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# PXS-6302 Solaria 2 Trial Results

**pharmaxis**

developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | 24 May 2023

Gary Phillips CEO

## Forward looking statement

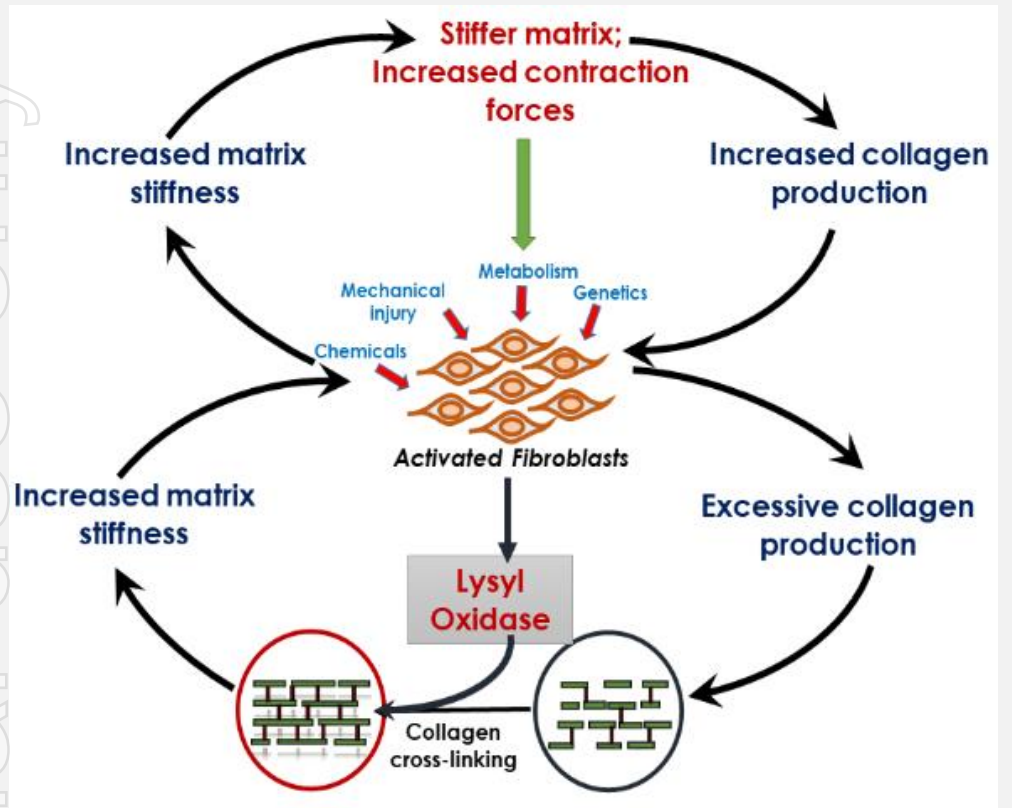
This document contains forward-looking statements, including statements concerning Pharmaxis' future financial position, plans, and the potential of its products and product candidates, which are based on information and assumptions available to Pharmaxis as of the date of this document. Actual results, 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.

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# 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

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# Established Scarring – PXS-6302 Phase 1c Trial (Solaria 2)

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

## DESIGN

## PATIENT DEMOGRAPHICS

## ENDPOINTS

<p><b>Phase 1c</b></p> <ul style="list-style-type: none"> <li>• 3 month</li> <li>• Objectives:             <ul style="list-style-type: none"> <li>○ Confirm PK/PD*, safety and efficacy of dose selected in dose escalation</li> </ul> </li> <li>• Double blind placebo controlled</li> </ul>	<p><b>42 Adult patients (18-60) with an established scar &gt; 1year:</b></p> <ul style="list-style-type: none"> <li>• Average age of scar; 12.8 years</li> <li>• Low to moderate severity</li> <li>• Included all surgery types.</li> <li>• Scar &gt; 10cm<sup>2</sup>.</li> <li>• Excluded patients with acute skin conditions or history of keloids</li> </ul>	<p><b>Primary:</b> Safety and tolerability</p> <p><b>Secondary:</b></p> <ul style="list-style-type: none"> <li>• Characterize PK/PD* parameters</li> </ul> <p><b>Exploratory:</b></p> <ul style="list-style-type: none"> <li>• Physical and visual skin and scar assessments</li> </ul>
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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 Completed March 2023

Study reported May 2023

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# PXS-6302 Phase 1c Trial (Solaria 2); Top line results

- **PXS-6302 was very well tolerated and demonstrated a good safety profile.**
  - No serious adverse events were reported
  - Two patients withdrew from the study; reversible rash
- **Mean inhibition of LOX activity 66% compared to baseline and placebo**
  - LOX measured 2 days post final dose
  - LOX is responsible for the cross linking of collagen fibres implicated in adverse scarring. (p<0.001)
- **Meaningful changes in the composition of the scars**
  - Patients in the active arm had a mean reduction in hydroxyproline 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\* score were seen between active and placebo groups after three months of treatment.

## PXS-6302 Phase 1c Trial (Solaria 2); Expert review

- Exploratory clinical study has significantly enhanced our understanding of the role of LOX enzymes in scarring and the scar process itself.
- PXS-6302 leads directly to an unprecedented change to the scar composition that we have not seen with any other form of treatment. We estimate that up to 50% of the excess collagen in these patients' scars has been removed.
- While the length of this Phase 1c safety study was not sufficient to change the appearance of an established scar the remodelling process will be ongoing and I'm confident we would see an improvement in scar appearance and physical characteristics if we observed them for longer.

### Professor Fiona Wood

Burns Service of Western Australia  
Director of the Burn Injury Research Unit  
University of Western Australia

# 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 (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

- 3 month phase 1c in established scars demonstrates good tolerability, full inhibition of LOX in skin and marked change in scar composition

■ 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); Next steps

- Positive data leads to extension of collaboration with Professor Wood's UWA team
- Wide vista of potential skin fibrosis indications opened up for clinical development. For example:
  - Younger scars
  - Scar prevention post surgery
  - Keloids
  - Dupuytren's
  - Surgical adhesions
- Further update on plans for skin scarring franchise mid 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 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 mid-year 2023	H1 2025

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# 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 haematological 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
- PXS-5505 phase 2a myelofibrosis monotherapy study – fully recruited
- PXS-4728 iRBD / neuro inflammation study commences recruitment



## H2 2023

- PXS-5505 phase 2a myelofibrosis study completed and reports safety and efficacy data
- PXS-5505 phase 2 myelofibrosis study add on to JAK inhibitor commences recruitment
- LOX topical drug PXS-6302 commences independent investigator patient studies – scar prevention

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