



Investor briefing; MF-101 myelofibrosis Final Interim Data

**pharmaxis**

developing breakthrough treatments for fibrosis and inflammation

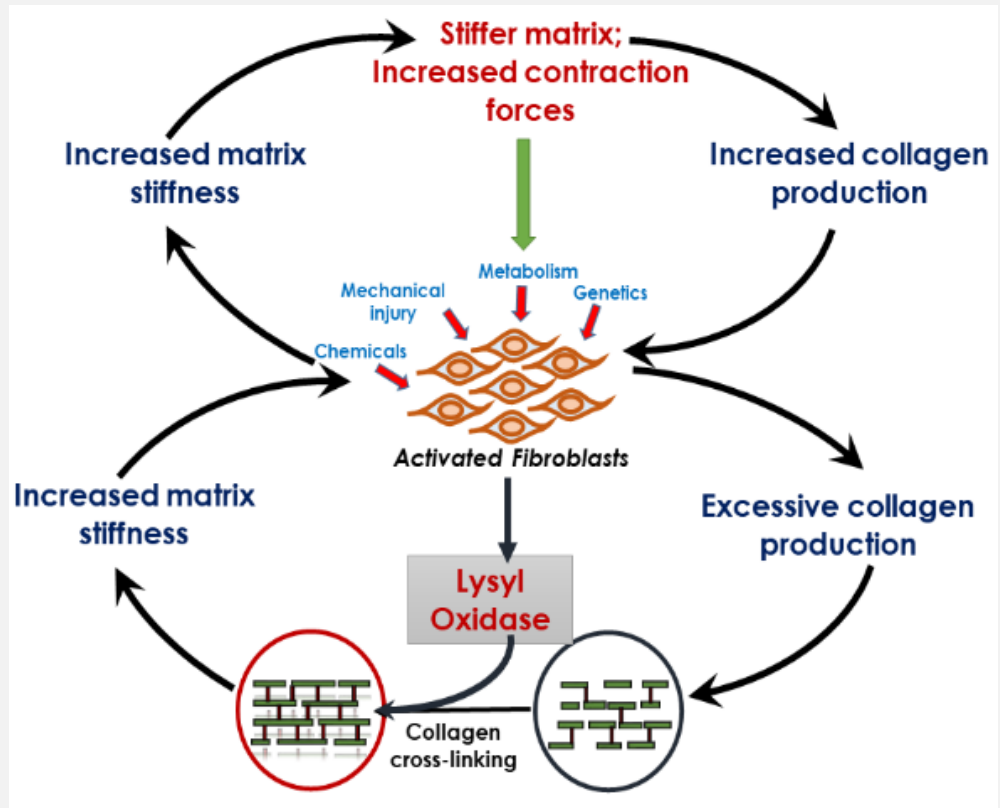
Investor Presentation | July 12 2023

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

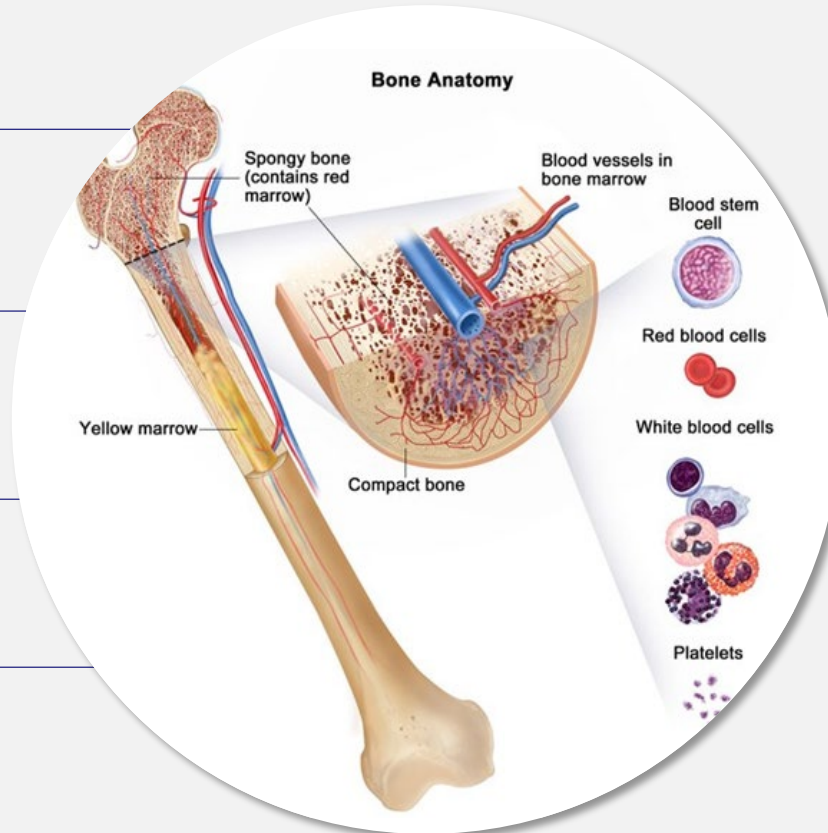
- Topical dosage form
- Patent filed - priority date 2019
- Strong pre clinical evidence in models of skin fibrosis and scarring
- Potential in prevention of scar formation and modification of existing scars
- Phase 1a (healthy volunteer) data demonstrates a safe, well tolerated drug that gives full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

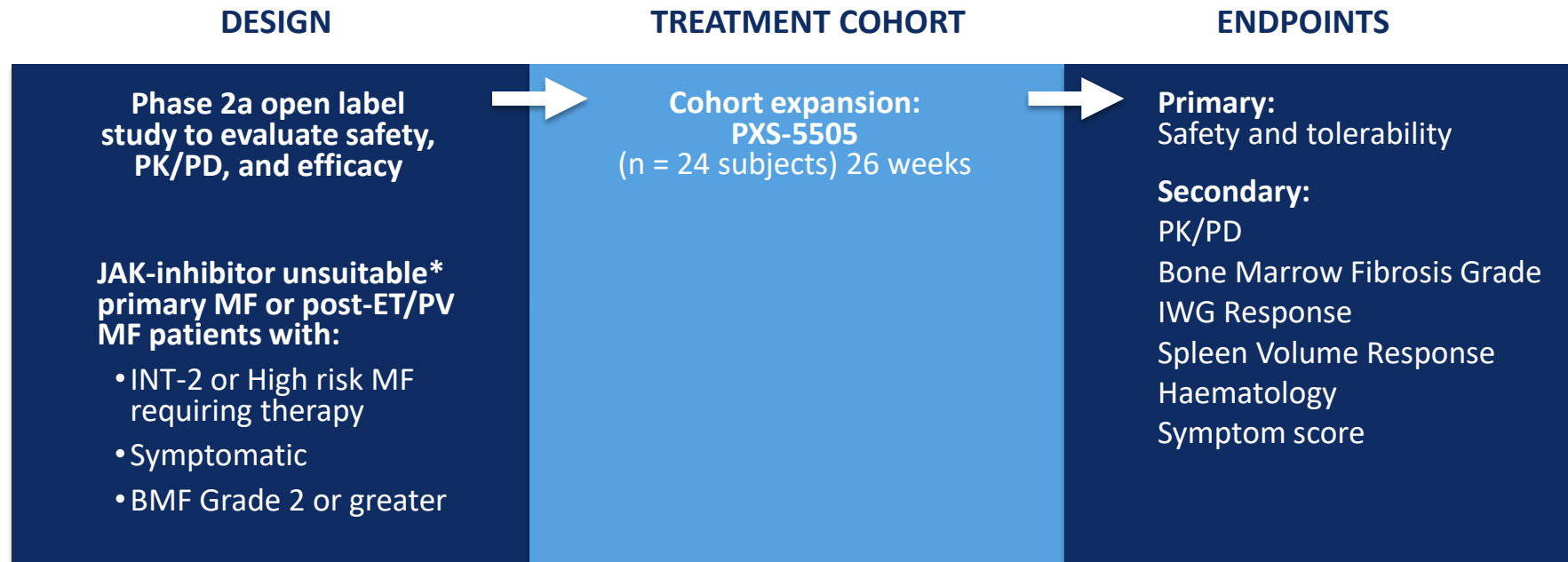
- Symptomatic relief plus some limited survival improvement. 75% discontinuation at 5 years
- Median overall survival is 14 – 16 months after discontinuation

## 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

# 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

## ■ 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 were treatment related

## ■ Efficacy

- 5/9 evaluable patients\* had improved bone marrow fibrosis scores of  $\geq 1$  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

## 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<br>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



# pharmaxis

developing breakthrough treatments for fibrosis and inflammation

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