



Investor Presentation

Gary Phillips CEO

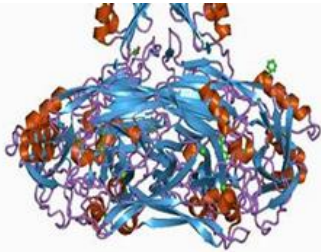
15 May 2017

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\$26.5m cash balance at Mar 2017; average annual cash usage \$1.4m/month
- Boehringer NASH phase 2 initiation milestone expected Q2 2017 €18m
- Boehringer 2nd indication phase 2 milestone of €10m expected H2 2017
- Market cap \$83m*
- 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



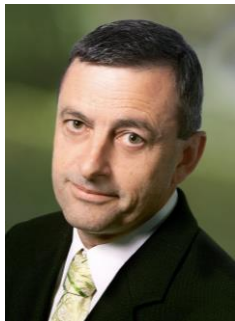
David McGarvey – CFO

- 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

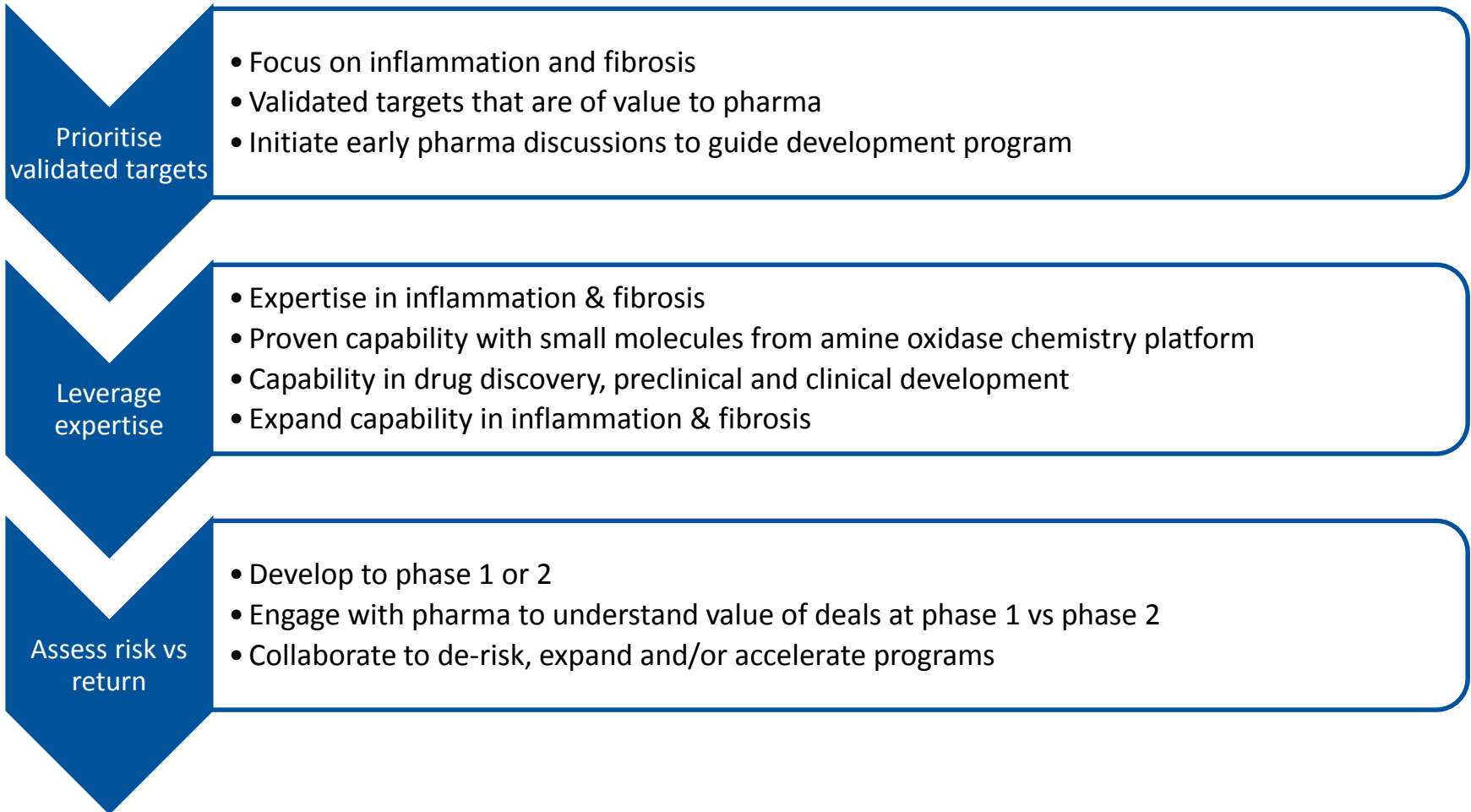
- **Malcolm McComas – Chair**
 - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- **Will Delaat – Non executive director**
 - former CEO of Merck Australia
 - former chair of Medicines Australia
- **Gary Phillips – Managing director**
- **Simon Buckingham – Non executive 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 <small>People and ideas for innovation in healthcare</small>	
RoW	Cystic fibrosis							Distributors	
Aridol	Asthma diagnosis							Distributors	
SSAO	NASH								
SSAO	2 nd indication								
<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					WOOLCOCK <small>LEADERS IN BREATHING & 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 in preclinical tox studies
- Two further lead candidates to enter preclinical in 2017

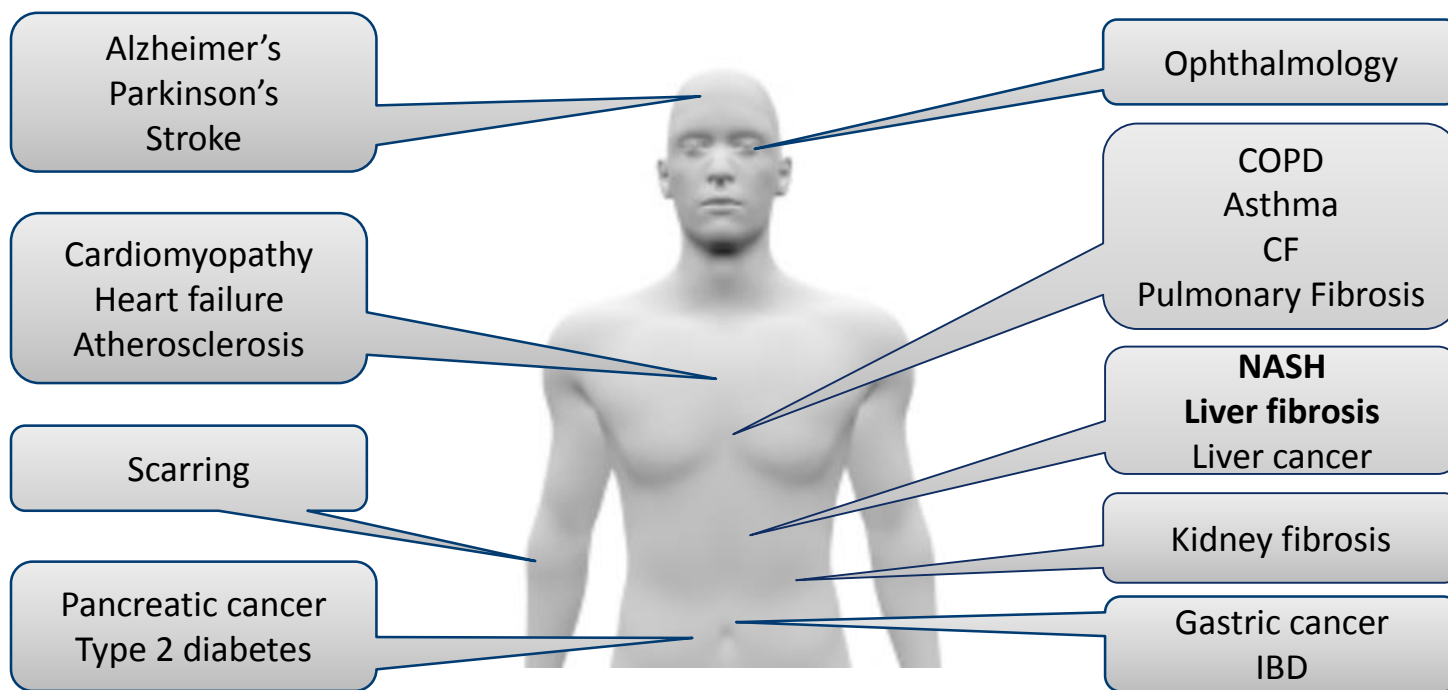


Partnering

- 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

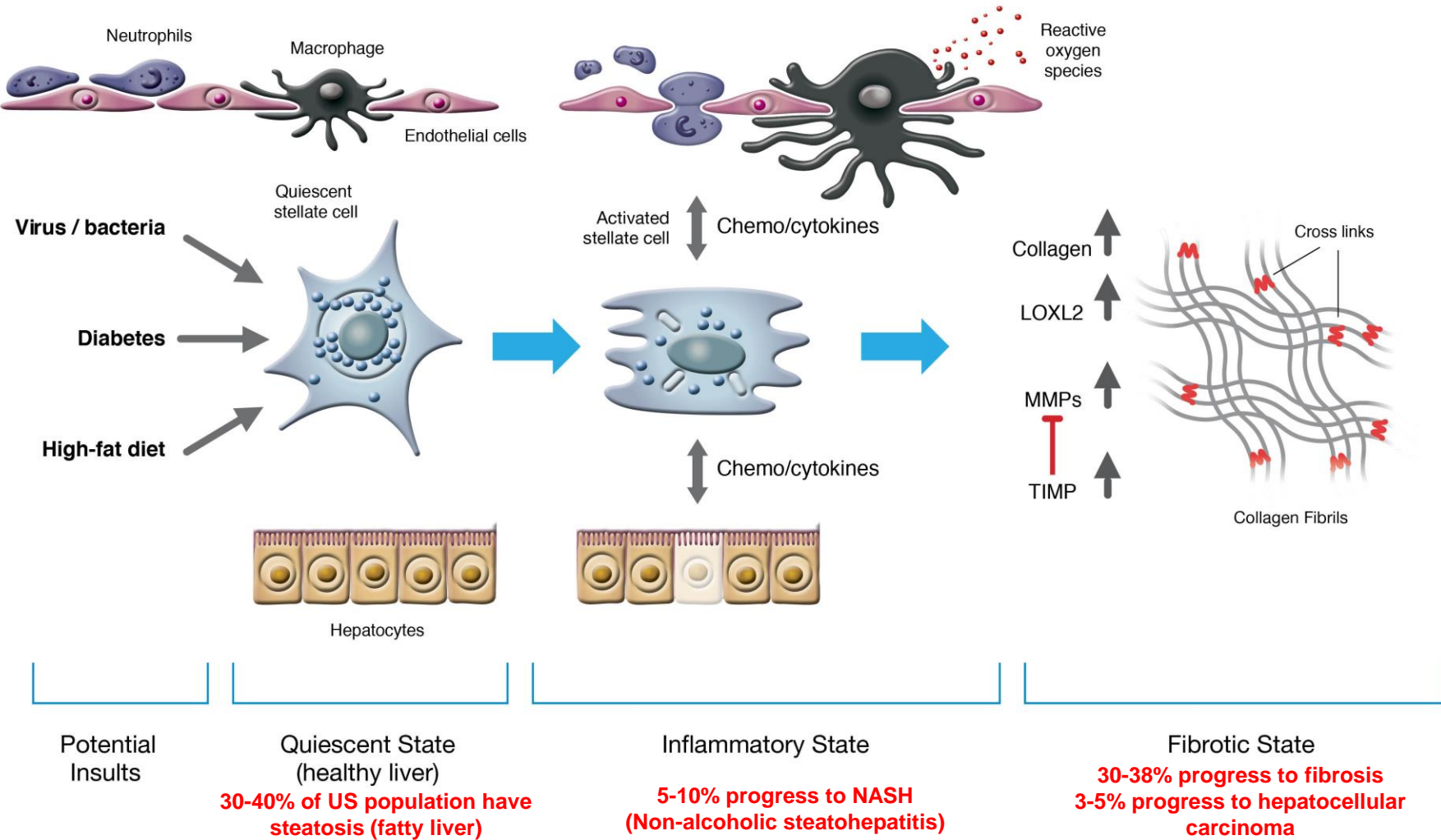
Drug discovery chemistry platform

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

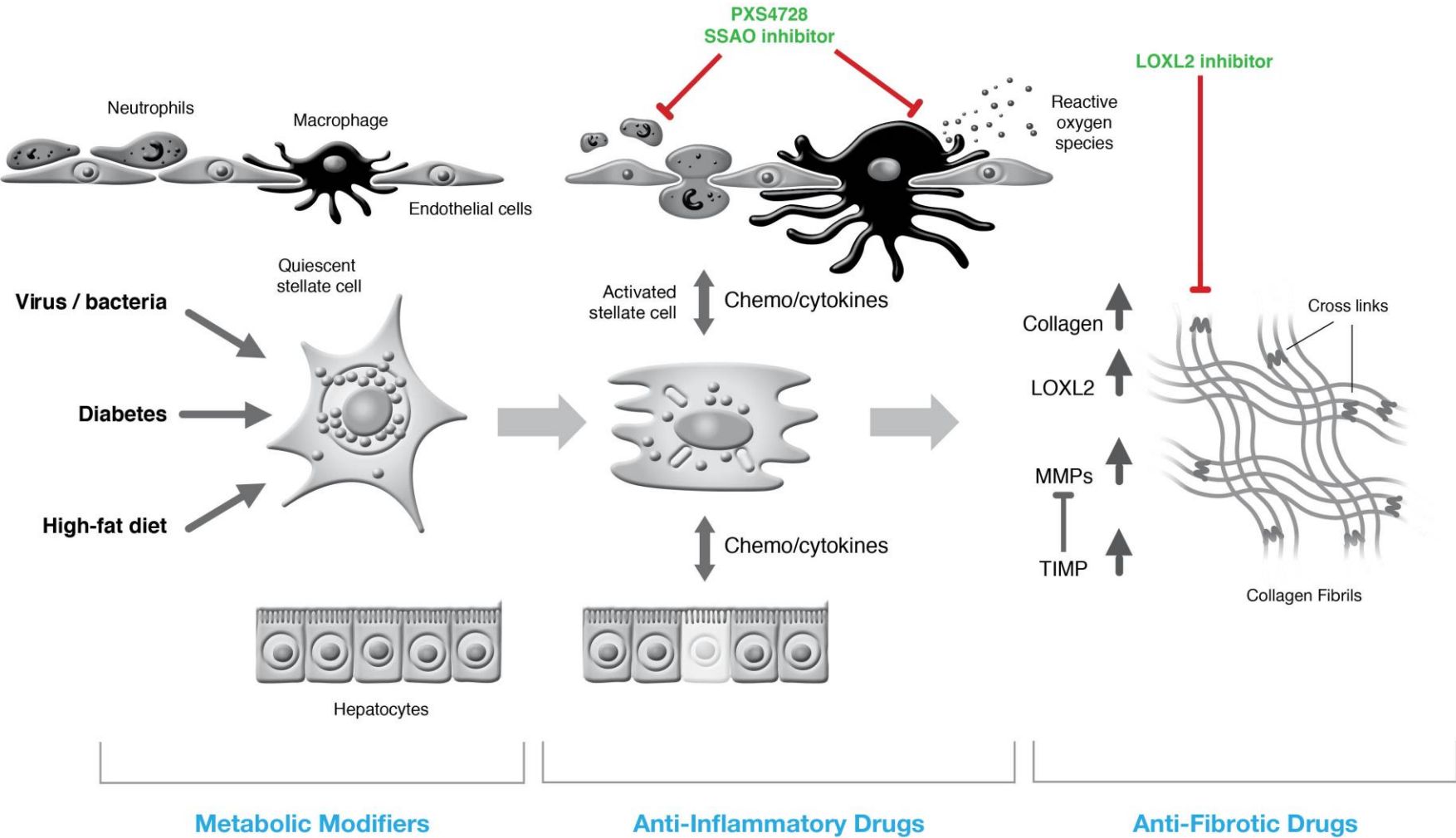


Current focus on Liver fibrosis and NASH

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

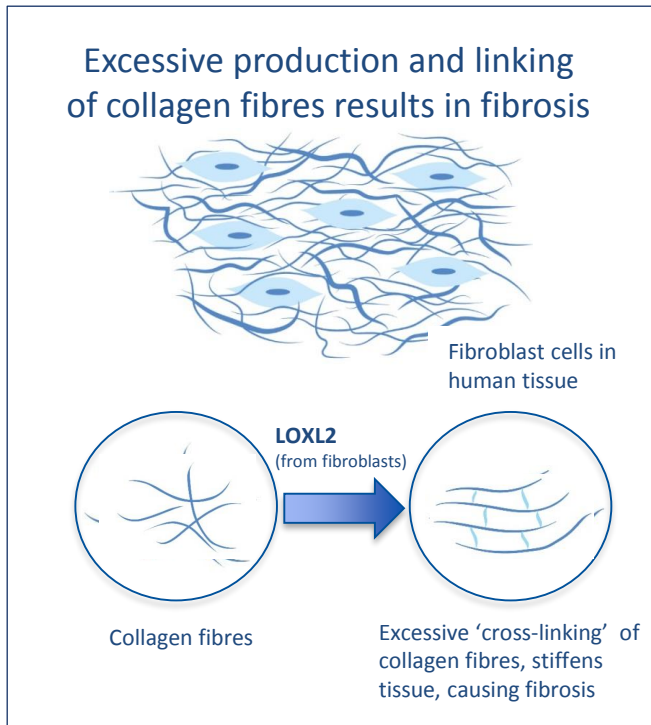
End of Phase 1 deal with Boehringer

- Potential milestones to approval: €418.5m (~A\$600m)
 - Upfront (May 2015): €27.5m (~A\$39m)
 - 1st Indication (NASH)
 - Commencement of phase 2 and 3: total €55m (~A\$80m)
 - Filing, regulatory & pricing approvals: total €140m(~A\$200m)
 - 2nd indication (commercial in confidence)
 - Additional milestone payments to approval total €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

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

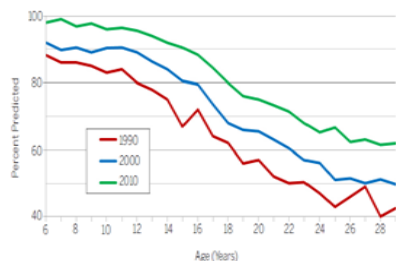
Fibrosis and NASH M&A

Attractive deal values for phase 1 and phase 2 clinical assets

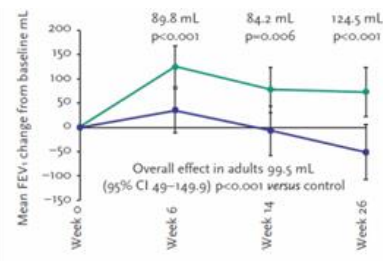
Acquirer	Company	Indication	Deal Type	Stage	Upfront (US\$M)	Potential (US\$M)
< 2 years ago						
Gilead	Nimbus	NASH - metabolic	Partnership	P1	400	1,200
Gilead	Phenex	NASH – metabolic	Asset Aqun	P2	U	470
Novartis	Conatus	NASH - inflammatory	Option	P2	50	650
Allergan	Tobira	NASH - inflammatory	Acquisition	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+
> 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	Acquisition	P1b	75	482
AZ	Regulus	NASH- metabolic +	License + equity	Pre	U	500

Bronchitol for cystic fibrosis

Overview



Median FEV₁, % 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%

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
- Top line results Q2 2017

Rest of world

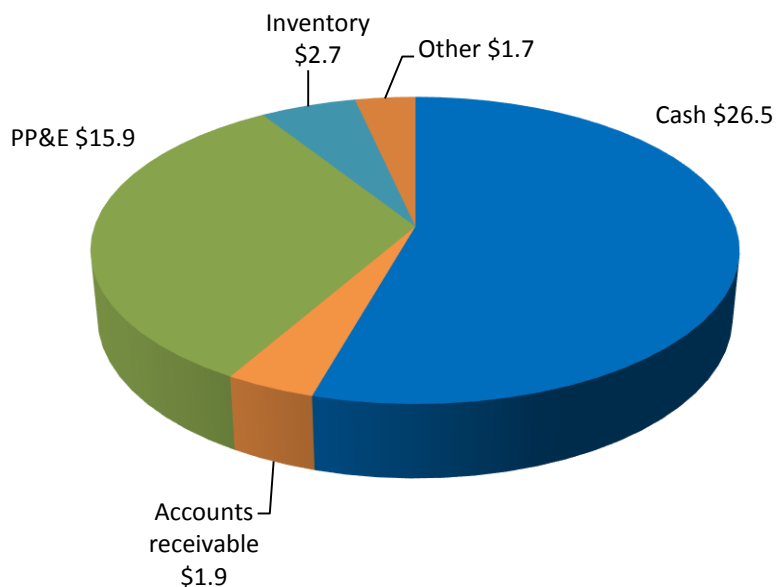
- Sold by Chiesi in UK & Germany
- Sold by PXS in Australia & Denmark
- Russian approval received Oct 2016 – first sale Q1 2017
- Pending approval/pricing/distributor appointments in Israel, Turkey, Brazil, Eastern European countries

Financials highlights

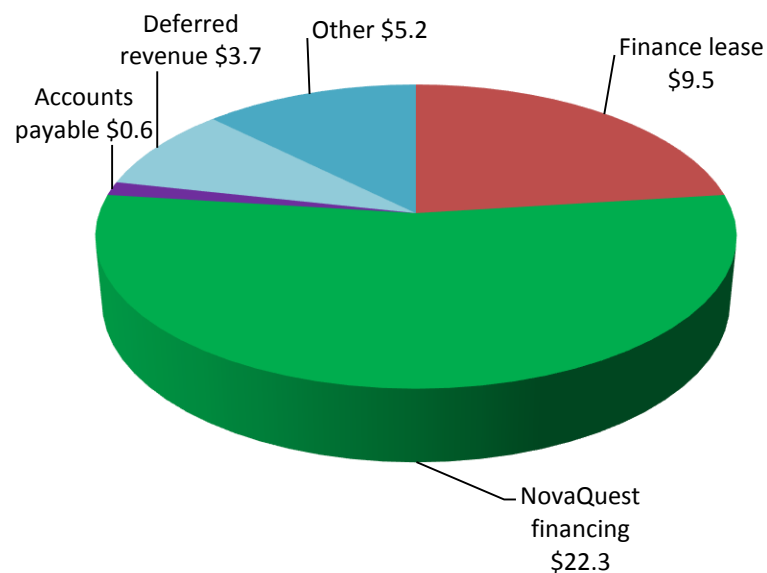
	A\$'000		Three months ended		Nine months ended	
			31-Mar-17	31-Mar-16	31-Mar-17	31-Mar-16
(unaudited)						
Income statements						
Sales revenue			2,267	1,705	3,957	5,432
Other revenue			1,879	3,149	7,099	8,793
Total revenue			4,146	4,854	11,056	14,225
Total expenses			(7,345)	(8,007)	(25,280)	(28,557)
Net profit (loss) after tax			(3,199)	(3,153)	(14,234)	(14,338)
Segment results – adjusted EBITDA						
Bronchitol & Aridol			(738)	(1,602)	(4,679)	(6,088)
<i>Bronchitol & Aridol – excluding net CF303</i>			<i>(899)</i>	<i>(1,046)</i>	<i>(3,743)</i>	<i>(3,645)</i>
New drug development			(1,892)	(870)	(4,313)	(2,717)
Corporate			(934)	(1,103)	(3,012)	(2,855)
Total			(3,564)	(3,575)	(12,004)	(11,660)
Statement of cash flows						
Cash inflow/ (outflow) from:						
Operations			(2,132)	(4,004)	(11,026)	(10,430)
Investing activities			(183)	(143)	(397)	(1,235)
Financing activities			(431)	(417)	(1,287)	(1,289)
Total cash used			(2,747)	(4,564)	(12,710)	(12,954)
Cash at bank			26,499	41,508	26,499	41,508

Balance sheet – 31 March 2017

Assets (\$49m)



Liabilities (\$41m)

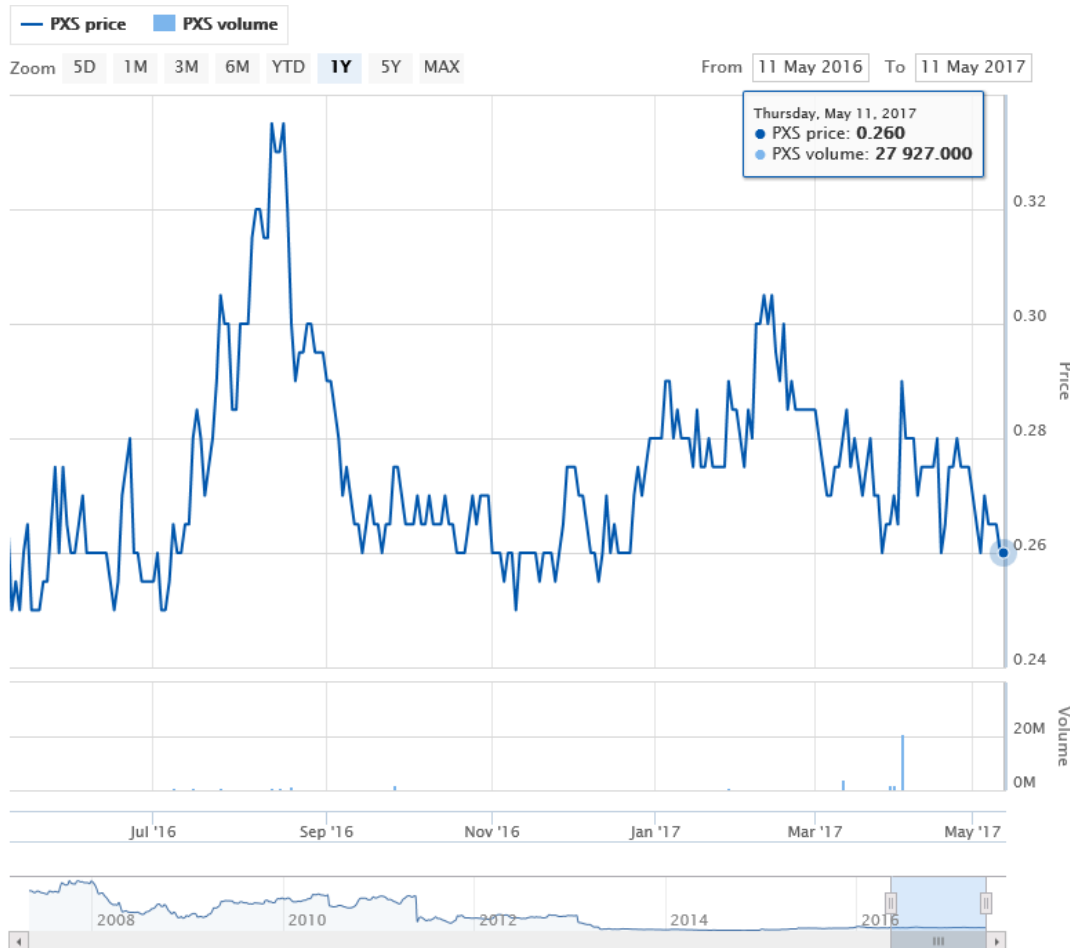


- Finance lease over 20 Rodborough Rd (to 2024)
- NovaQuest financing – not repayable other than as % of Bronchitol revenue

Shareholders & trading



ASX code: PXS



Shareholders (30 April 17)

- Shares on issue: 319m
- Employee options: 10.3m
- Institutional shareholders ~50%:
 - Australia/NZ: Australian Ethical (10%); Orbis (8%); Other (1%)
 - US - BVF Partners (19%); Other (2%)
 - UK - Montoya Investments (6%); Other (3%)

Shares traded to 30 April 17

- Three months: 39m
- Six months: 49m
- Year: 89m

Market capitalisation

- A\$83m (11 May 17)

News flow



Bronchitol – RoW



Bronchitol – US



New drug development

➤ LOXL-2 (NASH / IPF / Other)

➤ SSAO/MPO

➤ LOX (Scarring / Cancer)

➤ LOXL-2 (Cancer)

CY 2017

CY 2018

- PXS4728A NASH Phase 2 commences & €18M milestone payable (H1)
- PXS4728A 2nd indication Phase 2 commences & €10M milestone payable (H2)

- EU Paediatric label extension application

- CF303 – top line results (Q2)

- FDA submission

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

- Partner ≥1 compound

- Commence GLP tox program
- Phase 1 ready

- Commence phase 1 study

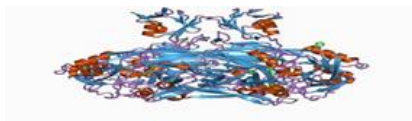
- Commence GLP tox program
- Phase 1 ready

- Commence phase 1 study

- 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: ~A\$25m at start of phase 2 NASH – Q2 2017
- 2nd indication milestone: at start of phase 2 (TBD)

LOXL2 program

- NASH market >\$35B
- Pulmonary fibrosis: market >\$1B
- Strong big Pharma interest in LOXL2 and PXS drug candidates
- Formal preclinical commenced Q4 2016
- Next step – commence phase 1 in H2 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\$26.5m cash at March 2017 with likely milestones of ~A\$42m in 2017