pharmaxis

Investor Presentation

Gary Phillips CEO

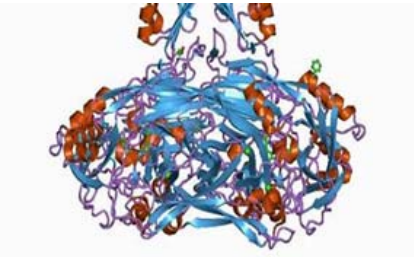
13 February 2017

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 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
  - LOXL2 inhibitor phase 1 ready in H1 2017
  - Two other drugs commencing preclinical tox in H1 2017

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

Boehringer Ingelheim

Chiesi

People and ideas for innovation in healthcare

Partnerships

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

Financial strength

- A$29m cash balance at Dec 2016; average annual cash usage $1.4m/month
- Boehringer NASH phase 2 initiation milestone expected Q2 2017 €18m
- Boehringer phase 2 milestone for second indication
- Market cap $96m*
- Institutional investor's ~50%
- Increasing Bronchitol sales globally in new and existing markets

* Note: Market Cap as of 9/02/17

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

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

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

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-Plank Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany

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.

Board of Directors

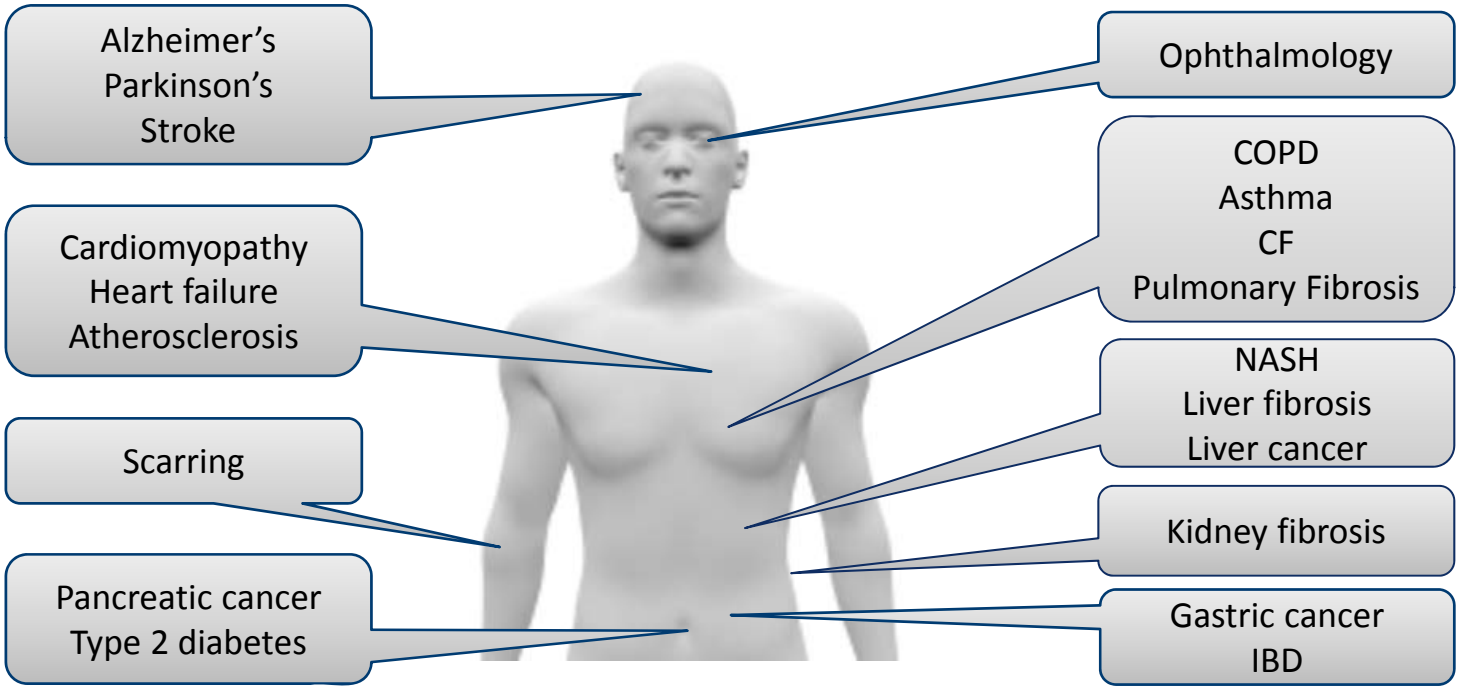
- **Malcolm McComas** – *Chair*
  - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- **Gary Phillips** – *Managing director*
- **Will Delaat** – *Non executive director*
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- **Simon Buckingham** – *Non 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							Chiesi
RoW	Cystic fibrosis							Distributors
Aridol	Asthma diagnosis							Distributors
SSAO	NASH						Boehringer Ingelheim	
SSAO	2nd indication						Boehringer Ingelheim	
Discovery								
SSAO/MPO	Respiratory & cardiovascular							
LOXL-2	NASH, fibrosis - liver, pulmonary, kidney							synairgen
LOXL-2 (other)	Cancer							Leading universities/academics assessing in cancer
LOX	Scarring+							
SSAO/MAO-B	Neuro inflammation+							
Orbital	Dry powder inhalation device							WOOLCOCK LEADERS IN BREATHING & SLEEP RESEARCH
ASM-8	Asthma							Seeking Partners

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

Pharmaxis drug discovery strategy

Building a biotech powerhouse in fibrosis and inflammation

Strategy

Drug discovery:

- 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 SSAO inhibitor drug taken to phase 1. Initial indication NASH. Partner developing second indication.
- One lead candidate recently entered preclinical
- Two lead candidates to enter preclinical H1 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

Our therapeutic focus is inflammation and fibrosis

Pharmaxis drug discovery

- NASH, liver, kidney and cardiac fibrosis (LOXL2)
- Respiratory, cardio vascular (SSAO/MPO)
- Neuro inflammation – Alzheimer's, Parkinson's, stroke (SSAO/MAO-b)
- Scarring (LOX)

Collaborations allow us to leverage our platform without losing focus

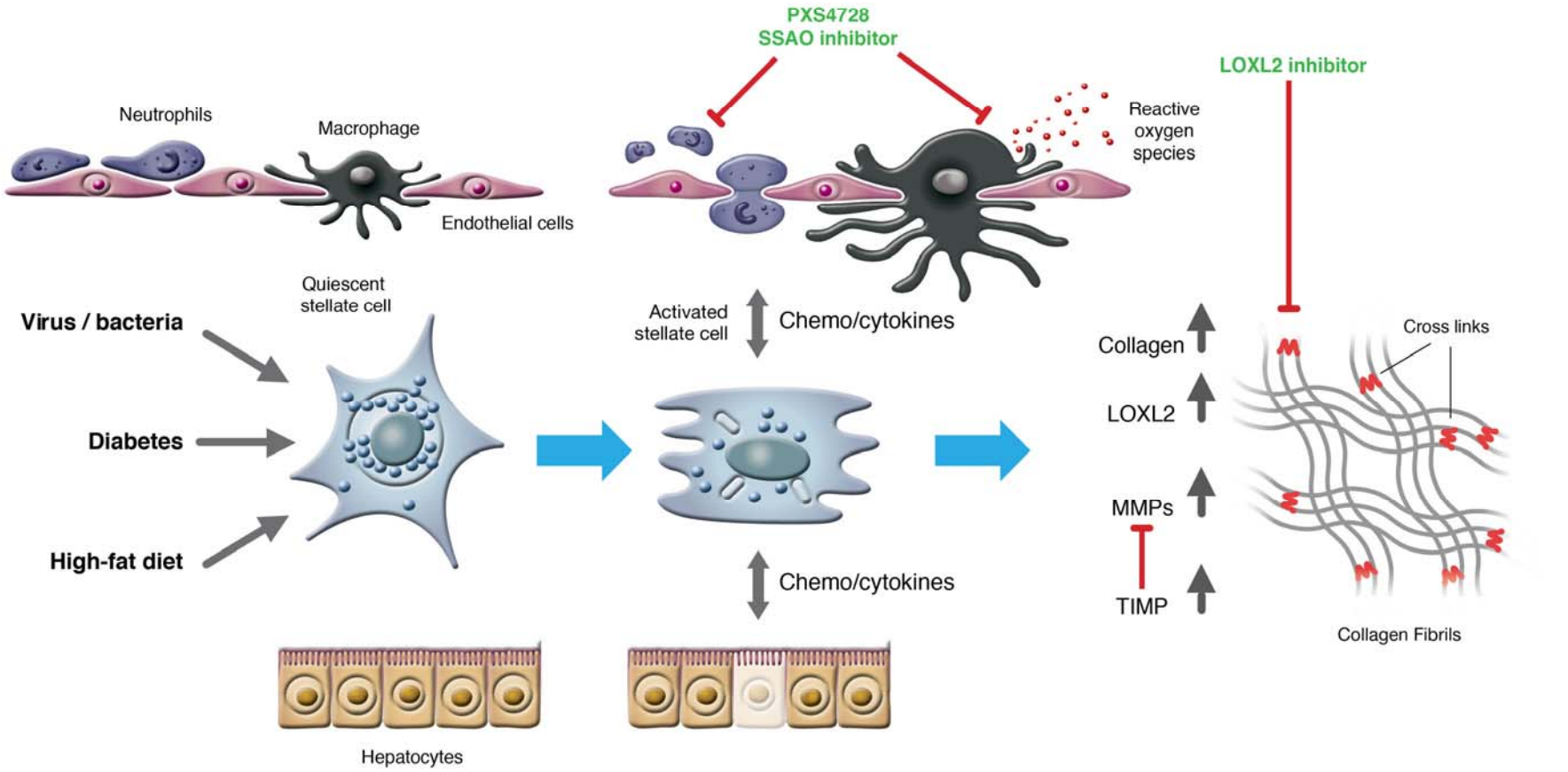
Collaboration with Synairgen

- Pulmonary fibrosis (LOXL2)

Exploratory academic collaborations (LOX/LOXL2)

- Cancer

Drugs targeting NASH → Cirrhosis



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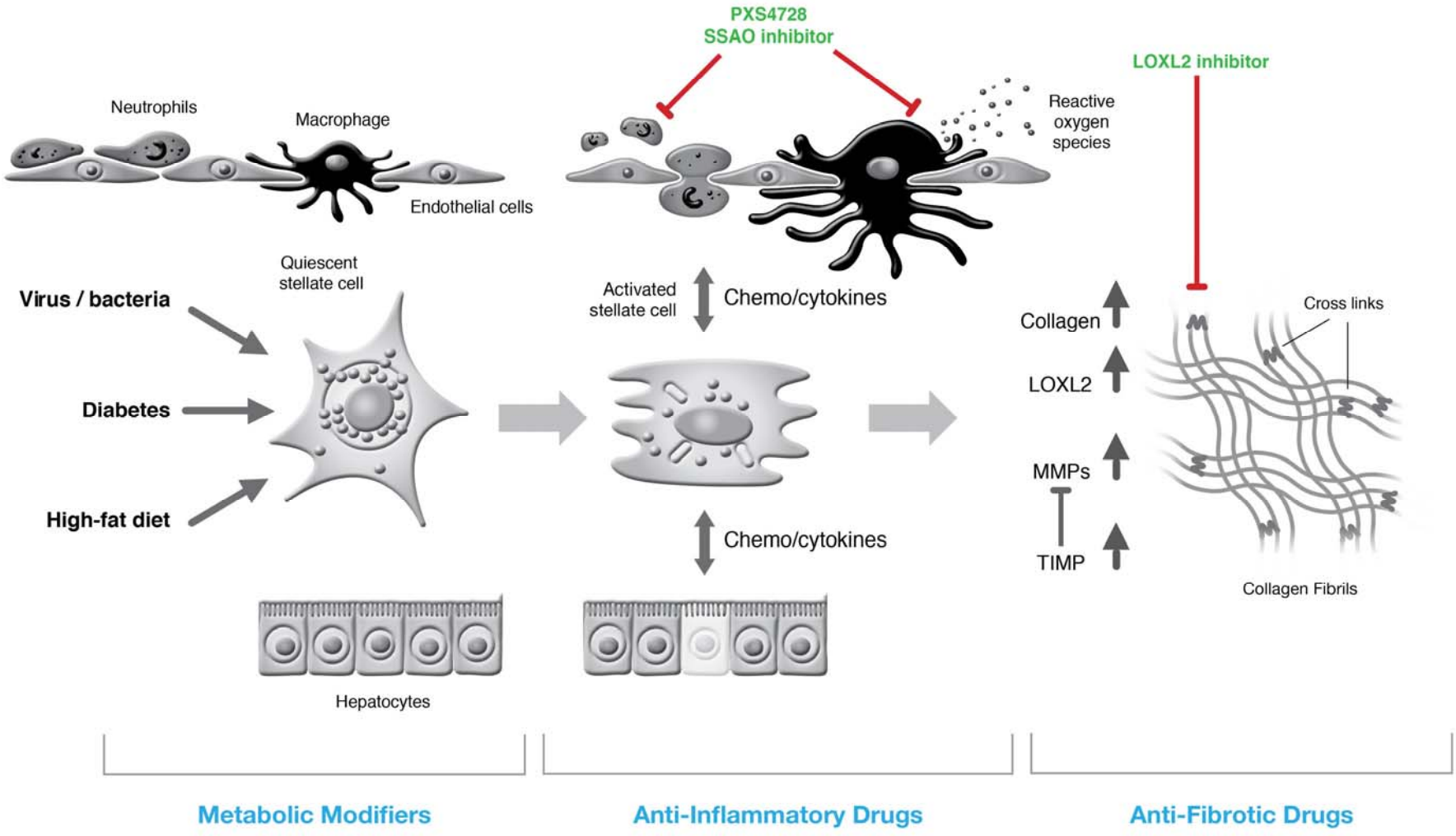
Drugs targeting NASH → Cirrhosis



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

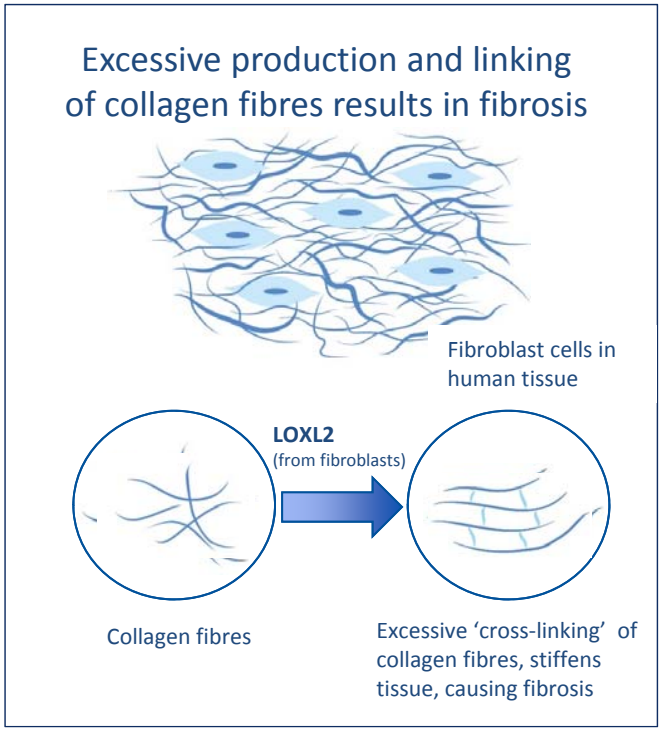
Competitive deal with Boehringer

- 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
  - 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

synairgen

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

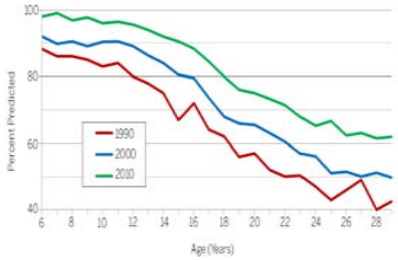
Fibrosis and NASH M&A

Attractive deal values for phase 1 and phase 2 clinical assets

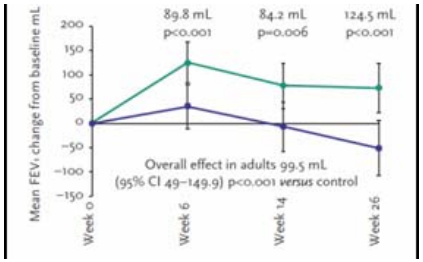
Acquirer	Company	Indication	Deal Type	Stage	Upfront (US$M)	Potential (US$M)
< 2 years ago						
Gilead	Nimbus	NASH - metabolic	Partnership	P1	400	1,200
Gilead	Phenex	NASH – metabolic	Asset Aqun	P2	U	470
Allergan	Tobira	NASH - inflammatory	Acquisition	P2	400	800
Allergan	Akarna	NASH - metabolic	Acquisition	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+
> 2 years ago						
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	Acquistion	P1b	75	482
AZ	Regulus	NASH- metabolic +	License + equity	Pre	U	500

Bronchitol for cystic fibrosis

Overview



Median FEV_1 % 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
- 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

CF204 trial results

- Paediatric age 6-17
  - Placebo-controlled
  - 8 weeks crossover design
  - standard therapy continued
- Primary endpoint:
  - Absolute change in FEV1: 3.42%; p=0.004
- Key secondaries
  - Absolute change in FEF25-75: 5.75% (p=0.005)
- Acceptable safety profile
  - Exacerbations and lung infection reduced by˜25%

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Bronchitol for cystic fibrosis

Partnering for success

Chiesi
People and ideas for innovation in healthcare

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 Q2 2017

Rest of world

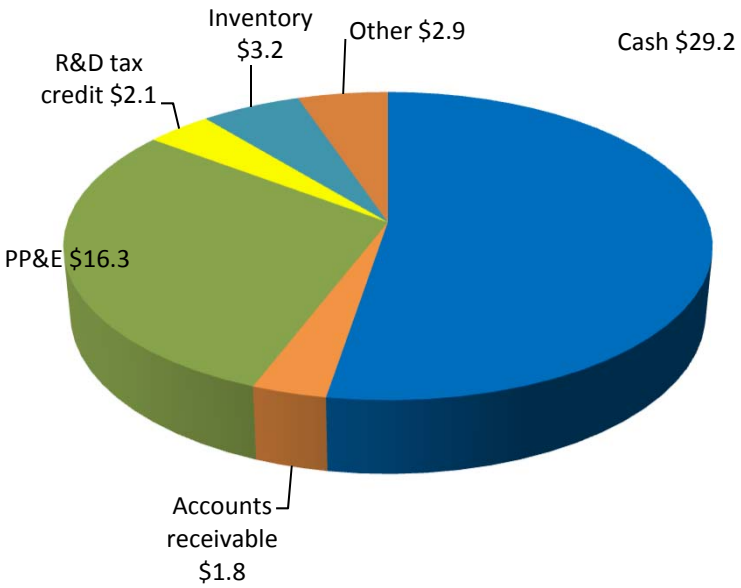
- Sold by Chiesi in UK & Germany
- Sold by PXS in Australia & Denmark
- Russian approval received Oct 2016 – first sale Q1 2017
- Pending approval/pricing/distributor appointments in Israel, Turkey, Brazil, Eastern Europe countries

Financials highlights

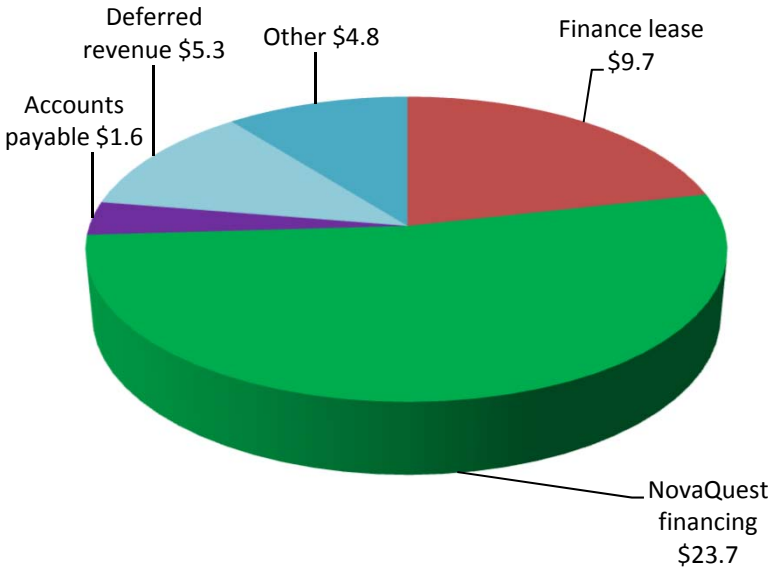
A$'000	Three months ended		Six months ended	
(unaudited)	31-Dec-16	31-Dec-15	31-Dec-16	31-Dec-15
Income statements				
Sales revenue	793	1,643	1,690	3,727
Other revenue	1,107	2,908	5,220	5,645
Total revenue	**1,900**	**4,551**	**6,910**	**9,372**
Total expenses	(8,850)	(9,567)	(17,945)	(20,550)
Net profit (loss) after tax	**(6,950)**	**(5,016)**	**(11,035)**	**(11,185)**
Segment results – adjusted EBITDA				
Bronchitol & Aridol	(2,489)	(3,334)	(3,941)	(4,485)
New drug development	(1,303)	(863)	(2,421)	(1,847)
Corporate	(850)	(1,330)	(2,078)	(1,753)
Total	**(4,642)**	**(5,527)**	**(8,440)**	**(8,085)**
Statement of cash flows	-	-		
Cash inflow/ (outflow) from:	-	-		
Operations	(2,721)	(2,994)	(8,894)	(6,426)
Investing activities	(64)	(646)	(214)	(1,092)
Financing activities	(430)	(430)	(856)	(872)
Total cash used	**(3,215)**	**(4,070)**	**(9,964)**	**(8,390)**
Cash at bank	**29,245**	**45,936**	**29,245**	**45,936**

Balance sheet – 31 December 2016

Assets ($55m)



Liabilities ($45m)



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

Shareholders & trading

ASX code: PXS

Shareholders (31 Dec 16)

- Shares on issue: 319m
- Employee options: 9.9m
- Institutional shareholders ~50%:
  - Australia - Orbis (16%); Australian Ethical (6%); Other (1%)
  - US - BVF Partners (14%); Other (2%)
  - UK - Montoya Investments (6%); Other (3%)

Shares traded to 31 Dec

- Three months: 12m
- Six months: 34m
- Year: 78m

Market capitalisation

- A$96m (9 February 17)

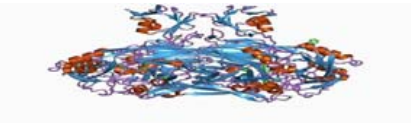
News flow

	CY 2017	CY 2018
Boehringer Ingelheim	PXS4728A Phase 2 commences & ~A$25M milestone payable (H1) BI advise timetable for second indication	
pharmaxis Bronchitol – RoW	EU Paediatric label extension application Russian sales commence	
Chiesi Bronchitol – US	CF303 – trial completion (Q1) CF303 – top line results (Q2)	Bronchitol approval
pharmaxis New drug development		
➢ LOXL-2	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 Complete GLP tox program – phase 1 ready	Commence phase 1 study
➢ LOX	Commence GLP tox program	Commence phase 1 study
➢ LOXL-2	Leading universities/academics assessing in kidney fibrosis, cancer and wound healing	

Pharmaxis opportunities for growth

Building a biotech powerhouse in fibrosis and inflammation

Boehringer Ingelheim



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 – Q2 2017

LOXL2 program

- NASH market >$35B
- Pulmonary fibrosis: market >$1B
- Strong big Pharma interest in LOXL2 and PXS chemistry
- Formal preclinical commenced Q4 2016
- Next step – phase 1 ready Q2 2017
- Synairgen collaboration increases value and shares risk

Discovery pipeline

- LOX
  - Scarring and severe fibrosis
  - Commence preclinical H1 2017
- SSAO/MPO
  - Respiratory and cardiovascular inflammation
  - Commence preclinical H1 2017

Bronchitol for CF

- Access large US CF market with Chiesi
  - CF303 trial reports in Q2 17
  - ~A$13m milestone payments on launch
  - High teens % share of in-market sales
- Growth from existing markets including Russia – sales commenced Q1 17
- New RoW markets opening over next 24 months

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$29m cash at December 16 with a likely milestone of A$25m due in Q2 17