

Investor briefing; MF-101 myelofibrosis Final Interim Data

pharmaxis

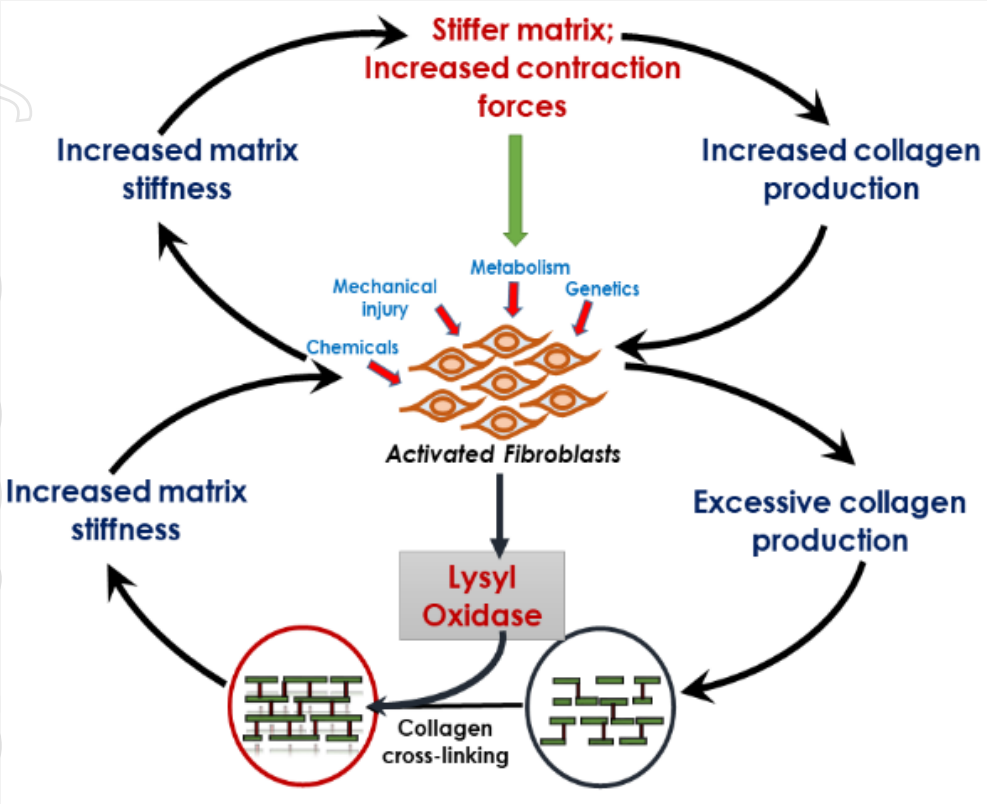
developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | July 12 2023
Gary Phillips CEO

Pharmaxis is the global leader in lysyl oxidase chemistry and biology

Multi year research program leveraged with extensive scientific collaborations worldwide has delivered 2 drugs in the clinic

Lysyl oxidases are the final stage in fibrosis



Tissue stiffening due to increases in collagen and number of cross-links which is a hallmark of fibrosis, is preventable through lysyl oxidase inhibition; at the heart of a true anti-fibrotic therapy

■ PXS-5505

- Oral dosage form – four capsules twice a day
- Patent filed - priority date 2018
- Strong pre clinical evidence in models of fibrosis and cancer
- INDs approved for myelofibrosis and hepatocellular carcinoma
- Potential in multiple cancer indications
- Phase 1 data demonstrates a safe, well tolerated drug that gives >90% inhibition of LOX enzymes

■ PXS-6302

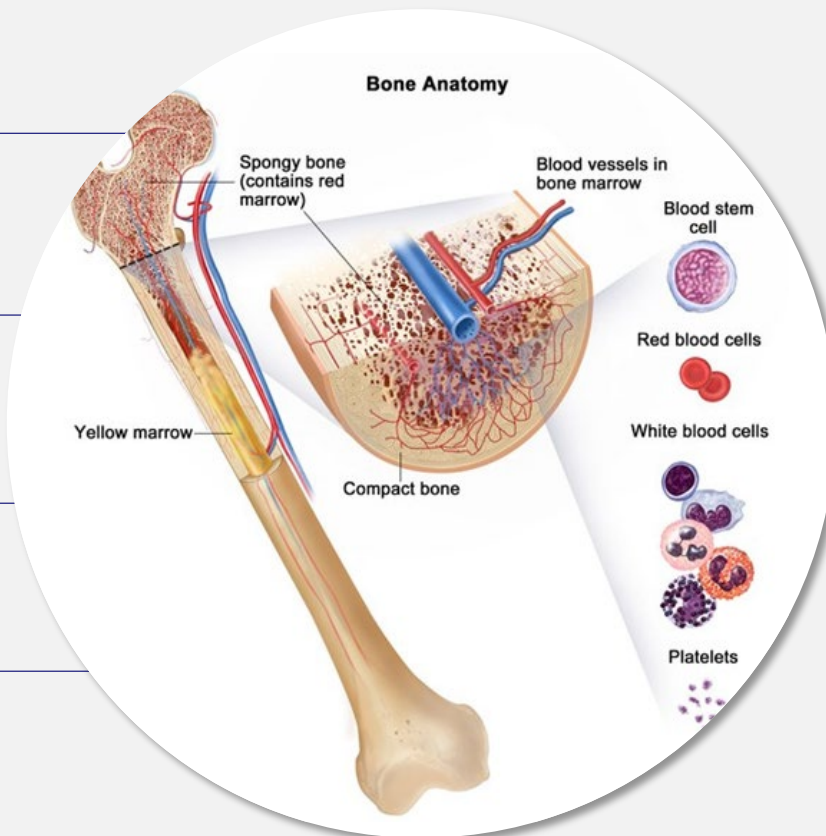
- Topical dosage form
- Patent filed - priority date 2019
- Strong pre clinical evidence in models of skin fibrosis and scarring
- Potential in prevention of scar formation and modification of existing scars
- Phase 1a (healthy volunteer) data demonstrates a safe, well tolerated drug that gives full inhibition of LOX enzymes in the skin with minimal systemic exposure

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
- 5 Years Median survival
- Age of onset typically from age 50
- 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:

- 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

Current Standard of Care; JAK inhibition

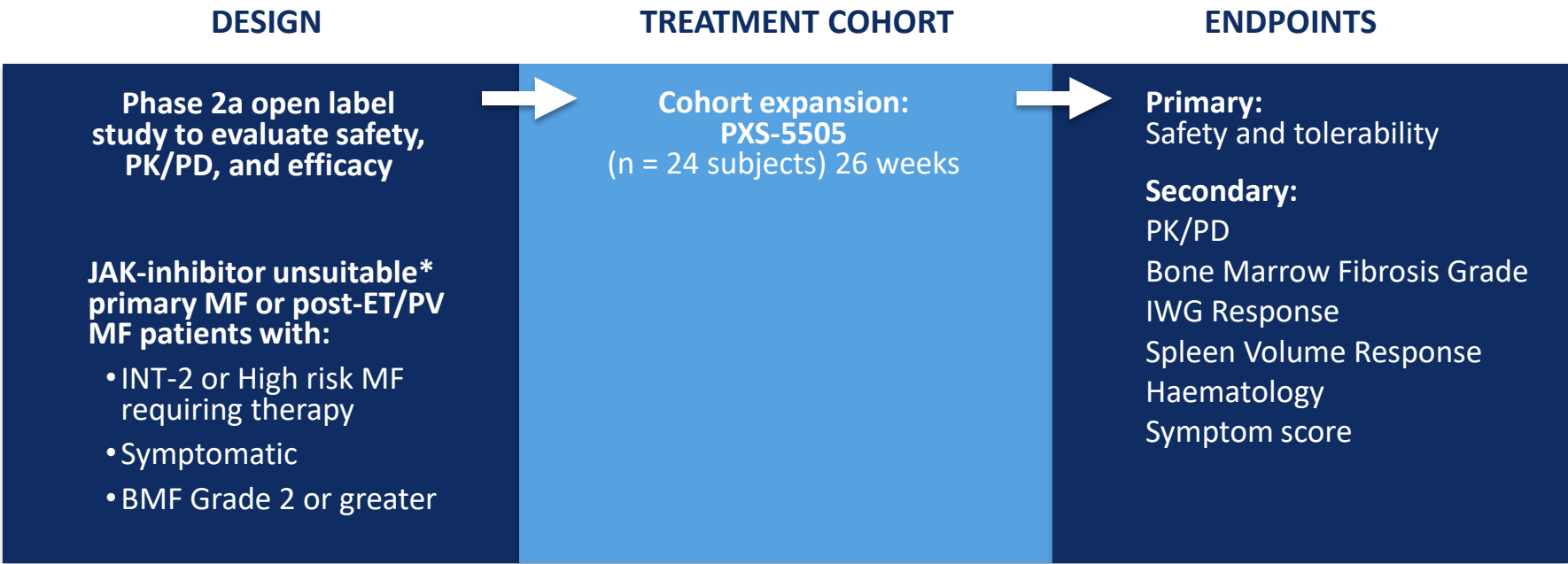
- Symptomatic relief plus some limited survival improvement. 75% discontinuation at 5 years
- Median overall survival is 14 – 16 months after discontinuation

Commercial Opportunity

- Current standard of care ; revenue ~US\$1b per annum

Myelofibrosis - PXS-5505 Phase 1/2a Trial

6 month monotherapy study with meaningful safety and efficacy endpoints



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

20 sites across 4 countries (Australia, South Korea, Taiwan, USA)

Study recruitment commenced Q4 2021

*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, PK pharmacokinetics, PD pharmacodynamics, IWG International Working Group Myeloproliferative Neoplasms

Myelofibrosis - PXS-5505 Phase 2a Trial (*INTERIM DATA*)

Very well tolerated with encouraging signs of clinical efficacy in JAK inhibitor unsuitable patients

■ Study status

- 21 out of a targeted 24 patients have been enrolled
- 10 patients having completed 24 weeks of treatment; none treatment related

■ 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

■ Efficacy

- 5/9 evaluable patients* had improved bone marrow fibrosis scores of ≥ 1 grade with 4 out of 5 fibrosis responders demonstrating stable hematological parameters and 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

*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.

PXS-5505 Phase 2 Trial (MF-101); Expert review

- “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.”



Dr. Lucia Masarova

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

PXS-5505 myelofibrosis clinical development plan: FDA feedback

- FDA Type C Meeting held in Q2 2023
- FDA reviewed all safety and efficacy data available at that time.
- Subject to protocol review FDA supported progression into a study in combination with a JAK inhibitor
- FDA provided guidance on the number of patients, treatment dosage, study duration and endpoints
- Trial protocol proposed to FDA
 - Uses existing trial sites; fast start up and minimal initiation costs
 - No dose escalation step; fastest route to meaningful data
- FDA feedback expected July 2023

Five trials to deliver near term value

Pipeline creates multiple opportunities in high value markets

	Indication	Addressable market (US\$)	Trial design	# patients	Status	Data
PXS-5505	Myelofibrosis (MF)	\$1 billion	Phase 2 open label 6 month study in JAK intolerant / ineligible myelofibrosis patients	24	Recruiting	Interim data released Significant data update mid 2023
			Phase 2 open label 6 month study in JAK intolerant / ineligible myelofibrosis patients	TBD	First Patient 2H 2023	TBD
PXS-6302	Modification of established scars	\$3.5 billion	Phase 1c 3 month placebo controlled study in patients with established scars (>1 year old)	50	Reported	H1 2023
	Scar prevention	\$3.5 billion	Phase 1c 3 month placebo controlled study in patients with scarring subsequent to a burns injury	50	First patient 2H 2023	2024
PXS-4728	Isolated REM sleep behaviours disorder (iRDB) and neuro inflammation	\$3.5 billion	Phase 2 double blind, placebo controlled study in patients with iRBD	40	First patient Q3 2023	H1 2025

News flow

Anticipated news flow

Strong and growing pipeline with advancement in studies expected to provide value inflection points in FY23



Q1 2023

- Pharmaxis strengthens Board with two new appointments
- PXS-5505 publication by KOL in hematological cancer myelodysplastic syndrome



Q2 2023

- PXS-5505: Encouraging FDA feedback on plans to progress to JAK inhibitor combination study
- LOX topical drug PXS-6302 top line data from established scars study
- PXS-5505 myelofibrosis monotherapy study: significant data update



H2 2023

- PXS-5505 phase 2 myelofibrosis study add on to JAK inhibitor commences recruitment
- Pan-LOX scar treatment and prevention clinical development update and trial initiation
- PXS-4728 iRBD / neuro inflammation study commences recruitment
- PXS-5505 phase 2a myelofibrosis study completed and reports safety and efficacy data at ASH





developing breakthrough treatments for fibrosis and inflammation

Pharmaxis Ltd ABN 75 082 811 630

www.pharmaxis.com.au



Contacts

Gary Phillips

Chief Executive Officer

gary.phillips@pharmaxis.com.au

David McGarvey

Chief Financial Officer

david.mcgarvey@pharmaxis.com.au