

# Annual General Meeting

29 November 2016



## Chairman's Address

Malcolm McComas



## Chief Executive Officer's Address

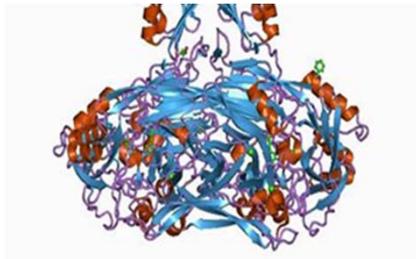
Gary Phillips

# 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 deliver value



## Drug development

- Focus on fibrosis and inflammation
- Strong Pharma interest in validated small molecule technology platform
- Three additional drugs acting on high value targets approaching the clinic over next 24 months



## 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
- Significant value milestones from existing partner deals near term
- Pipeline providing multiple future opportunities
- Synairgen collaboration developing additional indication



## Financial strength

- A\$32m cash balance at September 2016; average annual cash usage \$1.5m pm
- Boehringer phase 2 initiation milestone expected H1 2017 ~A\$25m
- Market cap \$84M\*
- 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 15 years' experience in pharmaceutical drug discovery and published more than 20 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 thirty 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 15 years experience in clinical trial design and management
- author of more than 60 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

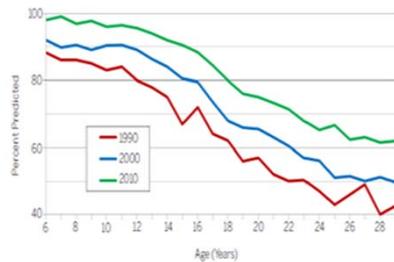
- **Malcolm McComas – Chair**
  - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- **Gary Phillips – Managing director**
- **Will Delaat – Non executive director**
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- **Simon Buckingham – Non executive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

# Pharmaxis product portfolio

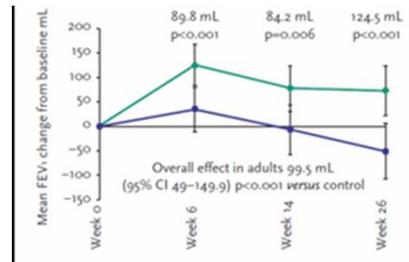
	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed	
Bronchitol US	Cystic fibrosis								
RoW	Cystic fibrosis							Distributors	
Aridol	Asthma diagnosis							Distributors	
SSAO	NASH+								
<u>Discovery</u>									
SSAO/MAO-B	Neuro inflammation								
SSAO/MPO	Respiratory inflammation								
LOXL-2	NASH, liver fibrosis								
LOXL-2 (IPF)	Pulmonary fibrosis				synairgen				
LOXL-2 (other)	Other fibrotic & cancer			Leading universities/academics assessing in kidney fibrosis and cancer					
LOX	Skin scarring								
Orbital	Dry powder inhalation device					Seeking Partners			
ASM-8	Asthma						Seeking Partners		

# 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

## CF204 trial results

- Paediatric age 6-17
  - Placebo-controlled
  - 8 weeks crossover design
  - standard therapy continued
- Primary endpoint:
  - Absolute change in FEV<sub>1</sub>: 3.42%; p=0.004
- Key secondaries
  - Absolute change in FEF<sub>25-75</sub>: 5.75% (p=0.005)
- Acceptable safety profile
  - Exacerbations and lung infection reduced by ~25%

# Bronchitol for cystic fibrosis

Partnering for success



## US market

- Largest CF market by value
- 28,103 CF patients
- 49.7% adults
- Bronchitol price target US\$20k per patient / year
- 7 year post launch market exclusivity

## US partner: Chiesi

- Fund CF303 up to US\$22m
- ~A\$13m milestone payment on launch, plus sales milestones
- High mid teens royalty % on in-market sales
- Mid teens % uplift on COGs
- Chiesi responsible for regulatory filing & commercialisation

## US trial: CF303

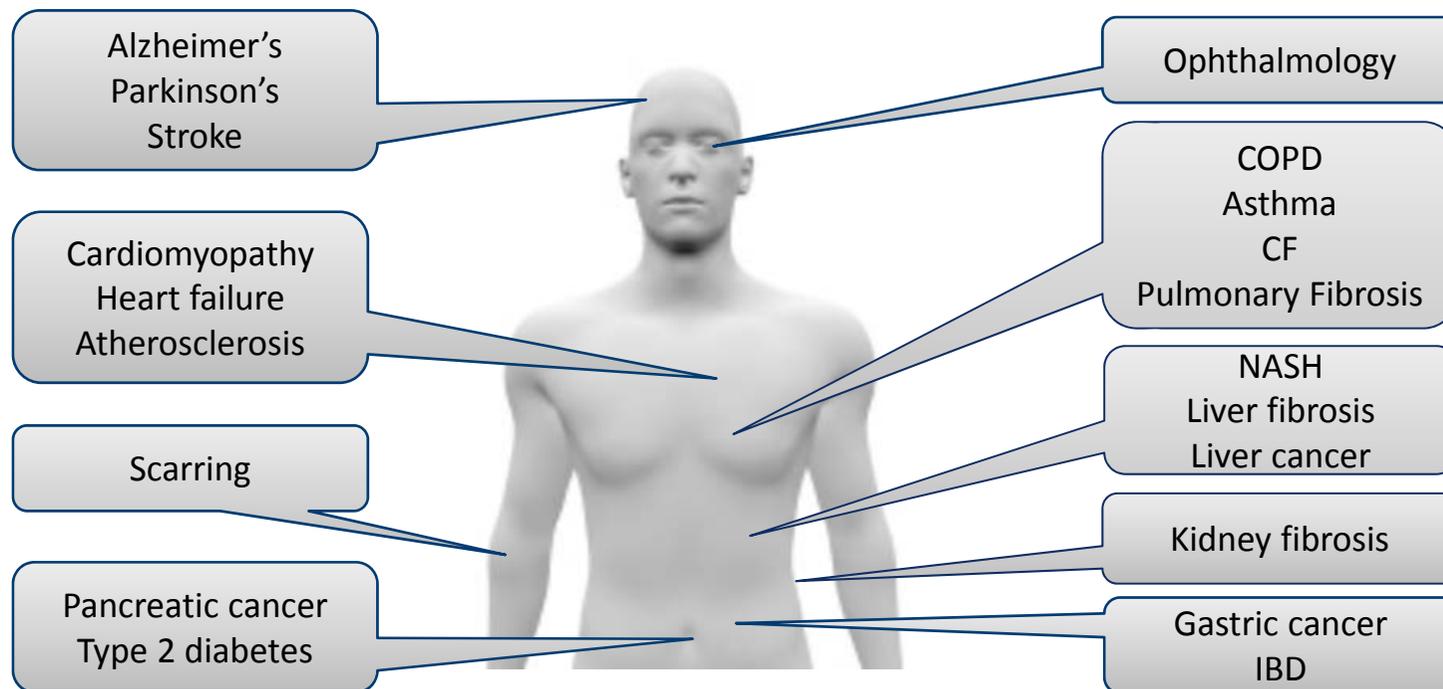
- Tie-breaker phase 3 trial commenced Q4 2014, managed by PXS
- 423 adult patients
  - 21 countries
  - 126 sites
- Design
  - Full consultation with FDA
  - Similar design to CF301/2
- Fully recruited July 2016
- Results H1 2017

## Rest of world

- Sold by Chiesi in UK & Germany
- Sold by PXS in Australia & Denmark
- Russian approval received Oct 2016 – first order received Nov
- Pending approval/pricing/distributor appointments in Israel, Turkey, Brazil, Eastern Europe countries

# Drug discovery

Applying amine oxidase chemistry to inflammation and fibrosis



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

# Drug discovery

Our therapeutic focus is inflammation and fibrosis



## Pharmaxis drug discovery

- NASH & liver fibrosis (LOXL2)
- Respiratory – COPD, asthma, cystic fibrosis (SSAO/MPO)
- Neuro inflammation – Alzheimer's, Parkinson's, stroke (SSAO/MAO-b)

Collaborations allow us to leverage our platform without losing focus



## Collaboration with Synairgen

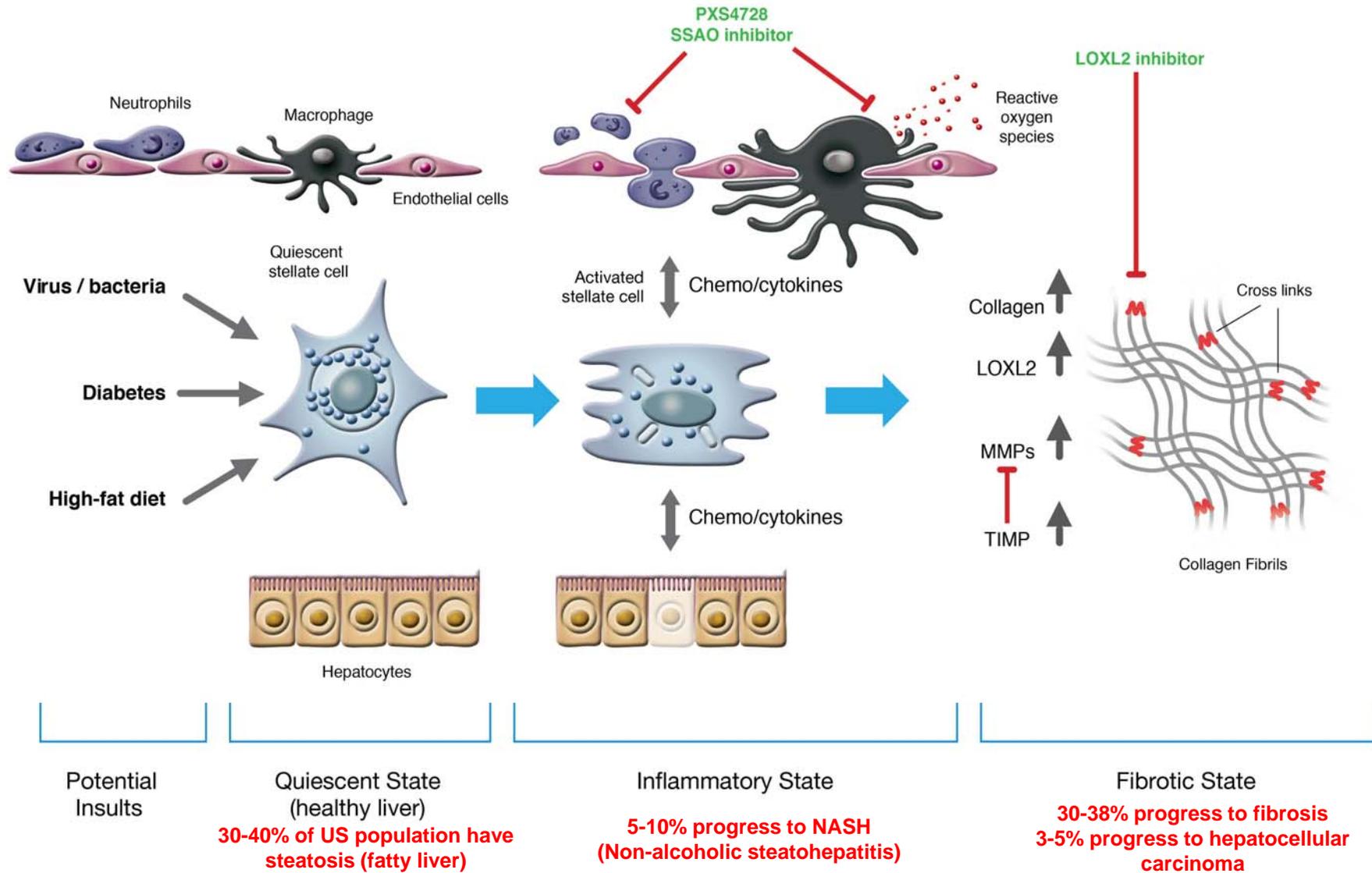
- Pulmonary fibrosis (LOXL2)



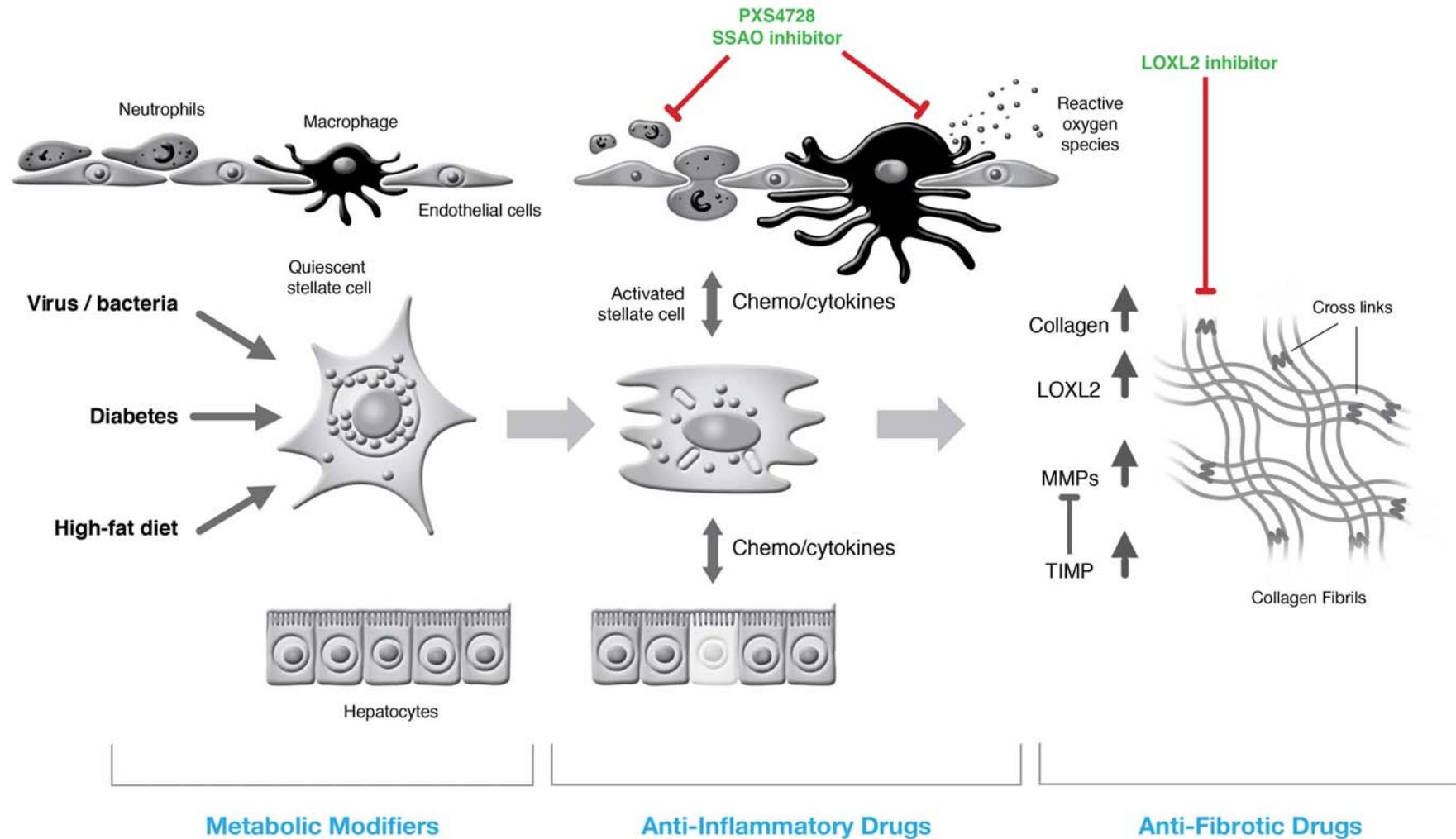
## Exploratory academic collaborations (LOX/LOXL2)

- Scarring
- Kidney fibrosis
- Some cancers

# 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
Immuron		Ph 2	
Shire	Ph 2		
Boehringer Ingelheim		Ph 1	
Other	Ph 2 x 3	Ph 2 x 3	

# SSAO for NASH



SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

## PXS-4728A

- Mechanism based inhibitor of SSAO
  - Small molecule inhibitor of SSAO (VAP-1)
  - Important inflammatory pathway in several diseases including NASH and COPD
- 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 scheduled H1 2017

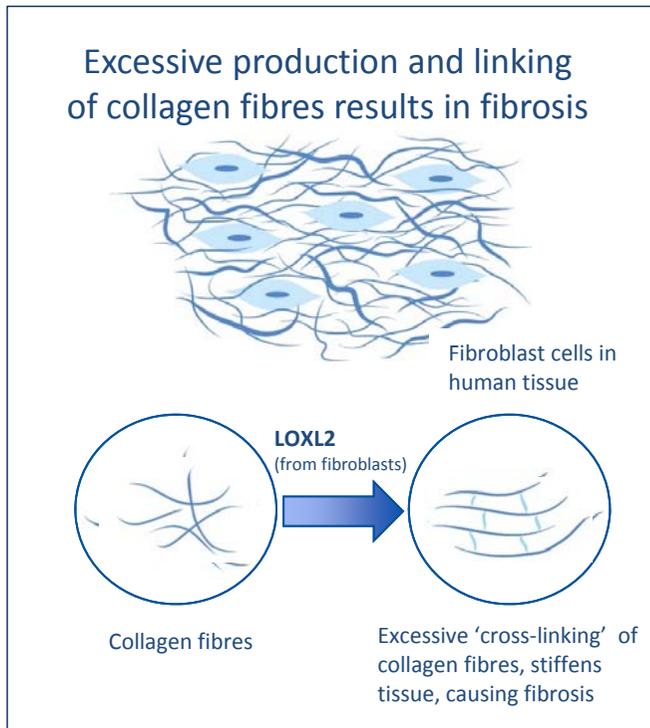
## Competitive deal with Boehringer

- Total potential payments to approval for 2 indications: €418.5m (~A\$600m)
  - Upfront (May 2015): €27.5m (~A\$39m)
  - Commencement of phase 2 and 3: up to total €55m (~A\$80m)
  - Filing, regulatory & pricing approvals: up to total €140m (~A\$200m)
  - Second indication: additional total milestone payments (€195m)
- Earn-out payments on annual net sales
  - Tiered percentages increasing from high single digits
  - Plus potential 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
    - Wound healing / Scarring
- Significant market opportunity
- Development status:
    - Pharmaxis discovery – patent filed 2016
    - Effective in pre clinical models of fibrosis and cancer
    - Lead candidate compounds identified
    - Preclinical toxicity studies scheduled 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

# 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
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

# News flow



**CY 2016**

**CY 2017**

**CY 2018**



**Bronchitol – RoW**



**New drug development**

**LOXL-2**

**SSAO/MAO-B**

**SSAO/MPO**

**LOX**

**LOXL-2**

PXS4728A Phase 2 commences & ~A\$25M milestone payable (H1)

EU Paediatric label extension application (H1)  
First Russian sales

CF303 – trial completion and report (H1)

Bronchitol approval

Nominate LOXL2 candidate(s) and commence full preclinical studies ≥1 programs

Commence ≥1 phase 1 studies  
Complete 1 phase 1 study

Partner ≥1 compound

Selection of indication

Selection of indication

Select compound to move into preclinical studies

Commence GLP tox program

Commence phase 1 study

Select compound to move into preclinical studies

Commence GLP tox program

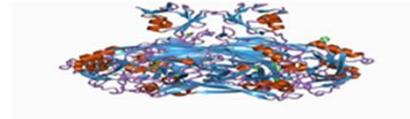
Commence phase 1 study

Leading universities/academics assessing in kidney fibrosis, cancer and wound healing

Select ≥1 compound to move into preclinical studies

# 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 (eg. kidney fibrosis, COPD)
- Next milestone: ~A\$25m at start of phase 2 – H1 2017

## LOXL2 program for pulmonary fibrosis

- Pulmonary fibrosis: market >\$1B
- Collaborate to phase 1 or 2 then seek partner
- Revenue share for phase 1 partnering deal: 50/50
- Next step – commencement of formal preclinical program H2 2016

## LOXL2 for NASH and other diseases

- NASH market >\$35B
- Strong big Pharma interest in LOXL2 and PXS chemistry
- Complimentary to SSAO program acquired by BI
- Next step – commencement of formal preclinical program H2 CY 2016

## Bronchitol for CF

- Access large US CF market with Chiesi
  - Chiesi funding CF303 to a cap of US\$22m
  - ~A\$13m milestone payments on launch
- High teens % share of in-market sales
- Growth from existing markets
- New markets opening over next 24 months, including large Russian market



# Financial Overview

David McGarvey CFO

# Financials – highlights

30 June 2016

A\$'000	2016	2015	2014
<b>Income Statements</b>			
Sales revenue	6,135	5,999	5,036
Other revenue	12,885	53,248	5,450
<b>Total revenue</b>	<b>19,020</b>	<b>59,247</b>	<b>10,486</b>
Expenses	(35,476)	(40,739)	(62,201)
Net profit (loss) before tax	(16,456)	18,508	(51,715)
<b>Net profit (loss) after tax</b>	<b>(16,463)</b>	<b>18,466</b>	<b>(51,818)</b>
<b>Segment results - adjusted EBITDA</b>			
Bronchitol & Aridol	(8,228)	(10,045)	(22,555)
New drug development	(2,625)	35,068	(1,620)
Corporate	(3,988)	(3,532)	(6,226)
	<b>(14,841)</b>	<b>21,491</b>	<b>(30,401)</b>
<b>Cash flow</b>			
Operations	(11,989)	21,780	(28,132)
Investing activities	(1,381)	(264)	(313)
Financing activities	(1,714)	(1,791)	(1,357)
	<b>(15,084)</b>	<b>19,725</b>	<b>(29,802)</b>
<b>Cash at bank</b>	<b>39,209</b>	<b>54,138</b>	<b>34,182</b>

## Highlights of 2016:

- Sales revenue maintained after appointment of distributors
- Other revenue continues to fund the business plan
- Reduction in expenses – see subsequent slides
- Business segments tracking to plan
- Cash flow tracking to plan
- 2016 investing included:
  - Additional R&D equipment
  - Manufacturing energy saving initiatives

# Financials – income statement revenue

30 June 2016

A\$'000	2016	2015	2014
<b>Revenue</b>			
Sales revenue			
Bronchitol	4,302	4,243	3,275
Aridol	1,833	1,715	1,752
Other products		41	9
	6,135	5,999	5,036
Other revenue			
Sale of drug candidate		40,603	
Clinical trial cost reimbursements	8,200	11,139	
Interest	1,213	721	1,735
R&D tax incentive	2,100	164	3,539
Other income	1,372	621	176
	<b>19,020</b>	<b>59,247</b>	<b>10,486</b>

## Highlights of 2016:

- Bronchitol sales maintained sharing of revenue with newly appointment distributors. Chiesi building inventory.
- Aridol sales growth without any sales/marketing investment
- 2015 included Boehringer Ingelheim acquisition or PXS4728A for \$41
- Clinical trial cost reimbursement (by Chiesi)
- R&D tax incentive available again - \$2.1m

# Financials – income statement expenses

30 June 2016

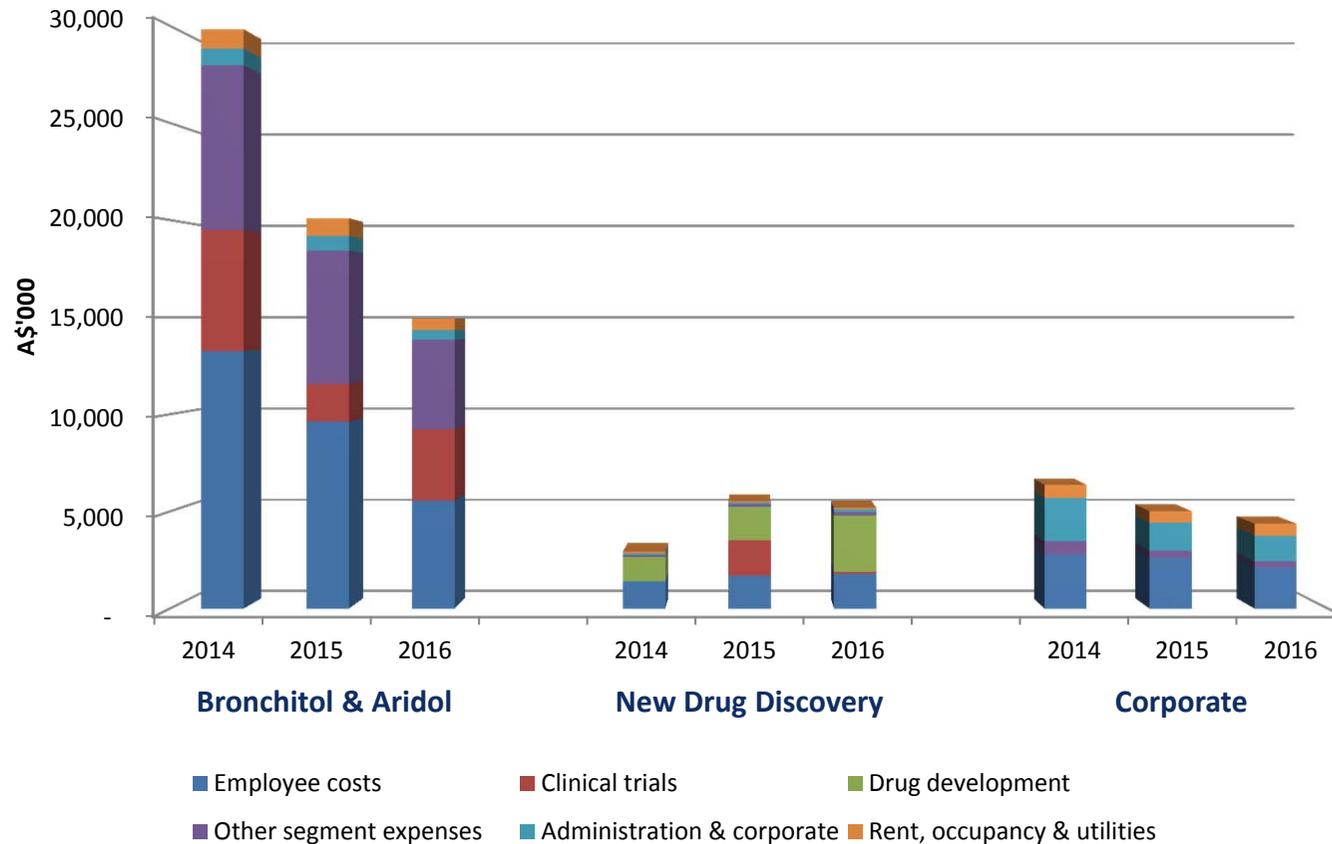
	A\$'000	2016	2015	2014
<b>Expenses</b>				
Employee costs		(10,529)	(14,111)	(19,376)
Administration & corporate		(2,082)	(3,316)	(3,379)
Rent, occupancy & utilities		(1,296)	(1,593)	(1,767)
Clinical trials		(11,955)	(11,315)	(6,221)
Drug development		(3,915)	(1,695)	(1,256)
Sales, marketing & distribution		(1,101)	(1,962)	(3,376)
Safety, medical and regulatory affairs		(1,707)	(1,723)	(1,852)
Manufacturing purchases		(1,928)	(1,736)	(2,142)
Other		(382)	(1,905)	(1,640)
Depreciation & amortisation		(3,028)	(3,406)	(5,131)
Foreign exchange gains & losses		(843)	(395)	(132)
Finance expenses		2,459	2,696	(7,146)
Impairment expenses		(174)	(277)	(8,783)
		<b>(35,476)</b>	<b>(40,739)</b>	<b>(62,201)</b>
Net profit (loss) before tax		(16,456)	18,508	(51,715)
Income tax expense		(7)	(42)	(103)
<b>Net profit(loss) after tax</b>		<b>(16,463)</b>	<b>18,466</b>	<b>(51,818)</b>

## Highlights of 2016:

- 2016 – first full financial year of new business
- Significant cost reductions from changes to business across most items
- Additional investment in drug development
- Additional investment in clinical trials – where Chiesi is funding US\$22m of forecast US\$26m cost of CF303
- Finance expense includes capitalised finance lease on 20 Rodborough Road (~\$0.7m pa) and credits in relation to the NovaQuest financing agreement in 2015 and 2016

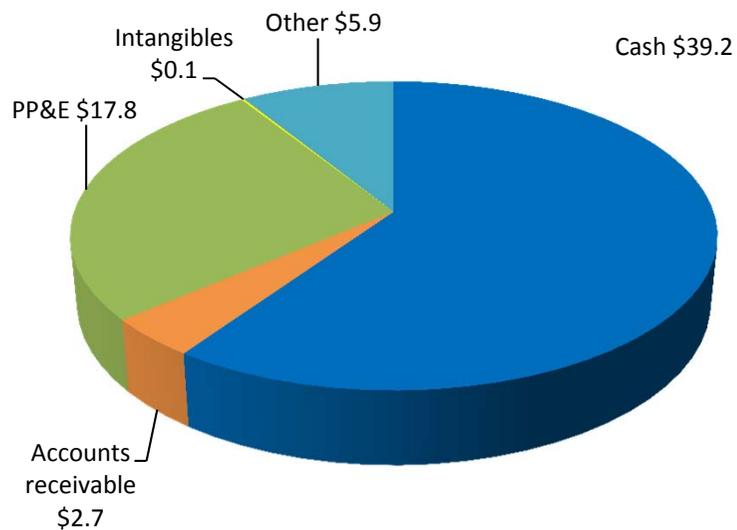
# Segment expenses: 3 year trends

excludes foreign exchange gains/losses and reimbursed clinical trial costs

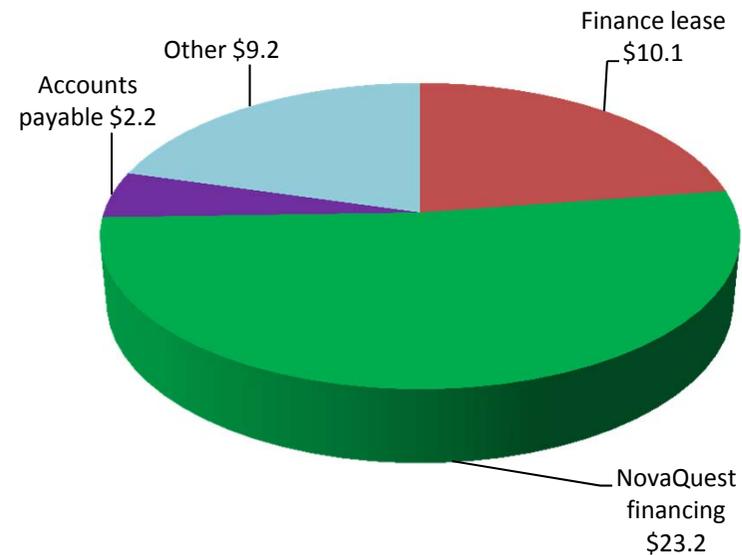


# Balance sheet – 30 June 2016

## Assets (\$66m)



## Liabilities (\$45m)



- Finance lease over 20 Rodborough Rd (to 2024, break possible in 2021)
- NovaQuest financing – not repayable other than as % of Bronchitol revenue

# Shareholders & trading

ASX code: PXS



## Shareholders (30 Sept 16)

- Shares on issue: 319m
- Employee options: 9.9m
- Institutional shareholders ~50%:
  - Australia - Orbis (17%); Australian Ethical (6%); Other (1%)
  - US - BVF Partners (13%)
  - US – other (2%)
  - UK - Montoya Investments (6%)
  - UK – other (3%)

## Shares traded to 30 Sept

- Three months: 22m
- Six months: 66m
- Year: 97m

## Market capitalisation

- A\$84m (30 Sept 16)



## Shareholder Questions



## Formal Business

# Resolution 1

Financial Report, Directors' Report and the Auditor's Report

**No shareholder vote is required**

# Resolution 2

Adoption of the Remuneration Report

**Ordinary resolution:**

***“That the remuneration report of the Company for the year ended 30 June 2016 is adopted.”***

# Resolution 2

## Adoption of the Remuneration Report

The Company has received:

- 150,616,995 proxy votes in favour of the resolution;
- 4,695,817 proxy votes against the resolution;
- 1,396,341 proxy votes abstaining from the resolution;
- 3,520,126 proxy votes excluded from voting;
- 399,224 proxies able to be voted by the chair/board which the chair/board intend to vote in favour of the resolution.

\* Voting exclusions apply

# Resolution 3

Re-election of Mr Malcolm McComas as a Non-Executive Director

**Ordinary resolution:**

***“That Mr Malcolm McComas, who retires and offers himself for re-election as a director of the Company, is re-elected as a non executive director of the Company.”***

# Resolution 3

## Re-election of Mr Malcolm McComas as a Non-Executive Director

The Company has received:

- 155,003,108 proxy votes in favour of the resolution;
- 5,288,837 proxy votes against the resolution;
- 229,834 proxy votes abstaining from the resolution;
- 396,724 proxies able to be voted by the chair/board which the chair/board intend to vote in favour of the resolution.

# Resolution 4

## Grant of Performance Rights to Mr Gary Phillips

### Ordinary resolution:

***“That for the purposes of the ASX Listing Rules and for all other purposes, approval is given for the grant of 827,000 zero grant price and zero exercise price employee options (Performance Rights) to Mr Gary Phillips under the Company’s performance rights plan, resolved to be granted by the Board in July 2016 and, upon exercise of those Performance Rights, the acquisition of 827,000 ordinary shares underlying those Performance Rights, in accordance with the terms of the performance rights plan and the explanatory statement accompanying the notice of meeting.”***

# Resolution 4

## Grant of Performance Rights to Mr Gary Phillips

The Company has received:

- 151,173,182 proxy votes in favour of the resolution;
  - 5,102,254 proxy votes against the resolution;
  - 1,347,364 proxy votes abstaining from the resolution;
  - 2,619,999 proxy votes excluded from voting;
  - 285,704 proxies able to be voted by the chair/board which the chair/board intend to vote in favour of the resolution.
- \* Voting exclusions apply



**Thank you for your participation**

2016 Annual General Meeting