



# Investor Presentation

Gary Phillips CEO

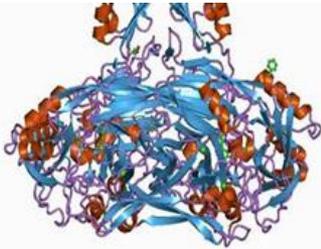
1 August 2017

# 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.

# Business overview

Built to create value



## Drug development

- Focus on fibrosis and inflammation
- Strong Pharma interest in validated small molecule technology platform
- Several new drugs acting on high value targets in current pipeline



## Management

- Management and Board with global experience & Pharma network
- Proven capability of executing global BD with major partners
- In house capability to run multi-centre international trials



## Partnerships

- First drug out licensed to Boehringer Ingelheim in globally competitive deal - total potential deal >A\$750m
- Synairgen collaboration for LOXL2
- Significant value milestones from existing partner deals near term
- Pipeline providing multiple future opportunities



## Financial strength

- A\$21.5m cash balance at June 2017; average annual cash usage \$1.5m/month
- Boehringer NASH phase 2 initiation milestone expected Q3 2017 €18m
- Boehringer 2nd indication phase 2 milestone of €10m expected H2 2017
- Market cap \$81m\*
- Institutional investor's 50%
- Increasing Bronchitol sales globally in new and existing markets

# 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 and funding 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

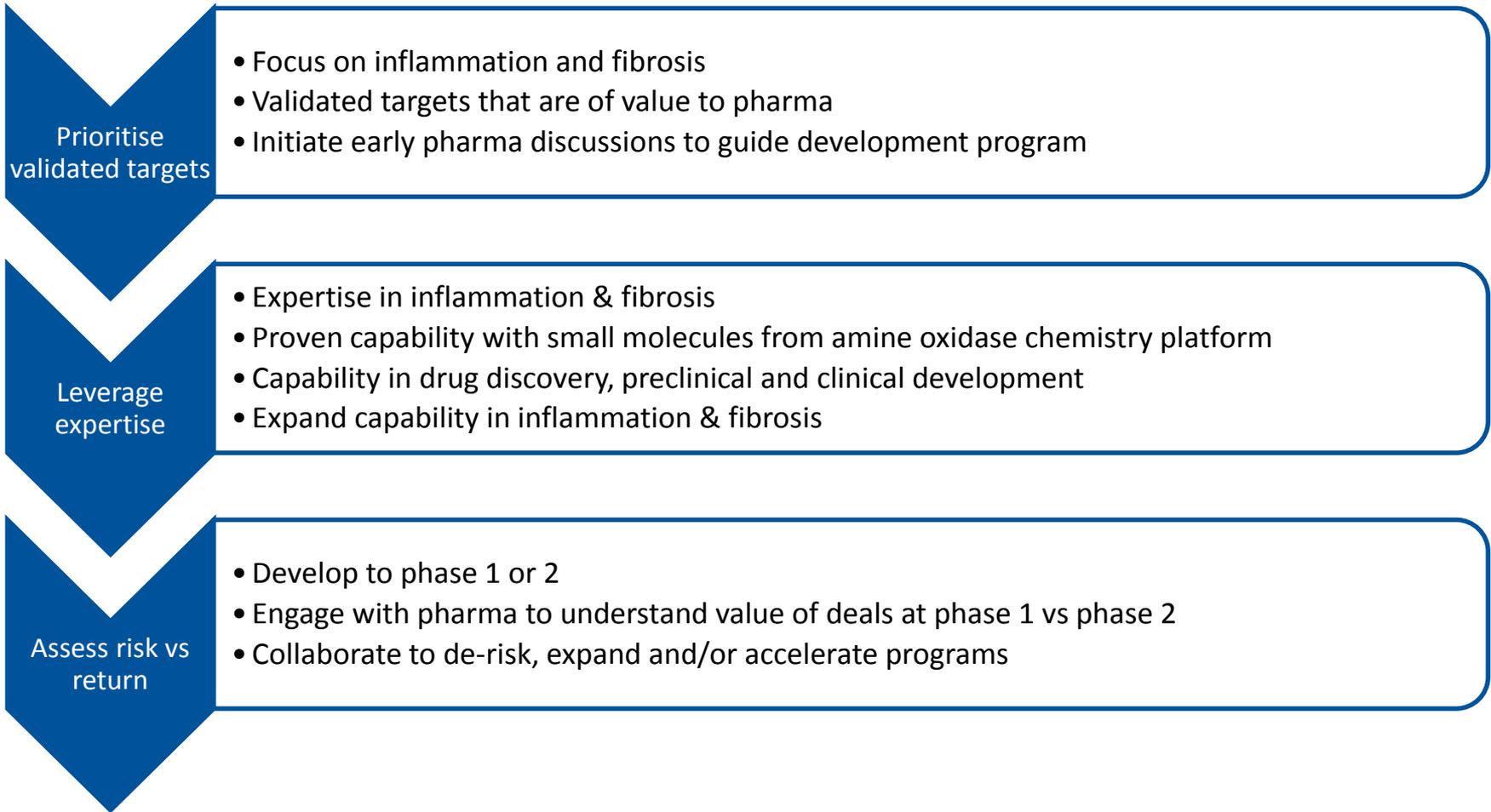
- Malcolm McComas – Chair**
  - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- Will Delaat – Non executive director**
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- Simon Buckingham – Non executive director**
  - former President Global Corporate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

# Pharmaxis product portfolio

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
Bronchitol US	Cystic fibrosis							<b>Chiesi</b> <small>People and ideas for innovation in healthcare</small>
RoW	Cystic fibrosis							Distributors
Aridol	Asthma diagnosis							Distributors
SSAO	NASH					<b>Boehringer Ingelheim</b>		
SSAO	2 <sup>nd</sup> indication					<b>Boehringer Ingelheim</b>		
<u>Discovery</u>								
LOXL-2	NASH, fibrosis - liver, pulmonary, kidney							
SSAO/MPO	Respiratory & cardiovascular							
LOX	Scarring+							
LOXL-2 (other)	Cancer			Leading universities/academics assessing in cancer				
Orbital	Dry powder inhalation device					<b>WOOLCOCK</b> <small>LEADERS IN BREATHING &amp; SLEEP RESEARCH</small>		
ASM-8	Asthma					Seeking Partners		

# Pharmaxis drug discovery strategy

Achieving value in the high risk world of early stage drug development



# Achievements to date

Building a biotech powerhouse in fibrosis and inflammation



## Drug discovery

- First in class SSAO inhibitor drug taken to phase 1.
  - Initial indication NASH.
  - Partner developing second indication.
- Two lead candidates completing preclinical tox studies
- Two further lead candidates to enter preclinical in 2017

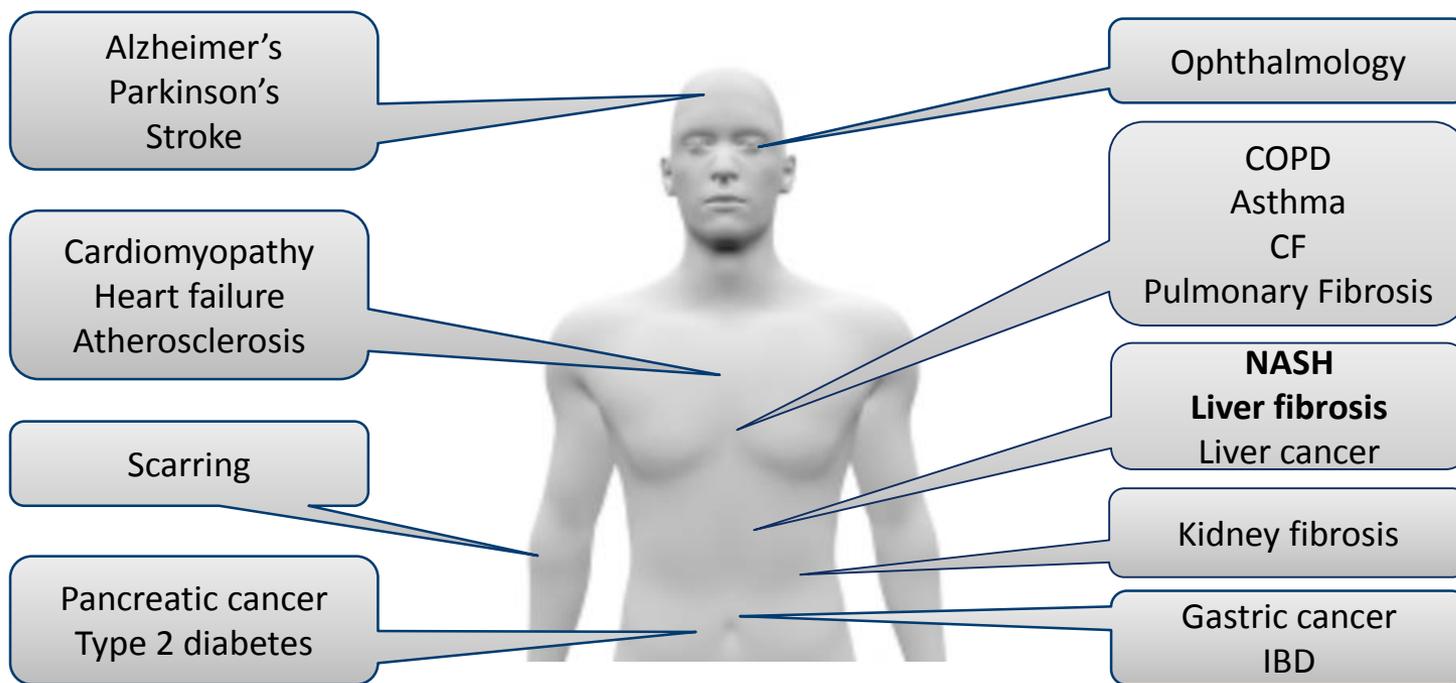


## Partnering

- In house BD expertise achieves valuable deal with Boehringer Ingelheim - A\$39m upfront, total potential > A\$750m
- Collaboration with Synairgen Research plc for early stage fibrosis program to widen spread of indications, enhance time to value inflection and spread risk

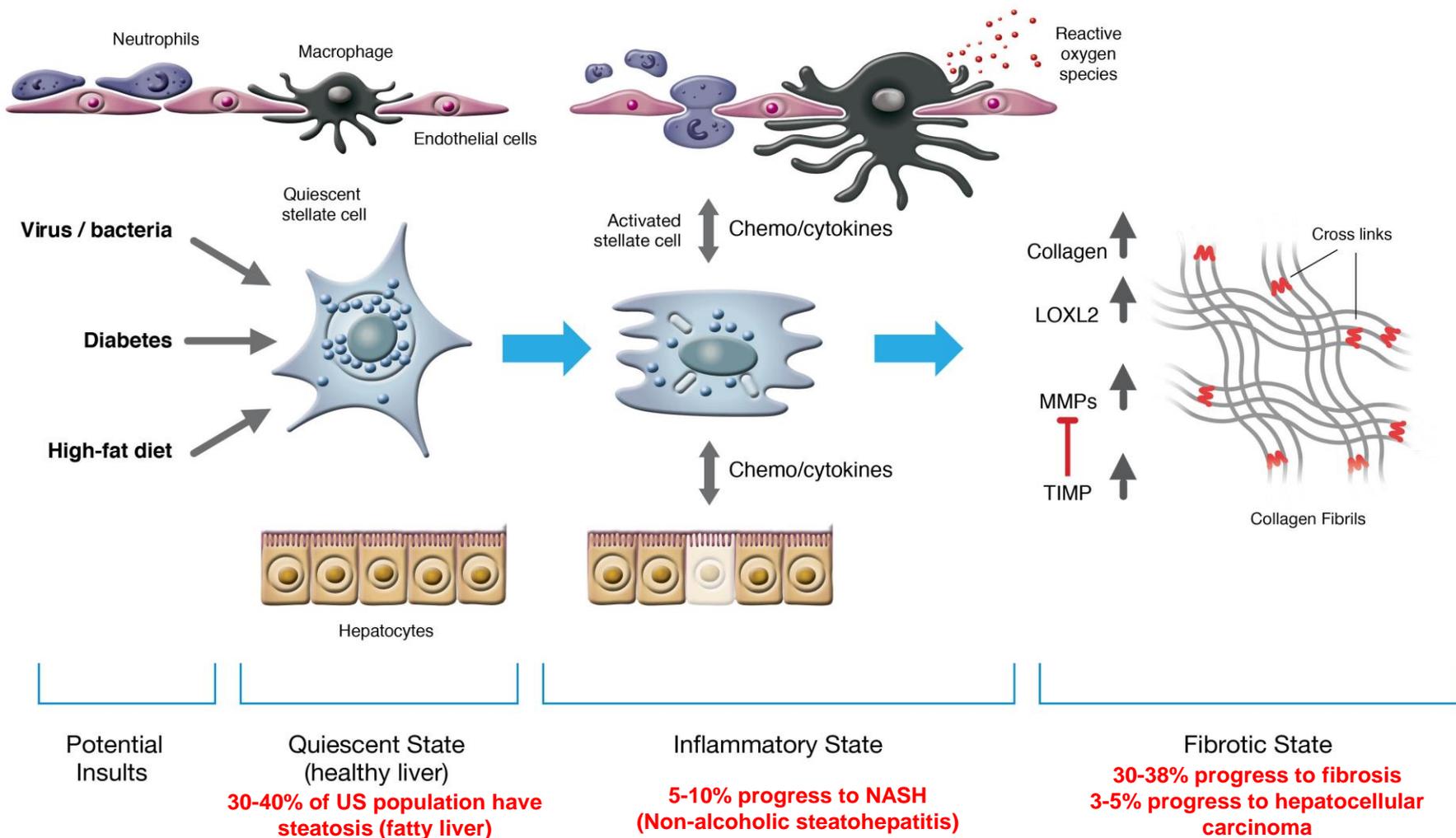
# Drug discovery chemistry platform

Amine oxidase enzymes; well validated targets in diseases with a high unmet medical need

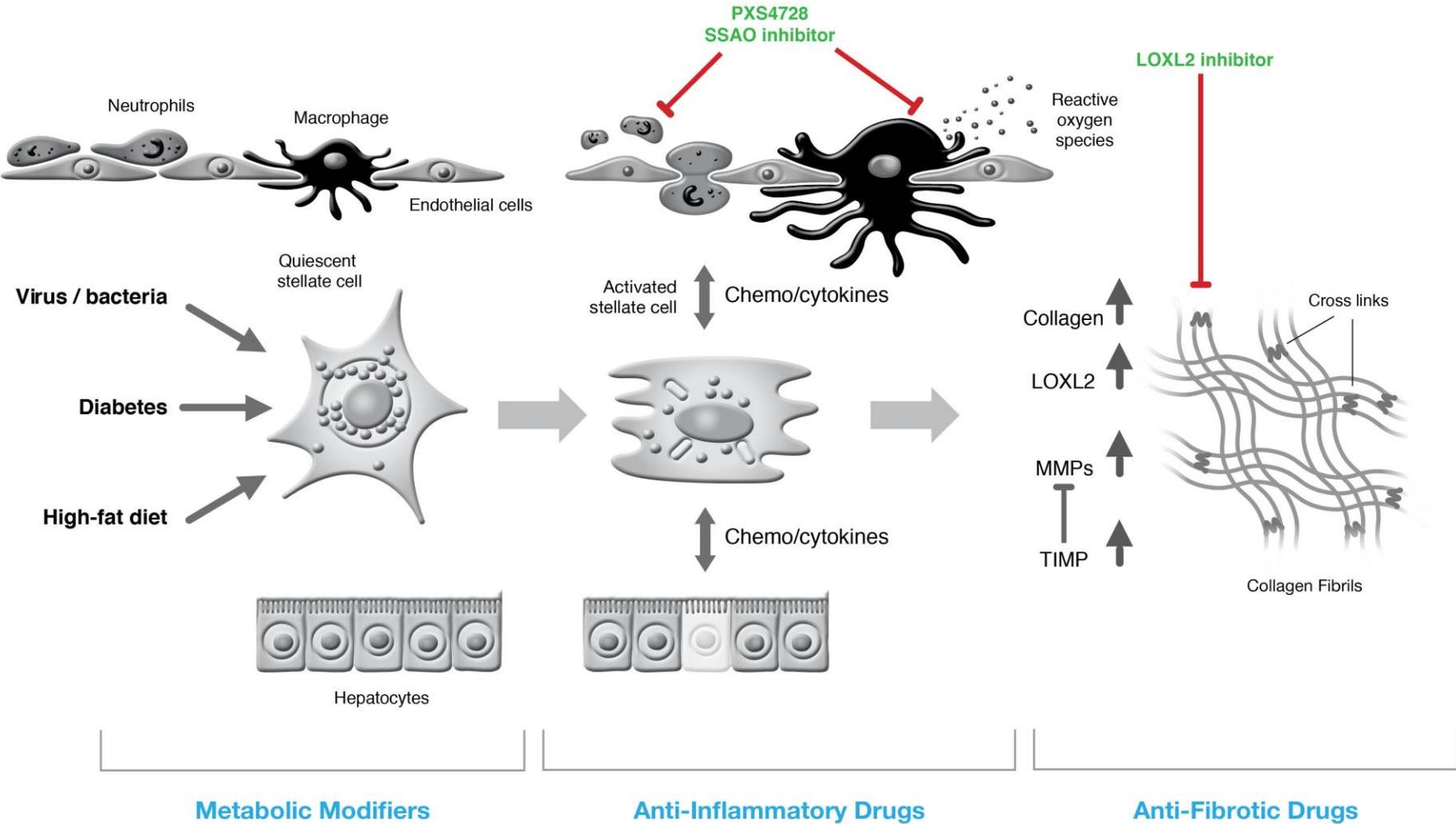


**Current focus on Liver fibrosis and NASH**

# Drugs targeting NASH → Cirrhosis



# Drugs targeting NASH → Cirrhosis



# Drugs in the clinic targeting NASH

Several large Pharma companies seeking to build competitive portfolios

	Metabolic modifiers	Anti-inflammatory	Anti-fibrotic
Intercept	Ph 3		
Genfit	Ph 3		
Galmed	Ph 2/3		
Allergan	Ph 2	Ph 2	
Gilead	Ph 2 x 2	Ph 2	
BMS	Ph 2		Ph 1
Galectin			Ph 2
Novartis	Ph 2		
AstraZeneca		Ph 2	
Shire	Ph 2		
Boehringer Ingelheim		Ph 1	
Other	Ph 2 x 3	Ph 2 x 4	

# SSAO for NASH



SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

## PXS-4728A

- Mechanism based inhibitor of SSAO
  - Small molecule oral drug
  - Important pathway in several inflammatory diseases of the liver, kidney, heart, eye and CNS.
- Development status
  - Pharmaxis discovery – patent filed 2012
  - Effective in pre clinical models of NASH and airway inflammation
  - Phase 1 study reported
    - orally bioavailable
    - long lasting enzyme inhibition after single dose
    - progressive dose response
  - Phase 2 NASH trial scheduled H1 2017

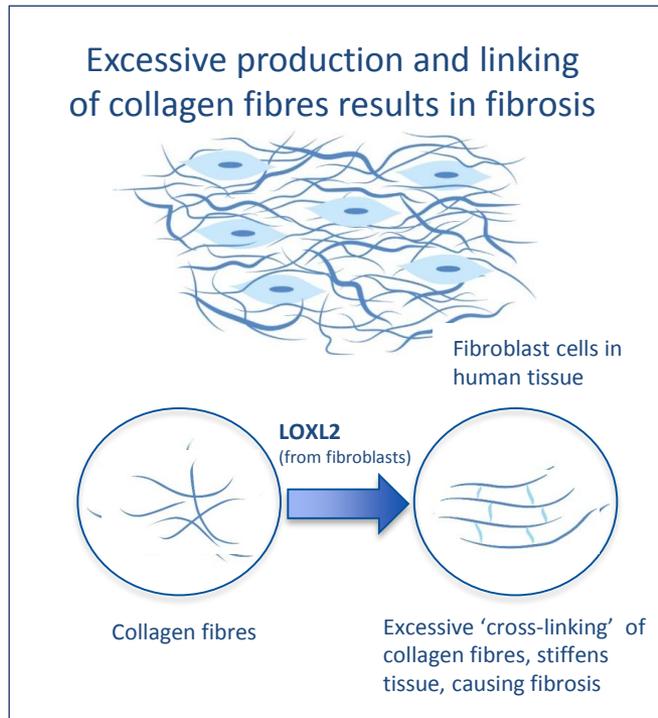
## End of Phase 1 deal with Boehringer

- Potential milestones to approval: €418.5m (~A\$600m)
  - Upfront (May 2015): €27.5m (~A\$39m)
  - 1<sup>st</sup> Indication (NASH)
    - Commencement of phase 2 and 3: total €55m (~A\$80m)
    - Filing, regulatory & pricing approvals: total €140m(~A\$200m)
  - 2<sup>nd</sup> indication (commercial in confidence)
    - Commencement of phase 2: €10m
    - Total milestone payments to approval: €195m (~A\$280m)
- Earn-out payments on annual net sales
  - Tiered percentages increasing from high single digits
  - Plus sales milestones

**External validation of PXS drug discovery and ability to negotiate valuable global deals**

# LOXL2 inhibition for NASH & other fibrotic diseases

## An attractive target and development program



- Potential indications:
    - NASH / Liver Fibrosis
    - Pulmonary fibrosis (IPF)
    - Cancer
    - Kidney
    - Cardiac fibrosis
- Significant market opportunity
- Development status:
    - Pharmaxis discovery – patent filed 2016
    - Effective in pre clinical models of fibrosis and cancer
    - Candidate compounds identified
    - Preclinical toxicity studies commenced Q4 2016 (significant de-risking step)
  - Competitive profile:
    - Novel target and mechanism of action
    - Once daily oral drug
    - Complete inhibition of LOXL2 versus partial inhibition by antibody
    - Selective inhibition over other amine oxidases Low cost of goods

# LOXL2 for pulmonary fibrosis



## Collaboration with Synairgen

### Idiopathic Pulmonary Fibrosis (IPF)

- IPF primarily affects people over the age of 50
- 5,000 patients have IPF in Australia
- 100,000 people with IPF in the US
- Prognosis is worse than that of many cancers
- Two drugs approved recently
  - Nintedanib (Boehringer Ingelheim)
  - Pirfenidone (Roche)
- Need for new therapies
- Current products expected to produce global revenues > \$1.1 billion by 2017

### Synairgen collaboration

- Access to
  - Synairgen's strength in fibrosis biology and respiratory clinical development - BioBank human tissue models technology platform
  - Clinical expertise at University of Southampton
- Faster time to value appreciation and partnering points of phase 1 or 2a
- Synairgen to fund pre clinical tox and phase 1
- Shares risk and reward based on investment in program
- Revenue share for IPF phase 1 partnering deal: 50/50
- Larger value partnering deal(s) from additional indications

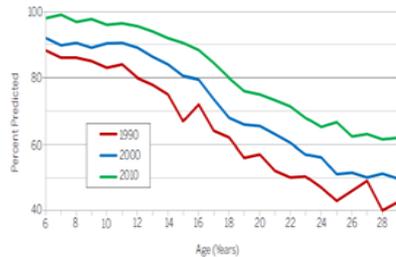
# Fibrosis and NASH M&A

Attractive deal values for phase 1 and phase 2 clinical assets

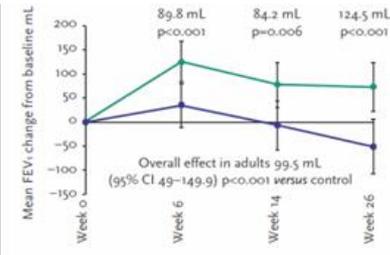
Acquirer	Company	Indication	Deal Type	Stage	Upfront (US\$M)	Potential (US\$M)
<b>&lt; 2 years ago</b>						
Gilead	Nimbus	NASH - metabolic	Partnership	P1	400	1,200
Gilead	Phenex	NASH – metabolic	Asset Aqun	P2	U	470
Novartis	Conatus	NASH - inflammatory	Option	P2	50	650
Allergan	Tobira	NASH - inflammatory	Acquisition	P2	400	800
Allergan	Akarna	NASH - metabolic	Acquisition	Pre	50	U
BMS	Promedior	IPF+	Acquisition	P2	150	1,250
BMS	Galecto	IPF	License	P1	U	444
BMS	Nitto Denko	NASH - fibrotic	License	P1	100	U
Boehringer	Inventiva	IPF+	License	Discovery	U	€189+
Boehringer	Pharmaxis	NASH - inflammation	Asset Aqun	P1	A\$40	A\$750+
<b>&gt; 2 years ago</b>						
BMS	Amira	IPF	Acquisition	P1	325	150
Gilead	Arresto	NASH – fibrosis +	Acquisition	P1	225	225
Biogen Idec	Stromedix	IPF	Acquisition	P2	75	487
Shire	Lumena	NASH – inflammatory	License	P1	260	U
Shire	Fibrotech	Diabetic nephropathy	Acquisition	P1b	75	482
AZ	Regulus	NASH- metabolic +	License + equity	Pre	U	500

# Bronchitol for cystic fibrosis

## Overview



Median FEV<sub>1</sub>, % Predicted versus Age



Pooled adult data from CF301 and CF302



## 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

## CF301/2 trial (adult)

- Total 317 adults
- FEV<sub>1</sub>
  - CF301; p=0.001
  - CF302; p=0.038
  - Pooled; p=0.001
  - rel % change = 4.7%
- Exacerbations
  - Pooled data
  - 26% reduction
  - 60% reduction in Bronchitol responders

## CF303 - trial for US

- 423 adult patients
  - 21 countries
  - 126 sites
  - Patients on best standard of care
- FEV<sub>1</sub>
  - Improved 54 ml (p=0.020)
  - Relative % change = 2.2% (p=0.025)
- Good safety profile
- Chiesi pursuing resubmission of NDA

# Bronchitol for cystic fibrosis

## Transitioning to profitability



### Business model

- 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%+
- Leverage fixed manufacturing costs by increasing volumes of Bronchitol & Aridol

### Sales growth

- Russian approval received Oct 2016 – first sale Q1 2017. Pricing approval expected H2 2017
- Chiesi appointed Italian distributor May 2017
- Turkey – launched CY 2016
- Pending approval/pricing in Israel, Brazil, Eastern European countries

### US market

- Largest CF market by value
- 28,103 CF patients
- 49.7% adults
- Bronchitol price target US\$20k per patient pa
- 7 year post launch market exclusivity
- Chiesi to file updated NDA 2018

### US partner: Chiesi

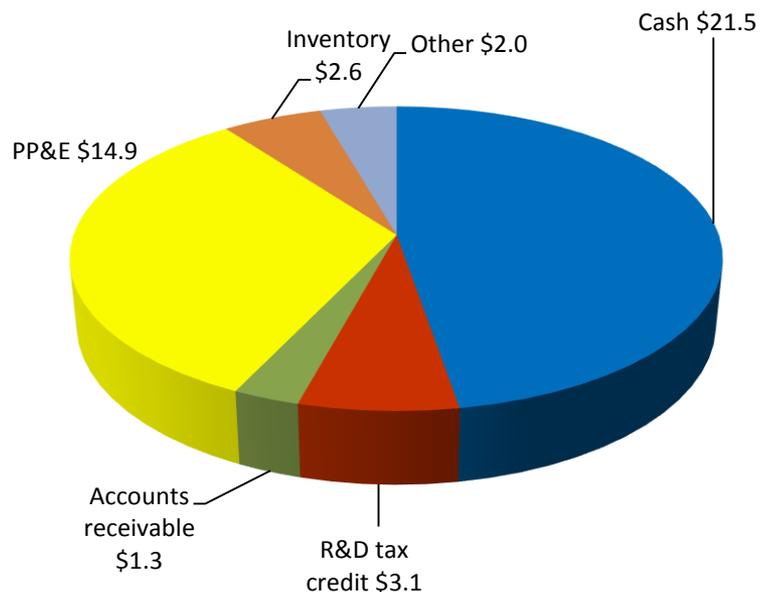
- Chiesi responsible for regulatory filing & commercialisation
- ~A\$13m milestone payment on launch, plus sales milestones
- PXS supplies US market from factory in Sydney
- PXS receives high mid teens % of in-market sales plus cost of goods

# Financials highlights

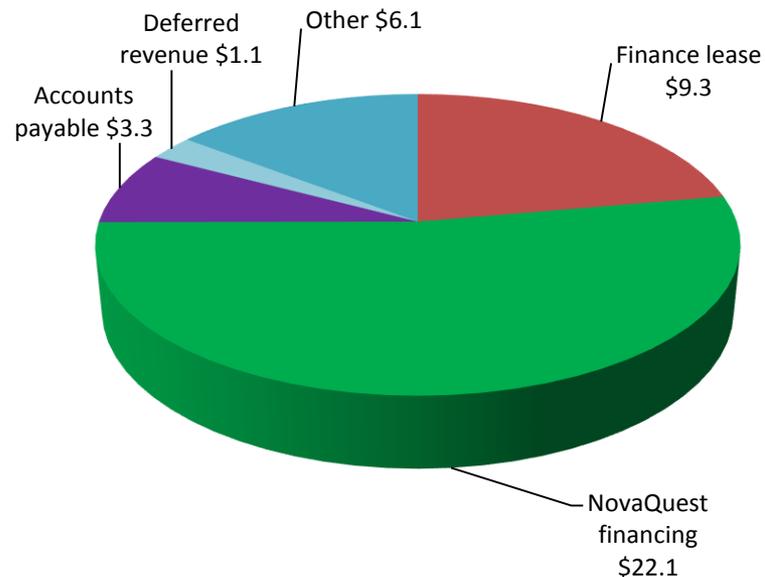
(unaudited)	A\$'000		Three months ended		Twelve months ended	
			30-June-17	30-June-16	30-June-17	30-June-16
<b>Income statements</b>						
Sales			866	703	4,823	6,135
Total revenue			6,945	4,795	18,001	19,020
Total expenses			(11,067)	(6,919)	(36,347)	(35,476)
Net profit (loss) after tax			<b>(4,122)</b>	<b>(2,124)</b>	<b>(18,346)</b>	<b>(16,463)</b>
<b>Segment results – adjusted EBITDA</b>						
Bronchitol & Aridol			(2,421)	(2,141)	(7,100)	(8,228)
<i>Bronchitol &amp; Aridol – excluding net clinical trial costs</i>			<i>(1,803)</i>	<i>(1,582)</i>	<i>(5,546)</i>	<i>(5,228)</i>
New drug development			199	92	(4,114)	(2,625)
Corporate			(1,005)	(1,132)	(4,017)	(3,988)
Total			<b>(3,227)</b>	<b>(3,181)</b>	<b>(15,231)</b>	<b>(14,841)</b>
<b>Statement of cash flows</b>						
Cash inflow/ (outflow) from:						
Operations			(4,228)	(1,559)	(15,181)	(11,989)
Investing activities			(328)	(146)	(725)	(1,381)
Financing activities			(434)	(425)	(1,721)	(1,714)
Total cash used			<b>(4,990)</b>	<b>(2,130)</b>	<b>(17,627)</b>	<b>(15,084)</b>
Foreign currency exchange rate changes impact on cash			(5)	(169)	(78)	155
<b>Cash at bank</b>			<b>21,504</b>	<b>39,209</b>	<b>21,504</b>	<b>39,209</b>

# Balance sheet – 30 June 2017

## Assets (\$45m)



## Liabilities (\$42m)

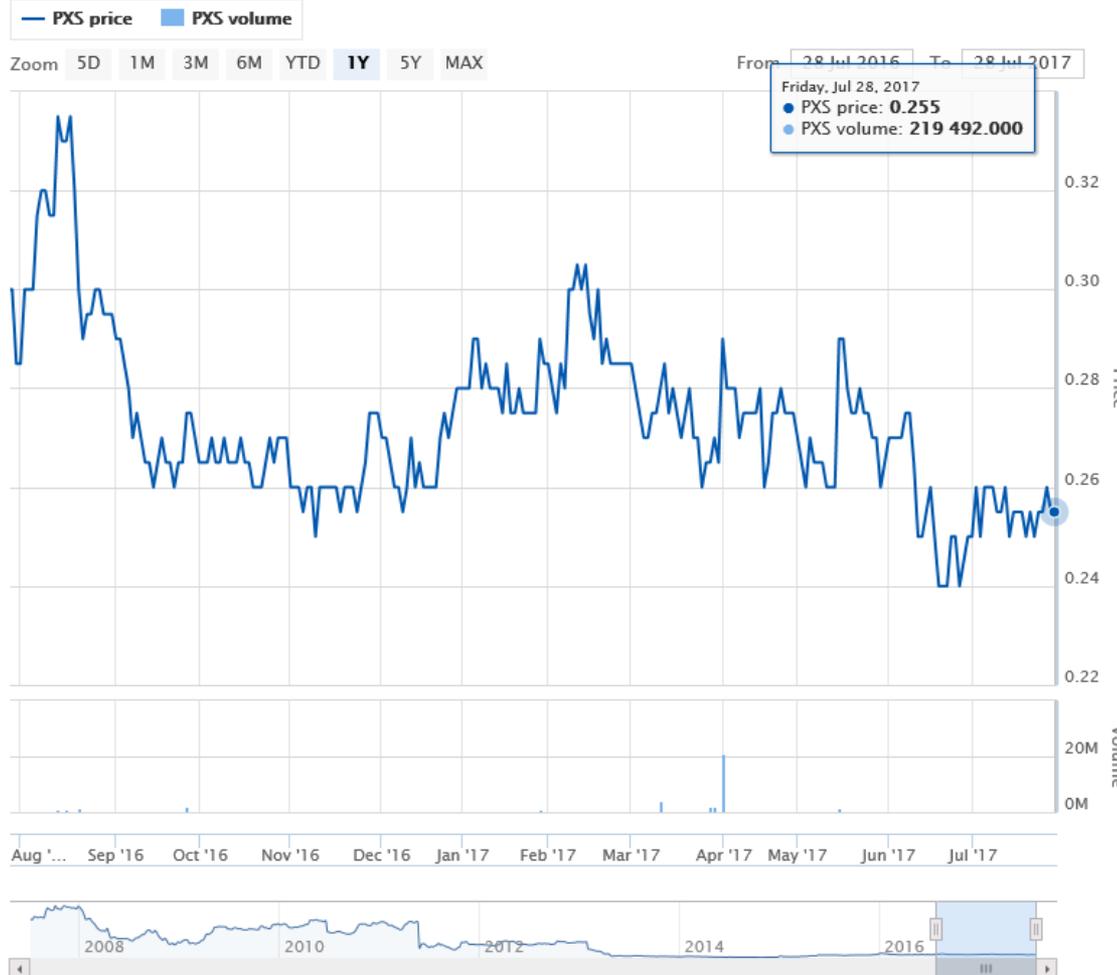


- Finance lease over 20 Rodborough Rd (to 2024)
- NovaQuest financing – not repayable other than as % of Bronchitol revenue

# Shareholders & trading



ASX code: PXS



## Shareholders (28 July 17)

- Shares on issue: 319m
- Employee options: 13m
- Institutional shareholders ~50%:
  - Australia/NZ: Australian Ethical (10%); Allan Gray (8%); Other (1%)
  - US - BVF Partners (19%); Other (2%)
  - UK - Montoya Investments (6%); Other (3%)

## Shares traded to 28 July 17

- Three months: 11m
- Six months: 49m
- Twelve months: 79m

## Market capitalisation

- A\$81m (28 July 17)

# News flow



CY 2017

CY 2018

- PXS4728A NASH Phase 2 commences & €18M milestone payable (mid year 2017)
- PXS4728A 2<sup>nd</sup> indication Phase 2 commences & €10M milestone payable (H2)



Bronchitol – RoW

- Russia – pricing approval



Bronchitol – US

- FDA re-submission



New drug development

➤ LOXL-2 (NASH / IPF / Other)

- Complete GLP tox program for ≥1 compounds
- Commence ≥1 phase 1 studies

- Complete 1 phase 1 study
- Partner ≥1 compound

➤ SSAO/MPO

- Commence GLP tox program
- Phase 1 ready

- Commence phase 1 study

➤ LOX (Scarring / Cancer)

- Commence GLP tox program
- Phase 1 ready

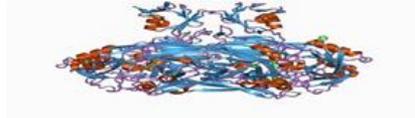
- Commence phase 1 study

➤ LOXL-2 (Cancer)

- Update from leading universities/academics assessing in cancer

# Pharmaxis opportunities for growth

Building a biotech powerhouse in fibrosis and inflammation



## SSAO program for NASH (fatty liver)

- NASH: US\$35B market by 2025
- Acquired by BI at phase 1 for A\$39m upfront, total >A\$750m
- BI to develop for NASH and other inflammatory indications
- Next milestone: €18m at start of phase 2 NASH – mid 2017
- 2<sup>nd</sup> indication milestone: € 10m at start of phase 2 (H2 2017)

## LOXL2 program

- NASH market >\$35B
- Pulmonary fibrosis: market >\$1B
- Strong big Pharma interest in LOXL2 and PXS drug candidates
- Formal preclinical to report Q3 2017
- Next step – commence phase 1 in H2 2017
- Synairgen collaboration increases value and shares risk

## Discovery pipeline

- LOX
  - Scarring and severe fibrosis
  - Commence preclinical H2 2017
- SSAO/MPO
  - Respiratory and cardiovascular inflammation
  - Commence preclinical H2 2017

## Bronchitol for CF

- Growth and profits over next 12 – 24 months from existing markets including Russia
- Chiesi pursuing access to large US CF market
  - ~A\$13m milestone payments on launch
  - High teens % share of in-market sales plus supply of product by PXS

# Conclusions

- **Strong therapeutic focus** in area of high unmet medical need and increasing interest to big Pharma
- **Productive R&D engine** and capacity to run multi-centre international studies
- **Track record** of value adding business development
- **Strong news flow** over the next 12 months
- **Strong balance sheet** – A\$21.5m cash at June 2017 with likely milestones of ~A\$42m in 2017