



# Investor Presentation

Gary Phillips CEO

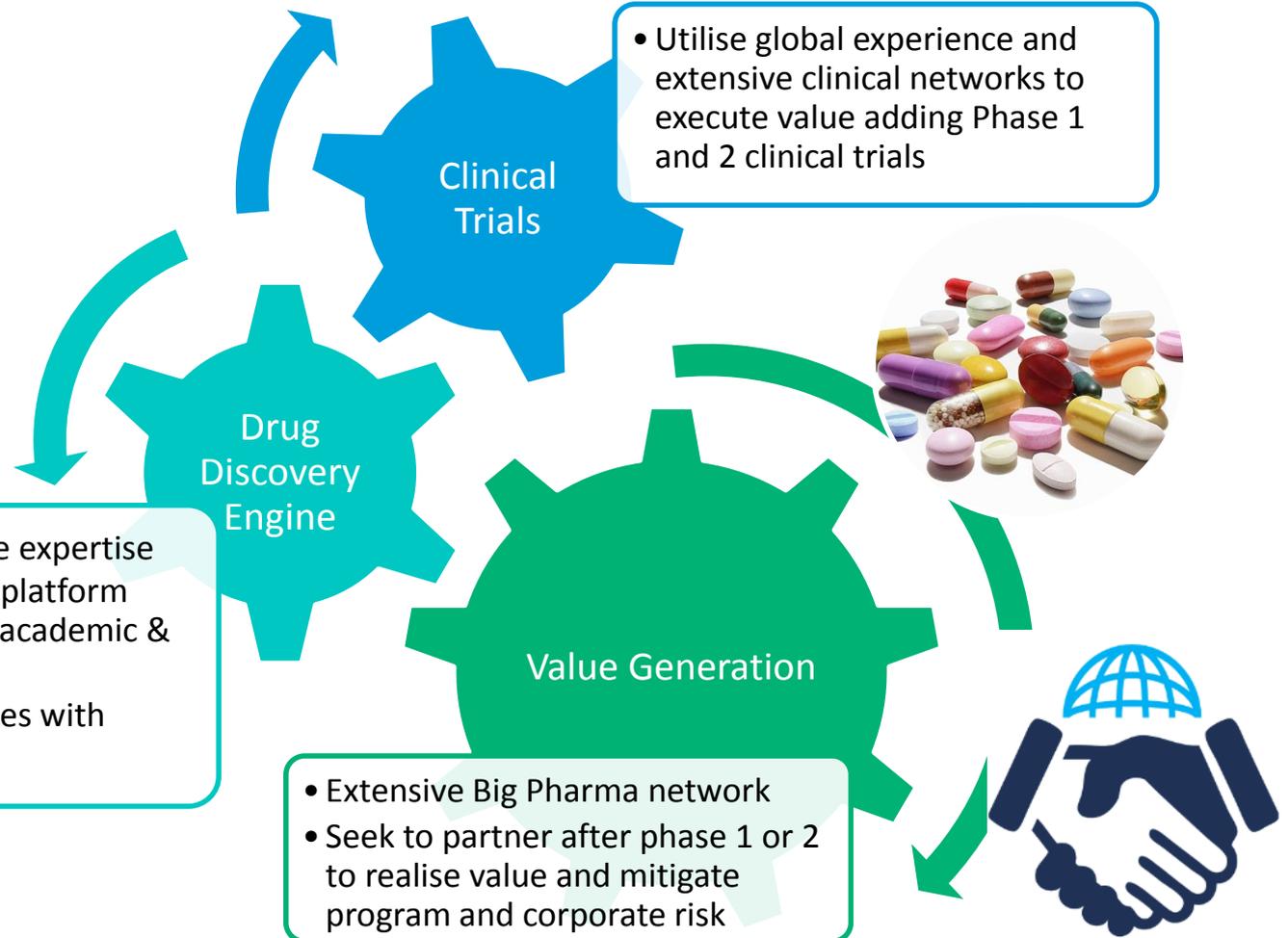
11 October 2018

# 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. 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 partnering our LOXL2 program or any of the other 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.

# 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 overview

## A globally recognised leader in drug development for fibrosis & inflammation

- A chemistry platform that has delivered a pipeline of oral small molecule drugs in preclinical and clinical development in diseases with large markets and high unmet need
- A **globally respected** translational **development team** delivering **best in class** drug development programs with international standard data packages
- A proven **track record** of achieving global partnering deals with multinational Pharmaceutical companies
- \$83m received to date from benchmark deal concluded with Boehringer Ingelheim in 2015 and worth a potential \$600m+ in development milestones for two indications (NASH and diabetic retinopathy) plus sales related payments (% and milestones)
- Commercial partnering process for phase 2 ready anti fibrotic LOXL2 inhibitor program expected Q4 2018
- Growing revenues from approved product sales (26% increase for FY 2018 to A\$6.1m) & milestones (A\$42m FY 2018)
- Strong balance sheet - A\$54m pro-forma cash balance (30 June 2018 post raising)
- Purpose built manufacturing and research facility in Sydney
- Strong institutional share register; including offshore specialist biotech funds

# 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 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



## 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 20 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

## Non Executive Directors

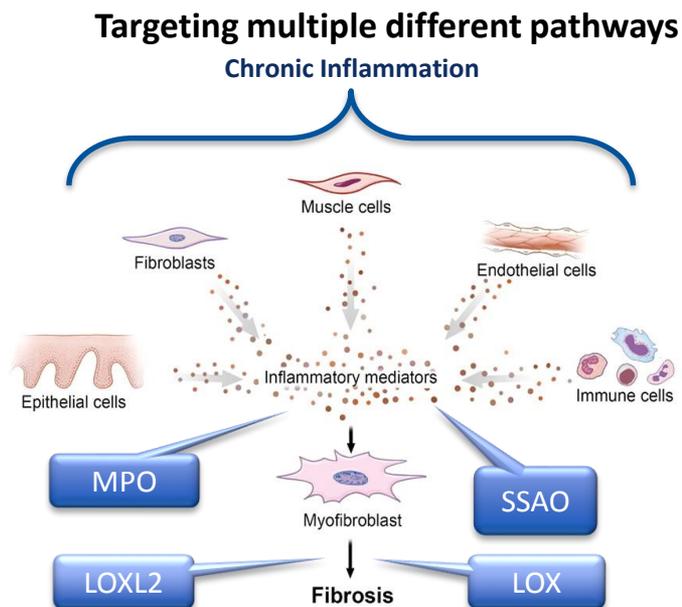
- Malcolm McComas – Chair**
  - former investment banker at Grant Samuel
- Kathleen Metters**
  - former head of worldwide basic research at Merck
  - former CEO of biopharmaceutical company Lycera Corp.
- Will Delaat**
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- Simon Buckingham**
  - former President Global Corporate and Business Development at Actelion

# 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. Chiesi planning to file with FDA Q4 2018. Subject to FDA approval, US partner Chiesi will launch commercially in the US.						
Bronchitol RoW	Cystic fibrosis	Bronchitol is currently sold in the UK, Germany and Italy by Chiesi; some other European countries and Russia by specialist distributors and by PXS in Australia and smaller countries						Direct & Dist
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.						Direct & Dist
<b>In the clinic</b>								
SSAO (PXS-4728A)	NASH	Sold to Boehringer Ingelheim in May 2015. Phase 2a trial commenced August 2017. PXS received payments of A\$68m to date.						
SSAO (PXS-4728A)	Diabetic retinopathy	Boehringer commenced dosing a Phase 2a trial in January 2018. PXS received A\$15m to date.						
LOXL-2	NASH, fibrosis - liver, lung, kidney, heart	Phase 1 trials in 2 compounds. First compound has reported positive results. Second compound has completed dosing – to report Q4 2018. Commercial partnering process Q4 2018.						
<b>Preclinical</b>								
SSAO/MPO	Inflammation	Dual inhibitor anti-inflammatory. Commenced pre-clinical tox Q4 2017.						
LOX - oral	Cancer	Anti-fibrotic. Completed pre-clinical tox studies Q4 2018.						
LOX – topical	Scarring	Anti-fibrotic. Effective in scarring models.						

# A pipeline of drugs for inflammation and fibrosis

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



## 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 developed IP
- Since 2015 the platform has delivered:
  - 1 compound in 2 x phase 2 trials (SSAO)
  - 2 compounds completing phase 1 trials (LOXL2)
  - 2 compounds in preclinical development approaching the clinic
    - LOX (oral)
    - SSAO/MPO

### 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 (oral)
- Scarring – LOX (topical)
- Inflammatory bowel disease – SSAO/MPO
- Respiratory – SSAO/MPO

# Key catalysts targeted for 2018/2019

## Pharmaxis value driving events

### 1. LOXL2 anti fibrotic program

- Phase 1 trials final stage completed October 2018
- Phase 2 enabling toxicity studies: 2 completed, 2 to report November 2018
- Partnering process - Q4 2018.

### 2. Boehringer Ingelheim acquired SSAO inhibitor (BI 1467335) to report **clinical proof of concept** in two major diseases as Phase 2 trials report in H1 2019

### 3. Two additional programs to enter the clinic

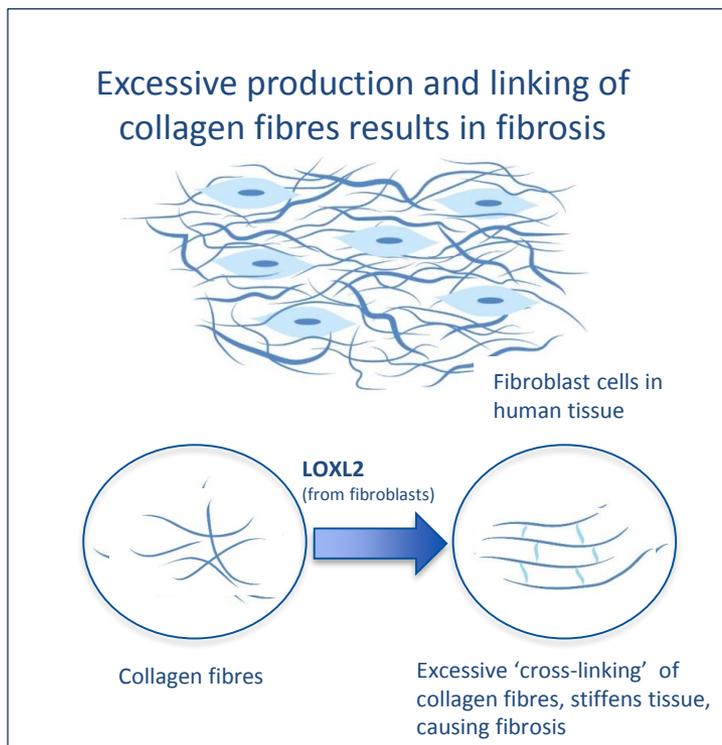
- LOX (oral) for pancreatic cancer and myelofibrosis to start clinical studies H1 2019
- SSAO/MPO combo scheduled to complete pre-clinical development in H1 2019

### 4. Others

- Bronchitol **FDA re-submission** by Chiesi in Q4 2018
- Other internal programs developing **additional compounds** to take into preclinical development
- Evaluating opportunities for in-license or acquisition of new programs in fibrosis and inflammation that **leverage PXS** research and commercialisation capabilities

# LOXL2 inhibition program

for NASH, IPF & other high value fibrotic diseases



- Potential indications / market size:
    - NASH / Liver Fibrosis; \$35b<sup>1</sup>
    - Pulmonary fibrosis (IPF); \$3.5b<sup>2</sup>
    - Kidney fibrosis
    - Cardiac fibrosis
- Significant market opportunity
- LOXL2 and fibrosis:
    - LOX family of enzymes are the final step in the fibrotic disease process
    - Clear association of increased levels of LOXL2 (and LOXL3) with disease progression in IPF, NASH and cardiac fibrosis
  - Competitive profile:
    - Novel target and mechanism of action
    - Once daily oral drug
    - Best in class drug with high level inhibition of LOXL2 enzyme for 24 hours from one dose
    - Only known drug in clinical development to inhibit LOXL3
    - Place of LOXL2 at the end of the fibrotic cascade provides opportunity to use in combination with other Pharma pipeline drugs

# LOXL2 inhibitor program

approaches “deal ready” status

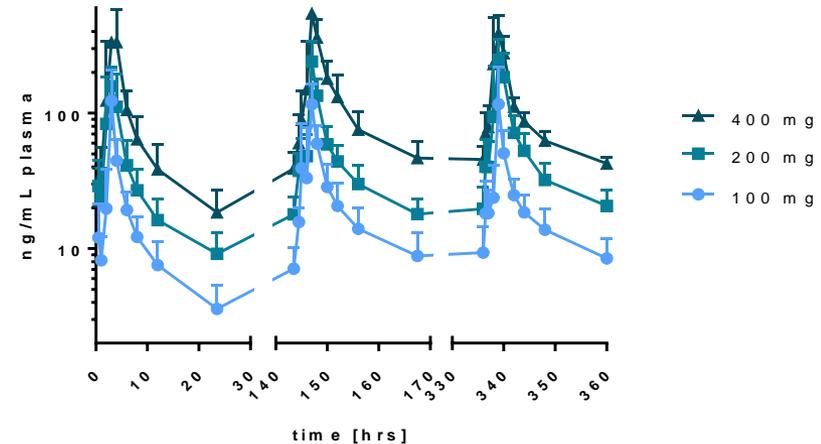
Feature	What Pharma values	PXS program status
Disease target	Independent validation	Multiple peer reviewed publications
Pre clinical proof of concept	2 or more different supportive animal models	Multiple supportive models across 5 different diseases. Further studies in progress
Dosing regimen	Ease of use	Oral once a day tablet or capsule
Patent	Composition of matter As long as possible	Composition of matter 2016 filing date; 100% PXS owned
Cost of Goods	Low	Small molecule with easy synthesis
# Compounds	1 plus backups	2 compounds in clinical development plus back ups
Toxicity	Wide therapeutic window As long as possible	28 day tox studies complete 13 week studies (2 species) 2 successfully completed; 2 in progress – report Q4 ‘18
Clinical phase	Phase 1 with target engagement Phase 2 ready	Both compounds completed Phase 1 dosing (stages 1 & 2) First compound results analysed and reported, second to report Q4 2018
Target engagement	Drug inhibits target	High levels of inhibition for 24 hours from a single dose

# LOXL2: Phase 1 Study in first of two compounds

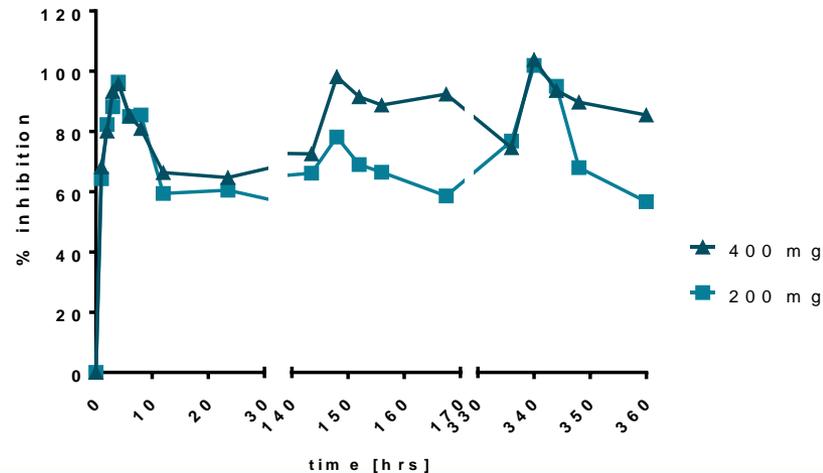
## Positive safety and PK/PD findings in SAD<sup>1</sup> and MAD<sup>2</sup> Phase 1 trial

- No adverse safety findings, compound was well tolerated
- Dose dependent increase in pharmacokinetic parameters
- Some accumulation occurred between Day 1 and Day 7 as expected from drug half life
- No further PK changes between Day 7 and Day 14
- Target engagement assay indicated that both compounds inhibited LOXL2 in a dose-related fashion.
- 24 hour inhibition is achieved with a single dose.
- Accumulation of compound over days increases plasma concentration and target engagement
- Once a day dosing causes >80% inhibition of the LOXL2 enzyme over 24 hrs
- Second compound SAD stage results consistent with first compound, MAD stage completed dosing – to report Q4 2018

Pharmacokinetics of first compound in MAD stage



Target engagement of first compound in MAD stage



# LOXL2 inhibitor program – partnering process

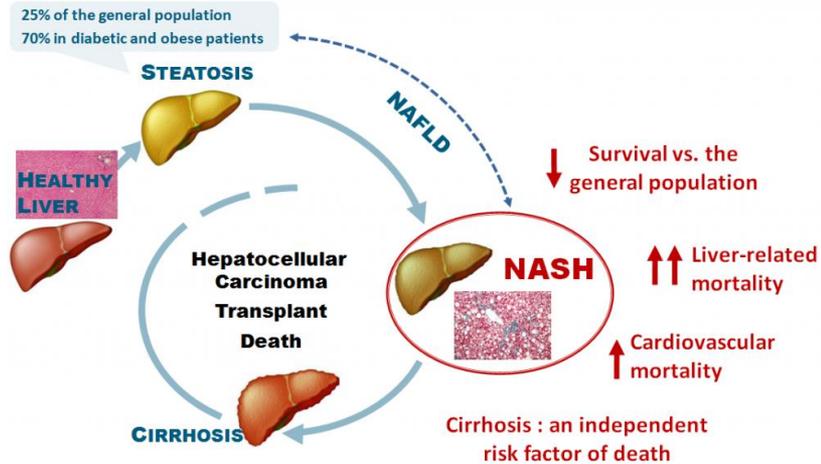
## Positive engagement with pharma companies

- Pharma company interest driven by search for:
  - Inhibitor to LOXL2 and LOXL3 enzymes,
  - Effective anti-fibrotic drug, and/or
  - Drugs to complement existing disease portfolio – lung, liver, kidney, heart, etc.
- Pharmaxis engagement with multiple potential partners on planning and progress of the LOXL2 program for over 2 years
- Pharmaxis data packaging will complete in Q4, including:
  - Data analysis of second stage of phase 1 trials for both compounds
  - 13 week tox studies (2 species) for both compounds
  - Additional disease models
- Data room has been available (under CDA) since Q4 2017
- Commercial partnering discussions expected Q4 2018

# SSAO (Boehringer Ingelheim): Pharmaxis poised to be a major player in diseases caused by complications of diabetes

Two diseases with high unmet need and large patient populations in Phase 2 studies

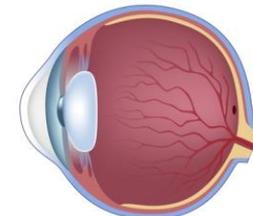
## NASH



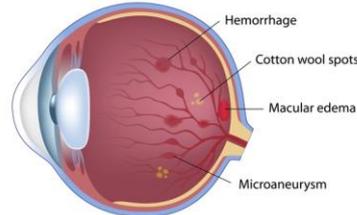
- Expected to become leading cause of liver transplant by 2020
- No approved treatments

## Diabetic Retinopathy

Normal

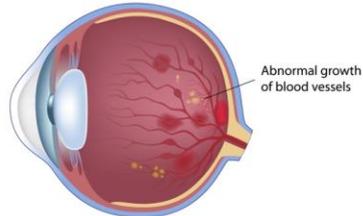


Diabetic Retinopathy



Nonproliferative Retinopathy

Proliferative Retinopathy



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

# SSAO: Phase 2 trials to show clinical proof of concept in H1 2019

Boehringer Ingelheim responsible for clinical development and commercialisation

## NASH

- Phase 2a trial expected to report H1 2019 – 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 peak sales potential of ~US\$2b [Analyst's estimate]

## Diabetic Retinopathy

- Phase 2a SSAO diabetic retinopathy expected to report H1 2019 – 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 peak sales potential of ~US\$800m [Analyst's estimate]

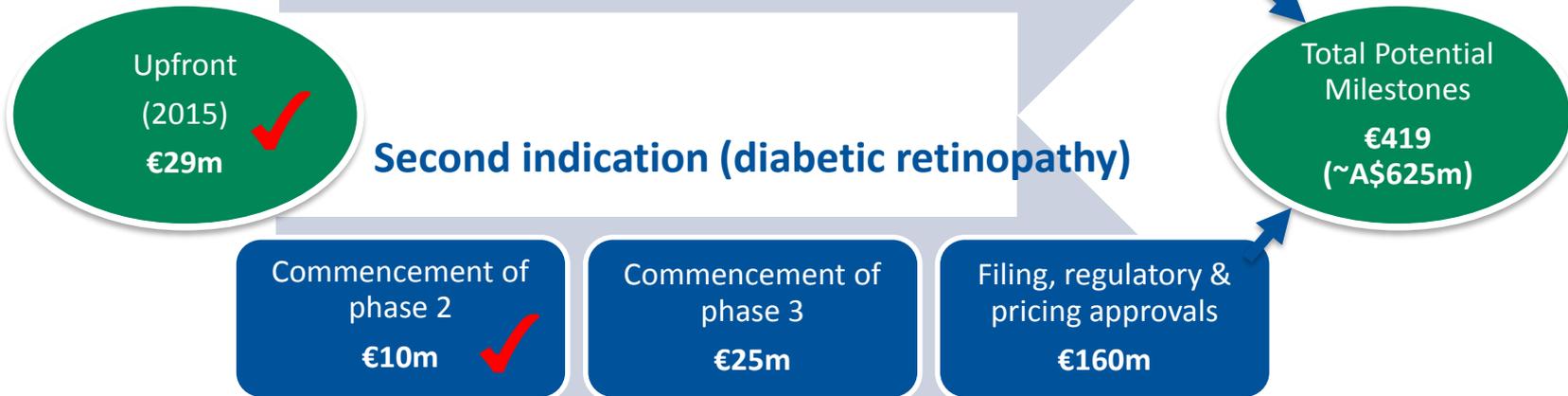
# SSAO: Boehringer Ingelheim deal

Deal structure illustrates value generating potential of Pharmaxis business model

## First indication (NASH)



## Second indication (diabetic retinopathy)



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

# More programs approaching the clinic

Program	LOX (oral)	Combo SSAO/MPO
Indication	Severe fibrotic indications: <ul style="list-style-type: none"> <li>pancreatic cancer</li> <li>myelofibrosis</li> </ul>	<ul style="list-style-type: none"> <li>Inflammatory bowel disease</li> <li>Respiratory disease</li> </ul>
Commercialisation	Partner after phase 2	Partner after phase 2
Status	<ul style="list-style-type: none"> <li>Effective in animal models of pancreatic cancer and myelofibrosis</li> <li>Completed preclinical 28 day tox</li> </ul>	<ul style="list-style-type: none"> <li>Commenced pre-clinical development Q4 2017</li> <li>Ongoing evaluation in various models of inflammation</li> </ul>
Next steps 2018/2019	<ul style="list-style-type: none"> <li>Additional animal models of pancreatic cancer and myelofibrosis</li> <li>Commence phase 1a (H1 2019)</li> </ul>	<ul style="list-style-type: none"> <li>Readouts from ongoing studies in various disease models of inflammation</li> <li>Review target indication and 28 day tox in conjunction with SAB</li> <li>Commence phase 1</li> </ul>

# Pharmaxis purpose built facility

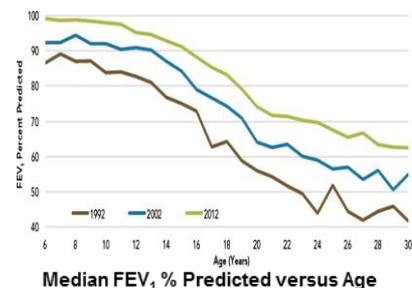
Pharmaxis has a purpose built manufacturing and drug development facility in Sydney

- Manufacturing and research facilities
- Productive R&D drug discovery engine
- Team of 17 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



## Cystic fibrosis

- Patients
  - US: 30,000;
  - Europe: 37,000;
  - Russia: ~10,000<sup>1</sup>
  - Australia: 3,500
  - Total world: ~100,000
- Disease characterised by poorly hydrated, tenacious, thick mucus
- Inexorable decline in lung function
- Frequent infections

1. Estimates vary from 7,000 to 30,000

## 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

- Distributors responsible for promotion & support
  - Chiesi in UK, Germany, Italy & Ireland
  - Other distributors in Russia, Eastern Europe, Middle East
  - PXS revenue share ~50%
  - Russian reimbursement decision H2 2018
- PXS direct in Australia and smaller markets

## Business model - US

- Phase 3 trial (CF303) reported June 2017
- Chiesi responsible for regulatory filing & commercialisation – preparing for launch
- File updated NDA - Q4 2018
- ~A\$13m milestone payment on launch
- PXS supplies US market from Sydney factory
- PXS receives high mid teens % of in-market sales plus cost of goods

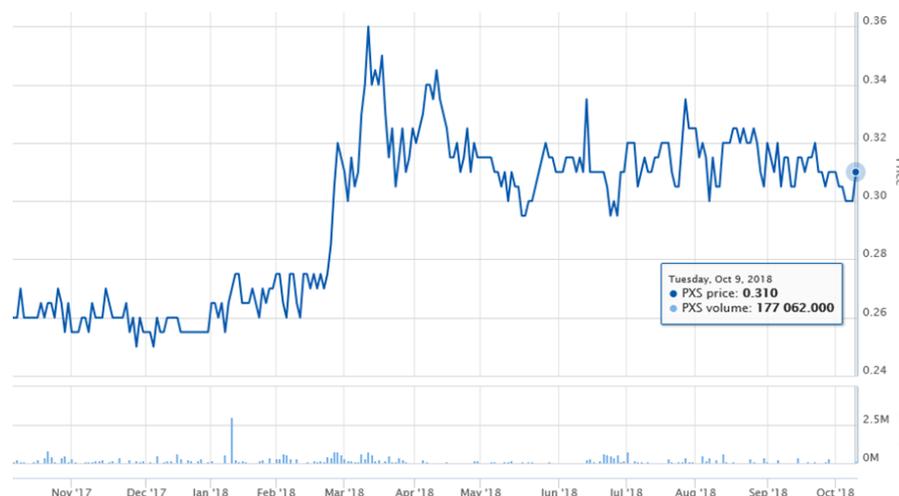
# Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap <sup>1</sup>	\$122m
Shares on Issue	394m
Employee Options <sup>1</sup>	16m
Liquidity (turnover last 12 months) <sup>1</sup>	48m shares
Share price <sup>1</sup>	\$0.32
Analyst valuation <sup>2</sup>	\$0.52
Cash Balance (30 June 18 Pro Forma Post Capital Raising)	A\$54m

Institutional Ownership	30 Sep 18
BVF Partners (US)	23%
Arix Bioscience (UK)	11%
Australian Ethical	7%
Allan Gray	5%
Montoya Investments (UK)	5%
Other Institutions	8%
<b>Total Institutional Ownership</b>	<b>59%</b>

1. As at 9 October 2018
2. Bell Potter Securities Research 30 April 2018



# Financials – highlights

30 June 2018

	A\$'000	2018	2017	2016	2015
<b>Income Statements</b>					
Sales revenue		6,094	4,823	6,135	5,999
Other revenue		44,739	13,178	12,885	53,248
Total revenue		<b>50,833</b>	<b>18,001</b>	<b>19,020</b>	<b>59,247</b>
Expenses		(44,413)	(36,437)	(35,476)	(40,739)
Net profit (loss) before tax		6,420	(18,436)	(16,456)	18,508
Net profit (loss) after tax		<b>6,428</b>	<b>(18,346)</b>	<b>(16,463)</b>	<b>18,466</b>
<b>Segment results - adjusted EBITDA</b>					
Bronchitol & Aridol		(3,786)	(7,100)	(8,228)	(10,045)
New drug development		28,771	(4,114)	(2,625)	35,068
Corporate		(13,466)	(4,017)	(3,988)	(3,532)
		<b>(11,519)</b>	<b>(15,231)</b>	<b>(14,841)</b>	<b>21,491</b>
<b>Cash flow</b>					
Operations		12,206	(15,262)	(11,989)	21,780
Investing activities		(884)	(723)	(1,381)	(264)
Financing activities		(1,753)	(1,721)	(1,714)	(1,791)
		<b>9,569</b>	<b>(17,706)</b>	<b>(15,084)</b>	<b>19,725</b>
<b>Cash at bank</b>		<b>31,073</b>	<b>21,504</b>	<b>39,209</b>	<b>54,138</b>

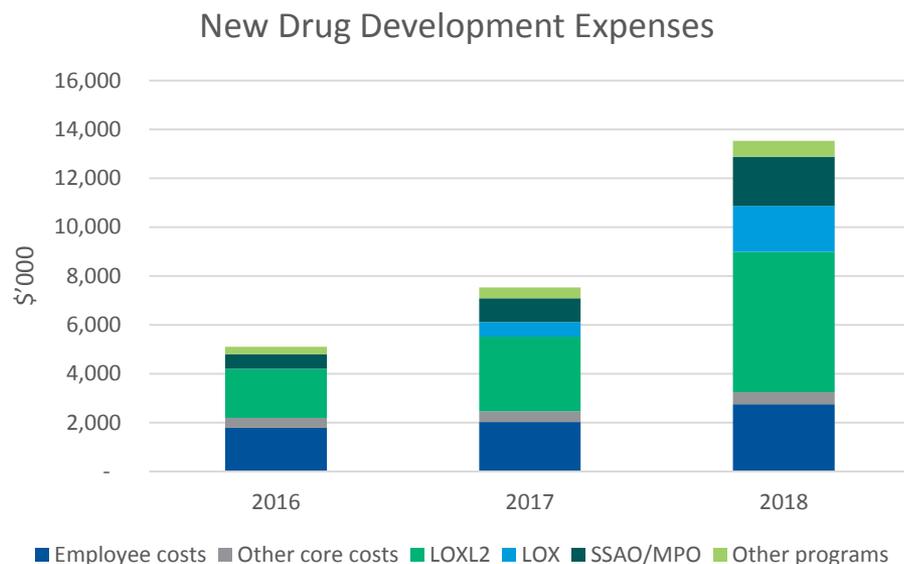
## Highlights of 2018

- Increased product sales
- Two milestone payments from Boehringer Ingelheim totaling \$42m
- Profit of \$6.4m
- Bronchitol & Aridol loss continued to reduce
- Increased investment in new drug development
- Corporate investment to increase revenue share of LOXL2 partnering
- Cash flow investing activities focused on drug discovery capability and manufacturing upgrades
- Cash flow financing activities – predominantly finance lease over facility at Frenchs Forest
- Closing cash of \$31m increased by \$24m placement completed September 2018.

Refer to June 2018 Quarterly Shareholder Update and 2018 Financial Statements for additional financial information

# New Drug Development

## Drug development and clinical trial expenditure by pipeline project

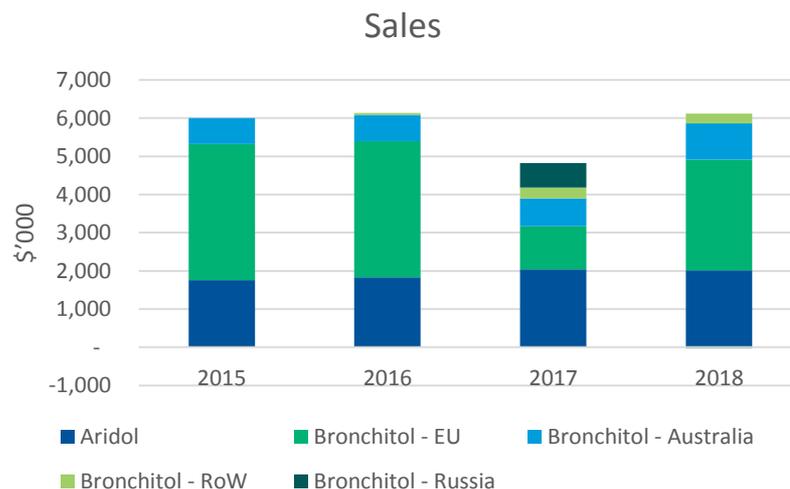
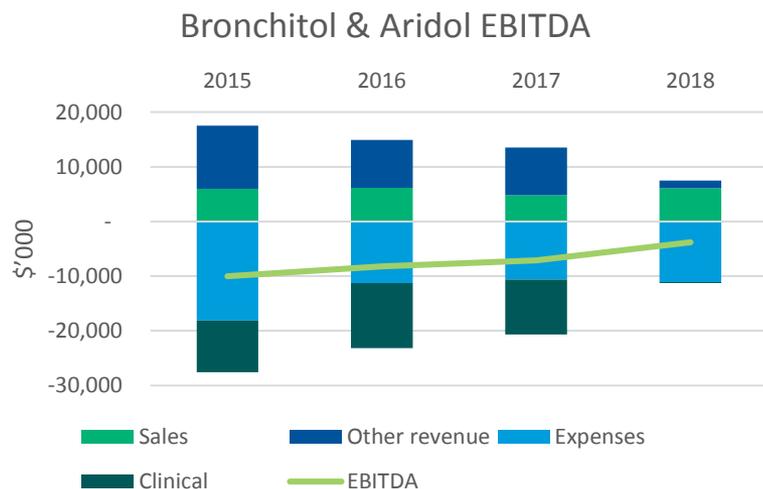


### Current status/planned expenditure

- LOXL2:
  - 2 compounds in phase 1 – will complete H2 2018
  - 13 week tox for both compounds
  - GMP material for rapid phase 2 start by partner
  - Other preclinical studies to report Q3/Q4 2018
- LOX (oral) in preclinical
  - Disease models – cancer
  - GLP tox – 1 month & 3 month
  - GMP material – for phase 1a/2a clinical trials
  - Plan to start phase 1/2a CY 2019
- SSAO/MPO in preclinical
  - Disease models - ulcerative colitis
  - GLP tox – 1 month
  - GMP material for phase 2 clinical trials

# Bronchitol & Aridol

## Segment profitability



### Path to profitability: increase revenue to leverage cost base

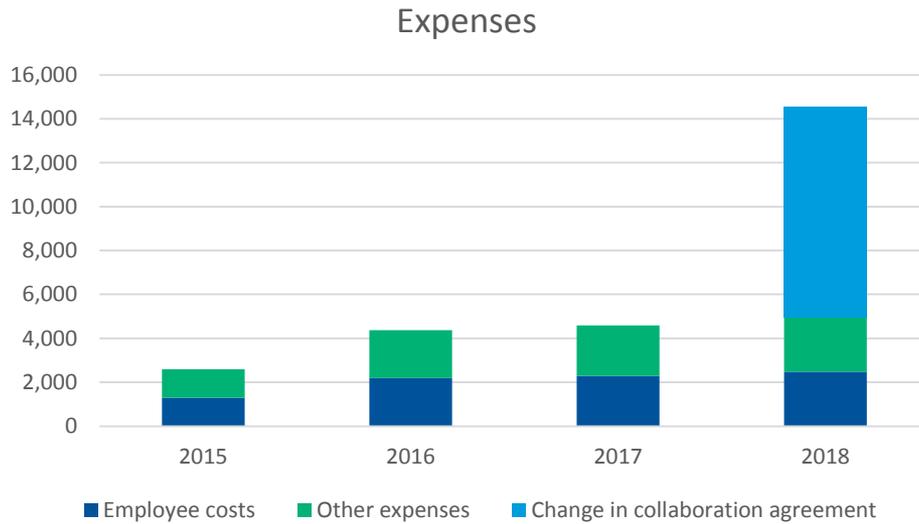
- Core cost base relatively fixed vs sales volume
- Reimbursement of Bronchitol in Russia key to rate of overall sales growth - decision Q3 2018
- US approval – Subject to FDA approval (~Q3 2019), launch Q4 2019 (US\$10m milestone)
- Aridol planned to re-launch in US Q4 2018 with specialist distributor. FDA inspection of factory Q3 2018
- Other Bronchitol sales growth opportunities
- Continued growth in major Bronchitol launched markets – UK, Germany & Australia
- Growth in other Bronchitol markets: Italy, Spain, CZ, Ireland
- Aridol in Canada – target launch Q3 2019

### Revenue

- 2015: Direct to pharmacy until June 15 (ie all sales revenue to PXS)
- 2016: EU sales via distributors at lower margin (~50%) to PXS. Chiesi builds inventory levels
- 2017: First sale to Russia (\$640k)
- 2018: Growth in EU (Chiesi UK & Germany) & Australia (expanded PBS coverage)
- Other revenue in all years is predominantly reimbursement of clinical trial costs by US partner

# Corporate

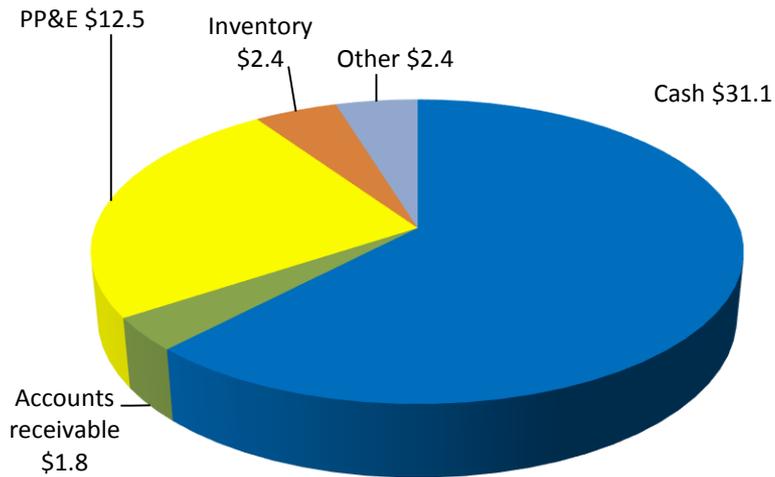
30 June 2018



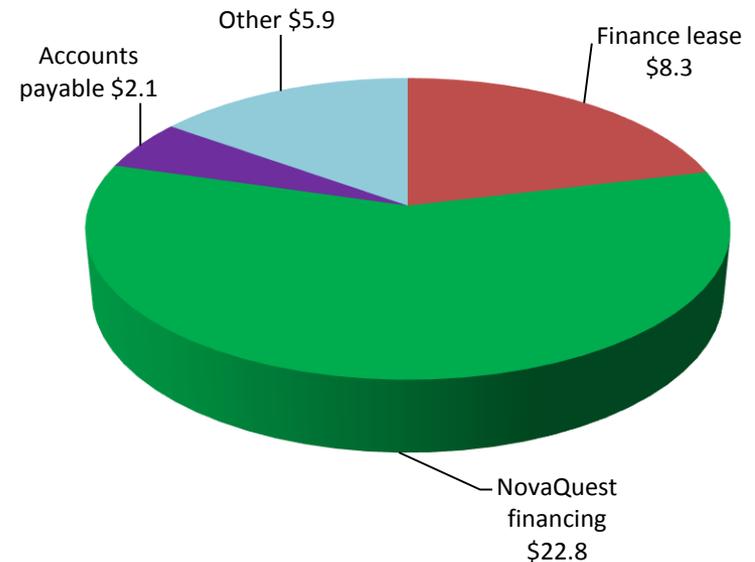
- Employee and other costs stable
- One-off expense in 2018 to change collaboration agreement with Synairgen

# Balance sheet – 30 June 2018

## Assets (\$50m)



## Liabilities (\$39m)



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

# Summary

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

- Pharmaxis have built a **successful platform of small molecule drugs targeting high value fibrotic and inflammatory indications**
- **Development pipeline** across various stages - one drug in two phase 2 trials, one drug program (two compounds) in phase 1 trials, two compounds in pre-clinical development approaching the clinic, additional drug candidates in discovery.
- 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 (SSAO) – A\$83m already received
- Next drug completing phase 1 trials and long term toxicity studies: **partnering negotiations planned Q4 2018**
- **Strong balance sheet - \$54m** pro-forma cash balance (30 June 2018 post raising)
- **Numerous catalysts over the next 18 months**

A decorative graphic on the left side of the slide consists of several overlapping hexagons in various shades of blue, creating a honeycomb-like pattern.

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