



# Investor Presentation

Gary Phillips CEO

30 January 2018

# 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. 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. 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.

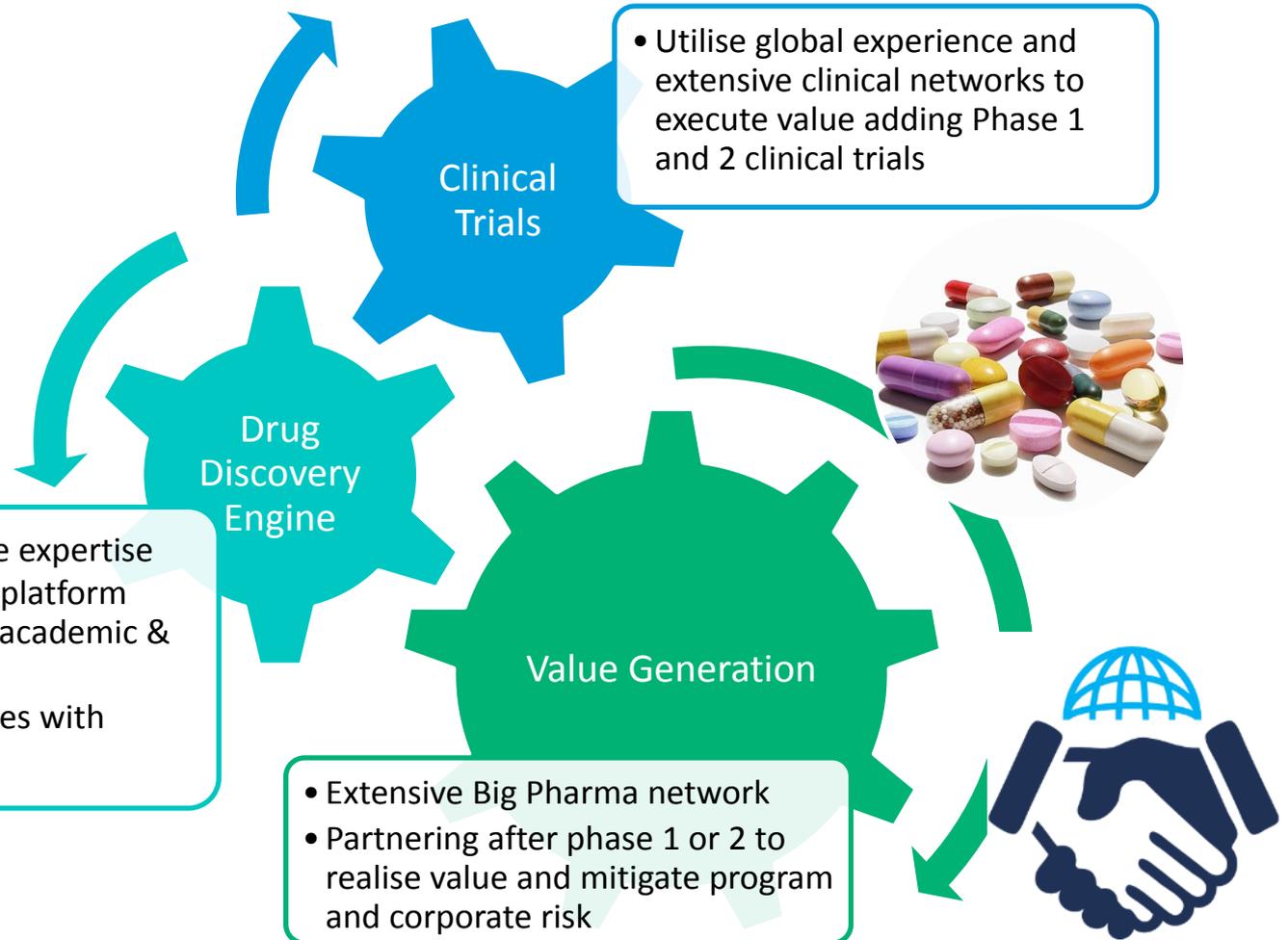
# Pharmaxis overview

A global leader in drug development for fibrosis & inflammation

- A portfolio of oral small molecule drugs across various stages of development in diseases with large markets and high unmet need managed by a globally respected translational development team
- A proven track record of achieving globally competitive partnering deals with multinational Pharmaceutical companies
- \$83m received to date from benchmark deal concluded with Boehringer Ingelheim for two diabetes related conditions worth potential \$600m+ plus sales related payments (% and milestones)
- Growing revenues from approved product sales (A\$2.5m in H1 FY18) & milestones (A\$42m FYTD 2018)
- Strong balance sheet - A\$25m cash at Dec 17 (plus A\$15m milestone received Jan 2018)
- Purpose built manufacturing and research facility in Sydney
- Strong institutional share register; including offshore specialist biotech funds

# pharmaxis

Pharmaxis has a successful track record of research, development and commercialisation of human healthcare products for the treatment and management of fibrotic and inflammatory diseases



# Pharmaxis portfolio

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
<b>Commercial</b>								
Bronchitol® US	Cystic fibrosis	Phase 3 trial met primary endpoint in 2017. Subject to FDA approval launch commercially in the US in 2018. Chiesi has responsibility (incurring all costs) for completing the New Drug Application with the FDA and US commercialisation.						
Bronchitol RoW	Cystic fibrosis	Bronchitol is currently sold in the UK, Germany and Italy by Chiesi; other European countries and Russia by specialist distributors and Australia by PXS. Bronchitol & Aridol business segment expected to transition to profitability over the next 12 to 24 months irrespective of any approval in the US – timing dependent on Russian sales growth. A\$3.5m revenue in CY17						Distributors
Aridol®	Asthma diagnosis	Aridol is approved and sold in Australia, South Korea and a number of European countries. Scheduled to re-enter US market in CY 2018 with specialist distributor and Canada in 2019. A\$2m revenue in CY17.						Direct & Dist
<b>In the clinic</b>								
SSAO (PXS-4728A)	NASH	Sold to Boehringer Ingelheim in May 2015. Phase 2a trial commenced August 2017. Total potential milestone payments of A\$290m during development program and further royalties and sales related milestones following approval. PXS received payments of A\$68m to date.						
SSAO (PXS-4728A)	Diabetic retinopathy	Boehringer commenced dosing a Phase 2 trial in January 2018. Total potential milestone payments of A\$290m during development and further royalties and sales related milestones following approval. PXS received A\$15m to date.						
LOXL-2	NASH, fibrosis - liver, lung, kidney	Phase 1 trials commenced in H2 2017 in 2 compounds. Expected to partner at end of Phase 1 – H2 2018.						
<b>Discovery</b>								
SSAO/MPO	Respiratory & cardiovascular	Dual inhibitor anti-inflammatory. Commenced pre-clinical tox Q4 2017. Targeting Phase 1 trial in 2018						
LOX	Scarring; cancer	Anti-fibrotic. Commenced pre-clinical tox studies Q4 2017. Targeting Phase 1 trial in 2018						

# Senior management

Significant experience in drug development, commercialisation and partnering



## Gary Phillips – CEO

- more than 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



## Wolfgang Jarolimek – Drug Discovery

- more than 18 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



## David McGarvey – CFO

- more than 30 years' experience building Australian based companies from inception to globally successful enterprises
- joined Pharmaxis as Chief Financial Officer and Company Secretary in December 2002
- previously Chief Financial Officer of the Filtration and Separations Division of US Filter (1998-2002), and Memtec Limited (1985-1998)
- commenced career at PriceWaterhouseCoopers



## Kristen Morgan – Alliance Management

- responsibility for alliance management and medical and regulatory affairs
- more than 19 years' experience in the pharmaceutical industry having previously held a senior role in medical affairs at Sanofi-Aventis, and a commercial sales role at GlaxoSmithKline.



## Brett Charlton - Medical

- more than 25 years experience in clinical trial design and management
- author of more than 80 scientific papers
- founding Medical Director of the National Health Sciences Centre
- previously held various positions with the Australian National University, Stanford University, the Baxter Centre for Medical Research, Royal Melbourne Hospital, and the Walter and Eliza Hall Institute

## Board of Directors

- **Malcolm McComas – Chair**
  - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- **Will Delaat – Non executive director**
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- **Simon Buckingham – Non executive director**
  - former President Global Corporate and Business Development at Actellion
- **Gary Phillips – Chief executive officer and managing director**
- **Kathleen Metters – Non executive director**
  - former head of global research at Merck

# Key catalysts for 2018

## Pharmaxis value driving events

1. Boehringer Ingelheim acquired drug (BI 1467335) reports clinical proof of concept in two major diseases as Phase 2 trials report in Q3 and Q4 2018
2. Pharmaxis owned anti fibrotic program realises significant valuation inflection point after completing first in man Phase 1 clinical studies in Q2 2018; major partnering deal planned H2 2018
3. Others
  - a) Bronchitol FDA re-submission by Chiesi in 2018
  - b) Pharmaxis portfolio valuation further boosted by two in house research programs reaching first in man phase 1 clinical study stage in H2 2018
  - c) Evaluating external opportunities for in-license or acquisition of new programs in fibrosis and inflammation



# Catalyst 1: Phase 2 trials to show clinical proof of concept in H2 2018

Boehringer Ingelheim responsible for clinical development and commercialisation

## NASH

- Phase 2a trial to report Q3 2018 – proof of efficacy in patients with moderate – severe disease
- Deutsche bank estimate market size of US\$35b by 2025
- First in class anti inflammatory SSAO inhibitor for NASH with sales potential of ~US\$2b [Analyst's estimate]

## Diabetic Retinopathy

- Phase 2a SSAO diabetic retinopathy to report Q4 2018 – proof of efficacy in patients with early stage disease
- Affects one third of diabetic patients world wide
- No approved treatments for early stage disease
- First in class anti inflammatory SSAO inhibitor for DR with sales potential of ~US\$800m [Analyst's estimate]

# Catalyst 1: Boehringer Ingelheim deal

Deal structure illustrates value generating potential of Pharmaxis business model

## First indication (NASH)

Commencement of  
phase 2  
€18m

Commencement of  
phase 3  
€37m

Filing, regulatory &  
pricing approvals  
€140m

**PLUS earn-out payments  
on annual net sales**

- Tiered percentages increasing from high single digits
- Plus sales milestones

Upfront  
(2015)  
€29m

## Second indication (diabetic retinopathy)

Commencement of  
phase 2  
€10m

Commencement of  
phase 3  
€25m

Filing, regulatory &  
pricing approvals  
€160m

Total Potential  
Milestones  
€419  
(~A\$625m)

- €57m (A\$83m) already received
- No further investment required from Pharmaxis

# Catalyst 2: LOXL2 inhibitor program approaches “deal ready” status

Key value drivers for H2 2018 deal

Feature	What do Pharma value?	PXS program status	
Disease target	Independent validation	Multiple peer reviewed publications	✓
Pre clinical proof of concept	2 or more different animal models	9 different models across 5 different diseases.	✓
Drug like qualities	No development flags	Cleared to develop	✓
Dosing regimen	Ease of use	Oral once a day tablet or capsule	✓
Patent	<ul style="list-style-type: none"> <li>• Composition of matter</li> <li>• As long as possible</li> </ul>	<ul style="list-style-type: none"> <li>• Composition of matter</li> <li>• 2016 filing date; 100% PXS owned</li> </ul>	✓
Cost of Goods	Low	Small molecule with easy synthesis	✓
# Compounds	1 plus backups	2 lead candidates plus back ups	✓
Toxicity	Wide therapeutic window As long as possible	28 day tox studies complete 13 week studies scheduled	✓
Clinical phase	Phase 1 with target engagement	Phase 1 commenced Q4 17	

**UPDATE:** Pharmaxis proprietary assay demonstrates significant enzyme inhibition at early stage of phase 1 studies

# Catalyst 2: LOXL2 inhibitor program - Deal Comparators

Anti Fibrotic market remains attractive

- Asset acquisition
  - Gilead acquisition of Nimbus asset for NASH post phase 1:- US\$1.2b
- Historical deal size in NASH market
  - Total average deal size: ~US\$850m
  - Upfront payments for phase 1 assets: US\$50m+
- Pharmaxis deal with Boehringer Ingelheim in 2015
  - Total deal size: US\$645m
  - Upfront payments : US\$33m

**UPDATE:** Pharmaxis has entered 'confidential due diligence' phase of partnering process with multiple large Pharma companies

# Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap <sup>1</sup>	\$86m
Shares on Issue	320m
Employee Options	14m
Liquidity (turnover last 12 months) <sup>1</sup>	62m shares
Share price <sup>1</sup>	\$0.27
Analyst valuation <sup>2</sup>	\$0.56
Cash Balance (proforma <sup>3</sup> 31 Dec)	\$40m

Institutional Ownership	%
BVF Partners (US)	20%
Australian Ethical	11%
Allan Gray	6%
Montoya Investments (UK)	6%
Other Institutions	8%
<b>Total Institutional Ownership</b>	<b>51%</b>

2yr Share Price Chart



1. 29 January 2018
2. Bell Potter Securities Research 11 January 2018
3. Includes \$15m BI milestone

# Financials highlights

(unaudited)	A\$'000	Three months ended		Six months ended	
		31-Dec-17	31-Dec-16	31-Dec-17	31-Dec-16
<b>Income statements</b>					
Sales of Bronchitol & Aridol		1,402	793	2,451	1,690
Sale of drug candidate		-	-	26,891	-
Total revenue		2,433	1,900	31,344	6,910
Total expenses		(17,859)	(8,850)	(25,432)	(17,945)
Net profit (loss) after tax		(15,419)	(6,950)	5,920	(11,035)
<b>Segment results – adjusted EBITDA</b>					
Bronchitol & Aridol		13	(2,489)	(1,447)	(3,941)
New drug development		(3,626)	(1,303)	20,872	(2,421)
Corporate		(10,654)	(850)	(11,635)	(2,078)
Total		<b>(14,267)</b>	<b>(4,642)</b>	<b>7,790</b>	<b>(8,440)</b>
<b>Statement of cash flows</b>					
Cash inflow/ (outflow) from:					
Operations		(13,023)	(2,721)	4,639	(8,894)
Investing activities		(125)	(64)	(235)	(214)
Financing activities		(436)	(430)	(863)	(856)
Total cash generated/(used)		<b>(13,584)</b>	<b>(3,215)</b>	<b>3,541</b>	<b>(9,964)</b>
<b>Cash at bank</b>		<b>25,045</b>	<b>29,245</b>	<b>25,045</b>	<b>29,245</b>

Refer to December 2017 Quarterly Shareholder Update for additional financial information



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