

ersonal use only



Investor Presentation

PHARMAXIS RELEASES PROMISING INTERIM  
DATA FROM SKIN SCARRING STUDY

**pharmaxis**

developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | 26 September 2022  
Gary Phillips CEO

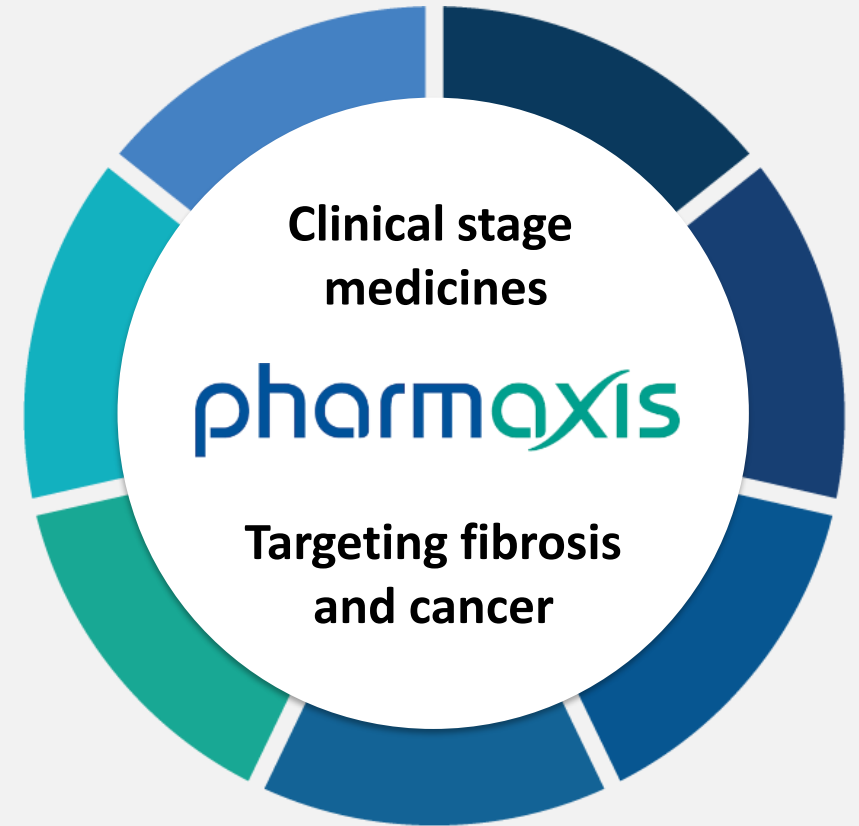
# Forward looking statement

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

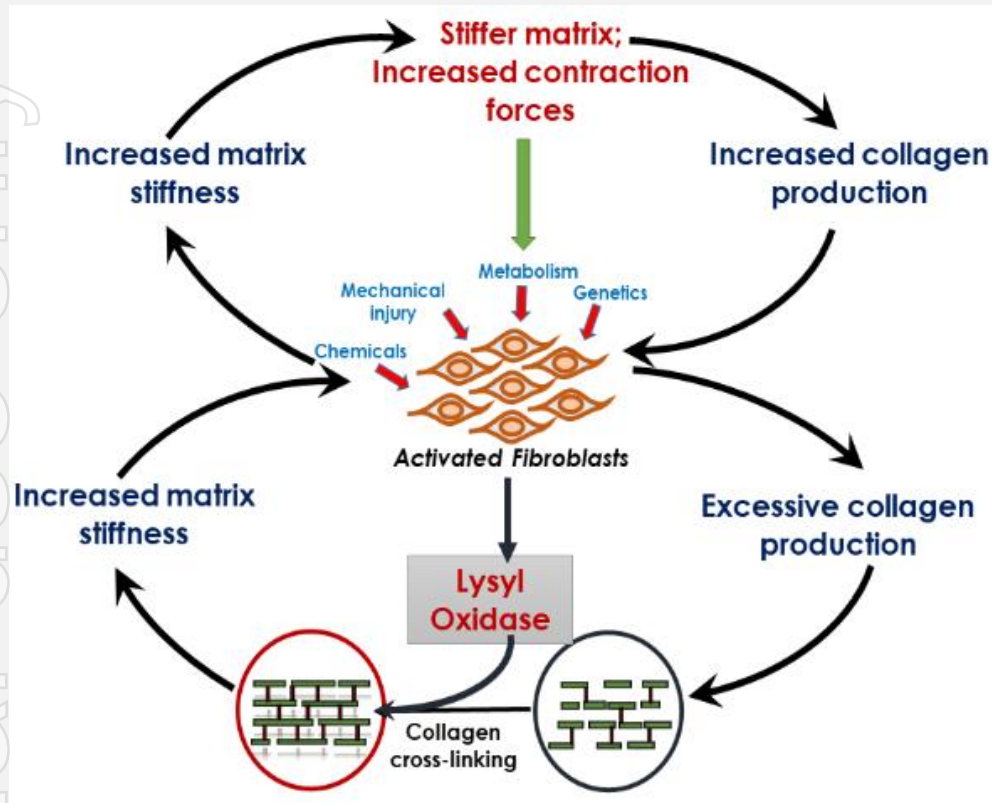
- Pharmaxis is a clinical stage drug development company targeting fibrosis and cancer indications with first in class or best in class small molecule drugs in markets of high value
- Pharmaxis is the global leader in fibrosis driven by lysyl oxidase enzymes having invested in a multi year research program leveraged with extensive external scientific collaborations
- Pharmaxis has 5 studies recruiting for 2022/2023 that will lead to near term value opportunities
  - Lead asset PXS-5505 is in a multinational phase 2 trial – a breakthrough clinical program with disease modifying potential in Myelofibrosis. > 50% recruited
  - US investigator led phase 2 trial in liver cancer with PXS-5505 as first line treatment added to existing chemotherapy to commence Q3 2022
  - Topical drug PXS-6302 trial in patients with potential to improve function and appearance of established scars. > 60% recruited
  - Additional PXS-6302 trial in scar prevention to commence recruitment in 1H 2023
  - Neuro inflammation drug PXS-4728 in phase 2 trial of patients with severe sleep disorder that leads to neurodegenerative diseases e.g. Parkinson's
- Specific corporate strategy delivering non-dilutive cash to fund development of clinical pipeline.
  - Orbital device, mannitol distribution and Parkinson's UK deals worth \$16m in 21 & 22.



# 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 is preventable through lysyl oxidase inhibition and at the heart of a true anti-fibrotic therapy

### ■ PXS-5505

- Oral dosage form – one capsule 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 1 data demonstrates a safe, well tolerated drug that gives full inhibition of LOX enzymes in the skin with minimal systemic exposure

# 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



*"In 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.

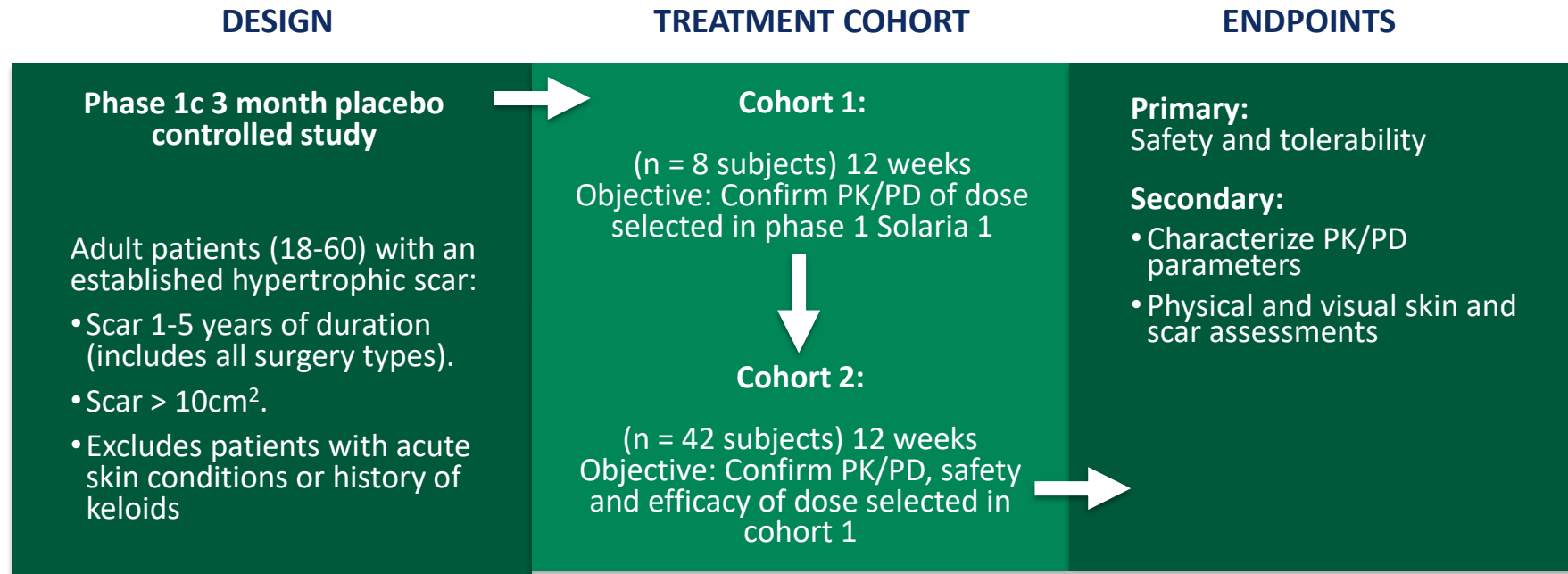
■ Commercial Opportunity

- 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 (Solaria 2) in established scars

3 month monotherapy study to assess dosage, tolerability and efficacy endpoints



Investigator initiated study (sponsor UWA) - long term collaboration with UWA to research and develop PXS-6302 supported by Australian NHMRC grants

Single site study in Perth Australia

Study budget to spend; A\$0.3m

Study recruitment commenced Q1 2022, study targeted to report H1 2023



# PXS-6302 Phase 1c Trial (Solaria 2) in established scars

3 month monotherapy study to assess dosage, tolerability and efficacy endpoints

DESIGN	TREATMENT COHORT	ENDPOINTS
<p><b>Phase 1c 3-month placebo controlled study</b></p> <p>Adult patients (18-60) with an established hypertrophic scar:</p> <ul style="list-style-type: none"><li>• Scar 1-5 years of duration (includes all surgery types).</li><li>• Scar &gt; 10cm<sup>2</sup>.</li><li>• Excludes patients with acute skin conditions or history of keloids</li></ul>	<p><b>Cohort 1:</b> Fully recruited</p> <p><b>Cohort 2:</b></p> <ul style="list-style-type: none"><li>• A total of 24 out of 42 patients have been enrolled</li><li>• Dosage regimen modified to reduce drug exposure but still maintain the overall high level of enzyme inhibition.</li></ul>	<p><b>Cohort 1:</b></p> <ul style="list-style-type: none"><li>• Skin biopsies show skin penetration and high inhibition of LOX</li><li>• Reduction in biomarkers of the scarring process suggests a disease modifying effect.</li><li>• Four patients withdrew after experiencing redness &amp; itchiness at the site of application that resolved on treatment cessation</li></ul>

*“We have noted positive changes in appearance and pliability of scars in those patients on active drug that now need to be confirmed by the results from the placebo controlled phase of this trial later this year.*

*We are learning a lot as we move from the promising pre-clinical work done at UWA and into the clinic where we have many patients who are in great need of a treatment that can improve both the cosmetic appearance of their scars and improve the functionality of their scarred skin; factors that have a huge impact on patient’s wellbeing.”*

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

# Anticipated news flow: 2022/2023

Multiple anticipated value inflection points

## Q4 2022

- PXS-5505 phase 1c liver cancer (HCC) study – starts recruitment
- PXS-5505 phase 2a myelofibrosis study – interim data
- PXS-5505 phase 2a myelofibrosis study – fully recruited
- PXS-5505 publications by KOL's in other cancers

## Q1 2023

- LOX topical drug PXS-6302 top line data from established scars study
- LOX topical drug PXS-6302 commences independent investigator patient studies – scar prevention
- PXS-4728 iRBD / neuro inflammation study commences recruitment

## Q2 2023

- PXS-5505 phase 2a myelofibrosis study completed and reports safety and efficacy data
- PXS-4728 iRBD / neuro inflammation study commences recruitment



Personal use only



# pharmaxis

developing breakthrough treatments for fibrosis and inflammation

Pharmaxis Ltd ABN 75 082 811 630

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



## Contacts

Gary Phillips  
Chief Executive Officer  
[gary.phillips@pharmaxis.com.au](mailto:gary.phillips@pharmaxis.com.au)

David McGarvey  
Chief Financial Officer  
[david.mcgarvey@pharmaxis.com.au](mailto:david.mcgarvey@pharmaxis.com.au)