



Investor Presentation



developing breakthrough treatments for fibrosis and inflammation

Investor Presentation | 31 January 2023
Gary Phillips CEO

Forward looking statement

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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.

December 2022 Quarter Update

- **Positive interim data from cancer drug PXS-5505 myelofibrosis phase 2a study**
 - 18 out of 24 patients recruited
 - Very well tolerated with encouraging signs of clinical efficacy in patients unsuitable for JAK inhibitors
 - Key opinion leaders and Industry show interest at ASH and JPM
- **Interim data and completed recruitment from skin scarring drug PXS-6302 in phase 1c study**
 - Recruitment of 50 patients completed in Dec 2022; top line results due Q2 2023.
 - Sub group of patients on active drug show changes to scar structure and physical changes noted by treating physicians
 - Developing protocols for follow up studies in other scar related indications
- **Pharmaxis strengthens Board with two new appointments**
 - Dr. Simon Green
 - Mr. Hashan De Silva
- **1H 2023 to deliver major trial updates**
 - PXS-5505 myelofibrosis open label 24 patient 6 moth trial to report major data update in Q2 2023
 - PXS-6302 established scars placebo controlled 50 patient 3 month study to report top line results in Q2 2023

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

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

DESIGN	TREATMENT COHORT	ENDPOINTS
Phase 2a open label study to evaluate safety, PK/PD, and efficacy JAK-inhibitor unsuitable* primary MF or post-ET/PV MF patients with: • INT-2 or High risk MF requiring therapy • Symptomatic • BMF Grade 2 or greater • Median survival after JAK-inhibitor discontinuation; approximately 1 year	Cohort expansion: PXS-5505 (n = 24 subjects) 26 weeks • A total of 18 patients have been enrolled • 6 patients having completed 24 weeks of treatment. • 6 patients have dropped out of the study due to a lack of clinical response.	Primary: PXS5505 has been well tolerated with no serious treatment related adverse events reported. Secondary: • 2/6 patients show clinically important improvement in symptoms. • 5/6 patients show either stable or improved bone marrow fibrosis scores of ≥ 1 grade. • 5/6 have stable or improved platelet and/or haemoglobin scores • No reductions were seen in spleen volume



“PXS-5505 continues to be very well tolerated in the clinic with no serious treatment related adverse events reported.

Though still early in the dose expansion phase of the study, PXS5505 appears to be stabilising and in some cases, improving the hemoglobin and platelet counts, which has also been associated with symptom improvements in those patients that were treated to 24 weeks.

This is encouraging given the poor prognosis seen after ruxolitinib discontinuation with a median overall survival of only 11-14 months typical of this study population. These results support further clinical investigation of PXS5505 in myelofibrosis.”

Dr Gabriela Hobbs MD,
Assistant Professor, Medicine, Harvard
Medical School & Clinical Director,
Leukemia Service, Massachusetts General
Hospital

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

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

DESIGN	TREATMENT COHORT	ENDPOINTS
Phase 1c 3-month placebo controlled study Adult patients (18-60) with an established hypertrophic scar: • Scar 1-5 years of duration (includes all surgery types). • Scar > 10cm². • Excludes patients with acute skin conditions or history of keloids	Cohort 1: Fully recruited Cohort 2: • A total of 38 out of 42 patients have been enrolled • Dosage regimen modified to reduce drug exposure but still maintain the overall high level of enzyme inhibition.	Cohort 1: • Skin biopsies show skin penetration and high inhibition of LOX • Reduction in biomarkers of the scarring process suggests a disease modifying effect. • Clinician notes positive changes in appearance and pliability • Four patients withdrew after experiencing redness & itchiness at the site of application that resolved on treatment cessation and informed the decision to reduce dosage frequency for Cohort 2

“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

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
	Hepatocellular Carcinoma (HCC)	\$7 billion	Phase 1c open label dose escalation study in newly diagnosed patients with unresectable HCC on top of standard of care (PD-L1 inhibitor + anti VEGF)	18	First Patient Q1 2023	2024
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	Fully Recruited	H1 2023
	Scar prevention post surgery	\$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 H1 2023	H1 2025

Board

Significant international pharmaceutical experience



Malcolm McComas – Chair

- Former investment banker and commercial lawyer
- Former MD Citi Group
- Has worked with many high growth companies across various industry sectors and has experience in equity and debt finance, acquisitions and divestments and privatisations
- Joined Pharmaxis Board in 2003
- Chair since 2012



Gary Phillips – Chief Executive Officer

- 30+ years' of operational management experience in the pharmaceutical and healthcare industry in Europe, Asia and Australia
- Joined Pharmaxis in 2003 and was appointed Chief Executive Officer in March 2013 at which time he was Chief Operating Officer
- Previously held country and regional management roles at Novartis – Hungary, Asia Pacific and Australia



Dr Kathleen Metters – Non-Executive Director

- Former Senior Vice President and Head of Worldwide Basic Research for Merck & Co. with oversight of all the company's global research projects
- In a subsequent role at Merck & Co she led work on External Discovery and Preclinical Sciences
- Former CEO of biopharmaceutical company Lycera Corp



Dr Neil Graham – Non-Executive Director

- Former VP of immunology and inflammation responsible for strategic program direction overseeing pipeline development and clinical programs at Regeneron (REGN:US)
- Former SVP program and portfolio management at Vertex Pharmaceuticals
- Former Chief Medical Officer at Trimeris Inc and Tibotec Pharmaceuticals



Dr Simon Green – Non-Executive Director

- Experienced senior global pharma executive with 30 years' of experience in the biotechnology industry.
- Actively involved in CSL's global expansion over a 17-year period where he held roles as Senior Vice President, Global Plasma R&D and General Manager of CSL's manufacturing plants in Germany and Australia.
- Prior to joining CSL he worked in the USA at leading biotechnology companies Genentech Inc and Chiron Corporation.



Hashan De Silva – Non-Executive Director

- Experienced life sciences investment professional with extensive knowledge of the biotech, pharmaceutical and medical technology sectors.
- Worked as associate healthcare analyst at Macquarie Group and lead healthcare analyst at CLSA Australia before joining Karst Peak Capital in February 2021 as head of healthcare research.
- Prior to moving into life science investment Hashan worked at Eli Lilly in various roles focused on the commercialisation of new and existing pharmaceuticals.

News flow

Anticipated news flow

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



Q1 2023

- PXS-5505 phase 1c liver cancer (HCC) study – starts recruitment
- PXS-5505 phase 2a myelofibrosis study – fully recruited



Q2 2023

- PXS-5505: significant data update
- LOX topical drug PXS-6302 commences independent investigator patient studies – scar prevention
- LOX topical drug PXS-6302 top line data from established scars study
- PXS-5505 publications by KOL's in other cancers
- PXS-4728 iRBD / neuro inflammation study commences recruitment



H2 2023

- PXS-5505 phase 2a myelofibrosis study completed and reports safety and efficacy data



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