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Investor Presentation

pharmaxis

Investor Presentation | 06 September 2022
Gary Phillips CEO

developing breakthrough treatments for fibrosis and inflammation

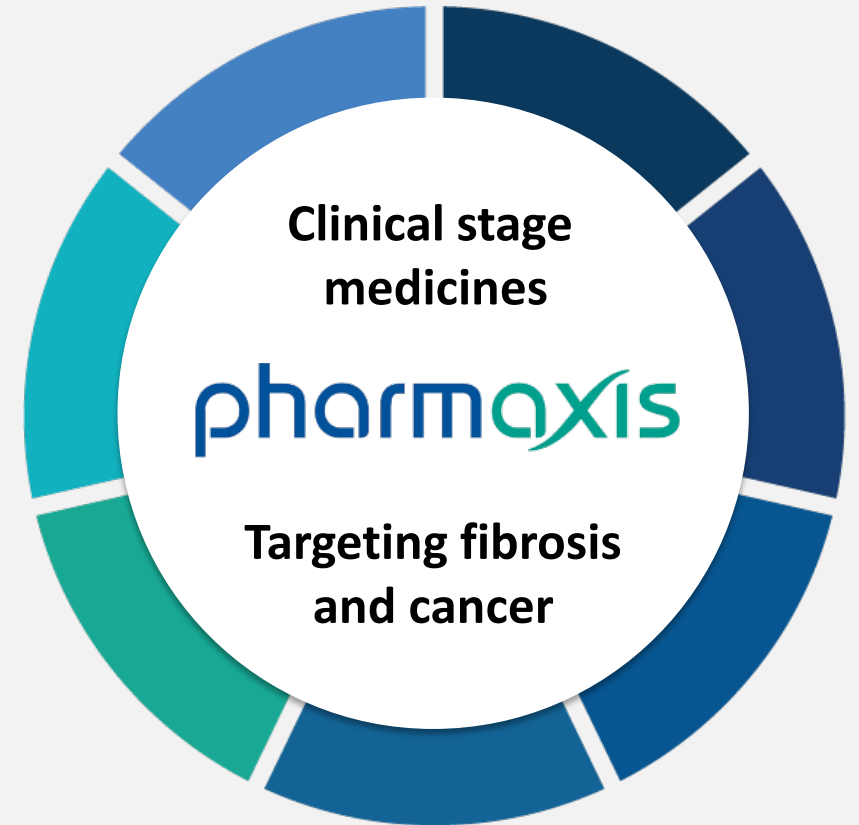
Forward looking statement

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 2021/22.



Experienced Scientific Leadership Team

Significant global experience in drug development, commercialisation and partnering

In senior management



Wolfgang Jarolimek – Drug Discovery

- 20+ years' experience in pharmaceutical drug discovery and published more than 30 peer reviewed articles
- Previously Director of Assay Development and Compound Profiling at the GlaxoSmithKline Centre of Excellence in Drug Discovery in Verona, Italy
- Spent 8 years as post-doc at the Max-Planck Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany



Dieter Hamprecht – Head of Chemistry

- 20+ years' experience with small molecule and peptide drug discovery, contributed to greater than 10 drug candidates brought to development and co-inventor of 50 patent families, co-author of 30+ scientific publications
- Previously Managing Director – Boehringer Ingelheim's research group in Milan
- Senior medicinal chemistry positions at GSK



Jana Baskar – Chief Medical Officer

- 20+ years' experience both in clinical medicine and the biopharmaceutical industry
- Broad therapeutic knowledge and significant clinical research expertise having worked in several different specialties
- Former Medical Director at Novartis Oncology in Australia; former Medical Director for IQVIA in Australia and New Zealand

On the board



Gary Phillips – CEO and Managing Director

- 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



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



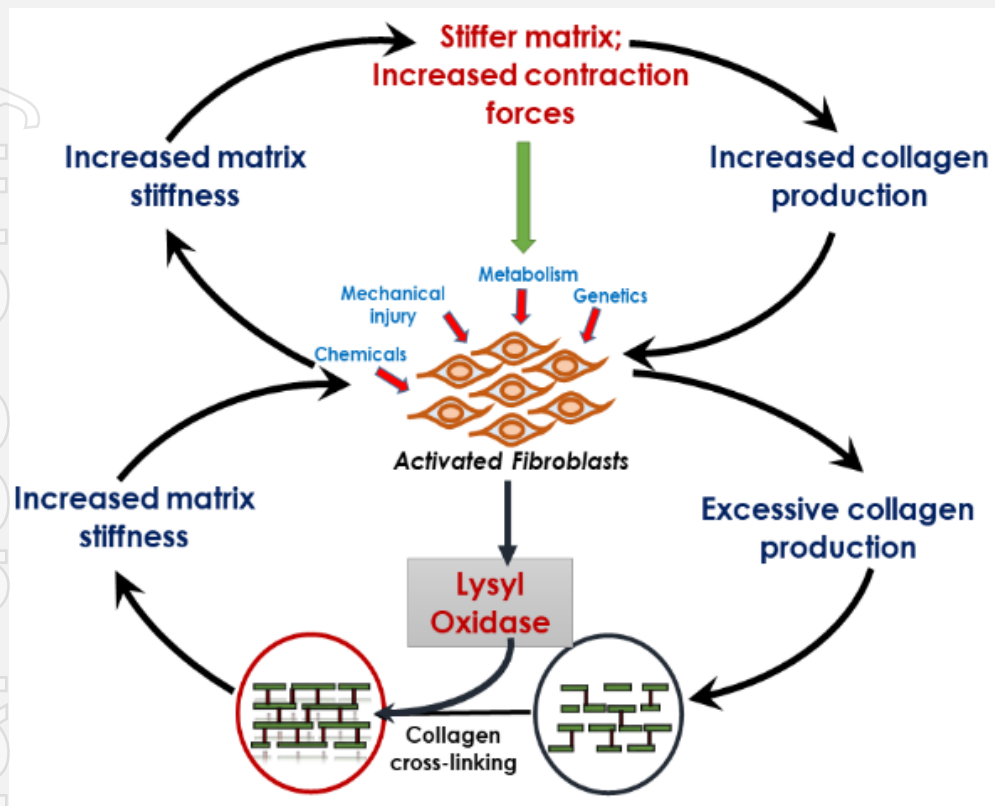
Neil Graham – Non Executive Director

- 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

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 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

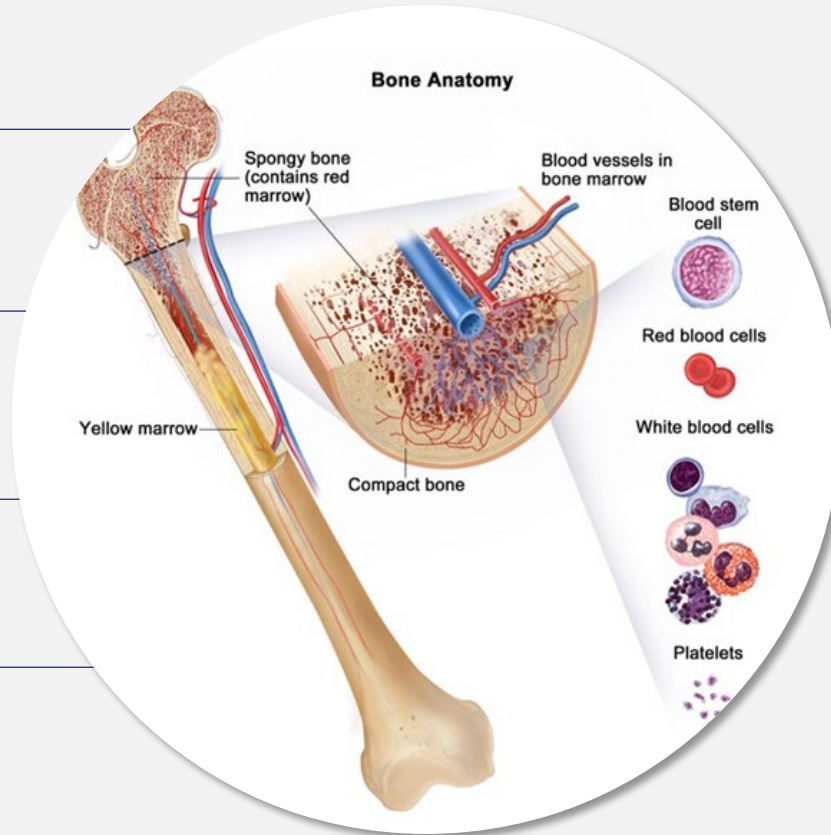
- Topical dosage form
- Patent 2019
- Strong pre clinical evidence in models of skin fibrosis and scarring
- Potential in prevention of scar formation and modification of existing scars
- Phase 1 data demonstrates a safe, well tolerated drug that gives full inhibition of LOX enzymes in the skin with minimal systemic exposure

Myelofibrosis background

A rare type of bone marrow cancer that disrupts your body's normal production of blood cells

KEY FACTS

- Affects 15 in 1m people worldwide
- 5 Years Median survival
- Age of onset 50 – 80
- 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

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

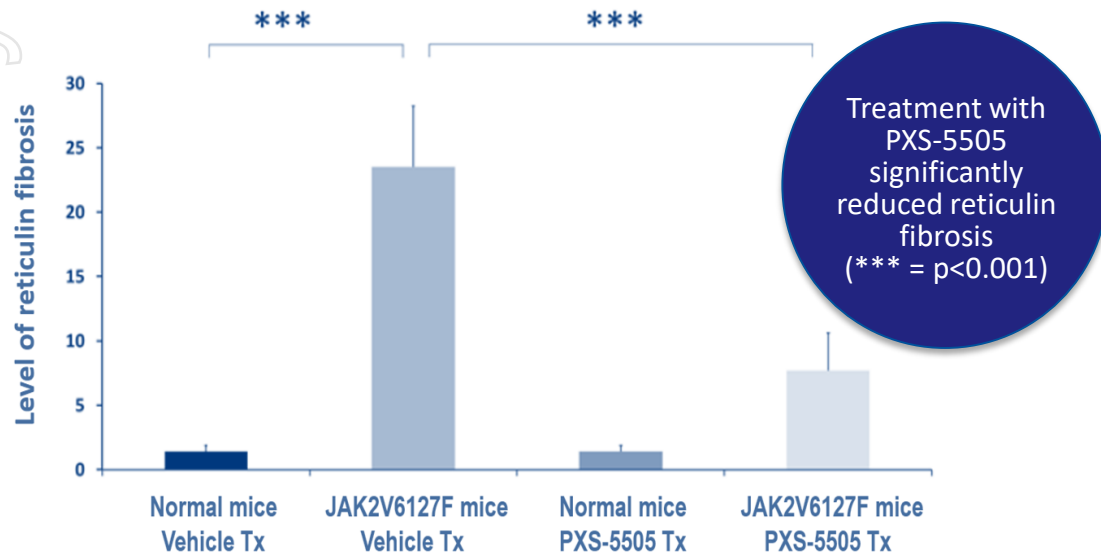
Commercial Opportunity

- Current standard of care; revenue ~US\$1b per annum

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

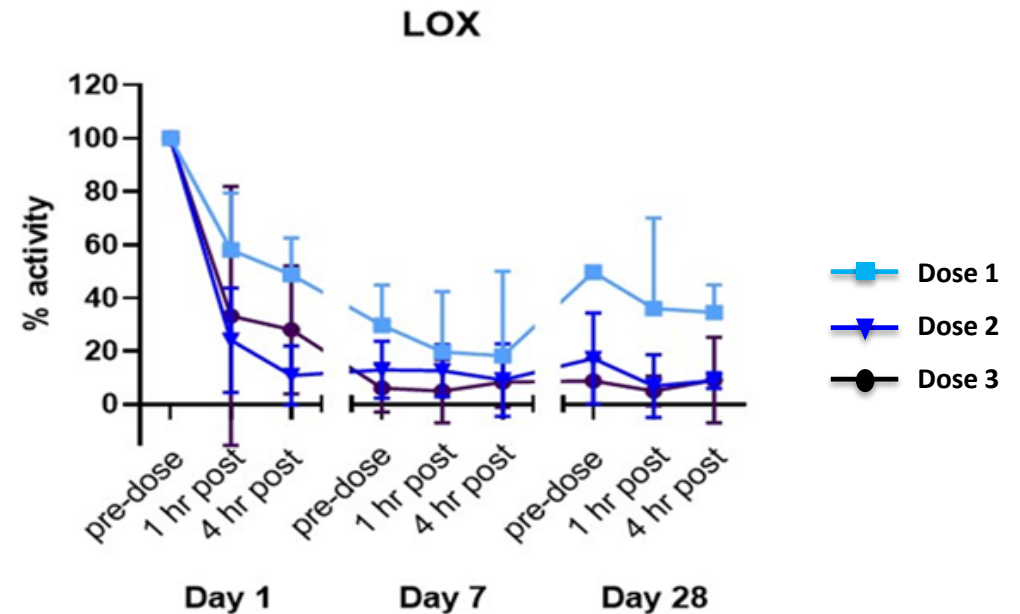


"None of the drugs approved to date consistently or meaningfully alter the fibrosis that defines this disease. PXS-5505 has a novel mechanism of action by fully inhibiting all LOX enzymes.

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 Hobbs¹

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



- Open label dose expansion in JAK-inhibitor unsuitable² primary MF or post-ET/PV MF patients
- Maximum of 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>

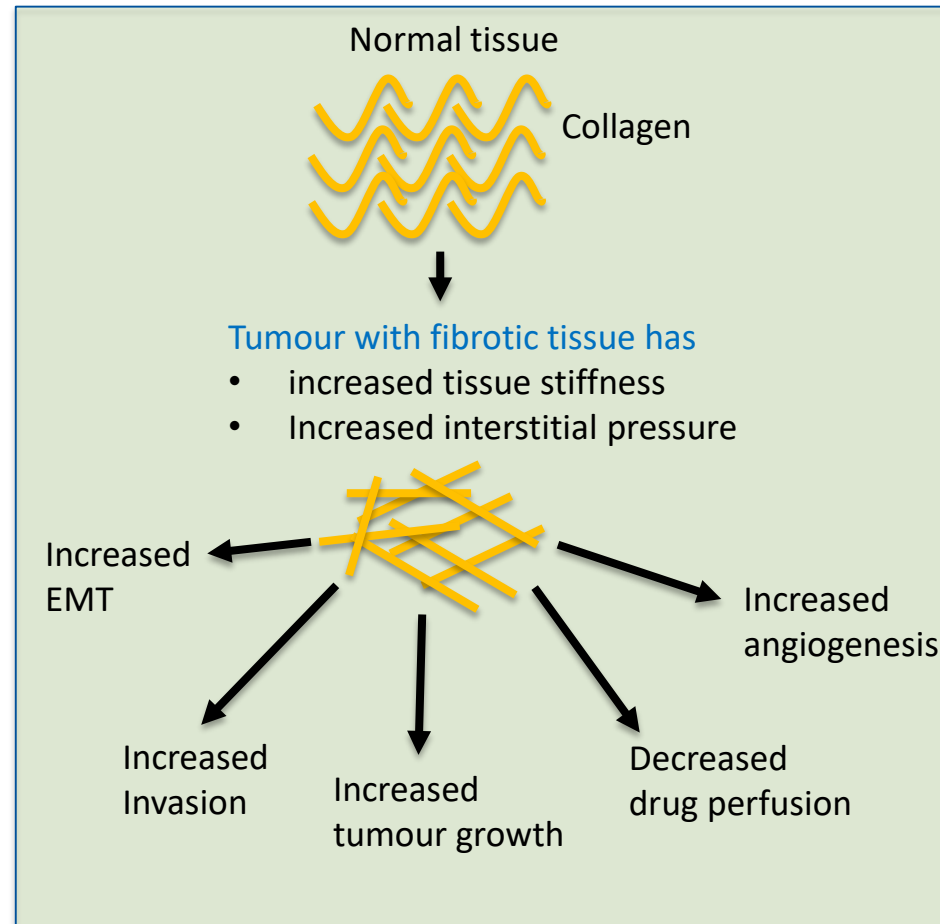
¹ Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital

² 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

Hepatocellular Carcinoma (HCC)

4th leading cause of cancer-related mortality worldwide with a 19.6% 5-year relative survival

- Primary liver malignancies have doubled in incidence over the last two decades.
- 4th leading cause of cancer-related mortality worldwide with a 19.6% 5-year relative survival
- HCC is a stromal (fibrotic) tumour
 - Accumulation of collagen cross-links increases stromal stiffening and interstitial fluid pressure reducing delivery of chemotherapy and immunotherapy
- Current standard of care
 - 20-30% are resectable at presentation with many patients relying on systemic therapy:
 - Tyrosine kinase inhibitors
 - PD-L1 inhibitors + anti-VEGF



- Pre-clinical data (Rochester Uni; Aug 2021)
 - Tumour tissue specimens show LOX enzymes are significantly elevated in human liver cancer and correlate with poor prognosis.
 - PXS-5505 with or without chemotherapy treatment in a pre-clinical model significantly **improves survival, delays tumor growth, and reduces intratumoral pressure.**
- **Commercial Opportunity**
 - Drugs market currently worth ~US\$2bn with rising incidence forecasted to drive growth to ~US\$7bn by 2027

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



“We now understand from our research that even scars which are stable and many years old are in fact replenishing a significant proportion of mature, stiff collagen in a matter of a few months.”

- 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 (data on file)

■ Clinical evidence

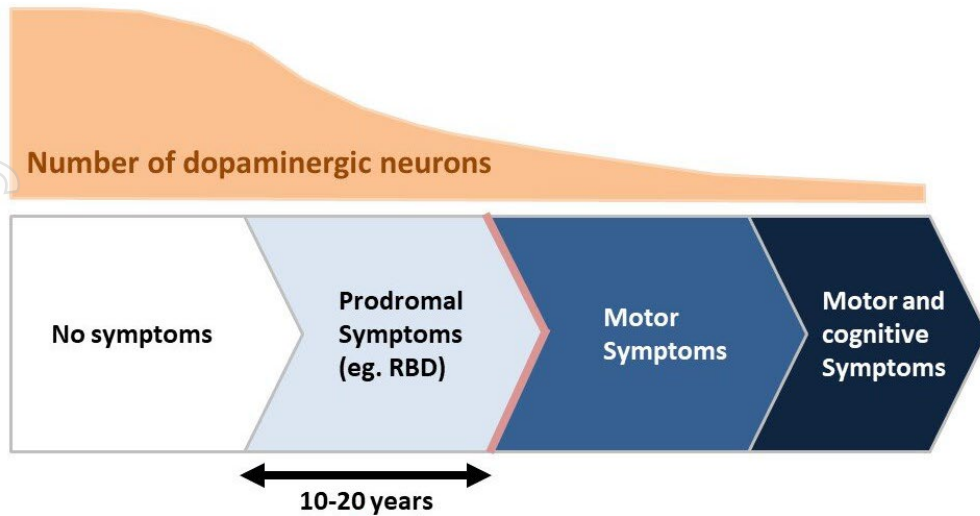
- 1 month phase 1a in healthy volunteers demonstrates good tolerability and full inhibition of LOX in skin.

■ Commercial Opportunity

- Total scar treatment market in 2019 exceeded US\$19b. Keloid and hypertrophic scar segment ~US\$3.5b

Using a sleep disorder to target Parkinson's Disease

SSAO inhibition proven effective mechanism against neuro inflammation and is neuro protective in pre clinical models

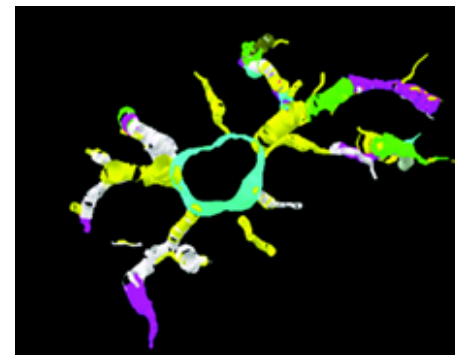


Parkinson's Disease and RBD

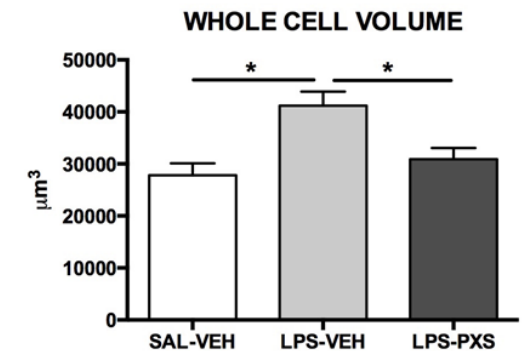
- More than 50% of dopaminergic neurons in the substantia nigra are lost at the onset of motor symptoms in PD.
- Prodromal symptoms, such as REM sleep behavior disorder (RBD), precede the onset of motor cognitive dysfunction by 10-20 years.
- 70% of iRBD patients transition to a neurodegenerative disease such as Parkinson's disease and Dementia with Lewy Bodies

PXS-4728 and neuro inflammation

- PXS-4728 has already undergone extensive development by Boehringer Ingelheim
- PXS-4728 inhibits SSAO and MAOB in the brain – both of which play a role in neurodegenerative diseases such as Parkinson's.
- Dual SSAO & MAO-B inhibition protects against neuronal degradation in pre clinical models²
- MAO-B inhibition alone (selegiline) does not offer any protection in the same model²



Activated microglia - reconstruction



Change in Microglia whole cell volume in the Substantia Nigra (SN) after LPS¹

Parkinson's UK to fund clinical trial in precursor to Parkinson's

PXS-4728 to proceed to phase 2 trial

Short and longer term commercial opportunities

- Current standard of care for iRBD is melatonin. There remains a high unmet need.
- >8% of 70 – 89 year olds have iRBD
- >70 % of iRBD patients develop Parkinson's disease and the related α -synuclein deposition disorders, dementia with Lewy bodies (DLB) and multiple system atrophy (MSA).

Clinical Trial

- 40 patients
- Randomised, double blind, placebo controlled clinical trial with iRBD
- 12 weeks of treatment with oral PXS-4728
- Two sites - University of Sydney and the University of Oxford
- Expected to commence dosing in H1 2023
- Efficacy endpoints for iRBD and neuroinflammation

Parkinson's UK Funding Agreement

- The funding agreement with Parkinson's UK entails up to £2.9m (~A\$5m) to be paid to Pharmaxis to run the phase 2 trial with advance payments received as the trial progresses.
- Pharmaxis is providing the study drug and the compound that will be used to measure inflammation in the brain scan of trial participants. The total is expected to cost approximately A\$5.8 million.
- Parkinson's UK will receive a return of up to 4 times their funding from royalties on future revenue Pharmaxis receives from commercialising PXS-4728 in neurological diseases and up to 2 times in other indications.

**Parkinson's
Virtual Biotech**

Four 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 2H 2022 Full data 1H 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 Q3 2022	1H 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	Recruiting	Q4 2022
	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 H1 2023	1H 2024
PXS-4728	REM sleep disorder and neuro inflammation		Phase 2 double blind, placebo controlled study in patients with iRBD	40	First patient H1 2023	2024

Aptar acquires Orbital high payload inhalation device

Further non-dilutive funds from Mannitol business unit



The Orbital technology is built on Pharmaxis patents, which allow powder payloads of up to 400mg or more to be inhaled by patients in divided doses without the need to reload. This unique platform was originally developed as a life cycle extending product for the Pharmaxis cystic fibrosis drug Bronchitol® (mannitol). However, it also meets an increasing global need to deliver high doses of other drugs, such as antibiotics, to the lungs.

- Orbital is a unique dry powder respiratory inhaler developed by Pharmaxis that delivers large payloads of drug in a convenient easy-to-use format.
- Following 12 months of technical and commercial evaluation, drug delivery solutions developer Aptar Pharma has paid US\$5.0 million (A\$ 7 million) to acquire the “Orbital” technology outright.
- Pharmaxis retains rights to inhaled mannitol products delivered via the Orbital inhaler. Pharmaxis has cross licensing rights in relation to use of the device in mannitol.

Shareholders & cash



Financial Information	31 Aug 22
ASX Code	PXS
Share price	\$0.076
Liquidity (turnover last 12 months)	100m shares
Market Cap	A\$42m
Pro forma ¹ cash balance (30 June 2022)	A\$21
Enterprise value	A\$21m
Clinical development program supported by:	
<ul style="list-style-type: none"> • Mannitol business* forecast to provide ongoing positive EBITDA growing to \$5m in 5 - 6 years • R&D tax credits • Strategy of partnering deals with pipeline assets 	
<ol style="list-style-type: none"> 1. Proforma cash includes cash of \$9m, estimated 2022 R&D tax credit of \$5 million (expected receipt H2 CY22), and Aptar option exercise fee A\$7m received August 2022 	

Institutional Ownership	30 June 22
BVF Partners LP	18.7%
Karst Peak Capital Limited	12.4%
D&A Income Limited	7.4%
Total Institutional Ownership	40.0%

Share Price



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
- LOX topical drug PXS-6302 top line data from established scars study
- PXS-6302 publications by KOL's in scarring

Q1 2023

- 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

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