



# Annual General Meeting

19 November 2015

# Chairman's Address

**Annual General Meeting  
November 2015**



# Investor Presentation

Annual General Meeting  
Sydney: 19<sup>th</sup> November 2015

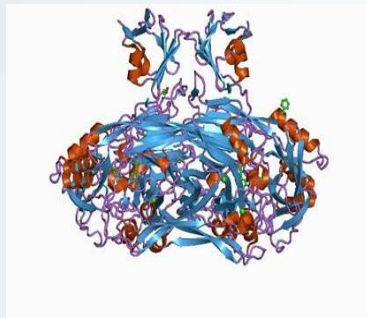
Gary Phillips CEO

# 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 today

new business focus already creating value



## Drug developer

- ❑ Leading position in amine oxidase chemistry and mechanism based inhibitors
- ❑ Proven capability in delivering quality programs to achieve phase 2 ready compounds
- ❑ Exciting pipeline of drug candidates for valuable targets



## Management

- ❑ Management team and Board with global experience
- ❑ Extensive pharma industry network
- ❑ Proven capability of executing global BD transactions with major partners
- ❑ Preclinical, early and late phase clinical experience



## Drug manufacturer

- ❑ Supplies Bronchitol to global markets via experienced commercial partners
- ❑ Financial risks shared
- ❑ Financial upside from accessing new markets – US, Russia
- ❑ Possibility to further rationalise manufacturing infrastructure



## Financial strength

- ❑ \$50m cash balance at September 2015
- ❑ Significant value milestones from existing partner deals within reach
- ❑ Growing institutional presence on share register - >45%

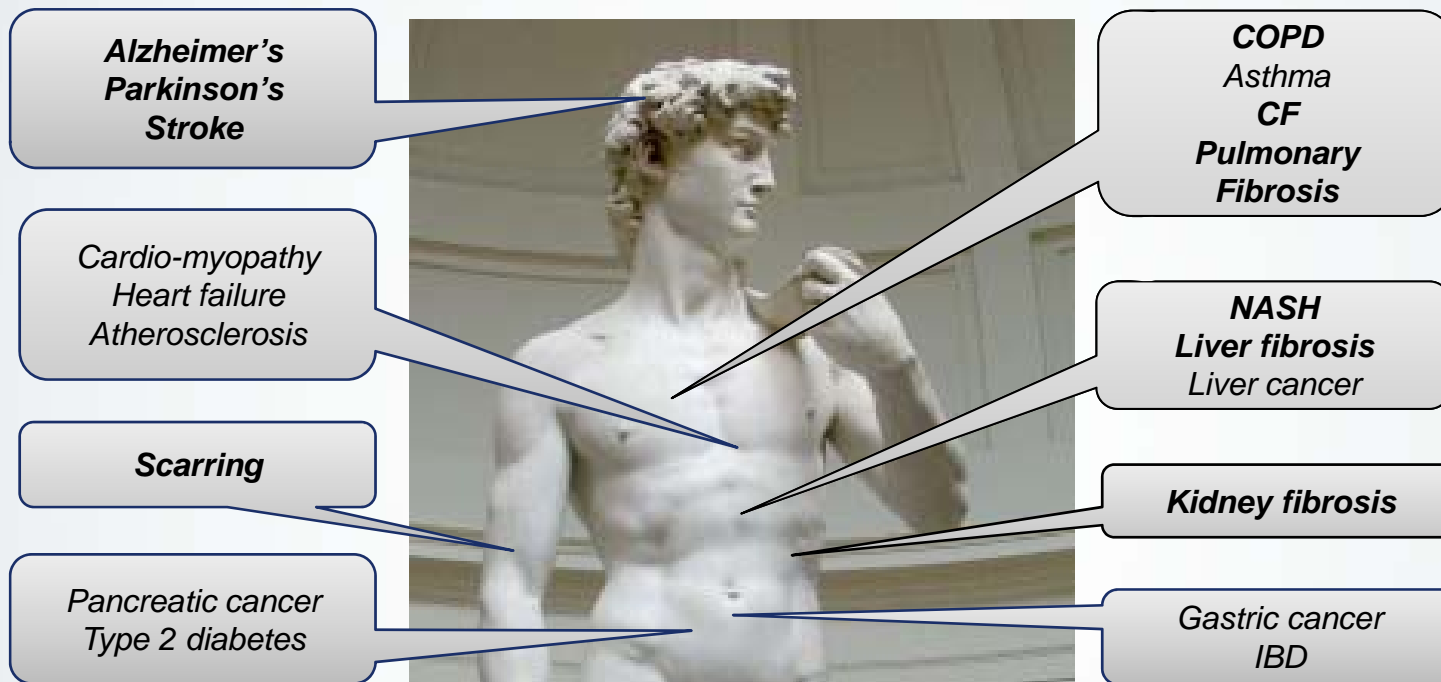
# Pharmaxis product portfolio

Product	Indication	Status
<i>LOXL2 inhibitor</i>	<i>NASH (fatty liver disease), Liver &amp; kidney fibrosis</i>	<i>Lead optimisation</i>
<i>LOXL2 inhibitor</i>	<i>Idiopathic pulmonary fibrosis</i>	<i>Lead optimisation; collaboration with Synairgen</i>
LOX/LOXL2 inhibitor	Fibrosis, cancer	Exploratory
<i>SSAO inhibitor</i>	<i>NASH</i>	<i>Successful phase 1 study reported; sold to Boehringer</i>
SSAO/MAOB inhibitor	Neuro inflammation; Alzheimer's, MS, etc.	Lead candidate selected
<i>SSAO/MPO inhibitor</i>	<i>Respiratory inflammation; Asthma, COPD</i>	<i>Exploratory</i>
Orbital	Dry powder inhalation device	Phase 1 – seeking partner
ASM8	Asthma	Phase 2 - seeking partner
Bronchitol US	Cystic Fibrosis	Partner: Chiesi, funding phase 3 study - currently underway
Bronchitol EU	Cystic Fibrosis	Partner: Chiesi (UK & Germany) - marketed
Bronchitol rest of world	Cystic Fibrosis	Marketed: Australia, CEE Approval pending; Brazil, Russia
Aridol	Asthma diagnosis	Marketed: Australia, EU, Korea

**amine  
oxidase  
chemistry  
platform**

# Our therapeutic focus

the inhibition of amine oxidase enzymes has broad potential application



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

# Pharmaxis drug discovery strategy

Building a biotech powerhouse in fibrosis and inflammation

## Strategy

### Drug discovery:

- ❑ Build a biotech powerhouse in fibrosis and inflammation
  - Prioritise validated targets
  - Multiple drugs from in-house amine oxidase chemistry platform
  - Develop to phase 1 or 2

### Partnering:

- ❑ Create value via
  - Licence out to Big Pharma with attractive 1<sup>st</sup> in class drugs post phase 1 or 2
  - Collaborate to de-risk and accelerate PXS programs
  - Collaborate on in-licensing programs



## Achievements to date

### Drug discovery:

- ❑ First in class NASH drug taken to phase 1
- ❑ Two further candidates in lead optimisation phase
- ❑ One lead candidate moving to preclinical

### 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



# SSAO for NASH



SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

## PXS 4728A

- ❑ Mechanism based inhibitor of SSAO
- ❑ 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 inhibition after single dose
    - progressive dose response
- ❑ Competitors:
  - Genefit – GF505 in Phase 2b NASH
  - Intercept - OCA (FXR agonist) in Phase 2b NASH
  - Gilead – FXR agonist in pre clinical

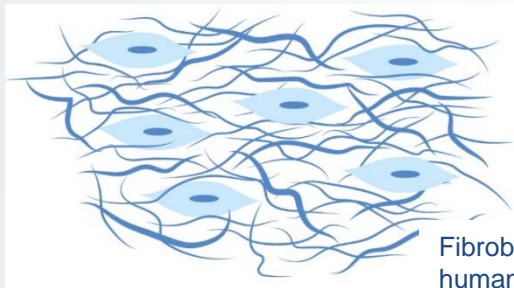
## Boehringer Ingelheim

- ❑ Excellent partner:
  - Boehringer leaders in metabolic disease
  - Industry leading development times
  - Boehringer responsible for all development, and commercialisation activities
- ❑ Competitive deal:
  - Total potential payments to approval for 2 indications: €418.5m (~A\$600m),
    - acquisition (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 starting in high single digits
    - plus potential sales milestones
- ❑ External validation of PXS drug discovery and ability to negotiate valuable global deals

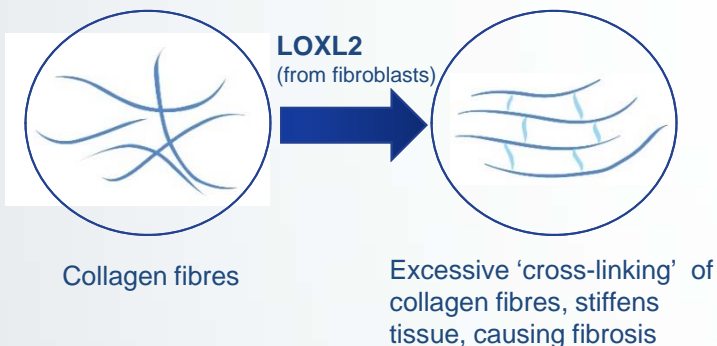
# LOXL2 inhibition for NASH & other fibrotic diseases

an attractive target and development program

Excessive production and linking of collagen fibres results in fibrosis



Fibroblast cells in human tissue



## Gilead – LOXL2 antibody

- Acquired Arresto program \$225m pre phase 1
- Now in broad phase 2b trial program
- Liver fibrosis; Idiopathic pulmonary fibrosis; Metastatic pancreatic cancer; Myelofibrosis; Solid tumours; Metastatic colorectal cancer

## ❑ Potential indications:

- ❑ NASH / Liver Fibrosis
- ❑ Pulmonary fibrosis (IPF)
- ❑ Cancer
- ❑ Wound healing

**Significant  
Market  
opportunity**

## ❑ Development status:

- ❑ Pharmaxis discovery – patent filed 2014
- ❑ Lead compounds with differentiated PK / PD profile identified
- ❑ Effective in pre clinical models of fibrosis and cancer

## ❑ Competitive profile:

- ❑ Novel target and mechanism of action
- ❑ Once daily oral drug
- ❑ Complete inhibition of LOXL2 versus partial inhibition by antibody
- ❑ Low cost of goods

# LOXL2 for pulmonary fibrosis



Collaboration with Synairgen

## Idiopathic Pulmonary Fibrosis (IPF)

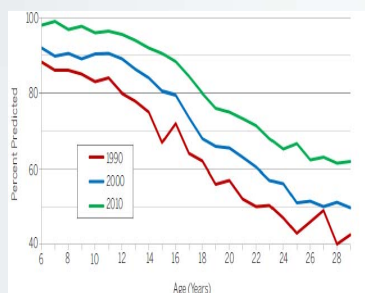
- ❑ IPF primarily affects people over the age of 50
- ❑ 5,000 patients have IPF in Australia
- ❑ 100,000 people with IPF in the US
- ❑ Prognosis is worse than that of many cancers
- ❑ Two drugs approved recently
  - Nintedanib (Boehringer Ingelheim)
  - Pirfenidone (Roche)
- ❑ Need for new therapies
- ❑ 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
  - 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
- ❑ Allows PXS to pursue further indications in parallel

# Bronchitol for cystic fibrosis

partnering for success



Median FEV<sub>1</sub>, % 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



## US

- ❑ Largest CF market by value
- ❑ 7 year post launch market exclusivity
- ❑ Tie-breaker phase 3 trial commenced Q1 2015, managed by PXS – to report 2016
- ❑ Chiesi (PXS partner) funding trial and responsible for regulatory filing & commercialisation



## Rest of world

- ❑ Sold by Chiesi in UK & Germany
- ❑ Sold by PXS in Australia & Denmark
- ❑ Pending approval/distributors appointed – Ireland, Russia, Israel, Turkey, Brazil, Eastern Europe
- ❑ Additional EU distributors to be appointed

# Pharmaxis opportunities

Building a biotech powerhouse in fibrosis and inflammation



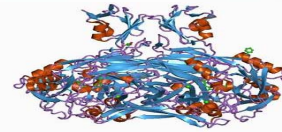
## SSAO program for NASH (fatty liver)

- ❑ NASH: US\$3.5B market by 2025
- ❑ PXS SSAO inhibitor of NASH successfully taken to phase 1
- ❑ Acquired by BI for A\$39m upfront, total >A\$750m
- ❑ BI to develop for NASH and other inflammatory indications (eg. kidney fibrosis, COPD)
- ❑ Next milestone: start of phase 2 ~end CY 2016



## 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 milestone – commencement of formal preclinical program ~ beginning CY 2016



## LOXL2 for NASH and other diseases

- ❑ Big pharma interest in NASH, LOXL2 and PXS approach
- ❑ Complimentary to SSAO program acquired by BI
- ❑ Next milestone – commencement of formal preclinical program ~ beginning CY 2016



## Bronchitol FOR CF in US

- ❑ US is largest CF market
- ❑ Partnered - Chiesi
- ❑ Chiesi funding CF303 to a cap of US\$22m
- ❑ \$25m milestone payments on launch and sales thresholds
- ❑ High mid teens royalty% on in-market sales
- ❑ Mid teens % uplift on COGs

# Major upcoming milestones

Cash funds (\$50m at 30 Sept) sufficient to reach near term valuable milestones

Calendar years

2015

2016

2017



☐ **PXS4728A Phase 2 commences**



☐ CF204 (paediatric) reports

☐ CF303 fully recruited  
☐ CF303 – last patient completes trial  
☐ CF303 – reports

☐ FDA decision on Bronchitol approval in US  
☐ **Bronchitol US launch**

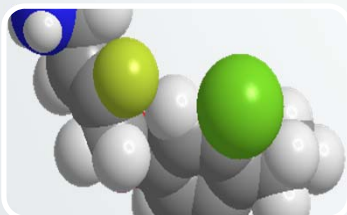


☐ Lead candidate for IPF identified

☐ Complete pre clinical program

☐ Commence phase 1  
☐ **Partner asset**

Drug discovery



☐ Lead LOXL2 candidate identified for NASH / Liver fibrosis  
☐ SSAO/MAOB disease indication nominated

☐ Complete pre clinical program  
☐ Complete pre clinical program

☐ Commence phase 1  
☐ **Partner Asset**  
☐ Commence phase 1  
☐ **Partner Asset**

# Financial Overview

**Annual General Meeting  
November 2015**



# Financials – income statements

30 June 2015

	A\$'000	2015	2014	2013
<b>Revenue</b>				
Sales revenue		5,999	5,036	3,237
Other revenue		53,248	5,450	8,370
Total revenue		59,247	10,486	11,607
Expenses		(40,739)	(62,201)	(55,142)
Profit/(loss) before tax		18,508	(51,715)	(43,535)
Income tax expense		(42)	(103)	(2)
Profit/(loss) after tax		18,466	(51,818)	(43,537)

## Highlights of 2015:

- The company generated a profit in 2015
- Significance of other revenue – generated by the business
- Reduction in expenses – see subsequent slides



# Financials – income statement revenue

30 June 2015

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

## Highlights of 2015:

- Bronchitol sales growth in challenging environment, plus Chiesi building inventory in EU
- Aridol sales maintained without any sales/marketing investment
- Boehringer Ingelheim acquires PXS4728A for \$41 million including \$1.8 million option fee (which derisked funding of phase 1 trial)
- Clinical trial cost reimbursement (by Chiesi) includes costs incurred by PXS in 2014 (\$4.7M)
- R&D tax incentive not available as PXS revenue too high (>\$20m)

# Financials – income statement expenses

30 June 2015

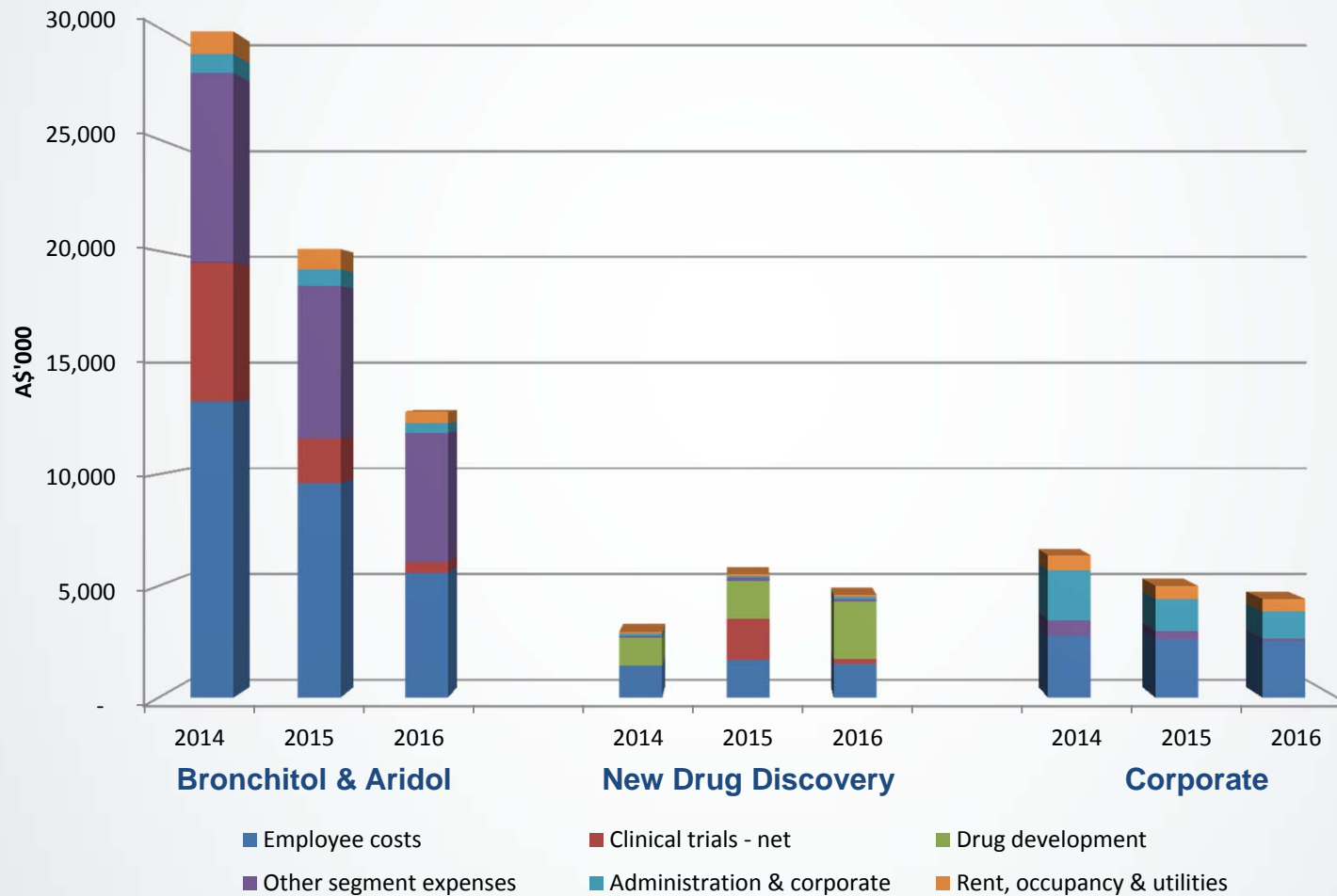
	A\$'000	2015	2014
<b>Expenses</b>			
Employee costs		(14,111)	(19,376)
Administration & corporate		(3,316)	(3,379)
Rent, occupancy & utilities		(1,593)	(1,767)
Clinical trials		(11,315)	(6,221)
Drug development		(1,695)	(1,256)
Sales, marketing & distribution		(1,962)	(3,376)
Safety, medical and regulatory affairs		(1,723)	(1,852)
Manufacturing purchases		(1,736)	(2,142)
Other		(2,300)	(1,772)
Depreciation & amortisation		(3,406)	(5,131)
Finance expenses		2,696	(7,146)
Impairment expenses		(277)	(8,783)
		(40,739)	(62,201)
Net profit (loss) before tax		18,508	(51,715)
Income tax expense		(42)	(103)
<b>Net profit(loss) after tax</b>		<b>\$18,466</b>	<b>(\$51,818)</b>

## Highlights of 2015:

- Significant cost reductions from changes to business
- Note Chiesi reimbursed \$7.5m of clinical trial costs
- Employee costs; sales, marketing and distribution; occupancy costs subsequent to closure of US office (Sept 14) and EU commercial infrastructure (May 15) and other initiatives
- FY 2015 only includes one month of completed EU cost reductions
- Clinical trials expense also includes phase 1 for PXS4728A (FY 2015: \$1.8m) and EU paediatric trial CF2024 (FY 2015: \$1.9m) – completes FY 2016
- Finance expense for FY 2015 includes capitalised finance lease on 20 Rodborough Road (\$0.7m) and a credit upon restatement of the NovaQuest financing agreement (\$3.4m)

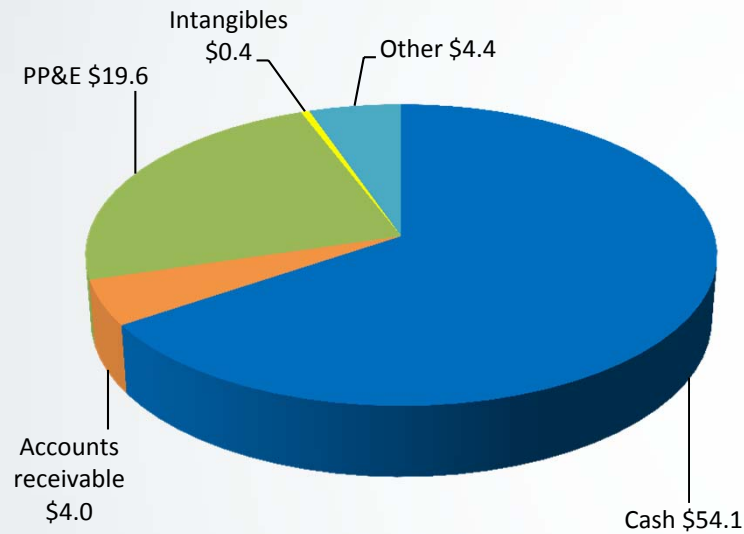
# Segment expenses: 2014, 2015 & annualised Q1 2016

excludes foreign exchange gains/losses and reimbursed clinical trial costs

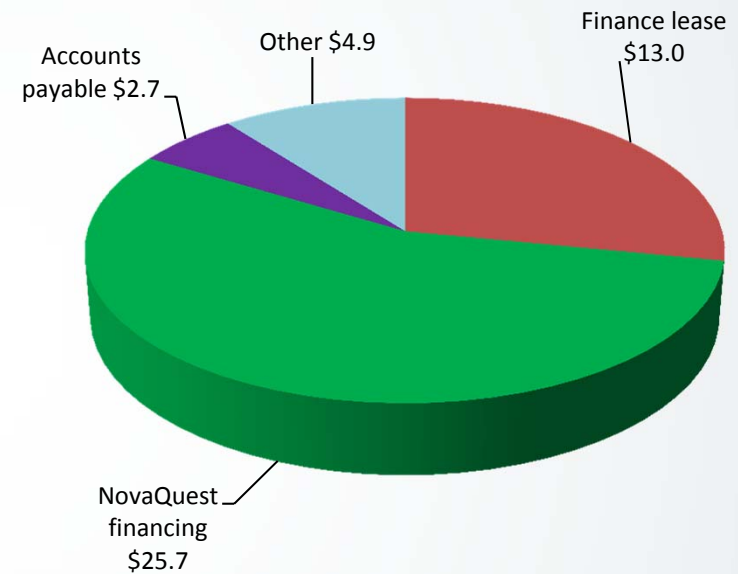


# Balance sheet – 30 June 2015

## Assets (\$83m)



## Liabilities (\$46m)



- Finance lease over 20 Rodborough Rd (to 2024, break possible in 2019)
- NovaQuest financing – amount received plus accrued charge. **Not repayable other than as % of Bronchitol revenue**

# PXS share trading for past 12 months

ASX code: PXS



18 Nov 14  
5.5cents

Shares traded: 53m shares      44m shares      75m shares

Total: 343m shares

## Shareholders

- ❑ Shares on issue: 317m (20 Aug 2015)
- ❑ Employee options: 5.9m (20 Aug 2015)
- ❑ Institutional shareholders (~48%):
  - ❑ Australia - Orbis (17%)
  - ❑ Australia - other (5%)
  - ❑ US - BVF Partners (12%)
  - ❑ US - other (5%)
  - ❑ UK - Montoya Investments (6%)
  - ❑ UK - other (3%)

## Market capitalisation

- ❑ \$70m

# Shareholder Questions

**Annual General Meeting  
November 2015**

# Formal Business

**Annual General Meeting  
November 2015**

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 2015 is adopted.”***

## Resolution 2

### **Adoption of the Remuneration Report**

The Company has received:

- 73,759,088 proxy votes in favour of the resolution;
- 3,313,023 proxy votes against the resolution;
- 276,029 proxy votes abstaining from the resolution;
- 1,286,793 proxy votes excluded from voting;
- 511,430 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 Dr Simon Buckingham as a Non Executive Director

#### Ordinary resolution:

***“That Dr Simon Buckingham, 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 Dr Simon Buckingham as a Non Executive Director**

The Company has received:

- 77,641,416 proxy votes in favour of the resolution;
- 751,219 proxy votes against the resolution;
- 242,298 proxy votes abstaining from the resolution;
- 511,430 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 1,626,000 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 2015 and, upon exercise of those Performance Rights, the acquisition of 1,626,000 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:

- 71,607,213 proxy votes in favour of the resolution;
- 6,956,858 proxy votes against the resolution;
- 14,502 proxy votes abstaining from the resolution;
- 60,000 proxy votes excluded from voting;
- 507,790 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 5

### Renewal of Proportional Takeover Provision

#### **Special resolution:**

***“That approval is given for the proportional takeover provision contained in article 45 of the current constitution of the Company to be renewed for a further three years from the date of the 2015 annual general meeting, as detailed in the explanatory statement accompanying the notice of meeting.”***

## Resolution 5

### **Renewal of Proportional Takeover Provision**

The Company has received:

- 76,935,756 proxy votes in favour of the resolution;
- 1,406,379 proxy votes against the resolution;
- 261,798 proxy votes abstaining from the resolution;
- 542,430 proxies able to be voted by the chair/board which the chair/board intend to vote in favour of the resolution.



## Resolution 6

### Amendments to Constitution

#### **Special resolution:**

*“That approval is given for the proportional takeover provision contained in article 45 of the current constitution of the Company to be renewed for a further three years from the date of the 2015 annual general meeting, as detailed in the explanatory statement accompanying the notice of meeting.”*

## Resolution 6

### **Amendments to Constitution**

The Company has received:

- 77,149,113 proxy votes in favour of the resolution;
- 1,393,760 proxy votes against the resolution;
- 95,700 proxy votes abstaining from the resolution;
- 507,790 proxies able to be voted by the chair/board which the chair/board intend to vote in favour of the resolution.

**Thank you for  
your participation**

**Annual General Meeting  
November 2015**