pharmaxis



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

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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



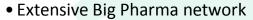
Clinical Trials Utilise global experience and extensive clinical networks to execute value adding Phase 1 and 2 clinical trials



- and in house chemistry platformEfficiencies from global academic & CRO networks
- Target high value diseases with validated targets



Value Generation



 Partnering after phase 1 or 2 to realise value and mitigate program and corporate risk



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-Plank 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



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



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.

Board of Directors

- Malcolm McComas Chair
 - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- **Gary Phillips** Chief executive officer and managing director

- 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
 - Kathleen Metters Non executive director
 - former head of global research at Merck

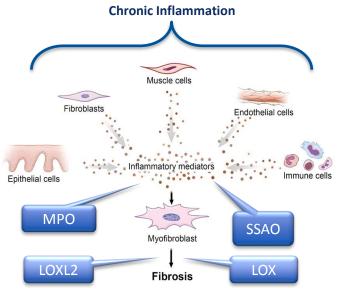
Pharmaxis portfolio

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
Commercial								
Bronchitol® US	Cystic fibrosis	responsibility (incurri	ary endpoint in 2017. Subjec ng all costs) for completing th	e New Drug Applica	ition with the FDA a	nd US commerciali:	sation.	Chiesi People and ideas for innovation in healthcare
Bronchitol RoW	Cystic fibrosis	Bronchitol is currently distributors and Austr	y sold in the UK, Germany and Palia by PXS. Bronchitol & Aric tive of any approval in the U	I Italy by Chiesi; otheolorise of the lorent business segmen	er European countr nt expected to trans	ies and Russia by spition to profitability	pecialist	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	
In the clinic								
SSAO (PXS-4728A)	NASH	potential milestone pa	elheim in May 2015. Phase 2 syments of A\$290m during do stones following approval. P	velopment progran	n and further royalt	ies (IIIIIII) T	Boehringer ngelheim	
SSAO (PXS-4728A)	Diabetic retinopathy	payments of A\$290m	ed dosing a Phase 2 trial in Ja during development and furt (S received A\$15m to date.				Boehringer ngelheim	
LOXL-2	NASH, fibrosis - liver, lung, kidney	Phase 1 trials commen at end of Phase 1 – H2	ced in H2 2017 in 2 compoun 2018.	ds. Expected to part	tner			
Discovery								
SSAO/MPO	Respiratory & cardiovascular		nmmatory. Commenced pre- orgeting Phase 1 trial in 2018					
LOX	Scarring; cancer	Anti-fibrotic. Commence 2017 . Targeting Phase	ced pre-clinical tox studies Q 1 trial in 2018	1				

A pipeline of drugs for inflammation and fibrosis

Pharmaxis has developed a commercial pipeline of small molecule drugs against high value targets

Targeting multiple different pathways



Key areas of current focus:

- NASH/liver fibrosis SSAO and LOXL2
- Diabetic retinopathy (DR) SSAO
- Pulmonary fibrosis (PF) LOXL2

Other active programs:

- Pancreatic cancer & myelofibrosis LOX
- Scarring LOX
- Cardiac fibrosis, Kidney fibrosis MPO
- COPD/asthma MPO

Pharmaxis Drug Discovery

Pharmaxis has developed a commercial pipeline of small molecule drugs for inflammation and fibrosis

- Amine oxidase enzymes are well validated as targets in diseases with a high unmet medical need
- Pharmaxis are global leaders in amine oxidase enzyme inhibition
- Pharmaxis owned IP
- Since 2015 the platform has delivered:
 - 1 compound in 2 phase 2 trials (SSAO)
 - 2 compounds in phase 1 trials (LOXL2)
 - 2 compounds planned to be phase 1 ready in 2018 (LOX and SSAO/MPO combo)

Key catalysts for 2018

Pharmaxis value driving events

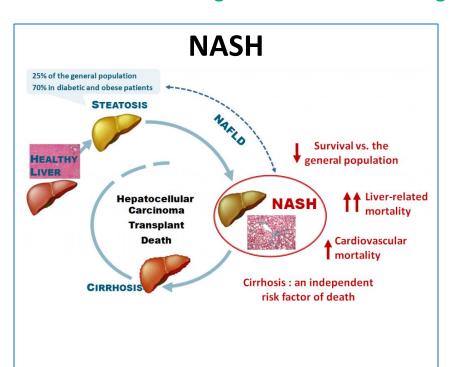
- Boehringer Ingelheim acquired drug (BI 1467335) reports clinical proof of concept in two major diseases as Phase 2 trials report in Q3 and Q4 2018
- Pharmaxis owned anti fibrotic program realises significant valuation inflection point after completing first in man Phase 1 clinical studies in Q2 2018; major partnering deal planned H2 2018

Others

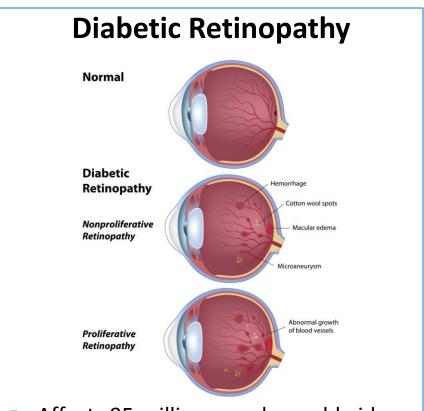
- a) Bronchitol FDA re-submission by Chiesi in 2018
- b) Pharmaxis portfolio valuation further boosted by two in house research programs reaching first in man phase 1 clinical study stage in H2 2018
- c) Evaluating external opportunities for in-license or acquisition of new programs in fibrosis and inflammation

Catalyst 1: Pharmaxis poised to be a major player in diseases caused by complications of diabetes

Two diseases with high unmet need and large patient populations



- Expected to become leading cause of liver transplant by 2020;
- No approved treatments



- Affects 95 million people worldwide
- No approved treatments for early stage disease

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]

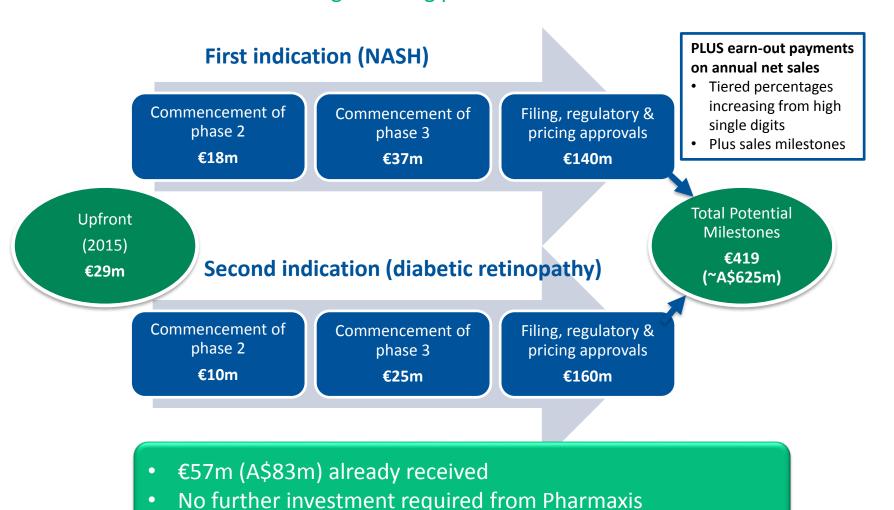
Diabetic Retinopathy

- 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

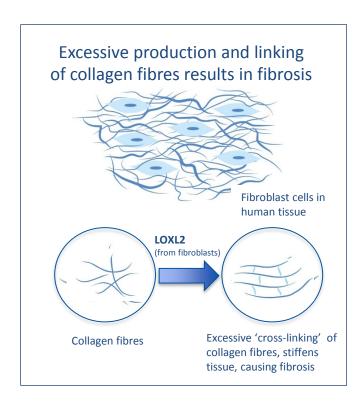


Deal structure illustrates value generating potential of Pharmaxis business model



Catalyst 2: LOXL2 inhibition program for NASH & other fibrotic diseases

An attractive target and development program



Potential indications:

- NASH / Liver Fibrosis
- Pulmonary fibrosis (IPF)
- Kidney
- Cardiac fibrosis

Significant market opportunity

Development status:

- Pharmaxis discovery patent filed 2016
- Effective in pre clinical models of fibrosis (liver, lung, kidney and heart)
- 2 lead candidate compounds commenced Phase 1 in Q4 2017
- Assay to measure target engagement

Competitive profile:

- Novel target and mechanism of action
- Once daily oral drug
- Complete inhibition of LOXL2 enzyme
- Opportunity to use in combination with other Pharma pipeline drugs

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	\checkmark
Patent	Composition of matterAs long as possible	Composition of matter2016 filing date; 100% PXS owned	√
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 reports mid 2018	

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

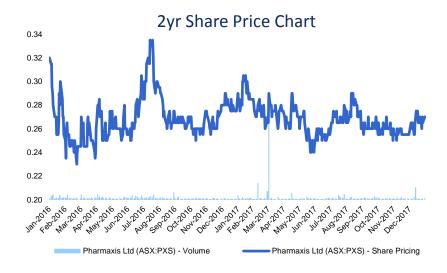
UPDATE: Pharmaxis has entered 'confidential due diligence' phase of partnering process with multiple large Pharma companies

Shareholders & trading



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

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



^{1. 29} January 2018

^{2.} Bell Potter Securities Research 1 February 2018

^{3.} Includes \$15m BI milestone



Drug development & other research initiatives

Pharmaxis product portfolio – new opportunities

New compounds expected to be ready for the clinic in 2018

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
<u>Discovery</u>								
SSAO/MPO	Respiratory & cardiovascular		mmatory. Commenced pre- rgeting Phase 1 ready in 201					
LOX	Scarring, cancer	Anti-fibrotic. Commence 2017 . Targeting Phase 1	ed pre-clinical tox studies Q4 ready in 2018					

Development strategy

- Identify indications from in-house development programs that have high value potential if developed to phase 2a / 2b
- Evaluate external opportunities for in-license or acquisition:
 - Lead optimisation or Phase 1 ready
 - Inflammation or fibrosis
 - Small molecule or biologic

Pharmaxis purpose built facility

Pharmaxis has a purpose built manufacturing and drug development facility in Sydney

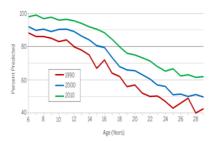
- Manufacturing and research facilities
- Productive R&D drug discovery engine
- Team of 15 scientists specialising in amine oxidase chemistry drug discovery and pre clinical development
- Capability to run global clinical trials
- Manufacturing and exporting approved products:
 - Bronchitol®
 - Aridol[®]
- Capacity for future growth





Bronchitol for cystic fibrosis

Overview



Median FEV₁ % Predicted versus Age

Cystic fibrosis

- Patients
 - US: 30,000;
 - Europe: 37,000;
 - Rest of world: 21,000
- Disease characterised by poorly hydrated, tenacious, thick mucus
- Rapid decline in lung function
- Frequent infections



Bronchitol

- Active ingredient mannitol delivered as an inhalable dry powder
- Restores airway surface liquid
- Mucus clearance enhanced
- Improves lung function
- Reduces incidence of lung infections



Business model - RoW

- Global Bronchitol distributors responsible for promotion & support
 - Chiesi in UK. Germany and Italy
 - Other distributors in Russia, Eastern Europe, Middle East
- PXS revenue share ~50%



Business model - US

- Phase 3 trial (CF303) reported June 2017
- Chiesi responsible for regulatory filing & commercialisation
- File updated NDA 2018
- ~A\$13m milestone payment on launch, plus sales milestones
- PXS supplies US market from Sydney factory
- PXS receives high mid teens % of in-market sales plus cost of goods

Summary

Pharmaxis is a global leader in drug development for fibrosis & inflammation

- Pharmaxis have built a successful platform of small molecule drugs targeting fibrosis and inflammation across various stages of development and approval
- Proven track record of early stage partnering and taking products through to commercialisation
- Potential to receive total up front and milestone payments of A\$625m plus further sales based payments from first deal – A\$83m already received
- Strong balance sheet \$25m at Dec 2017 and A\$15m milestone received
 Q1 2018
- Discovery pipeline targeting high value indications one drug in 2 phase 2 trials, one drug program(two compounds) in phase 1 trials, two compounds in pre-clinical development
- Numerous catalysts over the next 18 months

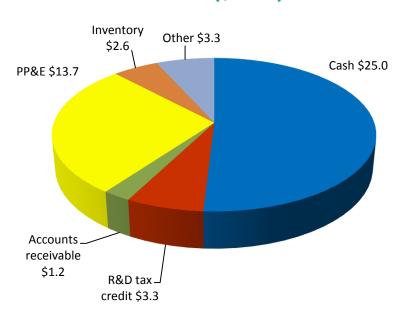
Financials highlights

A\$'000	Three months ended		Six mont	hs ended	
(unaudited)	31-Dec-17	31-Dec-16	31-Dec-17	31-Dec-16	
Income statements					
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)	
Segment results – adjusted EBITDA					
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	(14,267)	(4,642)	7,790	(8,440)	
Statement of cash flows					
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)	(13,584)	(3,215)	3,541	(9,964)	
Cash at bank	25,045	29,245	25,045	29,245	

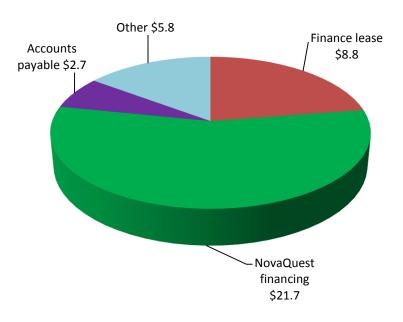
Refer to December 2017 Quarterly Shareholder Update for additional financial information

Balance sheet – 31 December 2017

Assets (\$49m)



Liabilities (\$39m)



- Finance lease over 20 Rodborough Rd (to 2024)
- NovaQuest financing not repayable other than as % of Bronchitol revenue



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