Investor Presentation

Gary Phillips CEO

8 September 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.

Pharmaxis overview

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

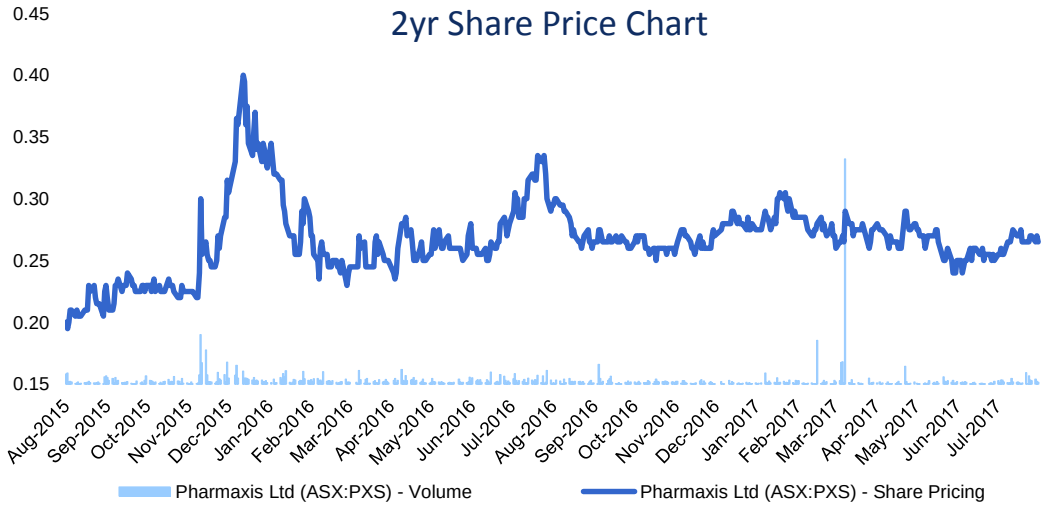
- Pharmaxis has built a **successful platform of small molecule drugs targeting fibrosis and inflammation** across various stages of development and approval
- Proven track record of **early stage partnering** and taking products **through to commercialisation** – delivered two products to market
- **Potential to receive total up front and milestone payments of A$625m** plus further sales based payments (% and milestones) from first deal – A$68m already received
- **Strong discovery pipeline targeting high value indications -** one drug in 2 phase 2 trials, one drug program to start phase 1 in 2017, three compounds in development
- **Growing revenues from approved product sales** (A$4.8m in FY17) **& milestones** (A$27m FYTD 2018)
- **Strong balance sheet - A$22m** at 6/17 plus A**$27m** milestone received Q3 2017 and **A$15m** milestone expected H2 2017
- Purpose built **manufacturing and research facility** in Sydney
- **Strong institutional share register**; including offshore specialist biotech funds

Shareholders & trading

Financial Information	
ASX Code	PXS
Market Cap[1]	$85m
Shares on Issue	319m
Employee Options	13m
Liquidity (2017 turnover YTD)[1]	68m shares
Cash Balance (proforma)	**$49m**

Institutional Ownership	%
BVF Partners (US)	20%
Australian Ethical	10%
Allan Gray	7%
Montoya Investments (UK)	6%
Other Institutions	7%
Total Institutional Ownership	**50%**

1. at 29 August 2017



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

Clinical Trials

- Utilise global experience and extensive clinical networks to execute value adding Phase 1 and 2 clinical trials

Drug Discovery Engine

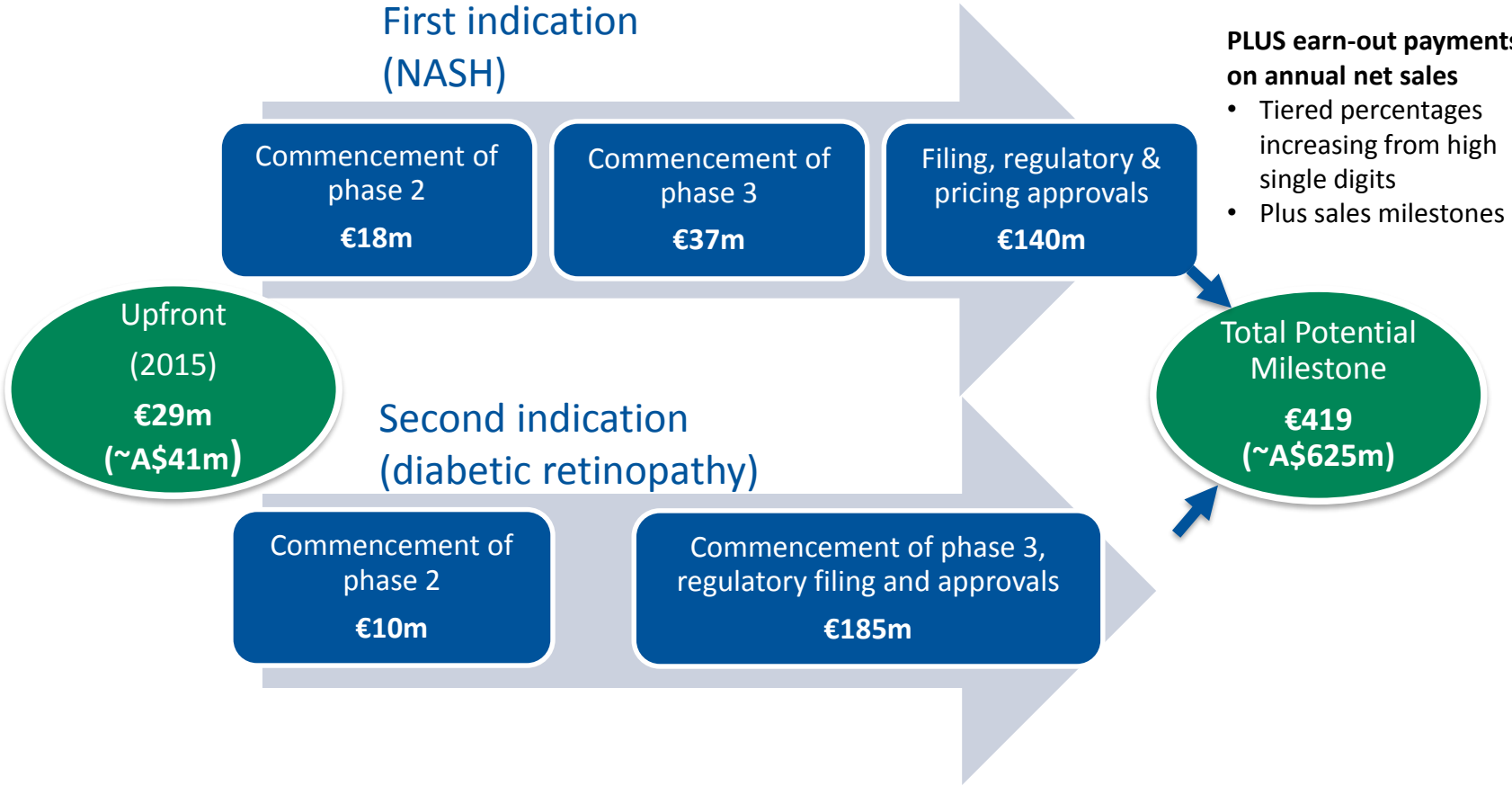
- Leverage small molecule expertise and in house chemistry platform
- Efficiencies from global academic & CRO networks
- Target high value diseases with validated targets

Value Generation

- Extensive Big Pharma network
- Partnering after phase 1 or 2 to realise value and mitigate program and corporate risk

Boehringer Ingelheim deal

Deal structure illustrates value generating potential of Pharmaxis business model



- Price sensitive interim news flow on trial results
- No further investment required from Pharmaxis

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. Subject to FDA approval launch commercially in the US in 2018 . Chiesi has responsibility (incurring all costs) for completing the New Drug Application with the FDA and US commercialisation.						Chiesi
Bronchitol RoW	Cystic fibrosis	Bronchitol is currently sold in the UK and Germany by Chiesi and recently added Italy (launch H2 17). Recently approved for sale in Russia. Bronchitol & Aridol business segment expected to transition to profitability over the next 12 to 24 months irrespective of any approval in the US. A$2.8m revenue in FY17						Distributors
Aridol®	Asthma diagnosis	Aridol is approved and sold in Australia, South Korea and a number of European countries. A$2m revenue in FY17.						Direct & Dist
In the clinic								
SSAO (PXS-4728A)	NASH	Sold to Boehringer Ingelheim in May 2015. PXS received payments of A$68m to date. Total potential milestone payments of A$290m during development program and further royalties and sales related milestones following approval.						Boehringer Ingelheim
SSAO (PXS-4728A)	Diabetic retinopathy	Boehringer Ingelheim commenced a Phase 2 trial in September 2017. Dosing of first patient triggers a €10m (A$15) to Pharmaxis. Total potential milestone payments of A$290m during development and further royalties and sales related milestones following approval.						Boehringer Ingelheim
Discovery								
LOXL-2	NASH, fibrosis - liver, pulmonary, kidney	Cleared preclinical development and set to enter phase 1 trials Q4 2017. Expected to partner at end of Phase 1 – H2 2018.						synairgen
SSAO/MPO	Respiratory & cardiovascular	Dual inhibitor with potential anti-inflammatory applications. Targeting Phase 1 trial in 2018						
LOX (topical)	Scarring	Commencing formal pre-clinical toxicology studies H2 2017 . Target Phase 1 trial in 2018						
LOX	Cancer	Assessing the utility of LOX in cancer						

Validated amine oxidase chemistry platform

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

Active Program Target Indications
Cardiac Fibrosis
COPD / Asthma
Kidney fibrosis
NASH / Liver fibrosis
Pancreatic cancer & myelofibrosis
Pulmonary Fibrosis
Scarring
Diabetic retinopathy

Pharmaxis Drug Discovery

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 owned IP
- Since 2015 the platform has delivered:
  - 1 compound in 2 phase 2 trials
  - 2 compounds to enter phase 1 in 2017
  - 2 compounds planned to enter phase 1 in 2018

Key areas of current focus are NASH and Pulmonary Fibrosis

Senior management

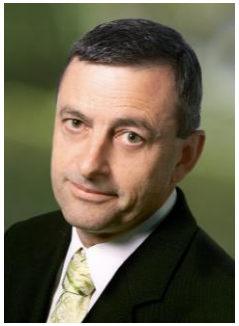
Significant experience in drug development, commercialisation and partnering

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** – *Chief executive officer and 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 Actellion
- **Kathleen Metters** – *Non executive director*
  - former head of global research at Merck

Key catalysts

Pharmaxis platform is built to deliver strong news flow

- Phase 2a SSAO (PXS-4728A) NASH trial commenced with first dosing in third quarter 2017 triggering €18m milestone payment (A$27m) from Boehringer Ingelheim. Trial to report mid 2018.
- Boehringer Ingelheim developing SSAO (PXS-4728A) for second indication (diabetic retinopathy). Phase 2 trial initiated in September 2017 – first patient dosed will trigger a milestone payment of €10m (A$15m)
- LOXL-2 program completed preclinical development, set to begin Phase 1 clinical trials in second half of 2017 and target partnering deal H2 2018
- Two further compounds with potential as first in class drugs in diseases with high unmet need planned to progress to Phase 1 in 2018
- Bronchitol FDA re-submission by Chiesi in 2018
- Productive R&D engine currently working on new drug discovery technologies
- Evaluating external opportunities for in-license or acquisition

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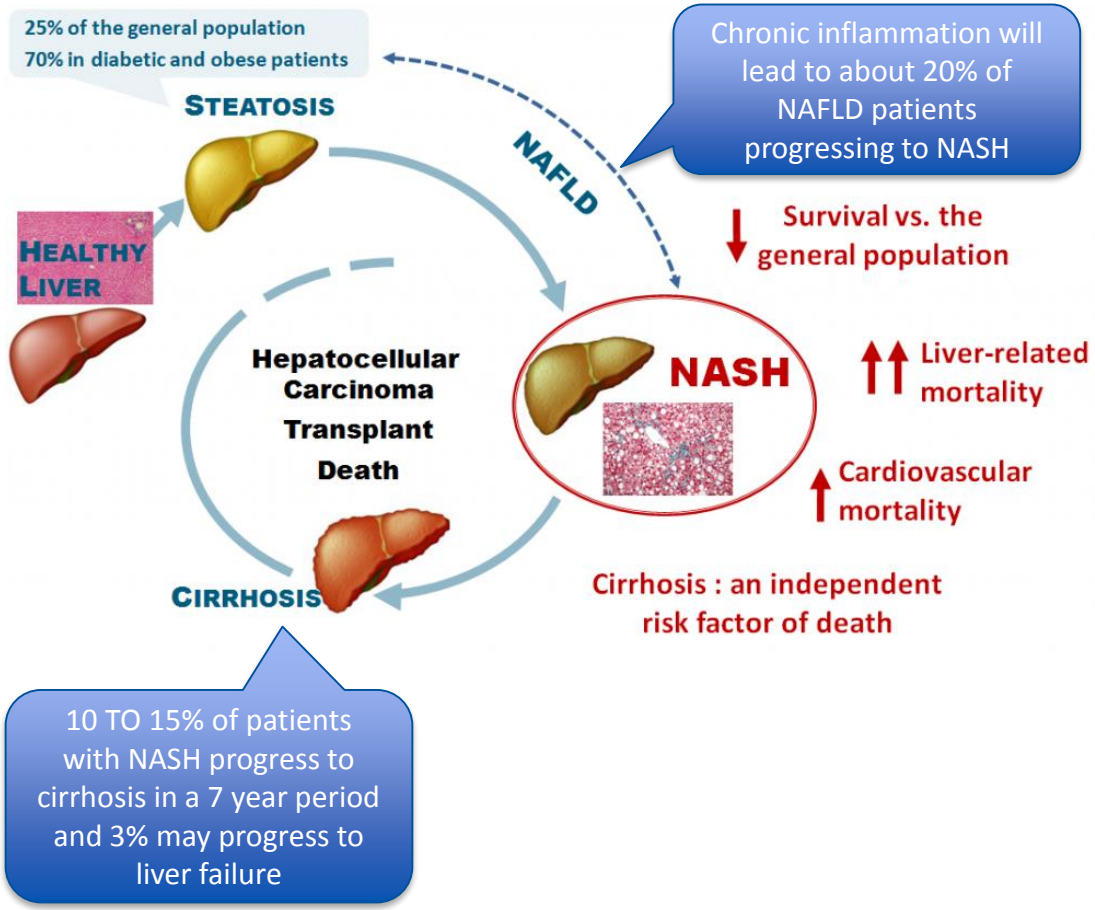
Key markets

NASH, Idiopathic Pulmonary Fibrosis and Diabetic Retinopathy

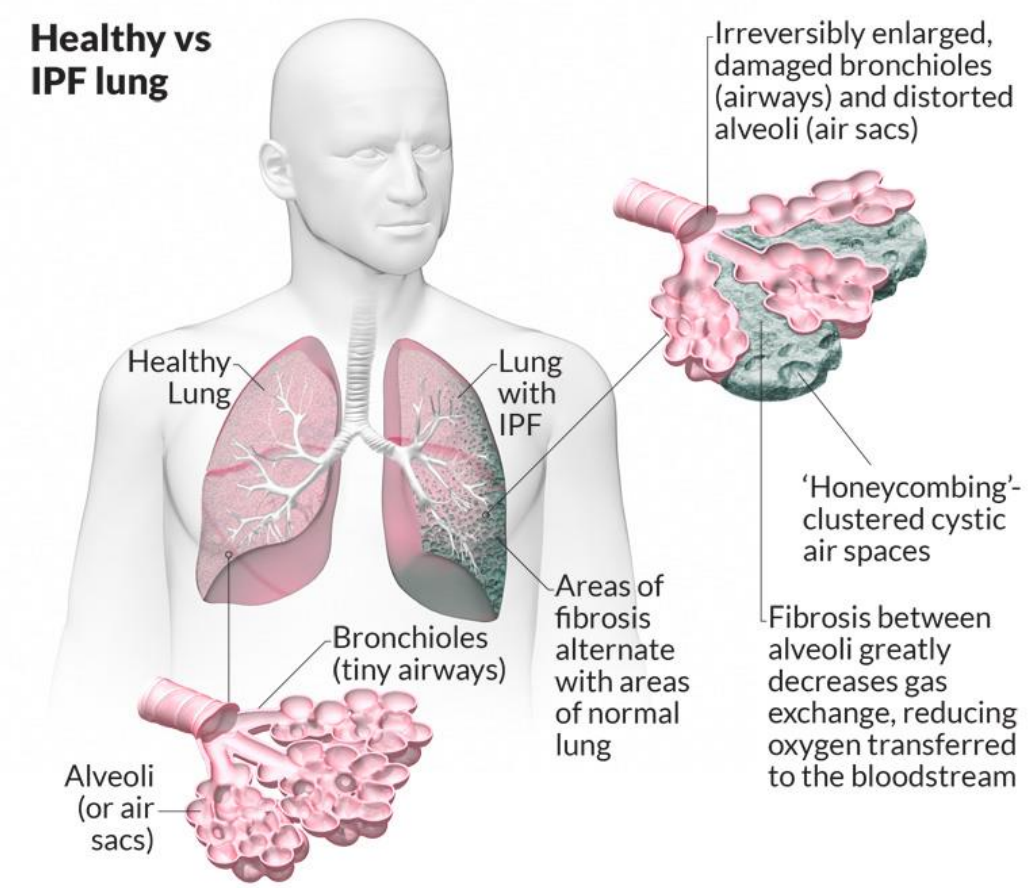
Disease focus - NASH



Nonalcoholic steatohepatitis

- NASH is a liver disease characterised by fat deposits, inflammation and tissue damage
- Risk factors are insulin resistance, type 2 diabetes, obesity, hypertension, high blood lipid levels and age
- Up to 16% of liver transplants in the US are due to NASH and by 2020 will overtake hepatitis C as the leading cause of liver transplant
- There no approved drugs
- Deutsche Bank predicts a global market >US$35b by 2025.

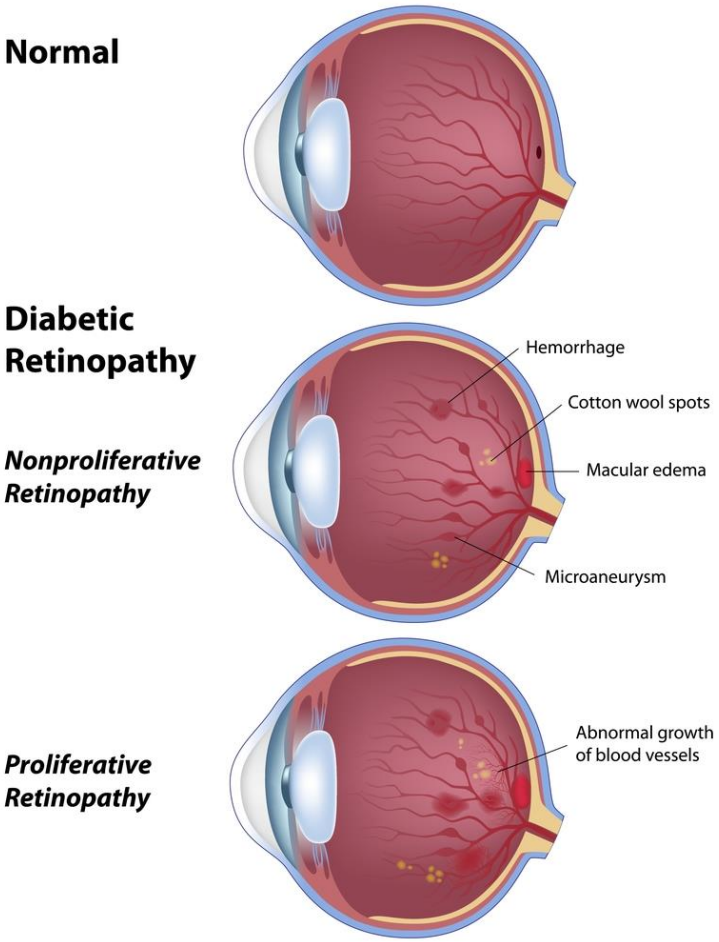
Disease focus - IPF



Idiopathic Pulmonary Fibrosis

- IPF primarily affects people over the age of 50
- 5,000 IPF patients in Australia
- 100,000 IPF patients 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

Disease focus – diabetic retinopathy

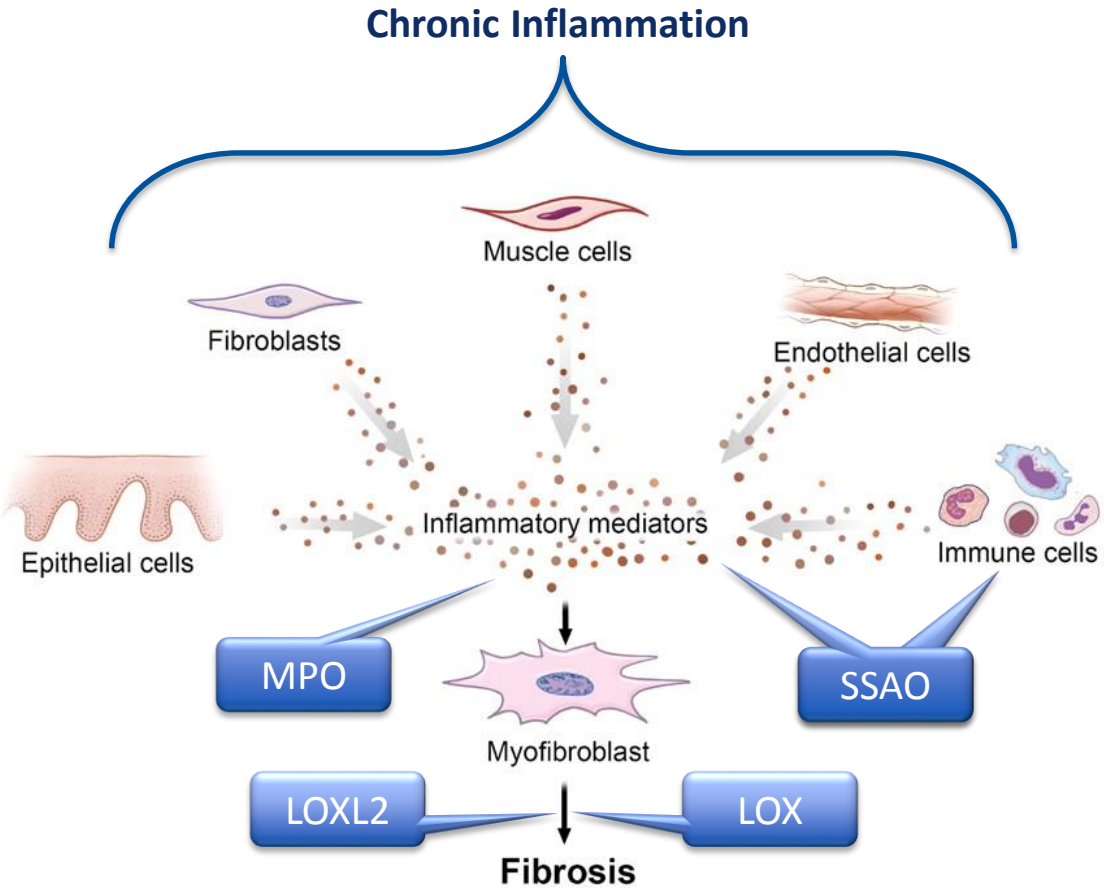


Diabetic retinopathy

- DR is the leading cause of vision-loss in adults aged 20-74
- Progresses from mild non-proliferative DR through to proliferative DR.
- Characterised by growth of new blood vessels on retina
- Diabetic macular oedema (DMA) can develop at all stages of DR
- Estimated 95 million people worldwide have DR – vision threatening to 1/3rd
- Urgent need for new therapies

A pipeline of drugs for inflammation and fibrosis

Targeting multiple different pathways



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In the clinic

Anti inflammatory drug PXS-4728A (SSAO inhibitor)

SSAO inhibitor: PXS-4728A

Boehringer Ingelheim

Anti inflammatory oral drug sold to Boehringer Ingelheim in May 2015

- 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 multiple pre clinical models inflammatory disease such as NASH and airway inflammation
  - Sold to BI in May 2015
  - Phase 2 trial in NASH commenced August 2017 (https://clinicaltrials.gov/ct2/show/NCT03166735)
  - Phase 2 trial in diabetic retinopathy initiated September 2017 (https://clinicaltrials.gov/ct2/show/NCT03238963)

External validation of PXS drug discovery and ability to negotiate valuable global deals

First indication: Phase 2a NASH study

Recruitment open and dosing commenced

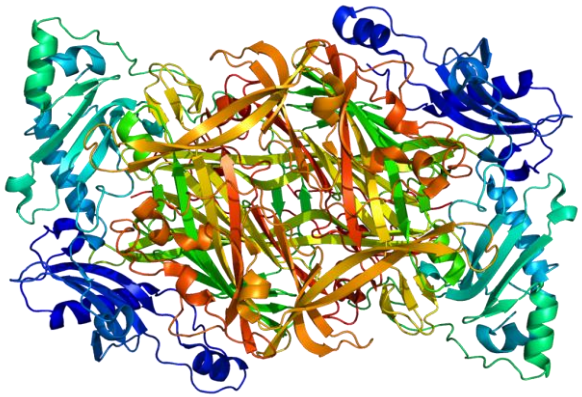
Study Design

- 150 patients with moderate to severe steatosis
- 4 doses placebo controlled
- 12 week duration
- Proof of mechanism and support of dose finding
- Safety evaluation in patients with clinical evidence of NASH
- Study expected to report by mid 2018

Boehringer Ingelheim

Target Product Profile

- AOC3 (SSAO) inhibitor for the treatment of NASH / liver fibrosis with anti-inflammatory and anti-oxidative stress activities
- Once a day oral dosage
- Indicated for treatment of NASH with liver fibrosis stages 2 & 3, or NAS ≥ 4



Structure of the AOC3 protein

Second indication: Phase 2a diabetic retinopathy study

Phase 2 trial initiated

Study Design

- 100 patients with moderately severe non-proliferative DR without centre involved diabetic macular oedema
- Placebo controlled
- 12 week duration
- Proof of mechanism and support of dose finding
- Safety evaluation in patients
- Study expected to report H2 2018

Target Product Profile

- AOC3 (SSAO) inhibitor for the reduction in retinal oxidative stress, hypoxia, inflammation, angiogenesis, advanced glycation end products, leading to stabilization and/or improvement of DR
- Once a day oral dosage
- Indicated for treatment of moderately severe and severe non-proliferative DR without centrally involved diabetic macular oedema

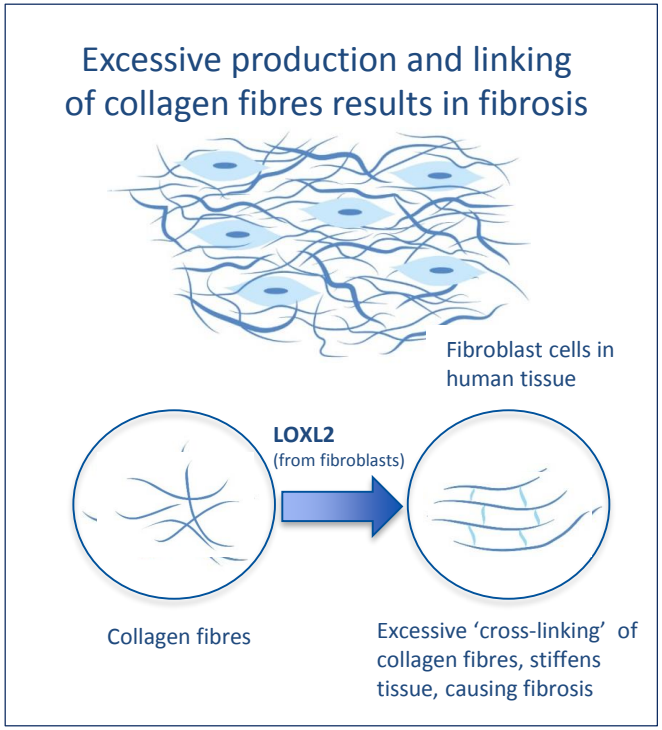
Boehringer Ingelheim

Approaching the clinic

Anti fibrotic program targeting the LOXL-2 enzyme for NASH, IPF and other fibrotic diseases

Pharmaxis LOXL2 inhibition for NASH & other fibrotic diseases

An attractive target and development program



- Potential indications:
  - NASH / Liver Fibrosis
  - Pulmonary fibrosis (IPF)
  - Kidney
  - Cardiac fibrosis

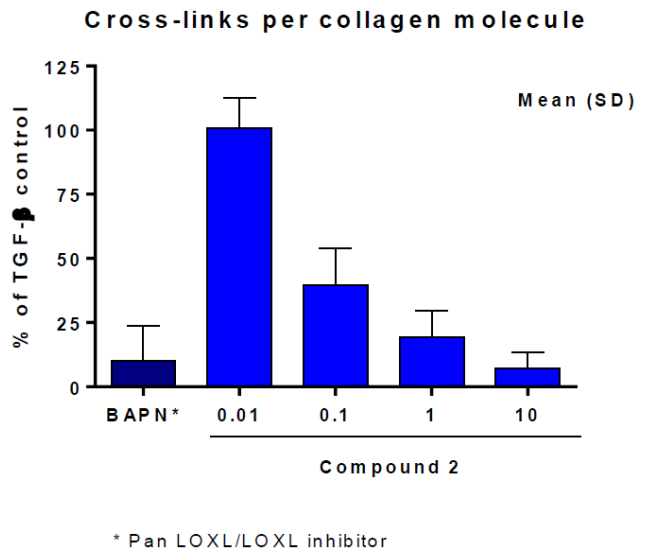
  Significant market opportunity
- Development status:
  - Pharmaxis discovery – patent filed 2016
  - Effective in pre clinical models of fibrosis and cancer
  - 2 candidate compounds completed pre-clinical trials and 28 day toxicity studies
  - Phase 1 clinical study due to commence in Q4 17
- Competitive profile:
  - Novel target and mechanism of action
  - Once daily oral drug
  - Complete inhibition of LOXL2 enzyme
  - Opportunity to use in combination with other Pharma pipeline drugs

Pharmaxis LOXL2 Synairgen collaboration

Collaboration with Synairgen

- Shares risk and reward based on investment in program
- Access to Synairgen's strength in fibrosis biology and human tissue models technology platform
- Faster time to value appreciation and partnering points of phase 1 or 2a
- Risk share - Synairgen funding pre-clinical tox and phase 1 of first compound
- Revenue share for IPF phase 1 partnering deal: 50/50
- Partnering deal(s) from additional indications (eg NASH) results in larger PXS deal share

Treatment of in vitro cultures using cells from IPF patients reduces collagen cross -linking



* Pan LOXL/LOXL inhibitor

In Vitro human IPF tissue data supports mechanism of action

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 Aqusn*	*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

LOXL2 inhibitor deal value drivers

Feature	What do Pharma value?	Pharmaxis LOXL2 program status
Disease target	Independent validation	Multiple references including Pharma company authored.
Pre clinical proof of concept	2 or more different animal models	9 different models across 5 different diseases.
Drug like qualities	No development flags	Cleared to develop
Dosing regimen	Ease of use	Oral once a day tablet or capsule
Patent	• Uncomplicated • Composition of matter • As long as possible	• 100% Pharmaxis owned • Composition of matter • 2016 filing date
Cost of Goods	Low	Small molecule with easy synthesis
# Compounds	1 plus backups	2 lead candidates plus back ups
Toxicity	Wide therapeutic window As long as possible	Phase 1 trials will inform 28 day tox studies complete
Clinical phase	Phase 1 or 2	Planned for phase 1 in H2 17

LOXL2 program is expected to be partnered at the end of phase 1 - estimated H2 2018

Drug development & other research initiatives

Pharmaxis product portfolio – new opportunities

New compounds expected to enter the clinic in 2018

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
Discovery								
SSAO/MPO	Respiratory & cardiovascular	Dual inhibitor with potential anti-inflammatory applications. Targeting Phase 1 trial in 2018						
LOX (topical)	Scarring	Commencing formal pre-clinical toxicology studies H2 of 2017. Target Phase 1 trial in 2018						
LOX	Cancer	Academic centres of excellence assessing the utility of LOX in cancer						

Development strategy

- Identify indications from in-house development programs that have high potential if developed to phase 2a / 2b
- Evaluate external opportunities for in-license or acquisition:
  - Phase 1 ready
  - Inflammation or fibrosis
  - Small molecule or biologic

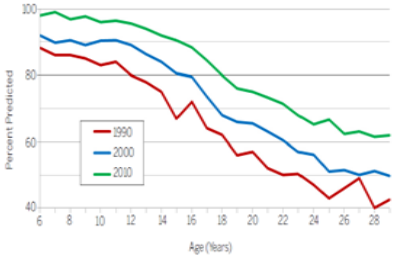
Pharmaxis purpose built facility

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

- Manufacturing and research facilities
- Productive R&D drug discovery engine
- Team of 15 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



Median FEV_1 % Predicted versus Age

Chiesi
People and ideas for innovation in healthcare

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

Business model - RoW

- Global Bronchitol distributors responsible for promotion & support
  - Chiesi in UK, Germany and Italy
  - Other distributors in Russia, Eastern Europe, Middle East
- PXS revenue share ~50%+

Business model - US

- Phase 3 trial (CF303) reported June 2017
- Chiesi responsible for regulatory filing & commercialisation
- File updated NDA - 2018
- ~A$13m milestone payment on launch, plus sales milestones
- PXS supplies US market from Sydney factory
- PXS receives high mid teens % of in-market sales plus cost of goods

Summary

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- Pharmaxis have built a **successful platform of small molecule drugs targeting fibrosis and inflammation** across various stages of development and approval
- Proven track record of **early stage partnering** and taking products **through to commercialisation** – delivered two products to market
- **Potential to receive total up front and milestone payments of A$625m** plus further sales based payments from first deal – A$68m already received
- **Strong balance sheet - $22m** at June 2017 plus **$27m** milestone received Q3 2017and **A$15m** milestone expected H2 2017
- **Discovery pipeline targeting high value indications** - one drug in 2 phase 2 trials, one drug program to start phase 1 in 2017, three compounds in development
- **Numerous catalysts over the next 18 months**

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