> <li>Protocol development underway</li> </ul>	TBD	TBD	~\$1 billion
Oral and Topical Pan-LOX inhibitors	Scar prevention	Phase 1c	<ul style="list-style-type: none"> <li>6 month placebo controlled trial</li> <li>Independent investigator trial</li> <li>Patients with scarring subsequent to burn injury (n=60)</li> </ul>	First patient Q4 2023	H1 2025	~\$3.5 billion
	Modification of established scars	Preclinical	<ul style="list-style-type: none"> <li>Plan to initiate Phase 1/2 trial</li> <li>Independent investigator trial</li> <li>Patients with keloid or hypertrophic scars</li> <li>Protocol under development</li> </ul>	TBD	TBD	~\$3.5 billion
PXS-4728	IRDB and Parkinson's Disease	Phase 2	<ul style="list-style-type: none"> <li>Double blind, placebo controlled</li> <li>Patients with Isolated REM sleep behaviours disorder IRBD (n=40)</li> <li>Majority funded by Parkinson's UK</li> </ul>	First patient Q4 2023	H1 2025	~\$3.5 billion



# News flow

## Recent and anticipated news flow

Strong and growing pipeline with advancement in studies expected to provide value inflection points

### Q4 2023

- PXS-5505 phase 2a myelofibrosis combination study (add on to JAK inhibitor) commences recruitment
- Pan-LOX scar prevention for burn injuries- clinical trial commences recruitment
- PXS-4728 iRBD / neuro inflammation study commences recruitment
- PXS-5505 phase 2a myelofibrosis study (monotherapy) completed and reports safety and efficacy data at ASH

### H1 2024

- PXS-5505 phase 2a myelofibrosis combination study (add on to JAK inhibitor) completes recruitment
- PXS-5505 Phase 1c myelodysplastic syndrome study commences recruitment
- Syntara skin scarring clinical development plan announced

### H2 2024

- PXS-5505 phase 2a myelofibrosis combination study (add on to JAK inhibitor) interim data with 6 months treatment.
- PXS-5505 phase 2a myelofibrosis study combination study reports safety and efficacy data at ASH
- Topical pan LOX inhibitor scar revision study commences recruitment

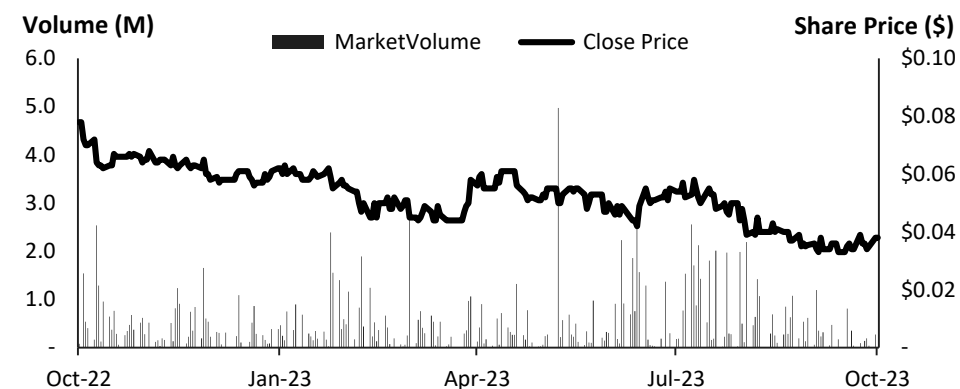
# Shareholders & Cash



Financial Information	30 Sept 23
ASX Code <sup>1</sup>	PXS
Share price	\$0.033
Liquidity (turnover last 12 months)	124m shares
Market Cap	A\$26m
Pro forma <sup>2</sup> Cash balance (30 June 2023)	A\$14m
Enterprise value	A\$12m
Clinical development program supported by: <ul style="list-style-type: none"> <li>• R&amp;D tax credits</li> <li>• Strategy of partnering deals with pipeline assets</li> </ul>	
1. Syntara ASX code will be SNT 2. Proforma cash includes cash of \$9.2m and 2023 R&D tax credit of \$5.2 million (expected receipt H2 CY23)	

Institutional Ownership	30 Sept 23
BVF Partners LP	14%
Karst Peak Capital Limited	12%
D&A Income Limited	11%
Platinum Investment Management Limited	8%
<b>Total Institutional Ownership</b>	<b>50%</b>

## Share Price



Q&A





Pharmaxis Ltd ABN 75 082 811 630

**Gary Phillips**

Chief Executive Officer

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

**David McGarvey**

Chief Financial Officer

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

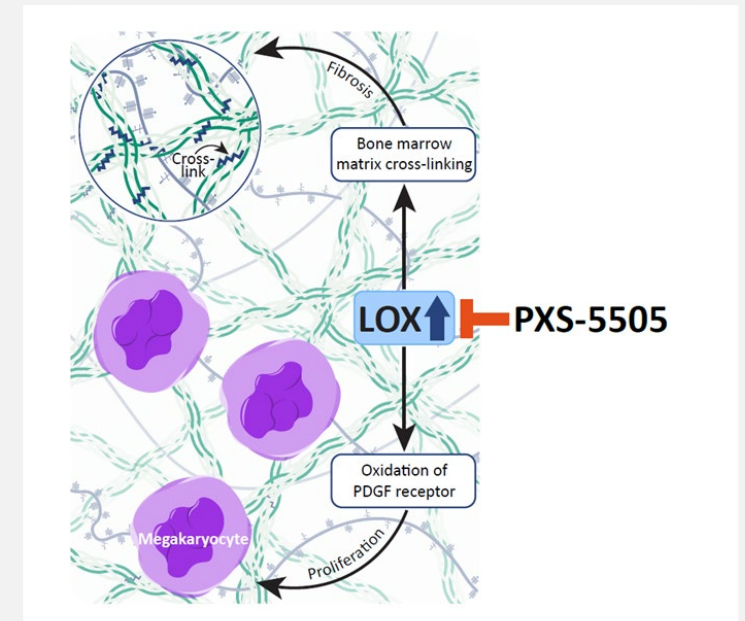
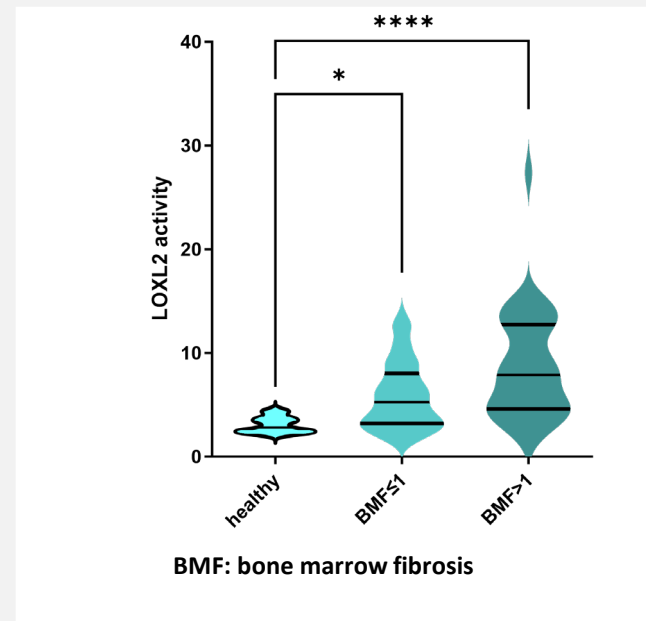
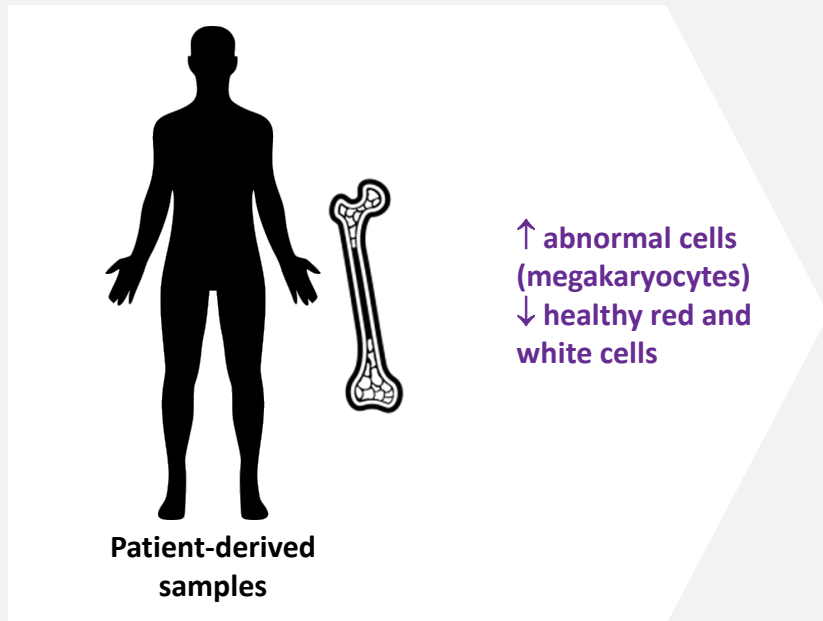


# APPENDIX

# Lysyl oxidases and MDS

Lysyl oxidases are a family of enzymes with different, non-conventional effects on cancer cells, including PDGF receptor activation

- Lysyl oxidase-like 2 (LOXL2) is a biomarker for myeloid neoplasms
- Lysyl oxidase (LOX) activity is increased in bone marrow aspirates

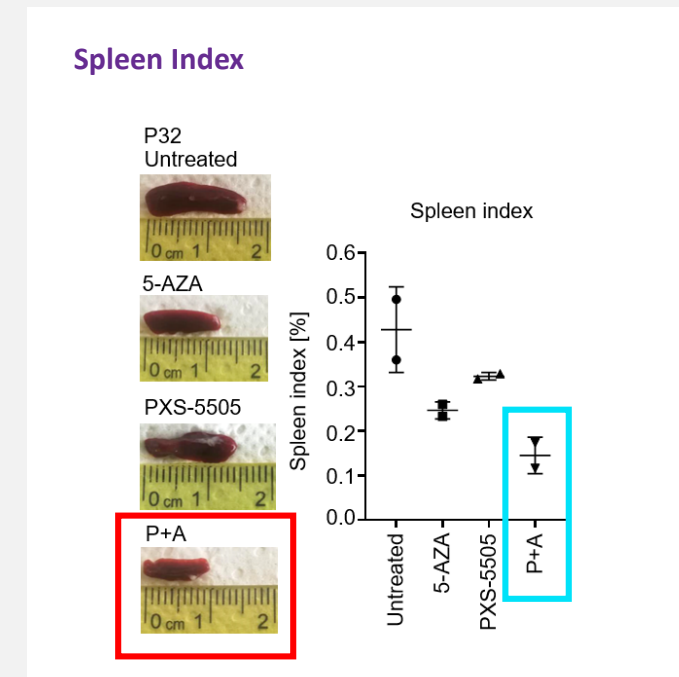
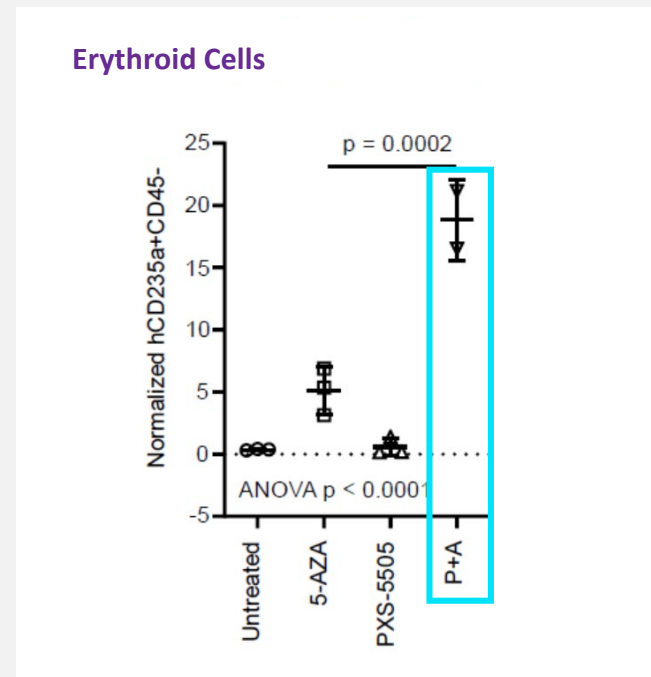
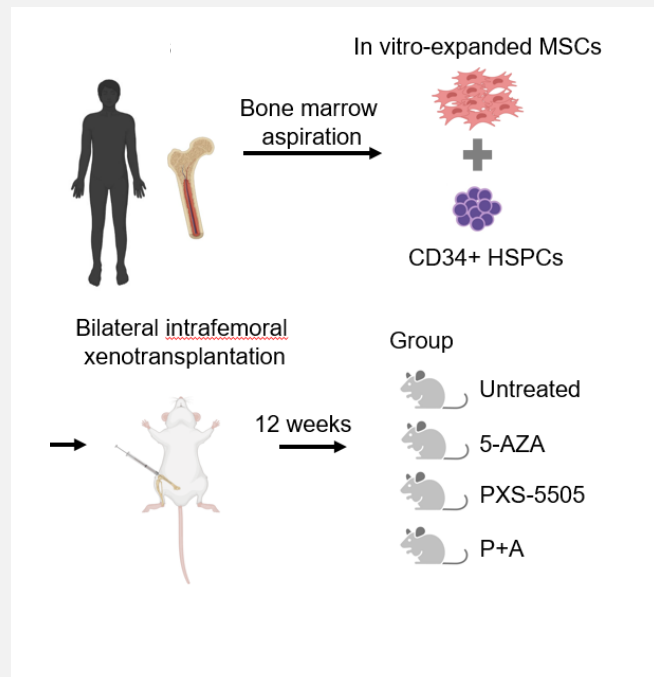


**Hypothesis:** Correction of elevated lysyl oxidase activity in the bone marrow microenvironment is of therapeutic value in MDS.



# Improved erythropoiesis *in vivo* MDS model with PXS-5505

Impressive *in vitro* efficacy of PXS-5505 on top of 5-AZA translates to substantial clinical improvements in xenograft animal models (closest mimic of human disease)



The response to 5-AZA (29%) is potently augmented by concomitant lysyl oxidase inhibition with PXS-5505 (65%) in xenograft models.