



## Investor Presentation

**pharmaxis**

developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | 28 October 2021  
Gary Phillips CEO

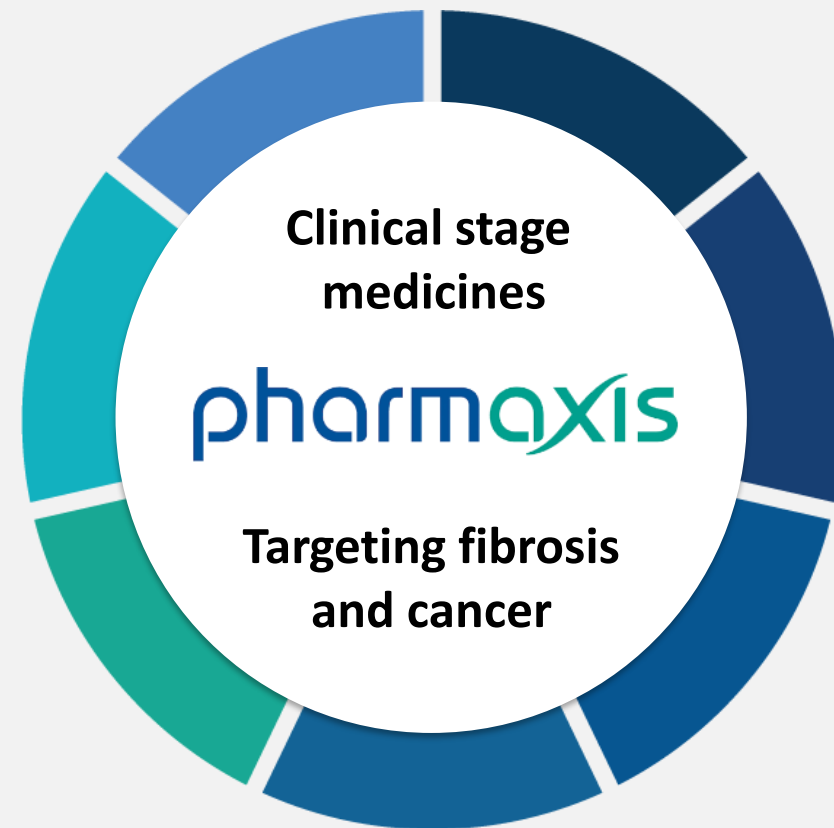
## Forward looking statement

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 2a trial – a breakthrough clinical program with disease modifying potential in Myelofibrosis
- PXS-5505 has demonstrated further potential in oncology as an adjunct to standard of care in difficult to treat tumours
- Anti-skin scarring drug PXS-6302 with potential to improve function and appearance progressing to phase 1c trial in patients with established scars and burns
- Specific corporate strategy to deliver non-dilutive cash and cost savings from commercial stage mannitol business
- Pharmaxis is in a strong position to fund its focused clinical program



## September 2021 Quarter Update

- **Cancer drug PXS-5505 progresses into myelofibrosis phase 2a study**

- Phase 1c dose escalation study completed with 3<sup>rd</sup> and highest dose demonstrating good tolerability profile and enzyme inhibition.
- Safety Committee endorsing decision to progress with highest dose into phase 2 dose expansion study.
- Recruitment commenced with dose escalation patients continuing into next phase.

- **Further data supporting the value of PXS-5505 in other cancers**

- The University of Rochester (NY) released the first data showing pre-clinical evidence of PXS-5505 significantly improving survival in liver cancer when added to existing chemotherapy drugs.

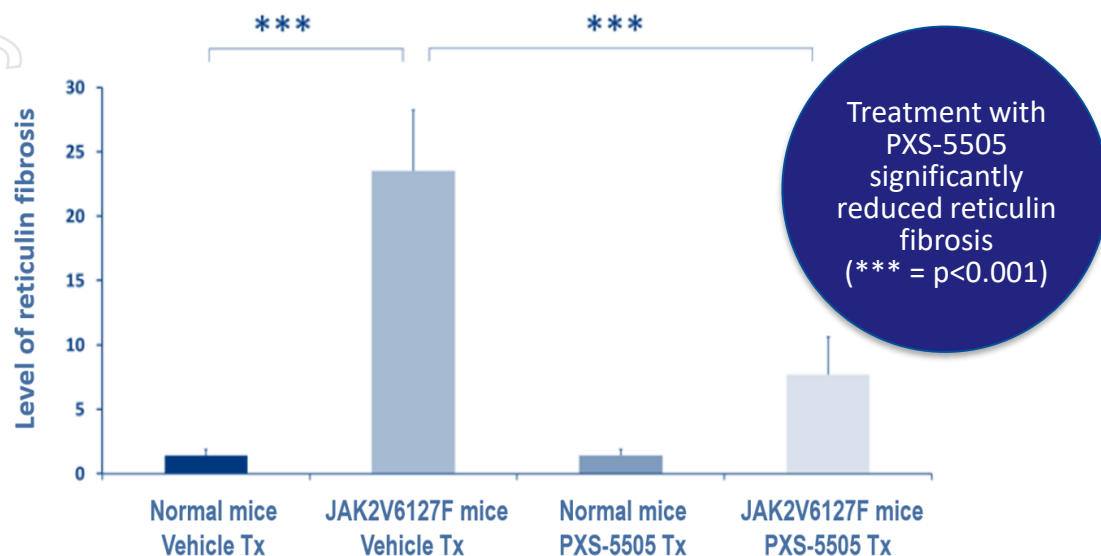
- **Anti scarring drug PXS-6302 clears phase 1 and ready for next step into patients**

- Phase 1 study of healthy volunteers at the University of Western Australia (UWA) in Perth demonstrated that PXS-6302 was well tolerated, produced a complete inhibition of the target enzymes in the skin and produced minimal inhibition of these same enzymes in the rest of the body.
- PXS-6302 will now progress to studies in patients with scars in the next quarter.

# PXS-5505; An effective and safe inhibitor of LOX in myelofibrosis patients

Pre clinical and clinical studies strongly support entry into long term phase 2 patient studies

## PXS-5505 attenuates hallmarks of primary myelofibrosis in mice

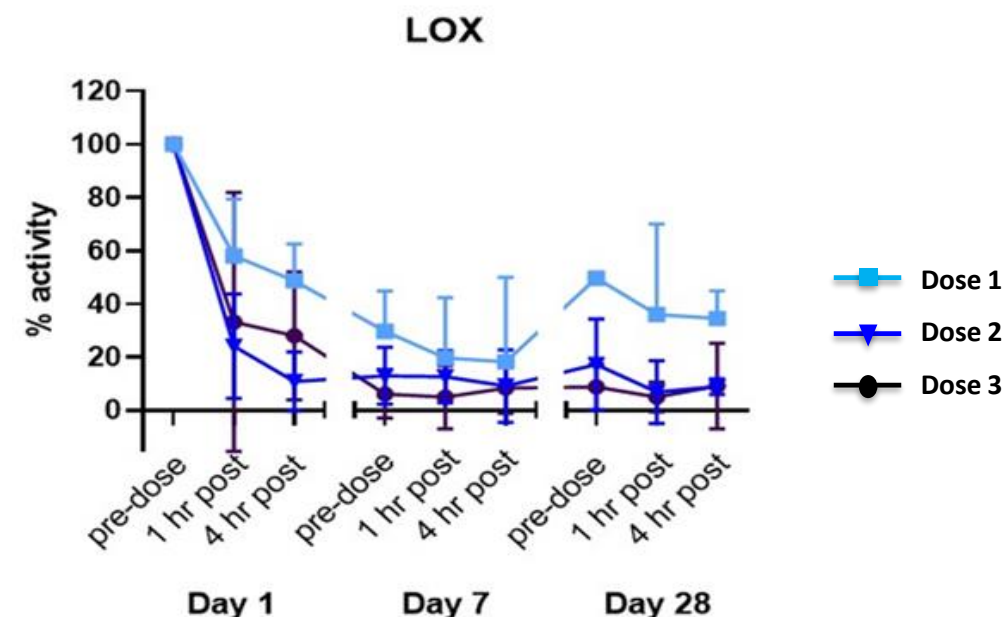


*"None of the drugs approved to date consistently or meaningfully alter the fibrosis that defines this disease. PXS-5505 has a novel mechanism of action by fully inhibiting all LOX enzymes.*

*Preliminary data thus far, demonstrate that PXS-5505 leads to a dramatic, >90% inhibition of LOX and LOXL2 at one week and 28 days. This confirms what's been shown in healthy controls as well as mouse models, that this drug can inhibit the LOX enzymes in patients. Inhibiting these enzymes is a novel approach to the treatment of myelofibrosis by preventing the deposition of fibrosis and ultimately reversing the fibrosis that characterizes this disease"*

Dr. Gabriela Hobbs<sup>1</sup>

## PXS-5505 – Phase 1c dose escalation in MF patients



- Open label dose expansion in JAK-inhibitor unsuitable<sup>2</sup> primary MF or post-ET/PV MF patients
- 3 patients on each dose for 28 days
- Good safety profile with no adverse events at highest dose
- >90% inhibition of LOX and LOXL2 at trough on highest dose at day 7 and 28

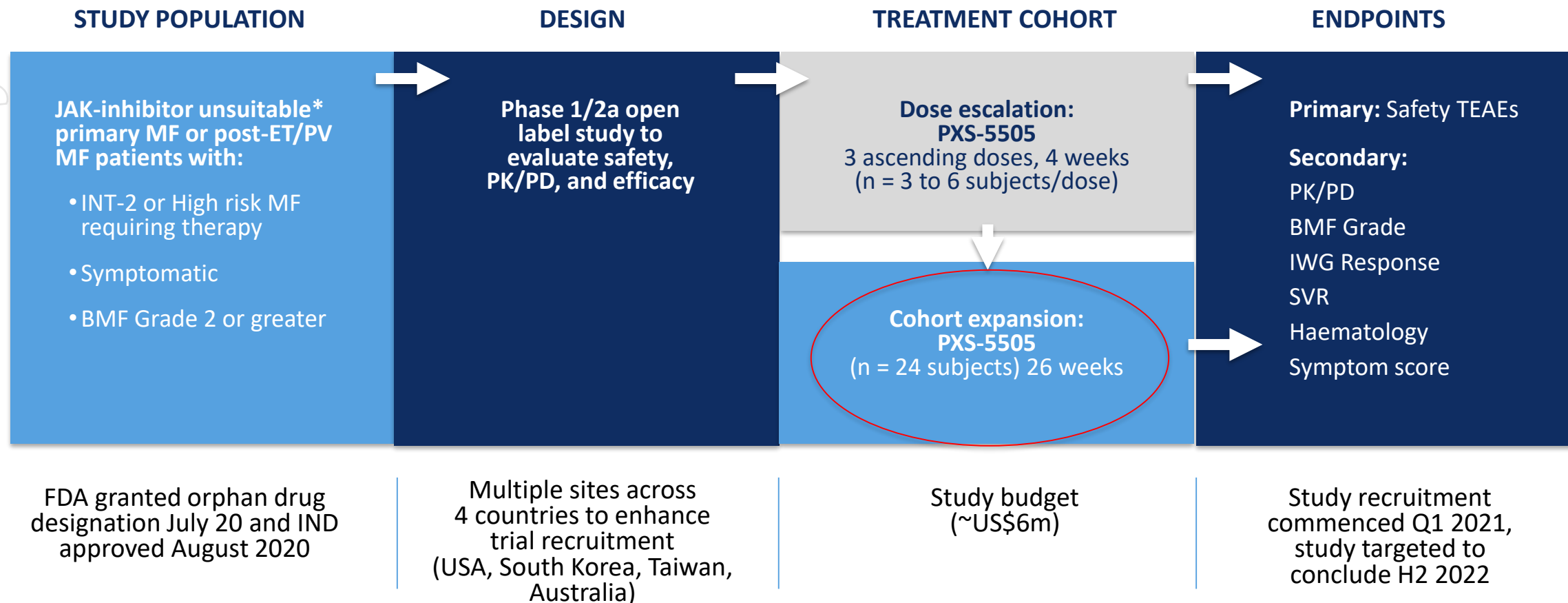
Ref Graph1: Leiva et al. Intl J Hemat 2019. <https://doi.org/10.1007/s12185-019-02751-6>

<sup>1</sup> Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital

<sup>2</sup> 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

# PXS-5505 Phase 1/2a Trial in myelofibrosis

6 month monotherapy study with meaningful safety and efficacy endpoints (phase 1c complete)



\*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

# Cash and capital structure

## Cash

- At 30 September A\$16m

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

### **Sale of Australian Bronchitol & Aridol distribution rights effective 1 July**

- A\$2m received July 2021

### **Further opportunities to extend cash runway**

- Previously announced (Russia) and potential cost savings from rationalization across mannitol business
- Pipeline supported by grants and R&D tax credit (~A\$5m 2020)
- Partnering deals with pipeline assets

## Share capital

- Current shares on issue 454m

## Enterprise value

- Market capitalisation at \$0.125 per share \$57m
- Less: net cash at 30 September (\$16m)
- Enterprise value \$41m

## Lead institutional shareholders

- BVF Partners LP 19.4%
- Karst Peak Capital Limited 12.1%
- D&A Income Limited 6.8%



# Anticipated news flow: 2021 – 2022

Multiple anticipated value inflection points over next two years

## Achieved 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

## Achieved H2 2021

- July 1: Sale of Australian Aridol and Bronchitol distribution rights
- Aug 5: University of Rochester paper – PXS-5505 significantly improves survival, delays tumor growth in pre-clinical cancer model
- Aug 17: Grant of option to Aptar for high payload inhaler – US\$275k fee, US\$2.5m exercise fee by 8/22
- Aug 31: Treatment to prevent wound and burns scars clears phase 1 trial – to progress into independent investigator phase 1c patient studies - burns and established scars
- PXS-5505 phase 1c shows good tolerability profile and strong inhibition of LOX and LOXL2

## H2 2021

- PXS-5505 phase 2a myelofibrosis study commences dosing
- LOX topical drug PXS-6302 commences independent investigator patient studies - burns and established scars
- Mannitol business simplification – realising annual cost savings
- PXS-5505 publications by KOL's in other cancers

## 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





developing breakthrough treatments for fibrosis and inflammation

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