pharmaxis

innovating for life

Investor Presentation

Hong Kong, London, Frankfurt & Amsterdam

26th November – 4th December 2015

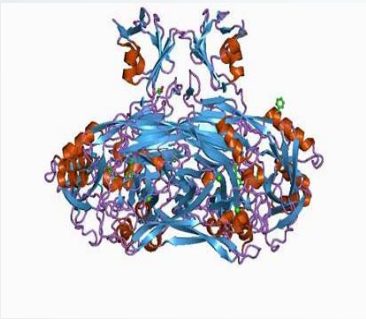
Gary Phillips CEO

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 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

Financial strength

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

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Board and management

experience that counts

Board

- Malcolm McComas – *Chair*
- Will Delaat
- Simon Buckingham
- Gary Phillips – *CEO*

Broad network and experience in capital markets

Biotech and Big Pharma commercial experience

Extensive business development networks

Experience of wide variety of partnering transactions

Management

- Gary Phillips – *CEO*
- David McGarvey – *CFO*
- Brett Charlton – *Medical*
- Wolfgang Jarolimek – *Drug Discovery*
- Kristen Morgan – *Alliance Management*

Biotech and Big Pharma commercial experience

Hands on experience across the whole of the Pharma value chain

Proven track record in business negotiations and deal making

Excellent industry and academic networks

Australian and international capital markets

Small cap companies

Refer to Pharmaxis website for further detail

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Pharmaxis product portfolio

Product	Indication	Status
LOXL2 inhibitor	*NASH (fatty liver disease), Liver & kidney fibrosis*	*Lead optimisation*
LOXL2 inhibitor	*Idiopathic pulmonary fibrosis*	*Lead optimisation; collaboration with Synairgen*
LOX/LOXL2 inhibitor	Fibrosis, cancer	Exploratory
SSAO inhibitor	*NASH*	*Successful phase 1 study reported; sold to Boehringer*
SSAO/MAOB inhibitor	Neuro inflammation; Alzheimer's, MS, etc.	Lead candidate selected
SSAO/MPO inhibitor	*Respiratory inflammation; Asthma, COPD*	*Exploratory*
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

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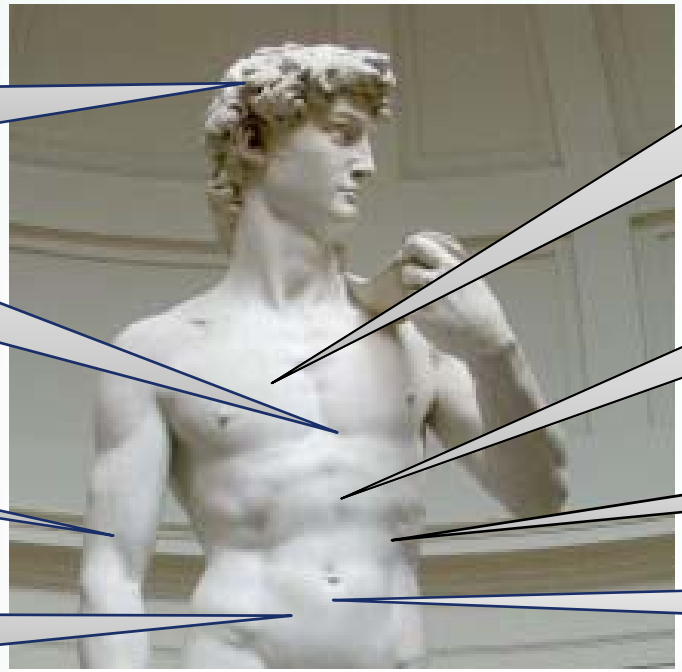
Our therapeutic focus

global leaders in amine oxidase enzyme chemistry



Alzheimer's
Parkinson's
Stroke

Cardio-myopathy
Heart failure
Atherosclerosis

Scarring

Pancreatic cancer
Type 2 diabetes

COPD
Asthma
CF
Pulmonary Fibrosis

NASH
Liver fibrosis
Liver cancer

Kidney fibrosis

Gastric cancer
IBD

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:

- ❑ Improve drug discovery hit rate by:
  - ➢ Prioritise validated targets
  - ➢ Multiple small molecule 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 1st 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
  - Small molecule inhibitor of semicarbazide-sensitive amine oxidase / vascular adhesion protein (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 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

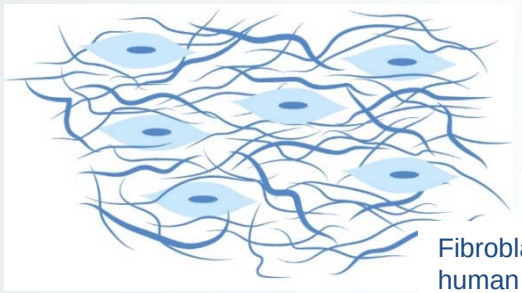
Boehringer Ingelheim

- 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

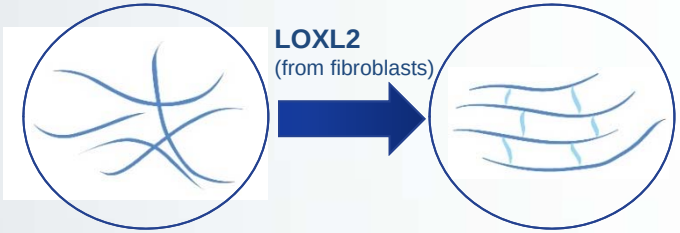
LOXL2 inhibition for NASH & other fibrotic diseases

an attractive target and development program

Excessive production and linking of collagen fibres results in fibrosis



Fibroblast cells in human tissue



Collagen fibres

Excessive 'cross-linking' of collagen fibres, stiffens tissue, causing fibrosis

- 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

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

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LOXL2 for pulmonary fibrosis

collaboration with Synairgen

Idiopathic Pulmonary Fibrosis (IPF)

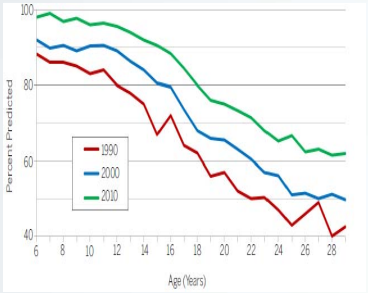
- IPF primarily affects people over the age of 50
- 5,000 patients have IPF in Australia
- 100,000 people with IPF in the US
- Prognosis is worse than that of many cancers
- Two drugs approved recently
  - Nintedanib (Boehringer Ingelheim)
  - Pirfenidone (Roche)
- Need for new therapies
- Current products 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_1 % 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

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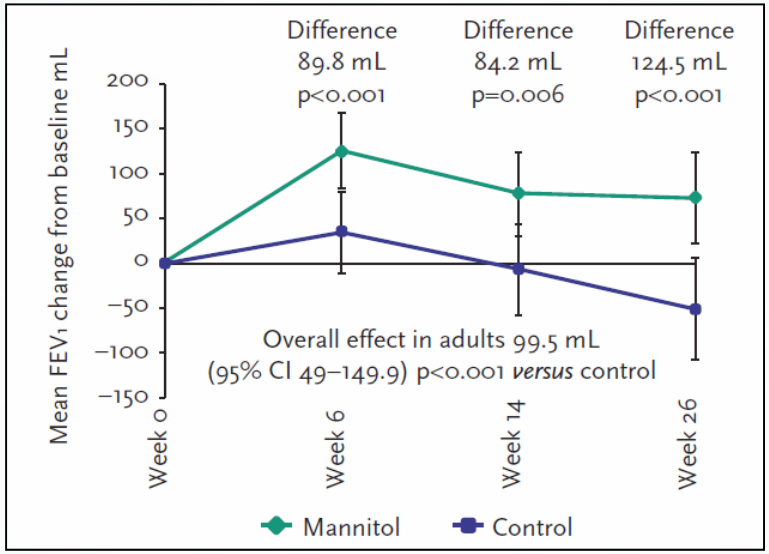
Bronchitol US opportunity

retained value – risk mitigated

US adult CF market
(2013 CFF patient registry)

- ❑ 28,103 CF patients
- ❑ 49.7% adults
- ❑ Pulmozyme use 85%
- ❑ Hypertonic saline use 63%
- ❑ Bronchitol price target US$20k per patient / year



Pooled adult data from CF301 and CF302

CF301/2 trial (adult) results

- ❑ FEV1
  - ❑ 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

- ❑ 440 adult patients
  - ❑ 20 countries
  - ❑ 120 sites
- ❑ Design
  - ❑ Full consultation with FDA
  - ❑ Similar design to CF301/2
- ❑ Fully recruited H1 2016
- ❑ Results H2 2016

Chiesi deal metrics

- ❑ CF303 funded to a cap of US$22m
- ❑ $25m milestone payments on approval and sales thresholds
- ❑ High mid teens royalty % on in market sales
- ❑ Mid teens % uplift on COGs

Pharmaxis opportunities

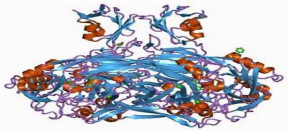
building a biotech powerhouse in fibrosis and inflammation

SSAO program for NASH (fatty liver)

- ❑ NASH: US$35B 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

Financials – income statements

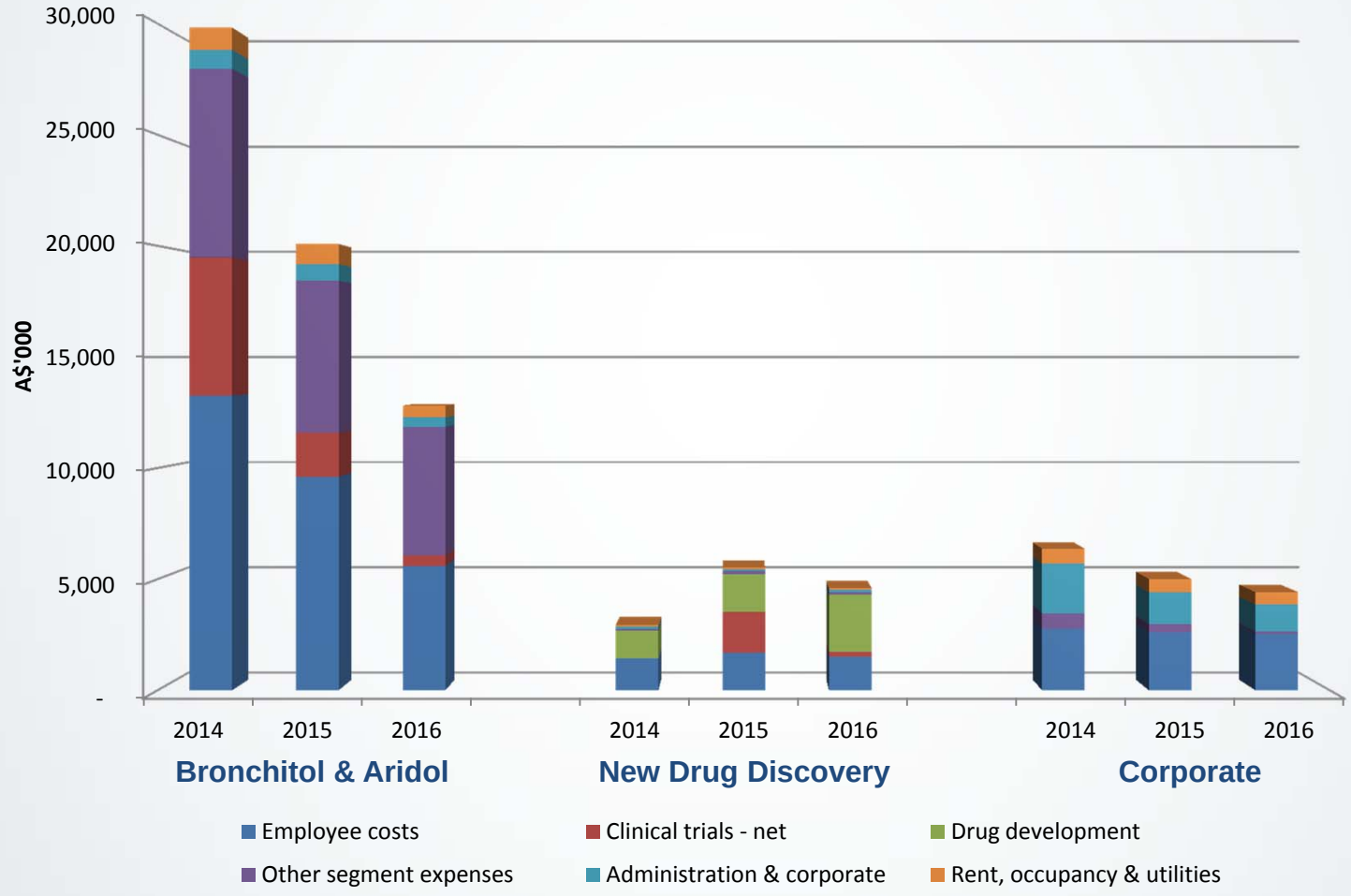
fiscal year: 30 June; currency: A$

A$'000	Fiscal years 2015	Fiscal years 2014		Quarter Sep 15
Revenue				
Sales revenue - Bronchitol	4,243	3,275		1,638
Sales revenue - Aridol & other	1,756	1,761		446
Sale of drug candidate (PXS4728A to Boehringer)	40,603			
Clinical trial cost reimbursements (by Chiesi)	11,139			2,168
Interest	721	1,735		319
R&D tax incentive & other income	785	3,715		218
	59,247	**10,486**		**4,821**
Expenses				
Employee costs	(14,111)	(19,376)		(2,811)
Administration, corporate, occupancy, utilities	(4,909)	(5,146)		(801)
Clinical trials (partly reimbursed by Chiesi)	(11,315)	(6,221)		(2,370)
Drug development	(1,695)	(1,256)		(639)
Sales, marketing & distribution	(1,962)	(3,376)		(324)
Safety, medical and regulatory affairs	(1,723)	(1,852)		(408)
Manufacturing purchases	(1,736)	(2,142)		(365)
Other	(2,300)	(1,772)		(2,340)
Depreciation & amortisation	(3,406)	(5,131)		(750)
Finance expenses	2,696	(7,146)		(175)
Impairment expenses	(277)	(8,783)		-
	(40,738)	**(62,201)**		**(10,983)**
Net profit (loss) before tax	**18,509**	**(51,715)**		**(6,162)**
Income tax expense	(42)	(103)		(7)
Net profit(loss) after tax	**18,467**	**(51,818)**		**(6,169)**

1. 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)

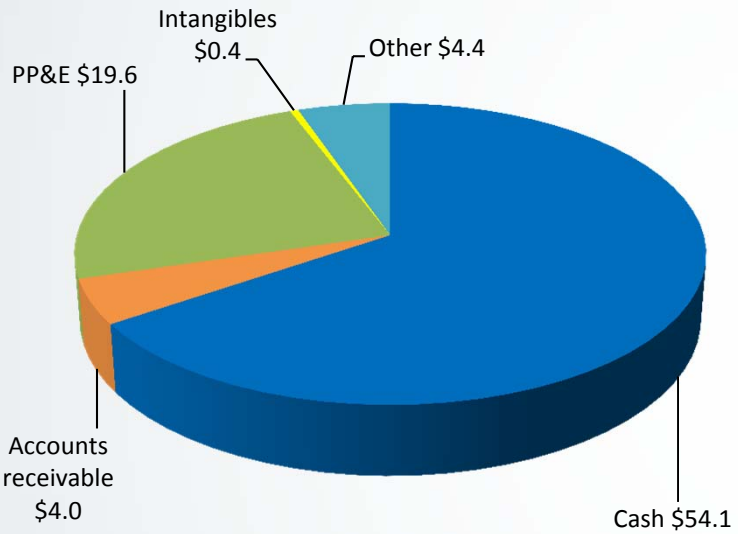
Segment expenses: 2014, 2015 & annualised Q1 2016

excludes foreign exchange gains/losses and reimbursed clinical trial costs



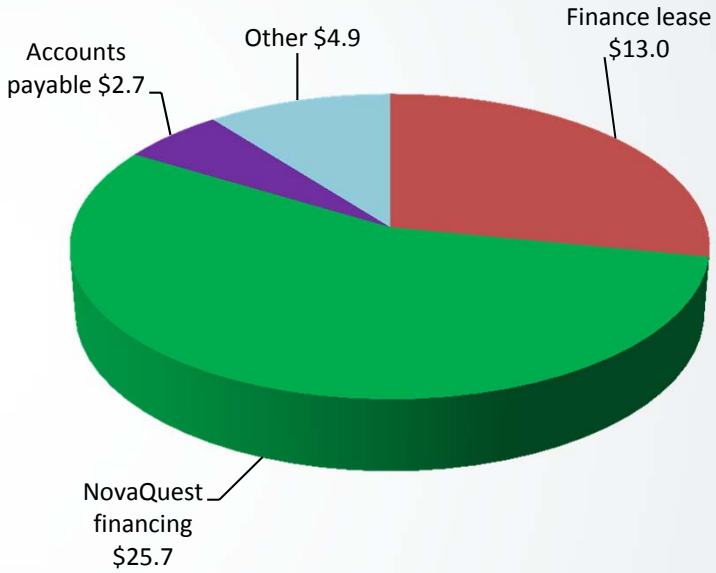
Balance sheet – 30 June 2015

Assets (A$83m)



- Cash at 30 September 2015: A$50.3m

Liabilities (A$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 - average of mid-single digit % of net in-country sales by distributors in US (7 years from launch) and EU (to March 2020) and share of any sales milestones received from Chiesi

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

- A$73m (19 Nov)

Major upcoming milestones

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

Calendar years	2015	2016	2017
Boehringer Ingelheim		❑ ***PXS4728A Phase 2 commences***	
Chiesi	❑ CF204 (paediatric) reports	❑ CF303 fully recruited ❑ CF303 – last patient completes trial ❑ CF303 – reports	❑ FDA decision on Bronchitol approval in US ❑ ***Bronchitol US launch***
synairgen	❑ Lead candidate for IPF identified	❑ Complete pre clinical program	❑ Commence phase 1 ❑ ***Partner asset***
iscovery	❑ Lead LOXL2 candidate identified for NASH / Liver fibrosis ❑ SSAO/MAOB disease indication nominated	❑ Complete pre clinical program ❑ SSAO/MAOB disease indication nominated ❑ Complete pre clinical program	❑ Commence phase 1 ❑ ***Partner Asset*** ❑ Commence phase 1 ❑ ***Partner Asset***

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