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innovating for life

Investor Presentation

19th May 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.

- ASX listed company
- Code: PXS
- Location: Sydney Australia
- Shares on issue: 314m
- Pro-forma cash at 31 March 15: $62m

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
  - Collaborate to de-risk and accelerate PXS programs
  - Collaborate on in-licensing programs
  - Licence out to Big Pharma with attractive 1st in class drugs post phase 1 or 2

Opportunities

- Milestone payments from Boehringer as PXS4728A progresses
- LOXL2 collaboration 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 RoW
- Resources for collaborating on selected in-licensing
- Further cost reduction

Achievements

- First in class NASH drug taken to phase 1
- In house BD expertise lands A$750m deal with A$39m upfront.
- Restructured Bronchitol business to reduce investment (>50%) and shorten time to profitability

Board and management

experience that counts

Board:

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

- Broad network and experience in capital markets
- Biotech and Big Pharma commercial experience
- Extensive business development networks
- Experience of wide variety of partnering transactions

Management:

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

- 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

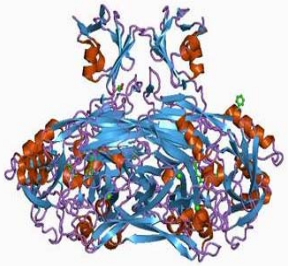
Refer to Pharmaxis website for further detail

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

Financial strength

- ❑ Healthy cash balance and reduced cash burn – long runway
- ❑ Significant value points within reach
- ❑ Cash strengthens negotiating position in future licensing activities

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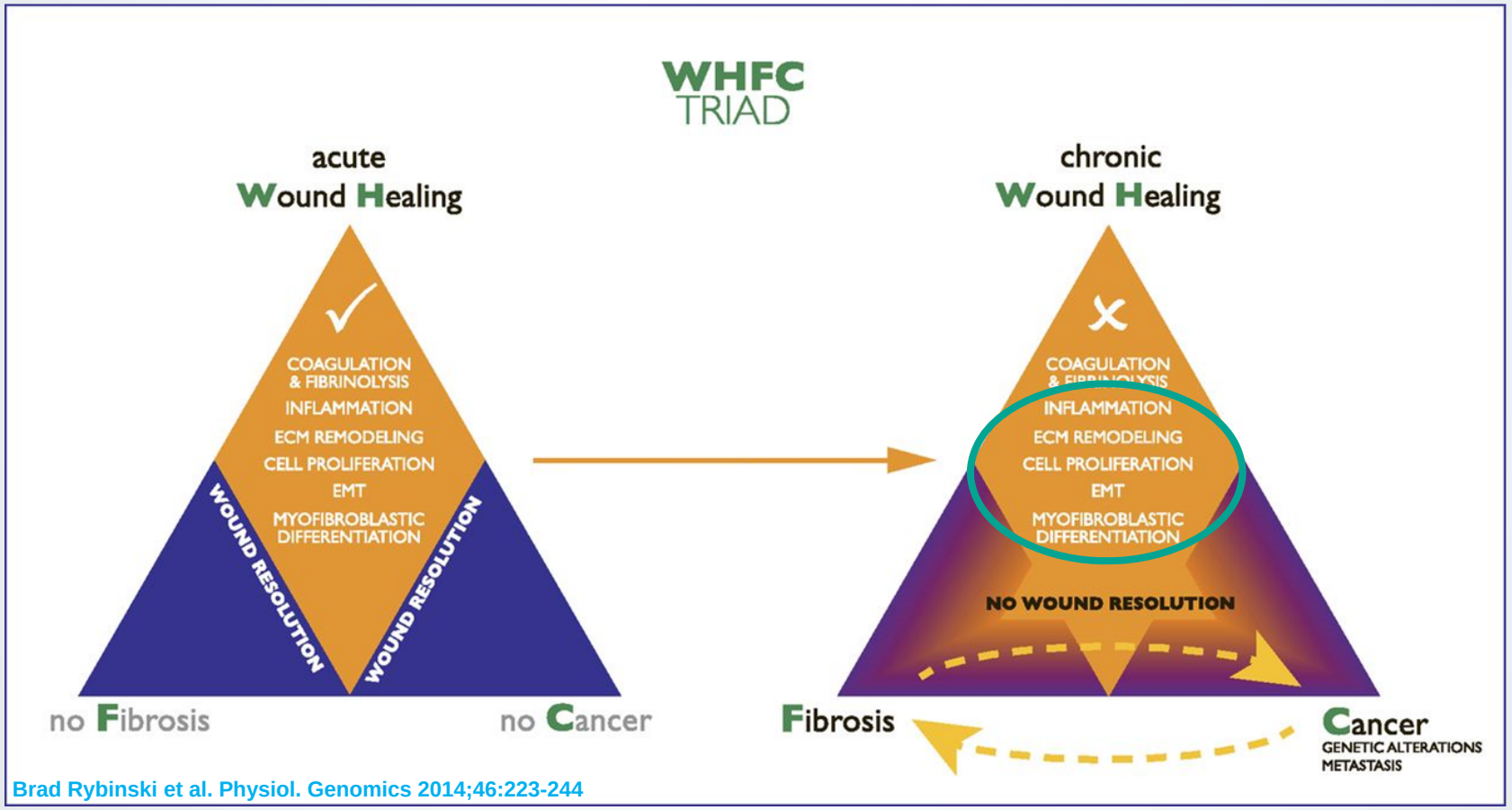
Our area of expertise



Brad Rybinski et al. Physiol. Genomics 2014;46:223-244

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Discovery capability

the engine of near term and multiple future value

- State of the art facilities opened 2009
- PC2 level
- Experienced Staff
  - Head of Drug Discovery; PhD, >12 years experience in large pharma (MSD, GSK)
  - Chemistry; Team of 4 PhD & 2 associates, >15 years experience in amine oxidase chemistry
  - Biology; Team of 4 PhD, > 20 years experience in assay development and compound screening, experts in inflammation and fibrosis biology
  - Clinical; Medical Director (>20 years experience), Senior clinical trial manager, Clinical trial administrator

Dr Wolfgang Jarolimek – head of drug discovery

Dr Brett Charlton – medical director

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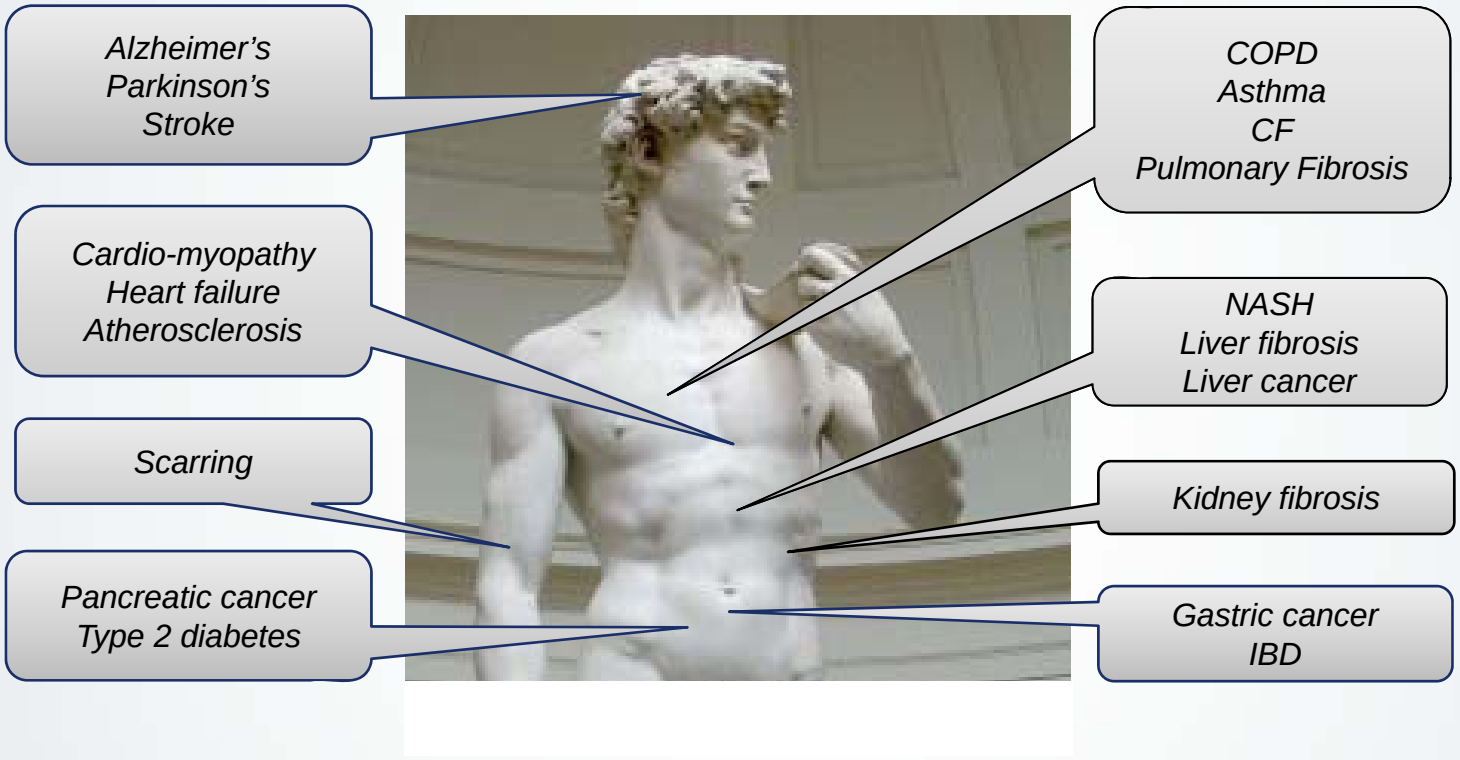
Our therapeutic focus

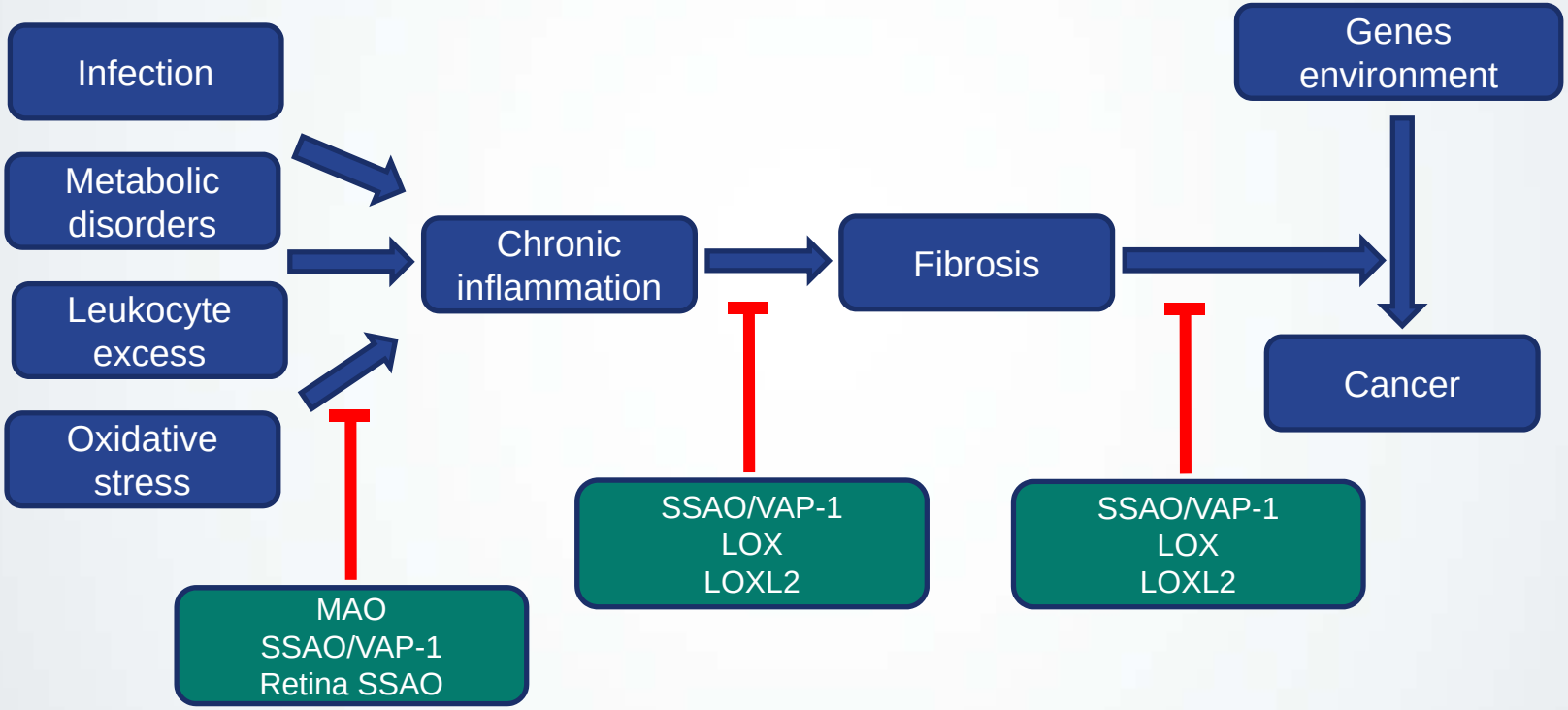
the inhibition of amine oxidase based enzymes has broad potential applications



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

Biology of amine oxidase platform

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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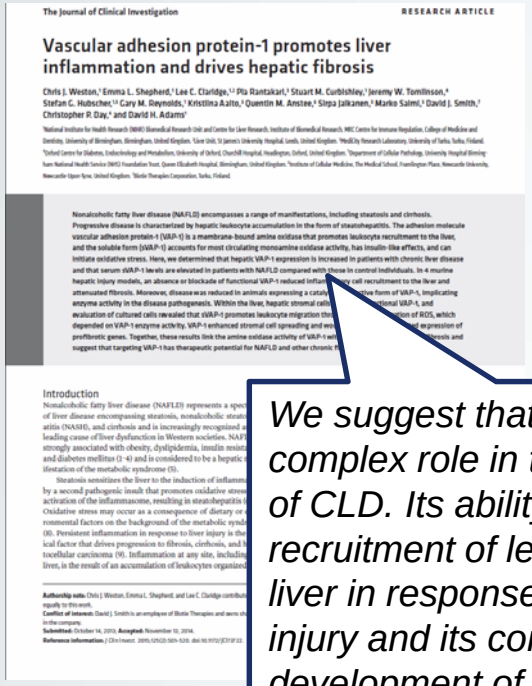


The Journal of Clinical Investigation — RESEARCH ARTICLE

Vascular adhesion protein-1 promotes liver inflammation and drives hepatic fibrosis

Chris J. Weston,[1] Emma L. Shepherd,[1] Lee C. Claridge,[1,2] Pia Rantakari,[3] Stuart M. Curbishley,[1] Jeremy W. Tomlinson,[4] Stefan G. Hubscher,[1,2] Gary M. Reynolds,[1] Kristiina Aalto,[3] Quentin M. Anstee,[4] Sirpa Jalkanen,[3] Marko Salmi,[3] David J. Smith,[5] Christopher P. Day,[4] and David H. Adams[1]

Nonalcoholic fatty liver disease (NAFLD) encompasses a range of manifestations, including steatosis and cirrhosis. Progressive disease is characterized by hepatic leukocyte accumulation in the form of steatohepatitis. The adhesion molecule vascular adhesion protein-1 (VAP-1) is a membrane-bound amine oxidase that promotes leukocyte recruitment to the liver, and the soluble form (sVAP-1) accounts for most circulating monoamine oxidase activity, has insulin-like effects, and can initiate oxidative stress. Here, we determined that hepatic VAP-1 expression is increased in patients with chronic liver disease and that serum sVAP-1 levels are elevated in patients with NAFLD compared with those in control individuals. In 4 murine hepatic injury models, an absence or blockade of functional VAP-1 reduced inflammatory cell recruitment to the liver and attenuated fibrosis. Moreover, disease was reduced in animals expressing a catalytically inactive form of VAP-1, implicating enzyme activity in the disease pathogenesis. Within the liver, hepatic stromal cells expressed abundant VAP-1, and evaluation of cultured cells revealed that sVAP-1 promotes leukocyte migration through the production of ROS, which depended on VAP-1 enzyme activity. VAP-1 enhanced stromal cell spreading and wound closure and induced expression of profibrotic genes. Together, these results link the amine oxidase activity of VAP-1 with inflammation and fibrosis and suggest that targeting VAP-1 has therapeutic potential for NAFLD and other chronic liver diseases.

Introduction

We suggest that VAP-1 plays a complex role in the pathogenesis of CLD. Its ability to promote the recruitment of leukocytes to the liver in response to initial liver injury and its contribution to the development of fibrosis suggest that targeting VAP-1 therapeutically, through Ab blockade or enzyme inhibitors, could prevent the progression of liver disease.

J Clin Invest. 2015;125(2):501–520. doi:10.1172/JCI73722

SSAO inhibition

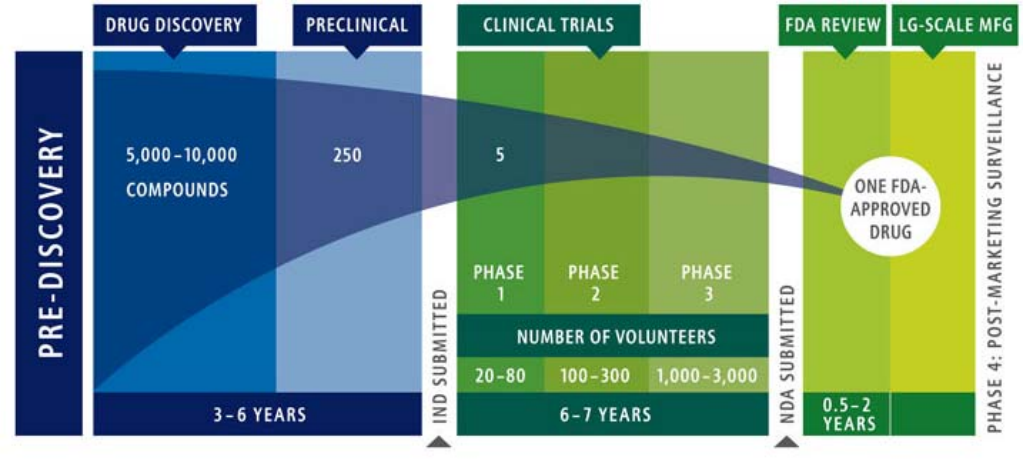
PXS4728A

- Primary indication: **NASH**
  - (~US$3.5b market by 2025)
- Other indication: COPD
  - (~US$12b current market)
- Development status:
  - 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 investment:
  - ~A$9m, R&D tax credit of ~A$2m
- Competitors:
  - Genefit – GF505 in Phase 2b NASH
  - Intercept - OCA (FXR agonist) in Phase 2b NASH
  - Gilead – FXR agonist in pre clinical

Boehringer Ingelheim

acquisition of PXS4728A

Average Drug Development Times



Source: Pharmaceutical Research and Manufacturers of America

- €27.5m (~A$39m) on acquisition of program in May 2015. Future payments for successful development and commercialisation:
  - up to €55m (~A$80m) on commencement of phase 2 and 3 clinical trials
  - up to €140m (~A$200m) on filing of applications for marketing approval and receipt of regulatory and pricing approvals
  - similar additional milestone payments for a second indication
  - earn-out payments on annual net sales at tiered percentages starting in the high single digits
  - commercialisation sales milestone payments
- Total potential payments to approval for 2 indications: €418.5m (~A$600m), plus potential sales milestones, plus potential earn-out at high single digit % of sales
- Boehringer responsible for all development, regulatory, manufacturing and commercialisation activities
- External validation of PXS drug discovery
- Demonstrates PXS ability to negotiate valuable global deals

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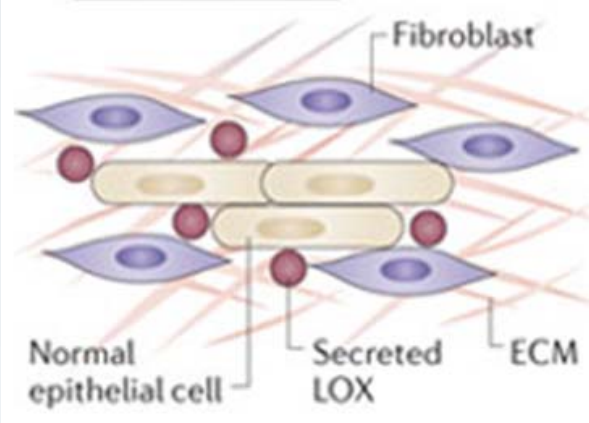
LOXL2 inhibition

an attractive target and development program



- LOXL2 is one of the Lysyl oxidase enzymes
- Lysyl oxidases cross-link collagen and elastin
- Excessive cross-linkage of collagen results in fibrosis

Gilead – LOXL2 antibody

- Acquired Arresto program $225m pre P1
- Now in broad phase 2b trial program
- Liver fibrosis; Idiopathic pulmonary fibrosis; Metastatic pancreatic cancer; Myelofibrosis; Solid tumours; Metastatic colorectal cancer

❑ Potential indications:

- ❑ Pulmonary fibrosis
- ❑ NASH
- ❑ Cancer
- ❑ Wound healing

❑ Development status:

- ❑ Lead compounds identified
- ❑ Effective in pre clinical models of fibrosis and cancer
- ❑ Formal toxicity studies by end 2015

❑ Collaboration objectives:

- ❑ Partner with strength in fibrosis biology and clinical
- ❑ Faster time to value appreciation points of phase 1/2a
- ❑ Partner to fund pre clinical tox and phase 1
- ❑ Shares risk
- ❑ Share reward based on investment in program
- ❑ Allows pursuit of further indications in parallel

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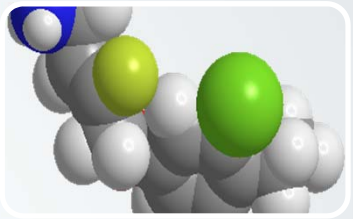
Drug development program

amine oxidase chemistry



LOX analogues

- Pharmaxis' platform enables the synthesis of inhibitors with different pharmacological and pharmacokinetic profile
- Selective LOXL2 inhibitor: lung, liver and kidney fibrosis, cancer
- Mixed LOX/LOXL2 inhibitor: cancer; severe lung and kidney fibrosis
- Selective LOX inhibitor: myelofibrosis, scarring
- Status:
  - Lead identification

SSAO – Neuro Inflammation

- SSAO/VAP-1 is also involved in Alzheimer's and Parkinson's Disease
- PXS dual SSAO/MAOB inhibitor diminishes brain inflammation in pre clinical models.
- Competition: Selective MAOB inhibitor phase 2 ready for Alzheimer's (Evotec / Roche)
- Status:
  - lead compound identified
  - formal pre-clinical program Q3 2015

SSAO – Respiratory

- SSAO/VAP-1 is upregulated in patients with respiratory disease such as CF and COPD
- PXS SSAO inhibitor is effective in pre clinical models.
- Potential to enhance efficacy through enhanced chemistry to target additional pathways.
- Status:
  - Lead identification

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
- 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

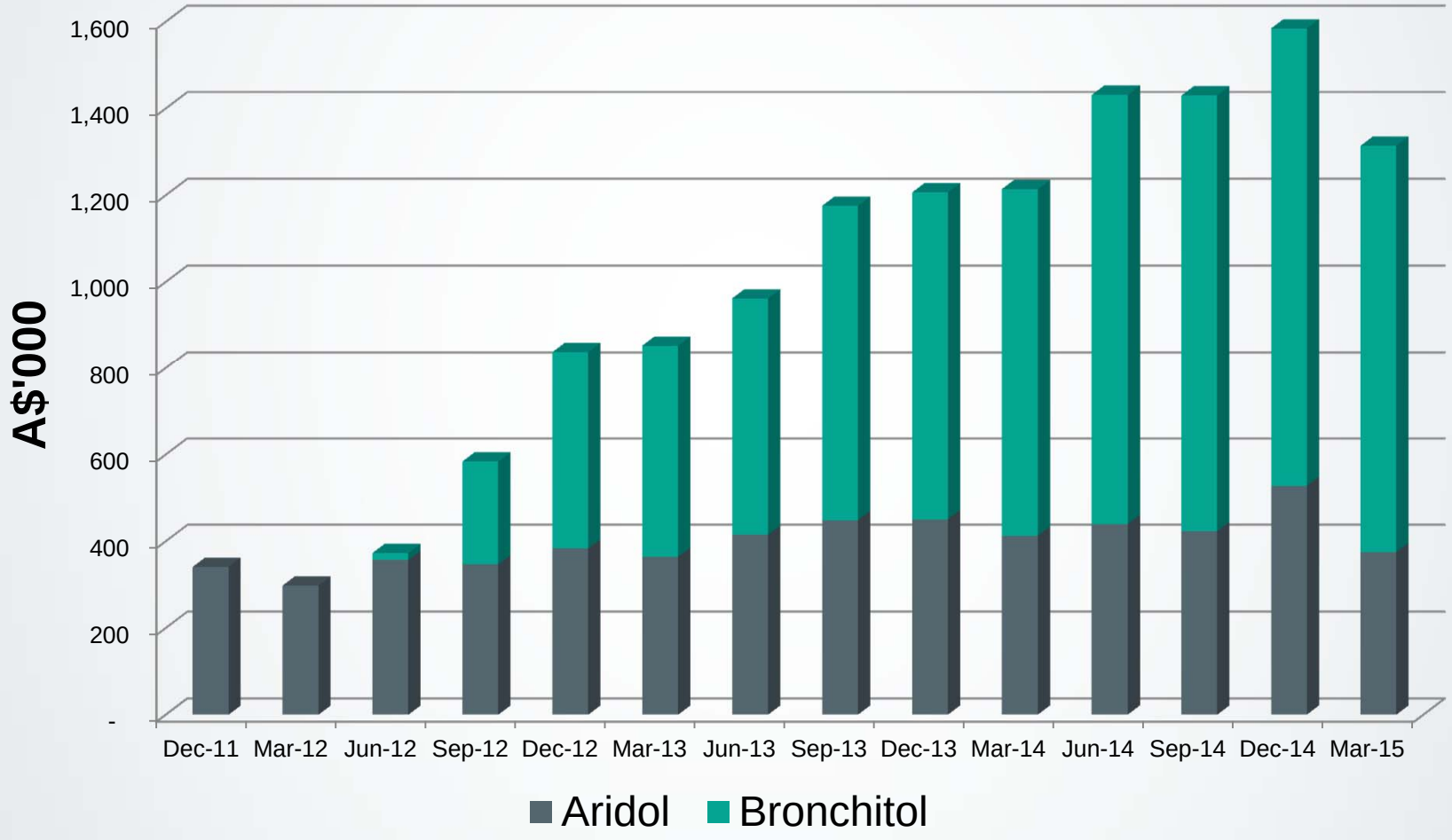
Rest of world

- 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

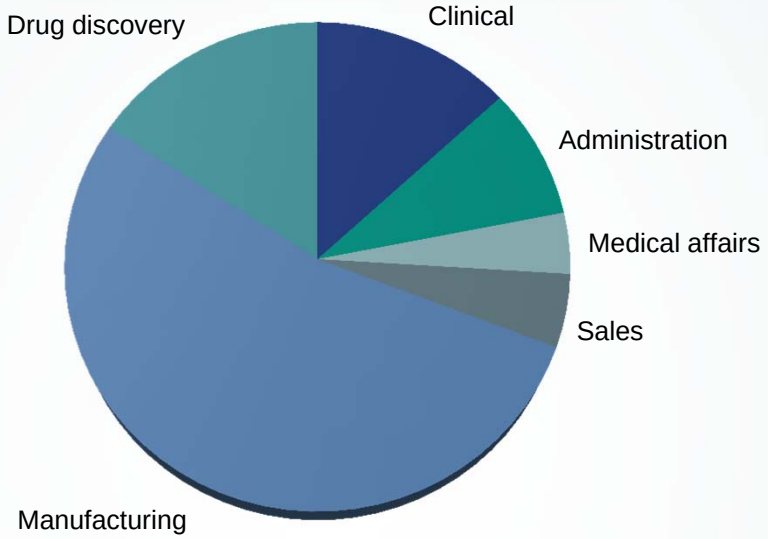
Quarterly sales

Aridol and Bronchitol



Employees

pro-forma March quarter 2015



Current number of employees (FTE's)	
Clinical	Supports CF303, CF204, drug discovery phase 1
Medical affairs	Supports Bronchitol & Aridol distributors worldwide
Sales	Australian sales and distributor liaison
Manufacturing	Commercial product, clinical trial material, support Chiesi NDA
Drug discovery	Chemistry & biology

Financials – income statement

31 March 2015

	Three months ended		Nine months ended	
A$'000	**31-Mar-15**	31-Mar-14	**31-Mar-15**	31-Mar-14
Revenue				
Revenue from sale of goods				
Bronchitol	**939**	801	**3,002**	2,284
Aridol	**372**	413	**1,323**	1,312
Other products	**3**	-	**29**	-
	1,314	1,214	**4,354**	3,596
Other revenue	**115**	385	**518**	1,394
Other income	**2,568**	622	**11,663**	2,613
	3,997	2,221	**16,535**	7,603
Expenses				
Employee costs	**(3,528)**	(4,585)	**(11,012)**	(14,833)
Administration & corporate	**(721)**	(704)	**(2,517)**	(2,440)
Rent, occupancy & utilities	**(378)**	(365)	**(1,191)**	(1,283)
Clinical trials	**(1,526)**	(974)	**(6,897)**	(2,344)
Drug development	**(284)**	(233)	**(817)**	(687)
Sales, marketing & distribution	**(287)**	(408)	**(1,613)**	(2,308)
Safety, medical and regulatory affairs	**(334)**	(423)	**(1,092)**	(1,226)
Manufacturing purchases	**(160)**	(426)	**(1,342)**	(1,441)
Other	**(1,424)**	(364)	**(2,240)**	(766)
Depreciation & amortisation	**(963)**	(1,336)	**(2,667)**	(3,808)
Finance expenses	**(241)**	(2,337)	**2,804**	(7,017)
Impairment expenses	**-**	-	**(277)**	-
Total expenses	**(9,846)**	(12,156)	**(28,861)**	(38,153)
Net Loss before tax	**(5,849)**	(9,935)	**(12,326)**	(30,550)
Income tax expense	**-**	(48)	**(95)**	(109)
Net Loss after tax	**(5,849)**	(9,983)	**(12,421)**	(30,659)

Financials – segment EBITDA

Pro-forma March quarter 2015

Segment	March 15	Adjust	Pro forma	Comment
Bronchitol EBITDA	(2,806)	1,256	(1,550)	See next slide
Drug discovery EBITDA	646	(1,376)	(730)	BI option grant fee ($1,789) and phase 1 trial of PXS4728A ($413k) - will complete by Q3 2015
Corporate/unallocated EBITDA	(2,601)	1,519	(1,082)	Unrealised FX loss on NQ investment ($1,666k), realised FX gains ($470), redundancy costs ($410k), share based payments (credit of $201k), business development costs ($114k)
Total EBITDA	**(4,761)**	**1,399**	**(3,362)**	
Interest & finance costs - net	(125)	-	(125)	
Depreciation & amortisation	(963)	**-**	(963)	
Loss before tax	**(5,849)**	**1,399**	**(4,450)**	

Financials - Bronchitol

Pro-forma March quarter 2015

Income statement	March 15	Adjust	Pro forma	Comment
Sales	**1,314**	**(310)**	**1,004**	Chiesi appointed distributor - reduced margin on UK & German sales
Other income & revenue	697	479	1,176	adjustment for Chiesi overpayment in December ($479k)
Employee costs	(2,424)	950	(1,474)	Reduction in EU staff costs
Administration & corporate	(144)	-	(144)	
Clinical trials	(1,113)	-	(1,113)	CF303 ($861k) & CF204 ($252k)
Other	(1,136)	137	(999)	EU sales & marketing costs
Total expenses	**(4,817)**	**1,087**	**(3,730)**	Includes CF303 costs reimbursed by Chiesi in other income
Segment EBITDA	**(2,806)**	**1,256**	**(1,550)**	

Financials

- Cash
  - Proforma cash – 31 March 15 (A$23m) plus Boehringer initial payment (A$39m): A$62 million
- Sales
  - From 1 June 2015 sales of Bronchitol will be in regular shipments to Chiesi and other distributors
- 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)
  - Royalties to RPA ~3.0%
- Other cost items:
  - CF204 to complete by March 2016. March 2015 external cost: $260k
  - Manufacturing cost review to leverage Chiesi relationship

Major upcoming milestones

near term valuable milestones

2015

- ❑ PXS4728A Phase 1 reports
- ❑ LOXL2 – lead candidate identified
- ❑ CF303 fully recruited

2016

- ❑ LOXL2 – lead candidate into preclinical
- ❑ LOXL2 – start phase 1
- ❑ CF303 – last patient completes trial
- ❑ CF303 – reports & file with FDA
- ❑ one Drug Discovery program reaches pre clinical valuation point

2017

- ❑ PXS4728A Phase 2 commences – milestone payment to PXS
- ❑ FDA decision on Bronchitol approval in US
- ❑ Bronchitol US launch – milestone payment to PXS
- ❑ one Drug Discovery program reaches clinical phase 1 valuation point

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