



Investor Presentation

Gary Phillips CEO

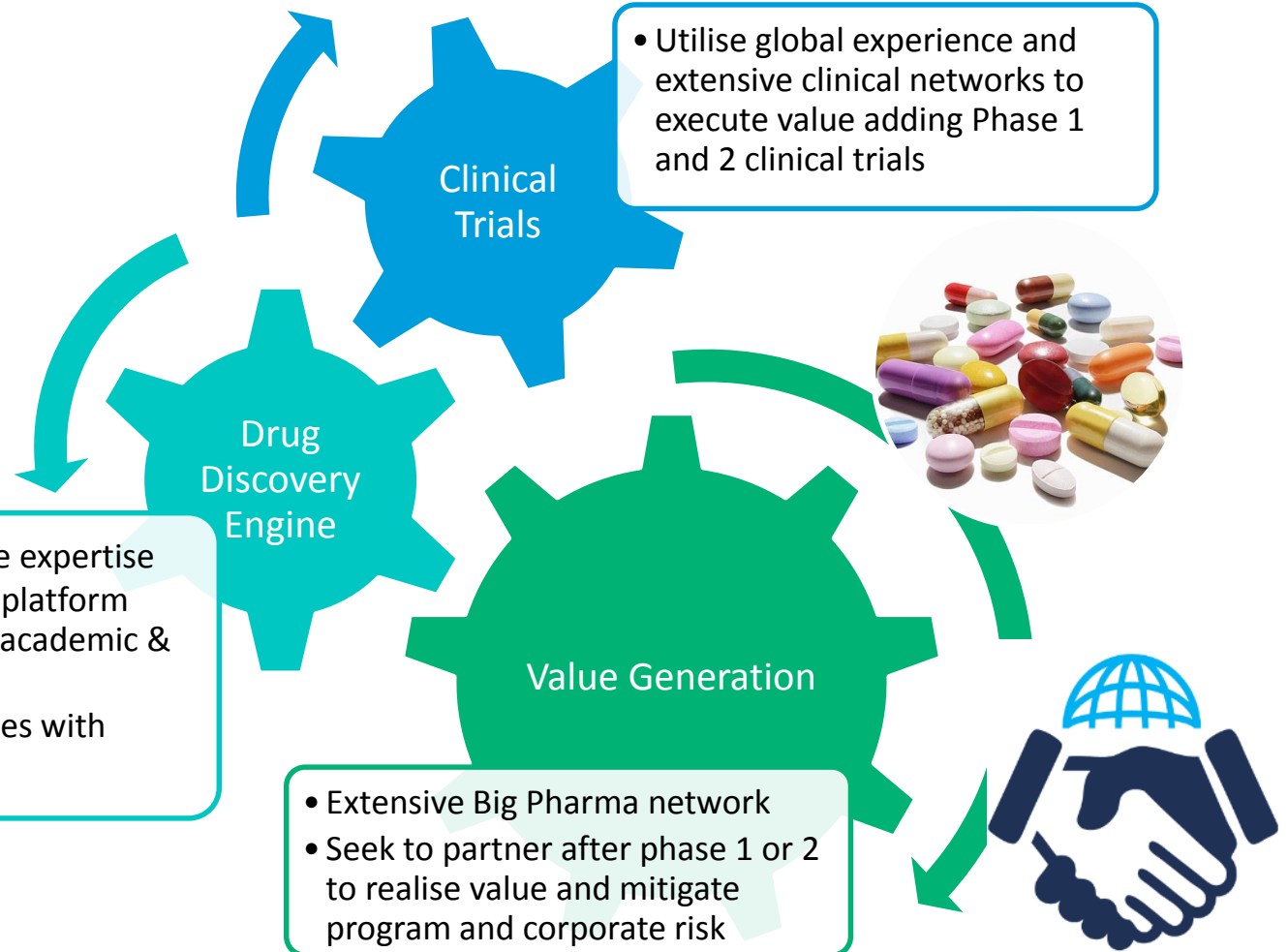
April 2019

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. All statements, other than statements of historical facts, are 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. For example, despite our efforts there is no certainty that we will be successful in partnering our LOXL2 program or any of the other products in our pipeline on commercially acceptable terms, in a timely fashion or at all. 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

Pharmaxis has a successful track record of research, development and commercialisation of human healthcare products for the treatment and management of fibrotic and inflammatory diseases



Pharmaxis overview

A globally recognised leader in drug development for fibrosis & inflammation

- A chemistry platform that continues to deliver a pipeline of oral small molecule drugs in preclinical and clinical development in diseases with large markets and high unmet need
- A **globally respected** translational **development team** delivering **best in class** drug development programs with international standard data packages
- A proven **track record** of achieving global partnering deals with multinational Pharmaceutical companies
- \$83m received to date from benchmark deal concluded with Boehringer Ingelheim in 2015 and worth a potential \$600m+ in development milestones for two indications (NASH and diabetic retinopathy) plus sales related payments (% and milestones)
- Commercial partnering process for phase 2 ready anti fibrotic LOXL2 inhibitor program commenced
- Growing revenues from approved product sales (26% increase for FY 2018 to A\$6.1m) & milestones (A\$42m FY 2018)
- Strong balance sheet - A\$42m cash balance (31 December 2018)
- Purpose built manufacturing and research facility in Sydney
- Strong institutional share register; including offshore specialist biotech funds

Senior management

Significant experience in drug development, commercialisation and partnering



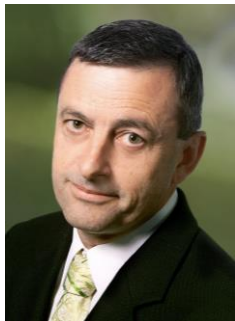
Gary Phillips – CEO

- 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 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 20 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






Kristen Morgan – Alliance Management


- more than 20 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.
- responsibility for alliance management and medical and regulatory affairs

Non Executive Directors

- **Malcolm McComas – Chair**
 - former investment banker at Grant Samuel
- **Will Delaat**
 - former CEO of Merck Australia
 - former chair of Medicines Australia
- **Kathleen Metters**
 - former head of worldwide basic research at Merck
 - former CEO of biopharmaceutical company Lycera Corp.
- **Edward Rayner**
 - over 20 years' experience in global capital markets

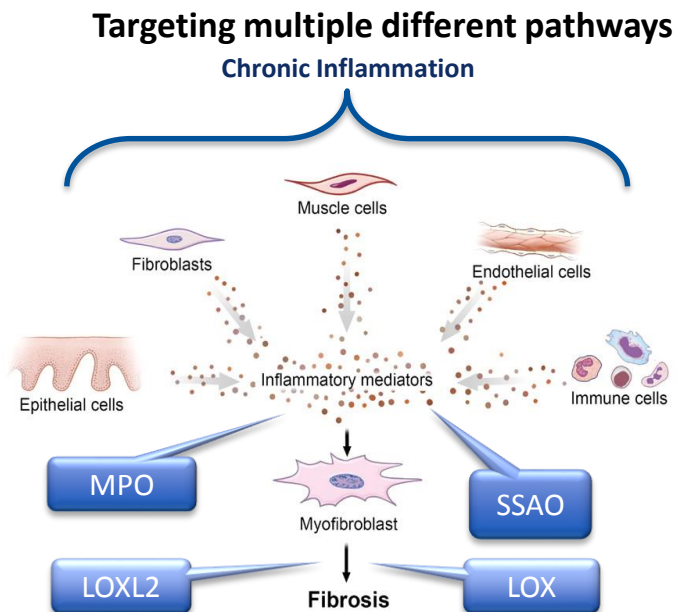
Pharmaxis portfolio

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
Commercial								
Bronchitol® US	Cystic fibrosis	Phase 3 trial met primary endpoint in 2017. Chiesi filed updated NDA with FDA in Q4 2018. Subject to FDA approval, US partner Chiesi will launch commercially in the US in 2019.						
Bronchitol RoW	Cystic fibrosis	Bronchitol is currently sold in the UK, Germany and Italy by Chiesi; in certain other European countries and Russia by specialist distributors; and by PXS in Australia and smaller countries						Direct & Dist
Aridol®	Asthma diagnosis	Aridol is approved and sold in US, Australia, South Korea and a number of European countries. Filed for Canadian approval - response expected mid 2019.						Direct & Dist
In the clinic								
SSAO (PXS-4728A)	NASH	Sold to Boehringer Ingelheim in May 2015. Phase 2a trial commenced August 2017. PXS received payments of A\$68m to date. Fully recruited.						
SSAO (PXS-4728A)	Diabetic retinopathy	Boehringer commenced dosing a Phase 2a trial in January 2018. PXS received A\$15m to date.						
LOXL-2	NASH, fibrosis - liver, lung, kidney, heart	Phase 1 trials in 2 compounds complete. Commercial partnering process commenced.						
LOX – oral	Cancer	Anti-fibrotic. Commenced Phase 1 February 2019.						
Preclinical								
LOX – topical	Scarring	Anti-fibrotic. Effective in scarring models.						

 Progress in last 12 months

A pipeline of drugs for inflammation and fibrosis

Pharmaxis has developed a commercial pipeline of small molecule drugs against high value targets



Key areas of current focus:

- NASH/liver fibrosis – SSAO and LOXL2
- Diabetic retinopathy (DR) - SSAO
- Pulmonary fibrosis (PF) – LOXL2

Other active programs:

- Pancreatic cancer & myelofibrosis – LOX (oral)
- Scarring – LOX (topical)
- Oral anti-inflammatory – SSAO/MPO; SSAO/MAO

Pharmaxis Drug Discovery

Pharmaxis has developed a commercial pipeline of small molecule drugs for inflammation and fibrosis

- Amine oxidase enzymes are well validated as targets in diseases with a high unmet medical need
- Pharmaxis are global leaders in amine oxidase enzyme inhibition
- Pharmaxis developed IP
- Since 2015 the platform has delivered:
 - 1 compound in 2 x phase 2 trials (SSAO)
 - 2 compounds phase 2 ready (LOXL2)
 - 1 compound commenced phase 1 (LOX oral)
 - 1 compound in pre-clinical development (LOX topical)

Key catalysts targeted for 2019/2020

Pharmaxis value driving events

1. LOXL2 anti fibrotic program

- Partnering process – H1 2019

2. Boehringer Ingelheim acquired SSAO inhibitor (BI 1467335) to report **clinical proof of concept** in two major diseases as Phase 2 trials report in H1 2019 and H1 2020

3. LOX (oral) for pancreatic cancer and myelofibrosis progressing in the clinic

- Phase 1 clinical trial (healthy subjects) commenced Feb
- Ready to start trials in patients by early 2020
- Plan to develop to phase 2

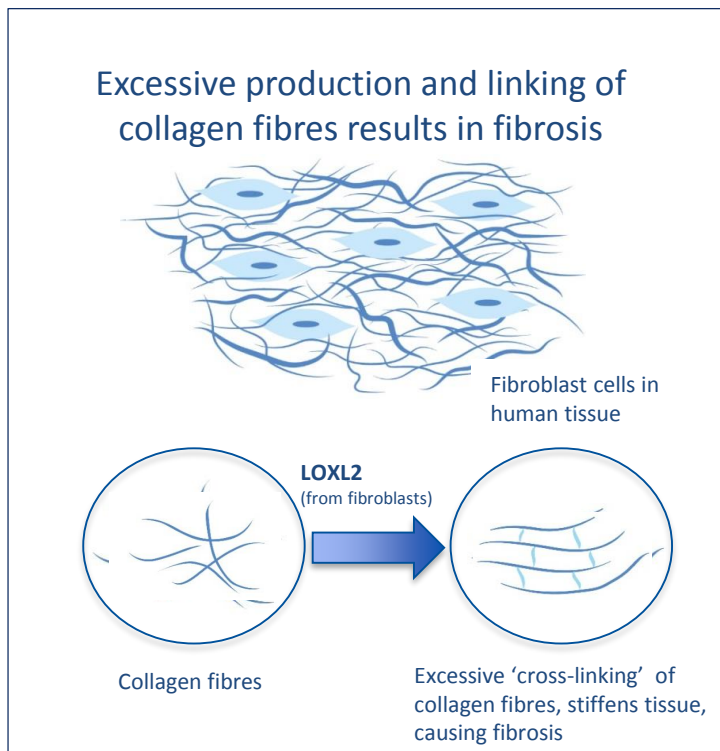
4. LOX (topical) for scarring ready to progress to phase 1 / clinical proof of concept study by early 2020

5. Others

- Bronchitol US - **FDA to complete review CY 2019**
- Other internal programs developing **additional compounds** to take into preclinical development
- Evaluating opportunities for in-license or acquisition of new programs in fibrosis and inflammation that **leverage PXS** research and commercialisation capabilities

LOXL2 inhibition program

for NASH, IPF & other high value fibrotic diseases



- Potential indications / market size:

- NASH / Liver Fibrosis; \$35b¹
- Pulmonary fibrosis (IPF); \$3.5b²
- Kidney fibrosis
- Cardiac fibrosis

Significant market opportunity

- LOXL2 and fibrosis:

- LOX family of enzymes catalyse the final step in the fibrotic disease process
- Clear association of increased levels of serum LOXL2 with disease progression in IPF, NASH and cardiac fibrosis

- Competitive profile:

- Novel target and mechanism of action
- Once daily oral drug
- Best in class drug with high level inhibition of LOXL2 enzyme for 24 hours from one dose
- Only known drug in clinical development to inhibit LOXL3
- Place of LOXL2 at the end of the fibrotic cascade provides opportunity to treat various fibrotic diseases and use in combination with other Pharma pipeline drugs

LOXL2 inhibitor program

“deal ready”

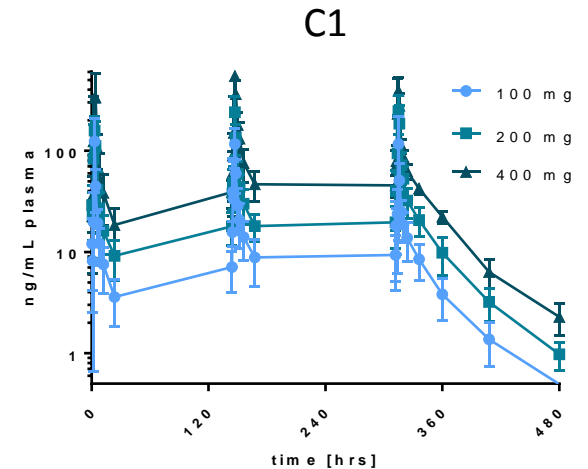
Feature	What Pharma values	PXS program status
Disease target	Independent validation	Multiple peer reviewed publications
Pre clinical proof of concept	2 or more different supportive animal models	Multiple supportive models across 5 different diseases. Further studies in progress
Dosing regimen	Ease of use	Oral once a day tablet or capsule
Patent	Composition of matter As long as possible	Composition of matter 2016 filing date; 100% PXS owned
Cost of Goods	Low	Small molecule with easy synthesis
# Compounds	1 plus backups	2 compounds in clinical development plus back ups
Toxicity	Wide therapeutic window As long as possible	13 week studies complete (2 species) Support progression to phase 2 studies
Clinical phase	Phase 1 with target engagement Phase 2 ready	Both compounds successfully completed Phase 1 clinical trials
Target engagement	Drug inhibits target	Very high levels of inhibition for 24 hours from a single daily dose over 14 days – achieved with both compounds

LOXL2: Phase 1 Studies in two compounds

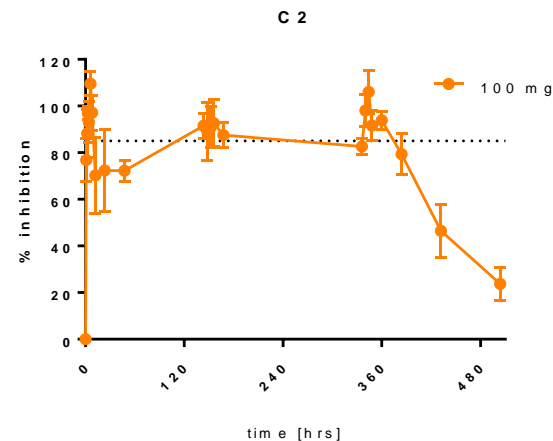
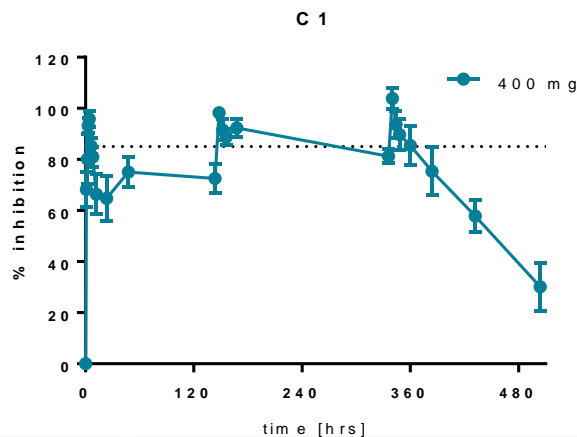
Positive safety and PK/PD findings in Phase 1 trials

- SAD and MAD for two compounds (C1 and C2) have been completed with 108 healthy subjects on drug
- C1 and C2 were well tolerated and no safety signals detected
- AUC and Cmax of both compounds increased with ascending dose
- C1 and C2 inhibited plasma LOXL2 at least 85% for 24 hrs after a single oral dose in MAD phase

Pharmacokinetics of first compound in MAD stage



Target engagement of two compounds in MAD stage



Repeated dosing resulted in >85% enzyme inhibition 24 hrs after last dose from Day 7 onwards.
Human effective doses will be equal or below the above doses.

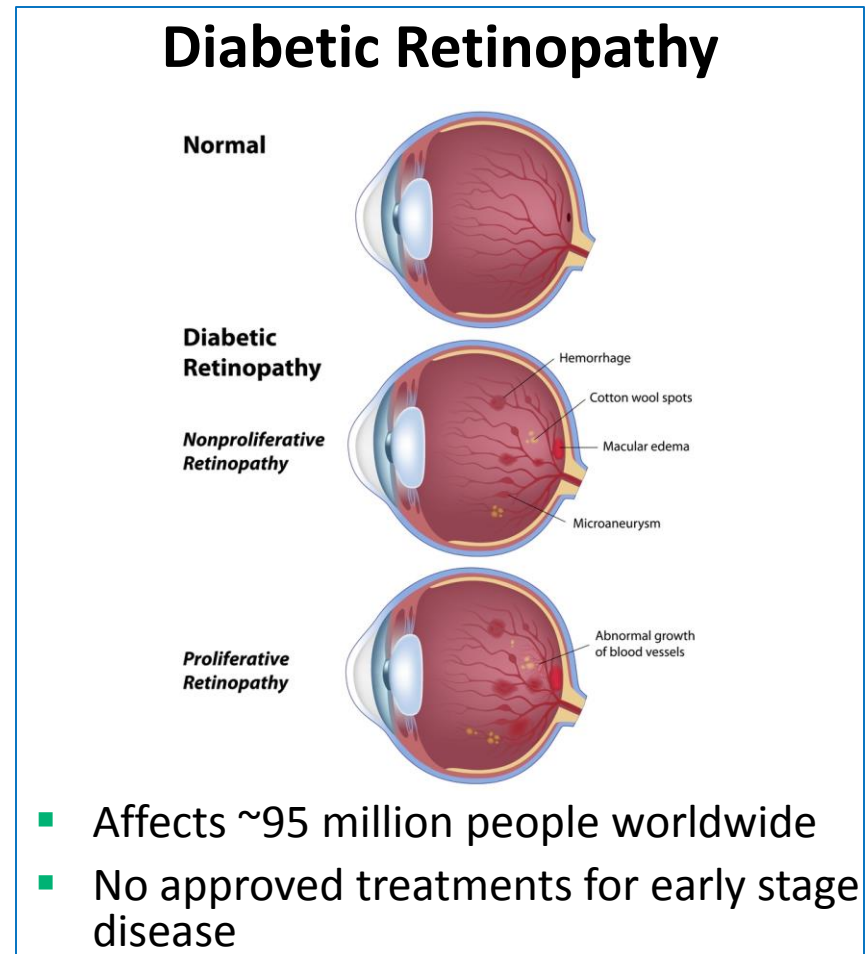
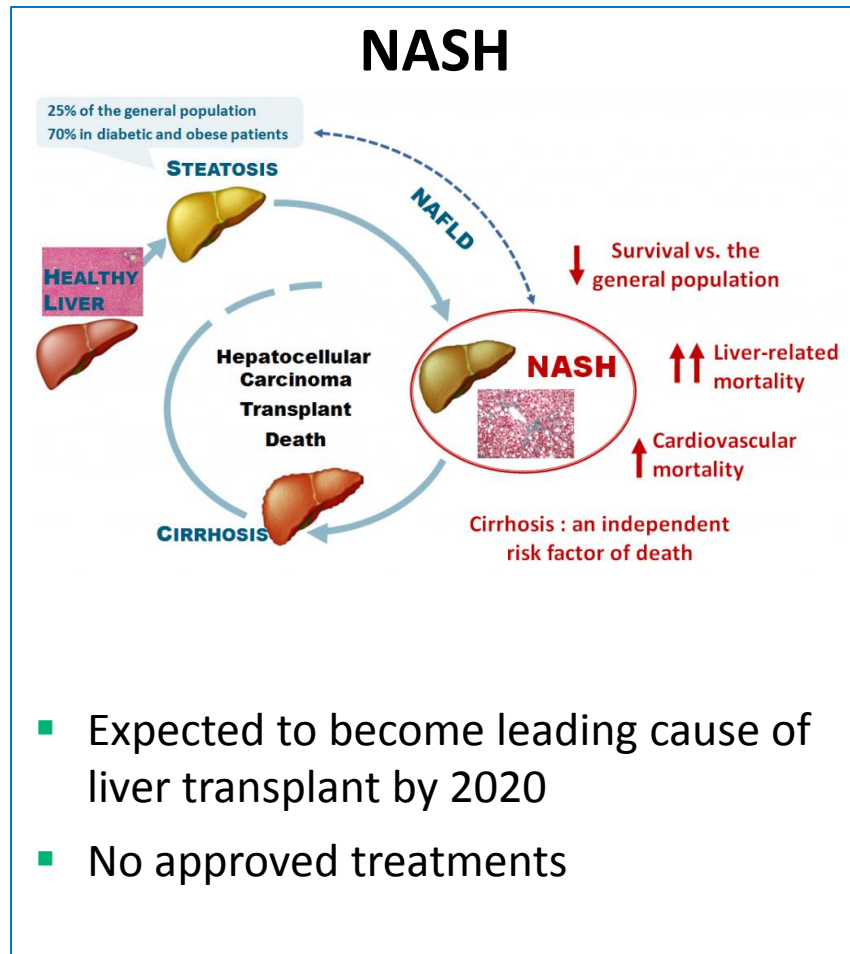
LOXL2 inhibitor program – partnering process

Positive engagement with pharma companies

- Pharma company interest driven by search for:
 - Safe and effective inhibitor to LOXL2 and LOXL3 enzymes,
 - Safe and effective anti-fibrotic drug, and/or
 - Drugs to complement existing disease portfolio – lung, liver, kidney, heart, etc.
- Pharmaxis has engaged with multiple potential partners during planning and progress of the LOXL2 program
- Pharmaxis data package completed in Q1 2019, including:
 - Full analysis of second stage of phase 1 trials for both compounds
 - 13 week tox studies (2 species) for both compounds
- Data room available (under CDA)
- Commercial partnering process commenced

SSAO (Boehringer Ingelheim): Pharmaxis poised to be a major player in diseases caused by complications of diabetes

Two diseases with high unmet need and large patient populations in Phase 2 studies



SSAO: Phase 2 trials to show clinical proof of concept in H1 2019

Boehringer Ingelheim responsible for clinical development and commercialisation

NASH

- Phase 2a trial expected to report H2 2019 – proof of efficacy in patients with moderate – severe disease
- Deutsche Bank estimate market size of US\$35b by 2025
- First in class anti inflammatory SSAO inhibitor for NASH with peak sales potential of ~US\$2b [Analyst's estimate]

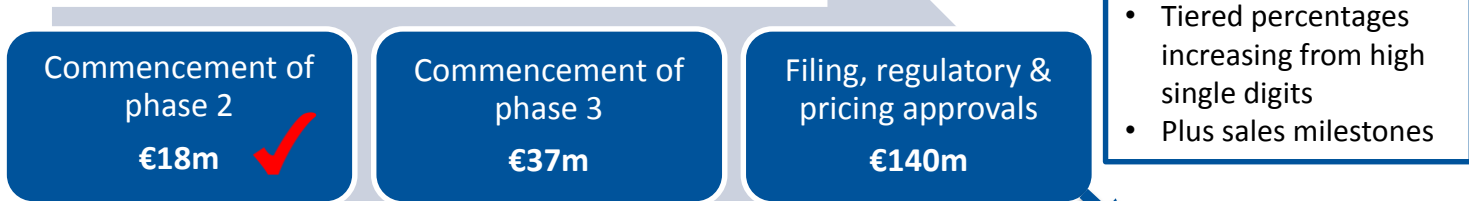
Diabetic Retinopathy

- Phase 2a SSAO diabetic retinopathy expected to report H1 2019 – proof of efficacy in patients with early stage disease
- Affects one third of diabetic patients world wide
- No approved treatments for early stage disease
- First in class anti inflammatory SSAO inhibitor for DR with peak sales potential of ~US\$800m [Analyst's estimate]

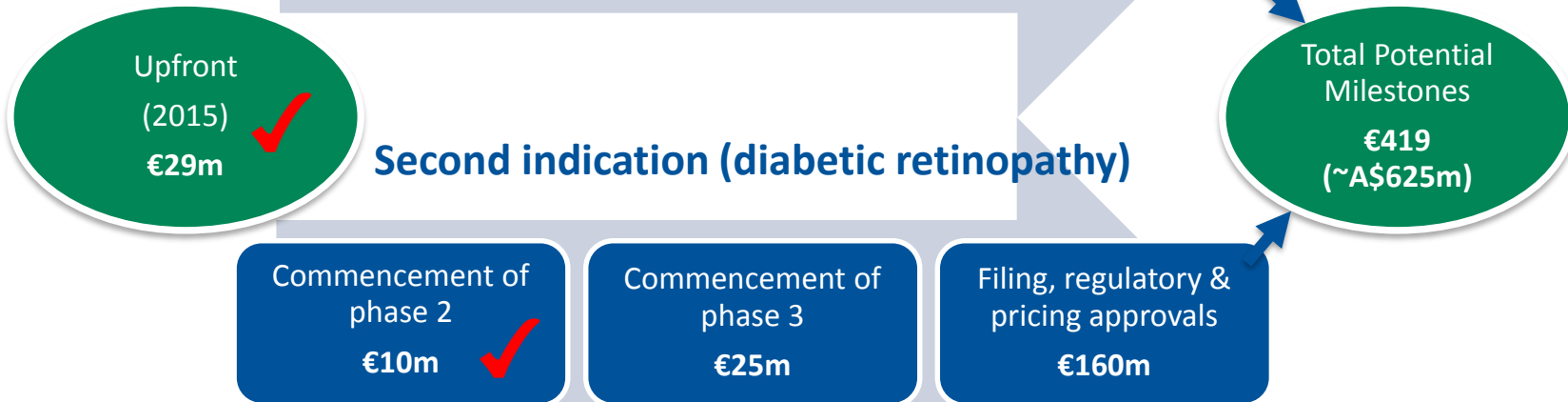
SSAO: Boehringer Ingelheim deal

Deal structure illustrates value generating potential of Pharmaxis business model

First indication (NASH)



Second indication (diabetic retinopathy)



- €57m (A\$83m) already received
- No further investment required from Pharmaxis

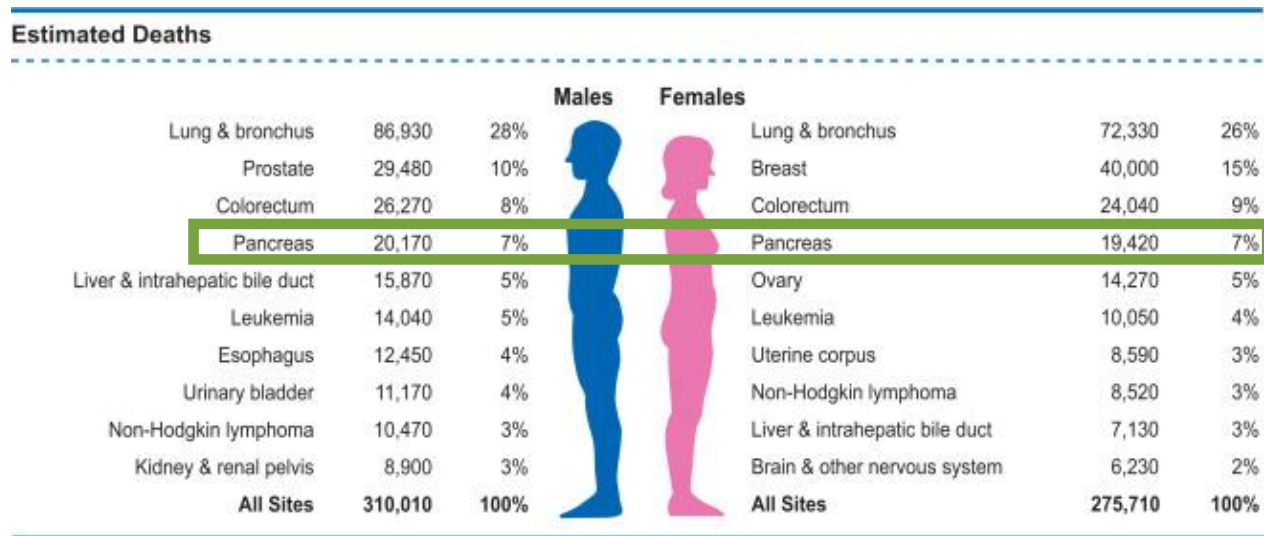
More programs approaching the clinic

Opportunities to fast track both programs into patient proof of clinical efficacy studies

Program	LOX (oral)	LOX (topical)
Indication	Severe fibrotic indications: <ul style="list-style-type: none"> pancreatic cancer myelofibrosis 	<ul style="list-style-type: none"> Scar revision Keloid Contraction of scarring from burn wounds
Commercialisation	Partner after phase 2	Partner after phase 2
Status	<ul style="list-style-type: none"> Commenced Phase 1 Feb 2019 Effective in reducing fibrosis in animal models of fibrosis and myelofibrosis 2018 patent priority date 	<ul style="list-style-type: none"> Lead candidate selected Initial stability of topical formulation Ongoing evaluation in various disease models of scarring Ongoing tox studies
Next steps 2019	<ul style="list-style-type: none"> Additional animal models of pancreatic cancer and myelofibrosis Complete 3 month tox studies Ready to commence phase 1c/2 study in pancreatic cancer patients by early 2020 	<ul style="list-style-type: none"> Complete full preclinical development Ready to commence phase 1 study by early 2020 Phase 1 study will test clinical efficacy in healthy volunteers with scarring

Pancreatic Cancer: A highly aggressive disease

4th most common cancer related death in men and women



Infographic: Siegel et al. *CA Cancer J Clin* (2016);7–30.

- Approximately 450,000 new cases of pancreatic cancer annually worldwide
- The median survival for advanced pancreatic cancer, treated with our best therapeutics is currently only 6-8 months
- The 5 year survival rate for pancreatic cancer is approximately 7-8%
- This statistic has barely improved in the last 25 years
- New treatments to improve outcome are an urgent unmet clinical need.

Pancreatic Cancer is a highly stromal (fibrotic) disease

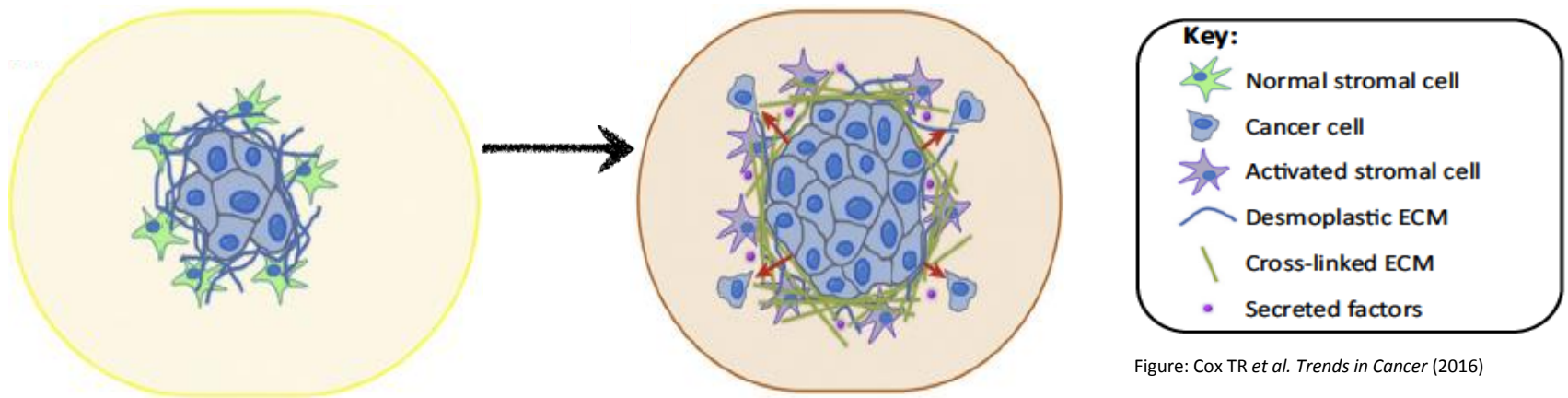
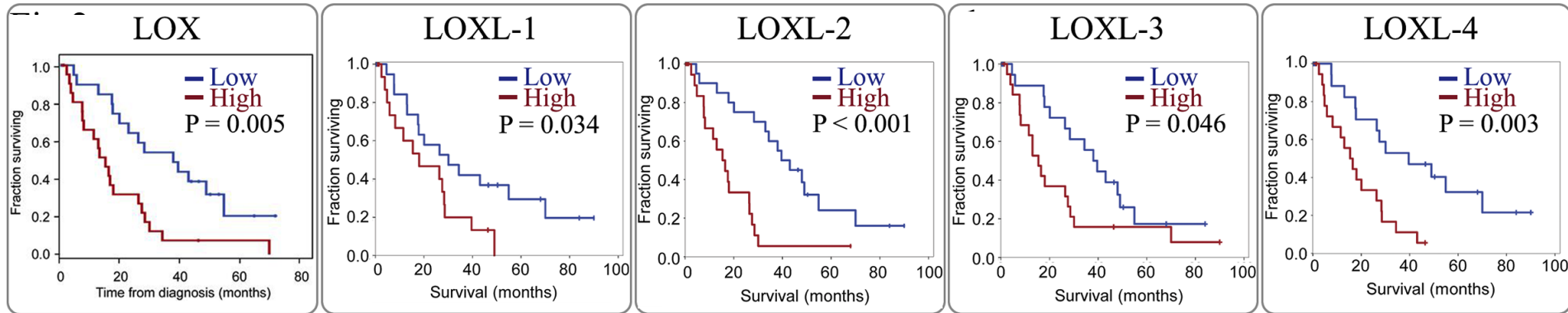


Figure: Cox TR *et al.* *Trends in Cancer* (2016)

- As pancreatic cancer progresses, an accompanying fibrotic response evolves within and around the developing tumour - decreasing the efficacy of existing therapies.
- The scar-like (fibrotic) tissue does this by changing the tumour in several ways;
 - Altering cancer cell behaviour, including making them more aggressive
 - Directly and indirectly altering cancer cell sensitivity to therapies
 - Acting as a physical barrier to the delivery of our adjuvant therapies
 - Providing a highway for cancer cells to spread (metastasise) around the body

The LOX family in pancreatic cancer

All LOX family members are elevated in PC and are associated with survival



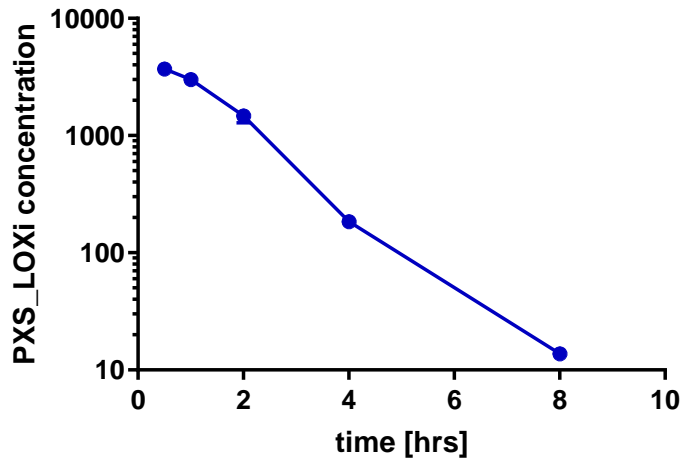
Data from Miller *et al. EMBO Mol. Med* (2015) - Kaplan-Meier analyses showing correlation of LOX family member expression and survival in the Glasgow patient cohort (microarray analysis of 400 cores from a total of 80 PDAC resections)

- Targeting a single family member (LOX) has shown some success previously in combination with chemotherapy (Miller *et al. EMBO Mol. Med* (2015))
- Preliminary *in vivo* data targeting the whole LOX family using Pharmaxis' new small molecule inhibitor suggests this maybe a more robust approach to improving efficacy of standard of care chemotherapy

Pharmaxis oral LOX inhibitor

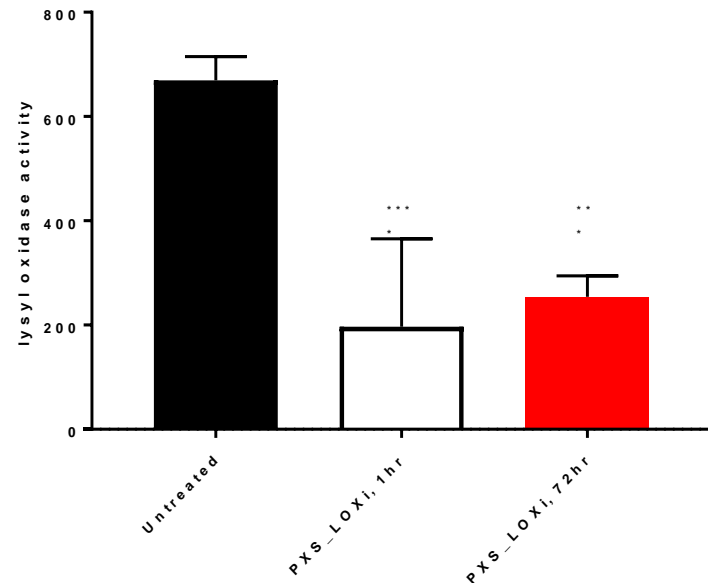
Significant and long lasting target engagement from a single dose

Pharmacokinetic properties



Single oral dose of 30 mg/kg in rat

Pharmacodynamic properties



Single oral dose of 30 mg/kg in rat

Excellent PK / PD properties in pre clinical studies now being validated in ongoing phase 1 clinical studies

Pharmaxis purpose built facility

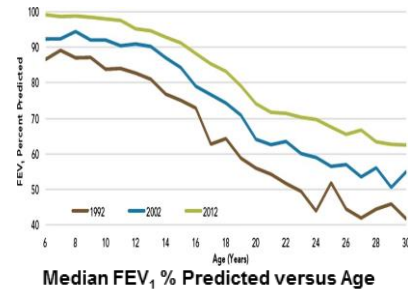
Pharmaxis has a purpose built manufacturing and drug development facility in Sydney

- Manufacturing and research facilities
- Productive R&D drug discovery engine
- Team of 17 scientists specialising in amine oxidase chemistry drug discovery and pre clinical development
- Capability to run global clinical trials
- Manufacturing and exporting approved products:
 - [Bronchitol®](#)
 - [Aridol®](#)
- Capacity for future growth



Bronchitol for cystic fibrosis

Overview



Cystic fibrosis

- Patients
 - US: 30,000;
 - Europe: 37,000;
 - Russia: ~10,000¹
 - Australia: 3,500
 - Total world: ~100,000
- Disease characterised by poorly hydrated, tenacious, thick mucus
- Inexorable decline in lung function
- Frequent infections

1. Estimates vary from 7,000 to 30,000

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
- PXS supplies all markets from Sydney factory

Business model - RoW

- Distributors responsible for promotion & support
 - Chiesi in UK, Germany, Italy & Ireland
 - Other distributors in Russia, Eastern Europe, Middle East
 - PXS revenue share ~50%
 - Russian reimbursement from 1 January 2019
- PXS direct in Australia and smaller markets

Business model - US

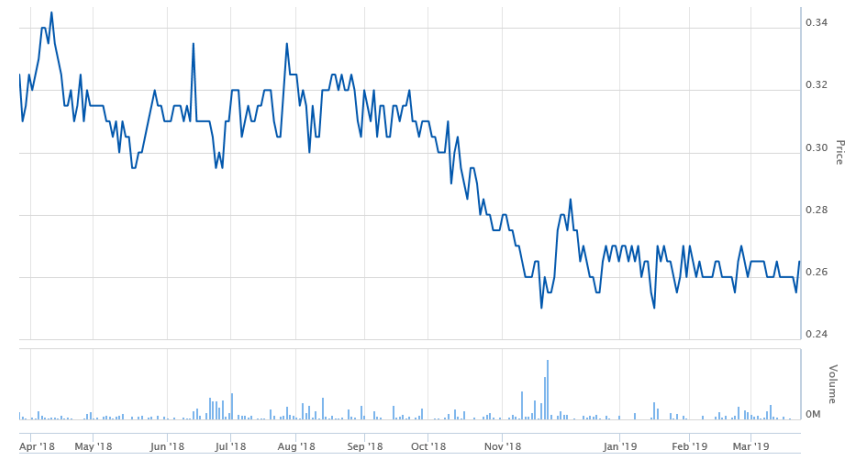
- Phase 3 trial (CF303) reported June 2017
- Chiesi responsible for regulatory filing & commercialisation – preparing for launch
- Updated NDA filed Q4 2018
- FDA advisory committee will review on May 8th 2019
- ~A\$13m milestone payment on launch
- PXS receives high mid teens % of in-market sales plus cost of goods

Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap ¹	\$104m
Shares on Issue	394m
Employee Options ¹	17m
Liquidity (turnover last 12 months) ¹	33m shares
Share price ¹	\$0.265
Analyst valuation ²	\$0.47
Cash Balance (31 December 18)	A\$42m

Institutional Ownership	25 Mar 19
BVF Partners (US)	22%
Arix Bioscience (UK)	11%
Australian Ethical	8%
D&A Income Limited	7%
Allan Gray	5%
Other Institutions	8%
Total Institutional Ownership	59%



1. As at 25 March 2019
2. Bell Potter Securities Research 15 February 2019

Financials – highlights

31 December 2018

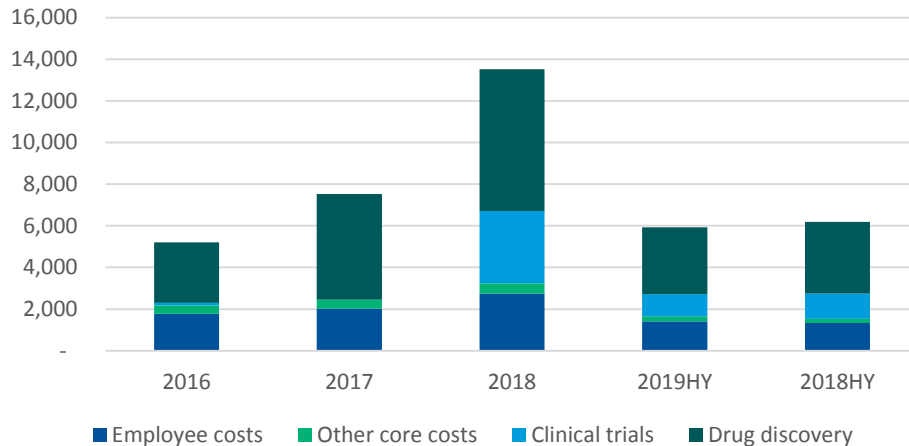
	A\$'000	Three months ended		Six months ended	
		31-Dec-18	31-Dec-17	31-Dec-18	31-Dec-17
(unaudited)					
Income statements					
Sales of Bronchitol & Aridol		1,337	1,402	2,237	2,451
Milestones from sale of drug					26,891
Total revenue		1,718	2,433	2,950	31,344
Total expenses		(6,772)	(17,860)	(15,537)	(25,432)
Net profit (loss) after tax		(5,054)	(15,427)	(12,587)	5,920
Segment results – adjusted EBITDA					
Bronchitol & Aridol		60	13	(1,789)	(1,447)
New drug development		(2,714)	(3,627)	(5,923)	20,872
Corporate		(1,000)	(10,653)	(2,098)	(11,635)
Total		(3,654)	(14,267)	(9,810)	7,790
Statement of cash flows					
Cash inflow/ (outflow) from:					
Operations		(4,071)	(13,023)	(10,280)	4,639
Investing activities		(229)	(125)	(562)	(235)
Financing activities		(455)	(436)	21,772	(863)
Total cash generated/(used)		(4,755)	(13,584)	10,930	3,541
Cash at bank		42,003	25,045	42,003	25,045

Refer to December 2018 Quarterly Shareholder Updates and 2019 Half Year Financial Report for additional financial information

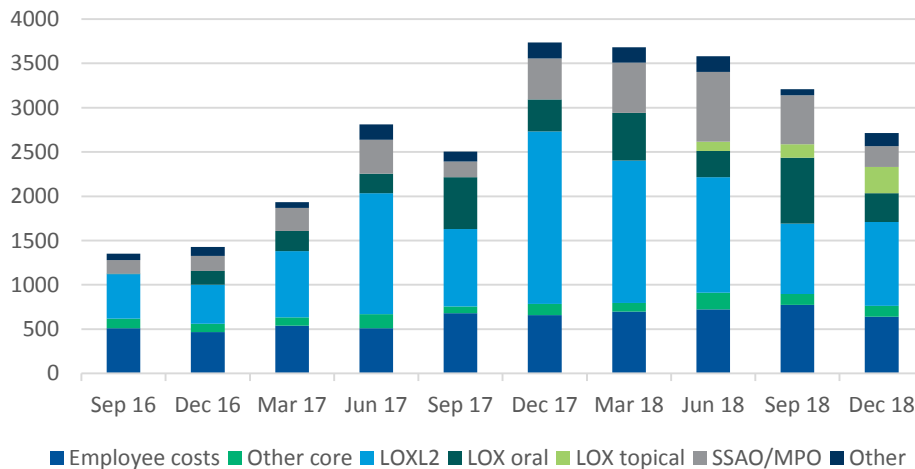
New Drug Development

Expenses (financial years ended 30 June)

New Drug Development Expenses



New Drug Development Expenses - Quarterly

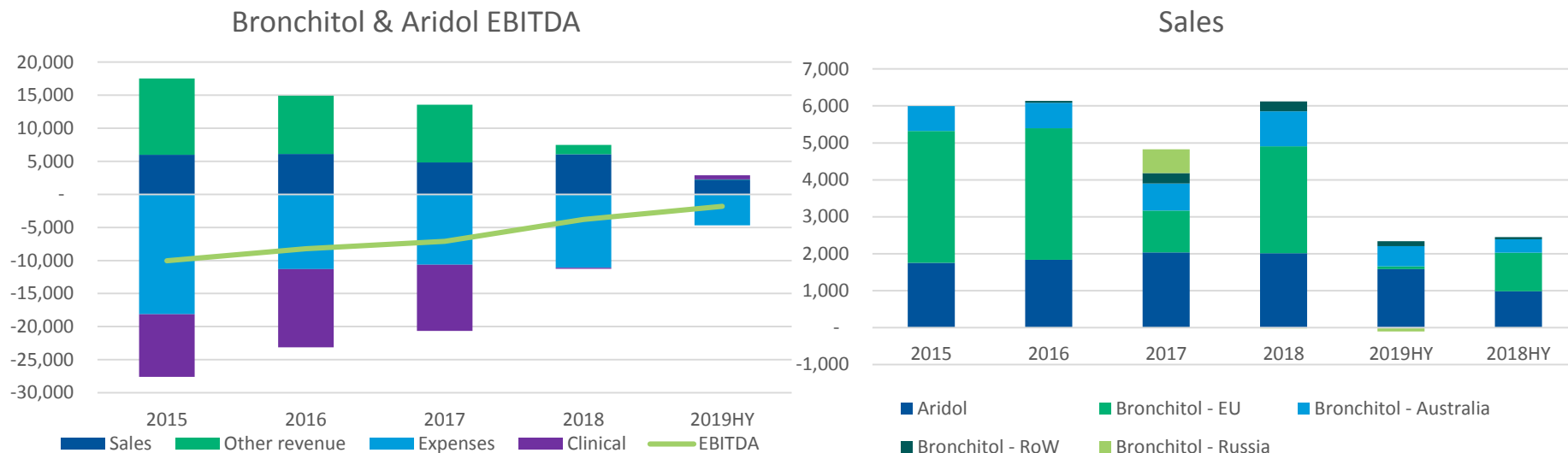


Current status/planned expenditure

- LOXL2:
 - 2 compounds completed phase 1 trials and 13 week tox in Dec 2018. Partnering package complete
- LOX (oral):
 - To commence phase 1 Q1 CY 2019
 - Disease models continue – cancer
 - Plan to advance into phase 2 clinical trials
- LOX (topical)
 - Commenced formal preclinical
- SSAO/MPO in preclinical
 - Evaluating potential indications

Bronchitol & Aridol

Segment profitability & sales analysis (financial years ended 30 June)



Path to profitability: increase revenue to leverage cost base

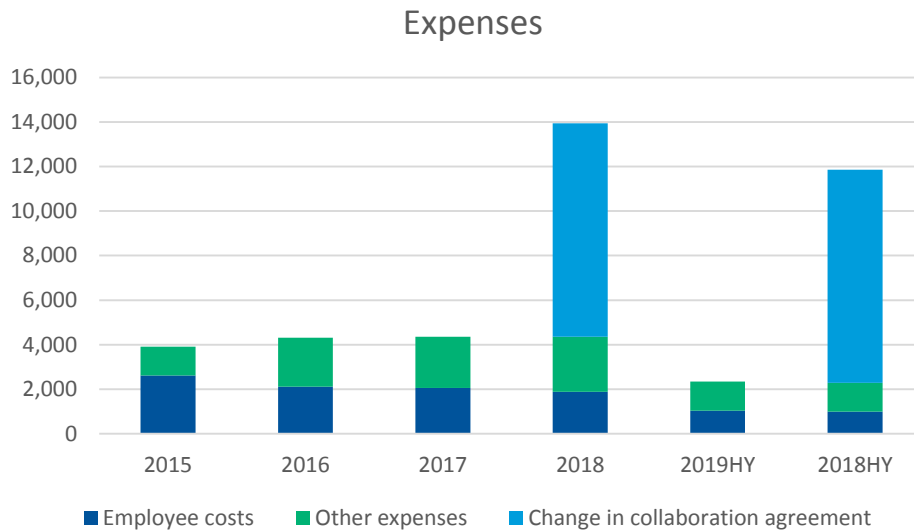
- Core cost base relatively fixed vs sales volume
- Reimbursement of Bronchitol in Russia key to rate of overall sales growth – achieved Q4 CY 2018
- US approval – Subject to FDA approval (~Q3 CY 2019), launch Q4 CY 2019 (US\$10m milestone)
- Other Bronchitol sales growth opportunities
- Growth from new markets: Bronchitol (Italy, Spain, CZ, Ireland); Aridol (US - Dec 18; Canada – target launch Q3 2019)
- FY 2019 includes reimbursement of CF303 clinical trial costs

Revenue

- FY 2015: Direct to pharmacy until June 15 (ie all sales revenue to PXS)
- FY 2016: EU sales via distributors at lower margin (~50%) to PXS. Chiesi builds inventory levels
- FY 2017: First sale to Russia (\$640k)
- FY 2018: Growth in EU (Chiesi) & Australia (expanded PBS coverage)
- FY 2019: Aridol includes US relaunch. Major distributor orders expected H2 FY 2019
- Other revenue in all years is predominantly reimbursement of clinical trial costs by US partner

Corporate

Expenses (financial years ended 30 June)

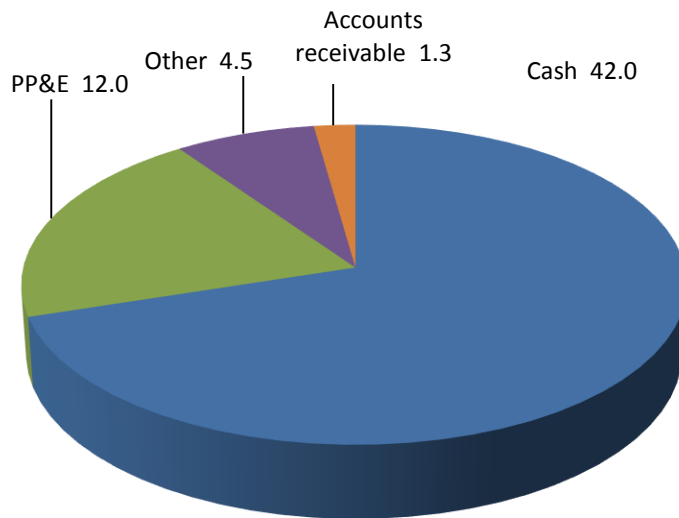


- Employee and other costs stable
- One-off expense in H1 FY 2018 to change collaboration agreement with Synairgen

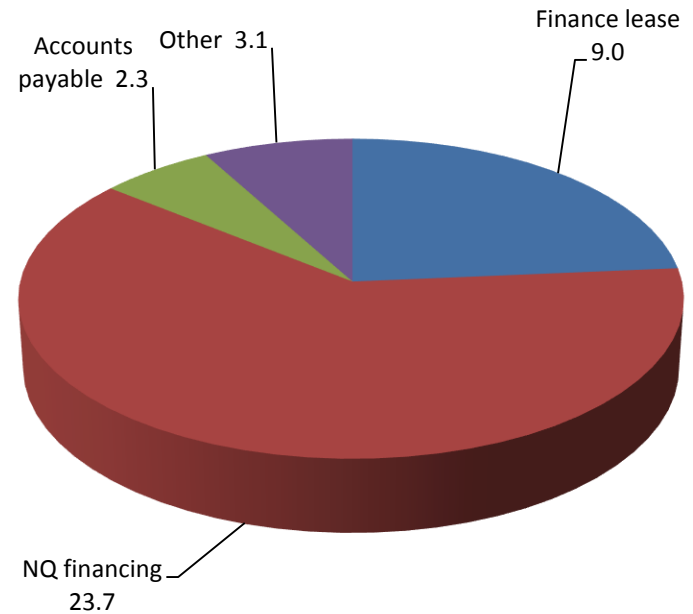
Balance sheet

31st December 2018

Assets (A\$59.9m)



Liabilities (A\$38.0m)



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

Summary

Pharmaxis is a global leader in drug development for fibrosis & inflammation

- Pharmaxis have built a **successful platform of small molecule drugs targeting high value fibrotic and inflammatory indications**
- **Development pipeline** across various stages
 - one drug in two phase 2 trials (BI)
 - one drug program ready to progress to phase 2
 - one compound commenced phase 1 Q1 2019
 - other compounds in pre-clinical development approaching the clinic, additional drug candidates in discovery.
- Proven track record of **early stage partnering** and taking products **through to commercialisation**
- **Potential to receive total up front and milestone payments of A\$625m** plus further sales based payments from first deal (SSAO) – A\$83m already received
- Next drug program completed phase 1 trials and long term toxicity studies: **partnering process commenced**
- FDA decision on Bronchitol NDA and US\$10m commercialisation milestone payment due in H2 2019
- **Strong balance sheet - \$42m** cash balance (31 December 2018)
- **Numerous catalysts over the next 18 months**



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