pharmaxis

innovating for life

Investor Presentation

7th August 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 overview

our path to value

Strategy

- Build a regional biotech powerhouse in fibrosis and inflammation
  - Multiple drugs from amine oxidase platform
  - Develop to phase 1 or 2
- Create value via partnering
  - 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

Opportunities

- Milestone payments from Boehringer as PXS4728A progresses in NASH
- Synairgen LOXL2 collaboration in pulmonary fibrosis to phase 1 or 2 and subsequent partnering
- 3 additional drug programs in drug discovery pipeline
- A stake in US commercialisation of Bronchitol (funded by partner) and sales by distributors in rest of world
- Resources for collaborating on selected in-licensing

Achievements

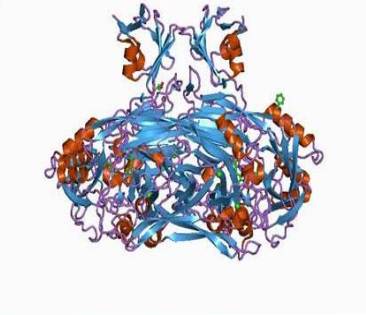
- First in class NASH drug taken to phase 1
- In house BD expertise lands deal - A$39m upfront, total up to A$750m
- Restructured Bronchitol business to reduce investment (>50%) and shorten time to profitability
- Attracted collaborators into early stage fibrosis program to widen spread of indications, enhance time to value inflection and spread risk

Pharmaxis today

building a regional biotech powerhouse in fibrosis and inflammation

Manufacturer

- ❑ Supplies Bronchitol to global markets via experienced commercial partners
- ❑ Financial risks minimised/shared
- ❑ Financial upside from accessing new markets
- ❑ Possibility to further rationalise manufacturing infrastructure



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

BD expertise

- ❑ Experienced management team and board
- ❑ Extensive Pharma industry network
- ❑ Proven capability of executing global transactions with major partners
- ❑ BI deal energises ongoing pharma interest in platform programs

Financial strength

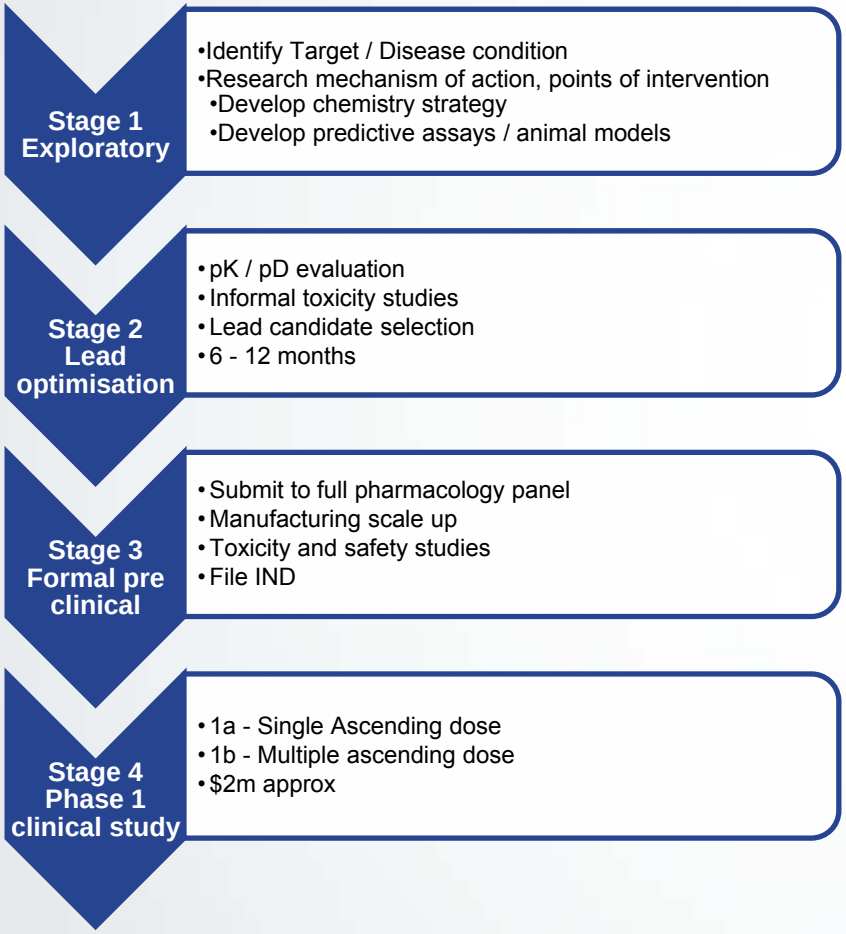
- ❑ $54m cash balance at June 2015 and reduced cash burn
- ❑ Significant value milestones from existing partner deals within reach
- ❑ Cash strengthens negotiating position in future licensing activities

Pharmaxis product portfolio

Product	Indication	Status	Partner
Aridol	Asthma diagnosis	Marketed: Australia, EU, Korea	Various
Bronchitol US	Cystic Fibrosis	Phase 3 study underway	Chiesi
Bronchitol EU	Cystic Fibrosis	Marketed	Chiesi
Bronchitol rest of world	Cystic Fibrosis	Marketed: Australia, CEE Approval pending; Brazil, Russia	Various
ASM8	Asthma	Phase 2	-
Orbital	Dry powder inhalation device	Phase 1	-
SSAO inhibitor	NASH	Phase 1	Boehringer
SSAO/MAOB inhibitor	Neuro inflammation; Alzheimer's, MS, etc.	Lead candidate selected	-
SSAO/MPO inhibitor	Respiratory inflammation; Asthma, COPD	Lead optimisation	-
LOXL2 inhibitor	NASH, Liver & kidney fibrosis	Lead optimisation	-
LOXL2 inhibitor (IPF)	Idiopathic pulmonary fibrosis	Lead optimisation	Synairgen
LOX/LOXL2 inhibitor	Fibrosis, cancer	Exploratory	
LOX inhibitor	Cancer, scarring	Exploratory	

Drug discovery strategy

Exploiting the amine oxidase chemistry platform



Drug discovery objective

at least one new drug candidate to complete formal pre clinical testing and be phase 1 ready each year

Projects	# of projects	$m / project
Exploratory	2 - 4	0.2
Lead optimisation	2 - 3	1.5
Formal pre-clinical	1	1.5

Indicative costs per project (including FTE & laboratory costs)

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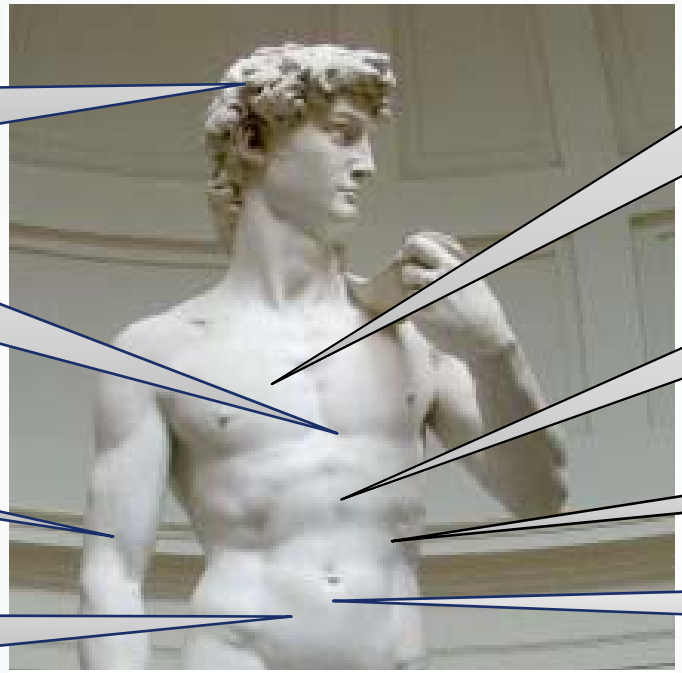
Our therapeutic focus

the inhibition of amine oxidase based enzymes has broad potential applications



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

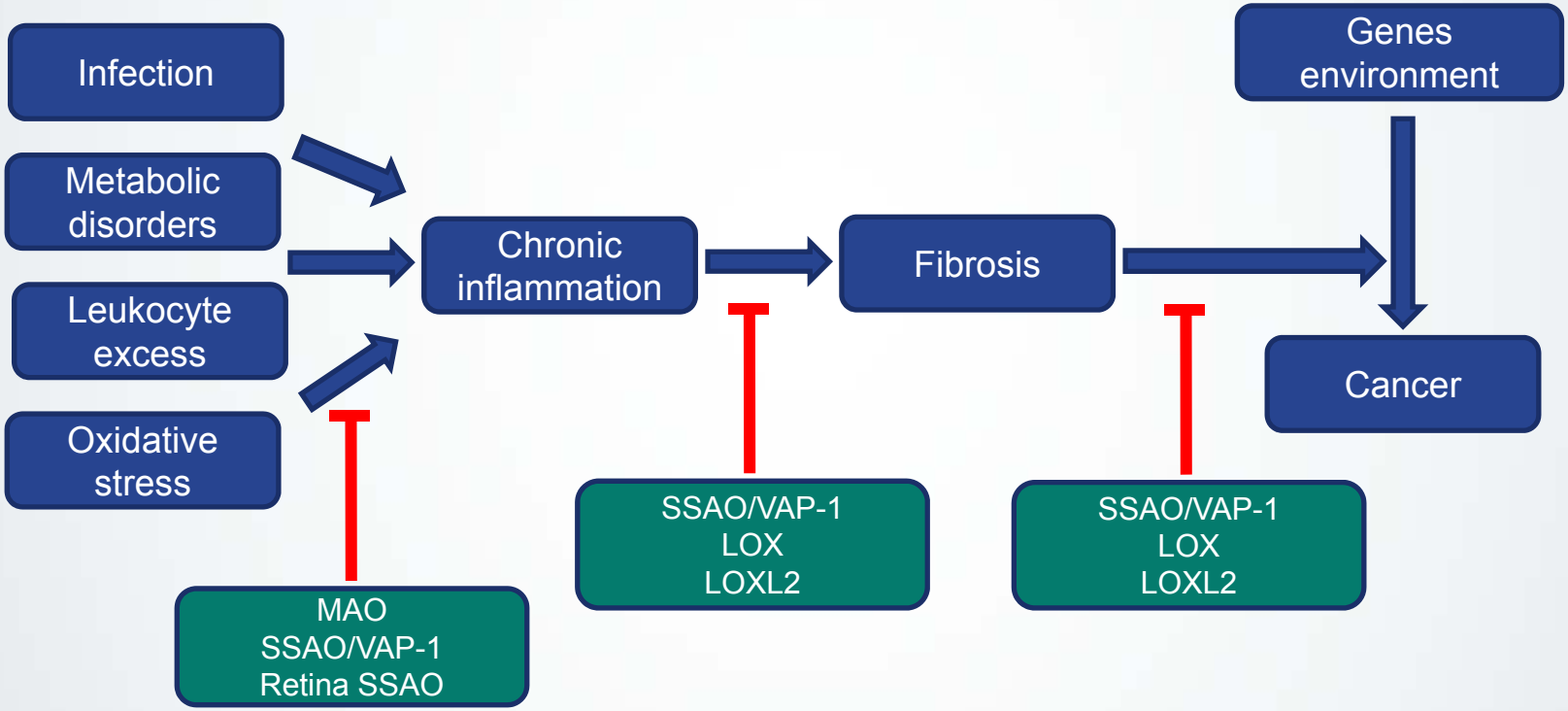
Kidney fibrosis

Gastric cancer
IBD

there is a strong **positive** correlation between increases in amine oxidase activity and these diseases.

Biology of amine oxidase based enzymes

amine oxidase based enzymes facilitate inflammatory and fibrotic processes



inhibition of these enzymes give multiple potential pathways to treat several important diseases

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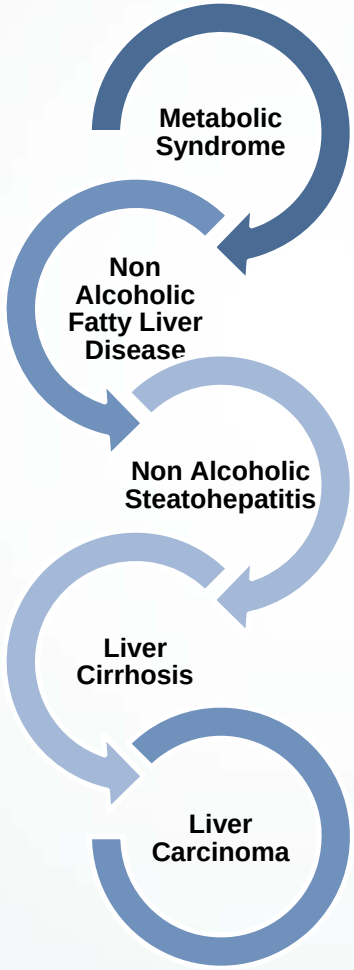
SSAO inhibition and NASH

a novel therapeutic target

Increasing levels of SSAO

- Modulates leucocyte migration
- Local generation of Reactive Oxygen Species
- Promotes inflammation
- Promotes fibrosis



- Primary indication: **NASH**
  - ~US$3.5b market by 2025
  - Estimated 6 million patients in US
- Development status:
  - Pharmaxis discovery – patent filed 2014
  - Effective in pre clinical models of NASH and airway inflammation
  - Completed single ascending dose stage of phase 1
    - orally bioavailable
    - long lasting inhibition after single dose
    - progressive dose response
- PXS total investment to phase 1:
  - ~A$9m
- Competitors:
  - Genefit – GF505 in Phase 2b NASH
  - Intercept - OCA (FXR agonist) in Phase 2b NASH
  - Gilead – FXR agonist in pre clinical

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Boehringer Ingelheim

acquisition of PXS4728A

Acquisition (May 2015).
- €27.5m (~A$39m)

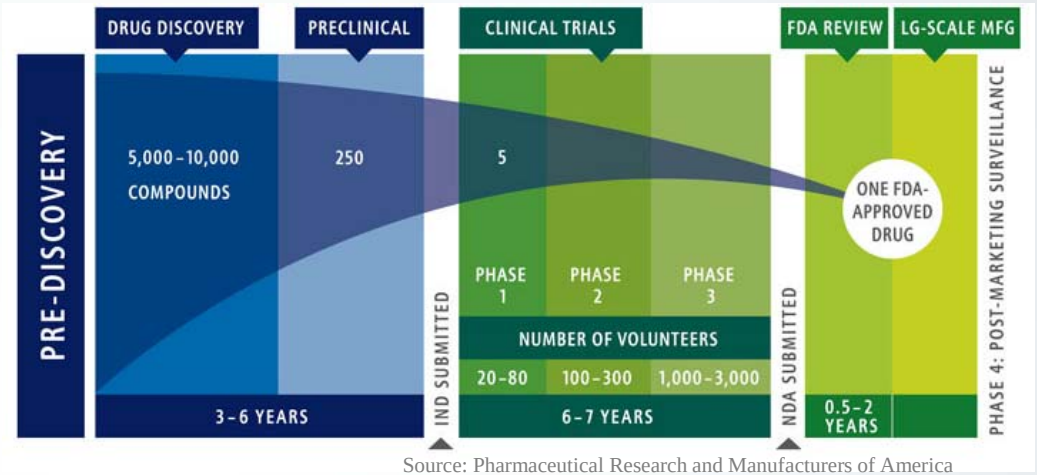
Commencement of phase 2 and 3
- up to total €55m (~A$80m)

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

Average drug development times



Source: Pharmaceutical Research and Manufacturers of America

- ❑ Excellent partner
  - ❑ Boehringer leaders in metabolic disease
  - ❑ Industry leading development times
  - ❑ Boehringer responsible for all development, and commercialisation activities
- ❑ Competitive deal
  - ❑ Demonstrates PXS ability to negotiate valuable global deals
  - ❑ Total potential payments to approval for 2 indications: €418.5m (~A$600m),
  - ❑ Plus potential sales milestones, and potential earn-out at high single digit % of sales
- ❑ External validation of PXS drug discovery

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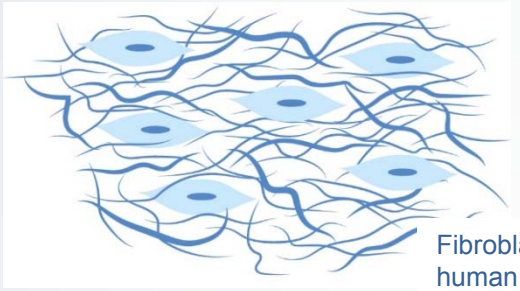
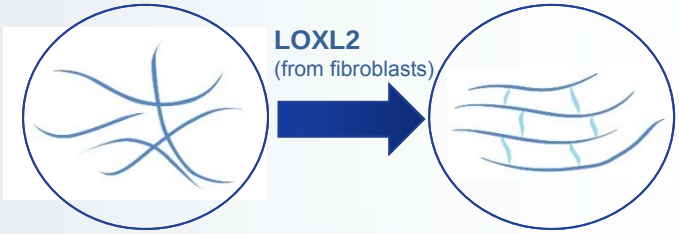
LOXL2 inhibition

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

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

❑ Potential indications:

- ❑ NASH / Liver Fibrosis
- ❑ Pulmonary fibrosis
- ❑ 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

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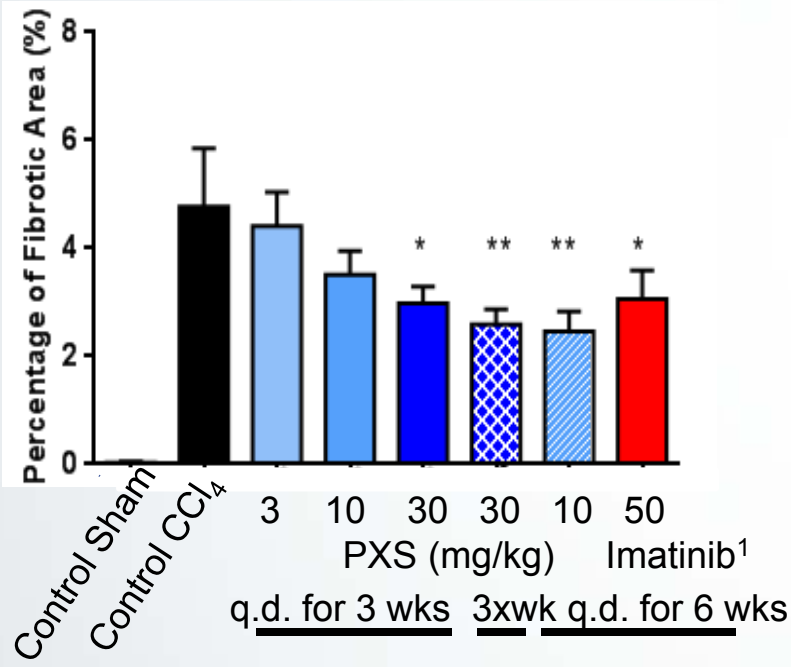
Drug development program – LOX / LOXL2

LOX / LOXL2 inhibitors for multiple indications

PXS reduces fibrosis in CCl4 liver disease model after disease established



[1] Positive control imatinib was dosed for 6 weeks, commencing ***before*** fibrosis established

LOX analogues (LOX, LOX/LOXL2)

Pharmaxis' platform enables the synthesis of inhibitors with different pharmacological and pharmacokinetic profiles

- Selective LOXL2 inhibitor: NASH, liver and kidney fibrosis
- Selective LOXL2 inhibitor: IPF
- Mixed LOX/LOXL2 inhibitor: cancer; severe liver and kidney fibrosis
- Selective LOX inhibitor: myelofibrosis, scarring
- Status:
  - NASH/ liver fibrosis: Lead optimisation
  - IPF: Lead optimisation with Synairgen
  - Cancer & scarring: Exploratory

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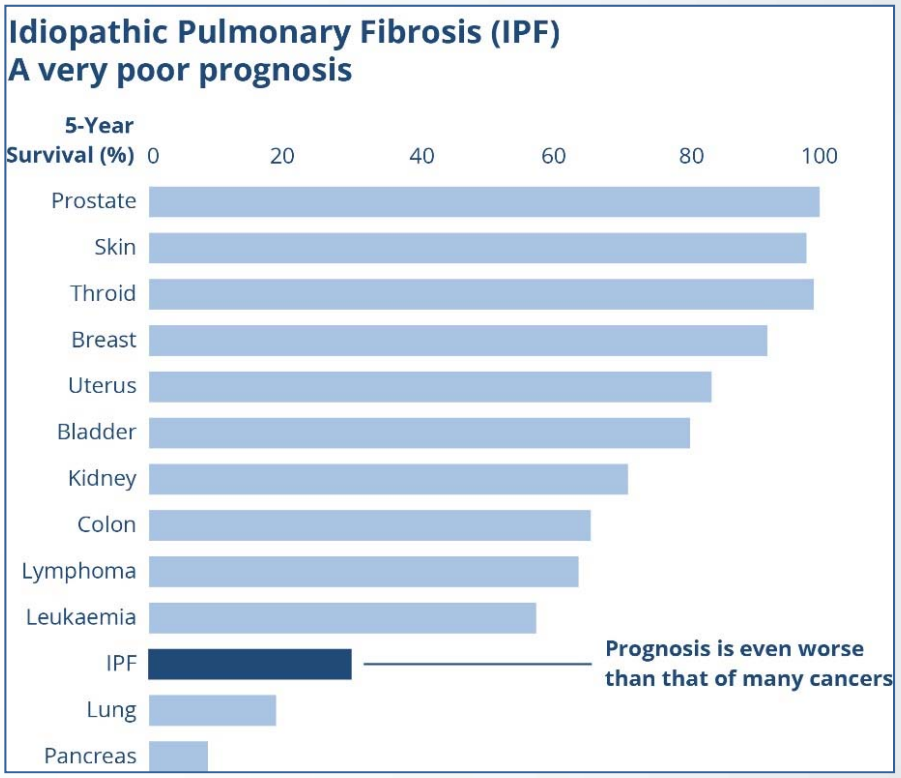
Idiopathic Pulmonary Fibrosis (IPF)

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



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Synairgen IPF collaboration

"Our collaboration with Synairgen will accelerate the development of a highly competitive once a day oral treatment for patients with IPF whilst enabling Pharmaxis to develop LOXL2 inhibitors for other valuable indications such as liver and kidney fibrosis, and cancer." - Gary Phillips PXS CEO

Synairgen overview

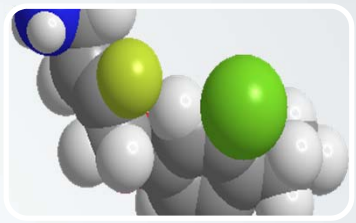
- Respiratory drug discovery and development company focussed on developing novel therapies
- Builds on expertise at University of Southampton
  - Professors Stephen Holgate, Donna Davies and Ratko Djukanovic
- Strategy to
  - develop assets via BioBank human tissue models technology platform;
  - out-license to global partners
- Inhaled IFN-β licensing deal signed with AstraZeneca in June 2014 (AZD9412)
- Well funded and quoted on AIM (LSE: SNG)

Collaboration objectives

- Synairgen has strength in fibrosis biology and respiratory clinical development
- Pharmaxis has expertise in developing small molecule LOXL2 inhibitors
- Faster time to value appreciation points of phase 1 or 2a
- Synairgen to fund pre clinical tox and phase 1
- Shares risk
- Share reward based on investment in program
- Allows pursuit of further indications in parallel

Drug development program - SSAO

SSAO inhibitor program yields two additional distinct opportunities



SSAO/MAOB – neuro inflammation

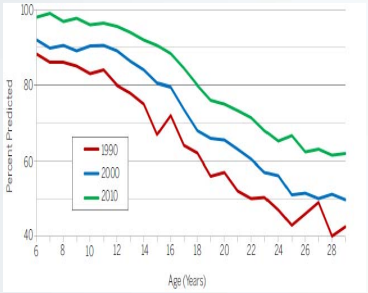
- SSAO/VAP-1 and MAOB enzymes are involved in Alzheimer's Disease, multiple sclerosis and cardiometabolic diseases
- PXS dual SSAO/MAOB inhibitor diminishes neuro inflammation in pre clinical models
- Status:
  - lead compound identified
  - screening to establish lead indication
  - formal pre-clinical Q1 2016

SSAO/MPO – respiratory

- SSAO/VAP-1 is upregulated in patients with respiratory diseases such as CF and COPD
- PXS SSAO inhibitors are effective in pre clinical models
- Potential to enhance efficacy through enhanced chemistry to target additional myloperoxidase (MPO) pathway
- Status:
  - lead optimisation

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

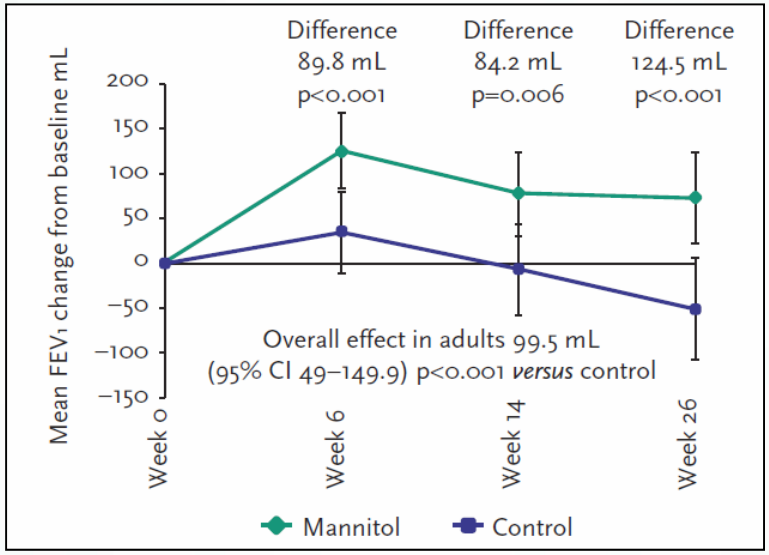
Refer to Pharmaxis website for more information

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 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 Dec 15
- ❑ Results Q3 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

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Financials – income statement

30 June 2015 (unaudited)

A$'000	2015	2014
Revenue		
Sales revenue		
Bronchitol	4,243	3,275
Aridol	1,715	1,752
Other products	41	9
	5,999	5,036
Other revenue	721	1,735
Other income	53,527	3,715
	60,247	10,486
Expenses		
Employee costs	14,111	19,376
Administration & corporate	3,316	3,379
Rent, occupancy & utilities	1,593	1,767
Clinical trials	11,315	6,221
Drug development	1,695	1,256
Sales, marketing & distribution	1,962	3,376
Safety, medical and regulatory affairs	1,723	1,852
Manufacturing purchases	1,736	2,142
Other	2,300	1,772
Depreciation & amortisation	3,406	5,131
Finance expenses	(2,696)	7,146
Impairment expenses	277	8,783
	40,739	62,201
Net profit (loss) before tax	19,508	(51,715)
Income tax expense	(42)	(103)
Net profit(loss) after tax	**19,466**	**(51,818)**

Highlights of 2015:

- Boehringer Ingelheim acquires PXS4728A for $41 million including $1.8 million option fee – derisked funding of phase 1 trial
- Bronchitol sales growth in challenging environment
- Aridol sales maintained without any sales/marketing investment
- Significant cost reductions from changes to business
  - Chiesi funds clinical trial (CF303) for US approval including reimbursement of 2015 costs ($7.5m) and 2014 costs ($4.7m)
  - Employee costs; sales, marketing and distribution; occupancy costs subsequent to closure of US office (Sept 14) and EU commercial infrastructure (May 15) and other initiatives
  - FY 2015 only includes one month of completed EU cost reductions
- Clinical trials expense also includes phase 1 for PXS4728A (FY 2015: $1.8m) and EU paediatric trial CF2024 (FY 2015: $1.9m) – completes FY 2016
- Other expenses includes a realised FX gain (FY 2015: $1.1m)
- Finance expense for FY 2015 of ($2.7m) relates to a restatement of the NovaQuest financing agreement

- For additional commentary refer to Quarterly Shareholder Update June 2014 – available on the Pharmaxis website
- For reconciliation of adjusted to EBITDA to net profit(loss) before tax – refer final slide

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Normalised cash loss

(unaudited)

A$'000	Total
Segment EBITDA FY 2015	
Bronchitol & Aridol[1]	(9,045)
Drug discovery	35,068
Corporate	(3,532)
Total	22,491
Interest income	721
	23,212
Items not recurring each year	
Chiesi reimbursement of prior year clinical trial costs	(4,482)
Boehringer Ingelheim acquisition of PXS4728A	(40,603)
FX gain	(1,100)
	(22,973)
Full year impact of changes to business made in FY 2015	6,500
Impact of not being eligible for R&D tax incentive in 2015[2]	4,385
	(12,088)
Note	
1. Includes clinical trial CF204 cost of $1.8m which completes in FY 2016	
2. In 2015 PXS not eligible for R&D tax incentive due to revenue exceeding $20m	

Financial - other

Financial notes

- Cash at 30 June 2015
  - $54 million
- Non current liabilities at 30 June 2015
  - Borrowings – $10 million. Capitalised finance lease of Frenchs Forest facility.
  - Other:
    - $1.9 million deferred lease incentive
    - $24.8 million financing agreement with NovaQuest – only receive payments based on the US and EU sales of Bronchitol over the term of the agreement.
- Bronchitol economics
  - EU / ROW: 50%+/- 10% of net selling price
  - US: $25m in total milestones payable to PXS on launch and on achievement of sales milestones; cost plus margin on COGS (mid-teens) plus share of net sales (mid to high teens)
  - NovaQuest 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
  - Royalties to RPA ~3.0%

Shareholders

- Shares on issue: 317m (4 Aug 2015)
- Employee options: 6.3m (4 Aug 2015)
- Institutional shareholders: >45%
- Major shareholders
  - Orbis/ Alan Gray: 18%
  - BVF Partners: 12%
  - Montoya Investments: 6%

ASX listed company (code: PXS)

Board and management

experience that counts

Board

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

Management

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

Broad network and experience in capital markets

Biotech and Big Pharma commercial experience

Extensive business development networks

Experience of wide variety of partnering transactions

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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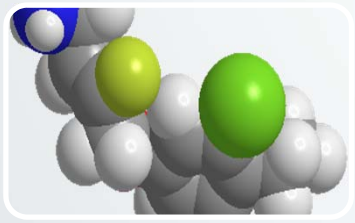
Major upcoming milestones

near term valuable milestones

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

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Financials – segment reconciliation

30 June 2015

A$'000	2015	2014
Segment EBITDA		
Bronchitol & Aridol	(9,045)	(22,555)
Drug discovery	35,068	(1,620)
Corporate	(3,532)	(6,226)
Total	**22,491**	**(30,401)**
Reconciling items:		
Interest revenue	721	1,735
Finance costs	2,696	(7,146)
Depreciation and amortisation expense	(3,406)	(5,131)
Impairment of patents and other assets	(277)	(8,783)
Redundancy costs	(544)	-
Non-recurring legal expenses	(1,032)	(177)
Unrealised gains/(losses) on NovaQuest	(1,493)	108
Share-based payment expenses	353	(1,920)
Net profit(loss) before income tax	**19,509**	**(51,715)**