



Investor Presentation

Australia Biotech Invest
Melbourne: 6 October 2015

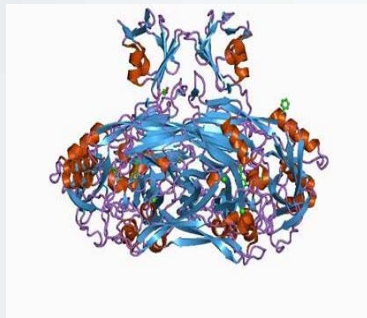
Gary Phillips CEO

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 today

new business focus already creating value



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



Management

- ❑ Management team and Board with global experience
- ❑ Extensive pharma industry network
- ❑ Proven capability of executing global BD transactions with major partners
- ❑ Preclinical, early and late phase clinical experience



Drug manufacturer

- ❑ Supplies Bronchitol to global markets via experienced commercial partners
- ❑ Financial risks shared
- ❑ Financial upside from accessing new markets – US, Russia
- ❑ Possibility to further rationalise manufacturing infrastructure

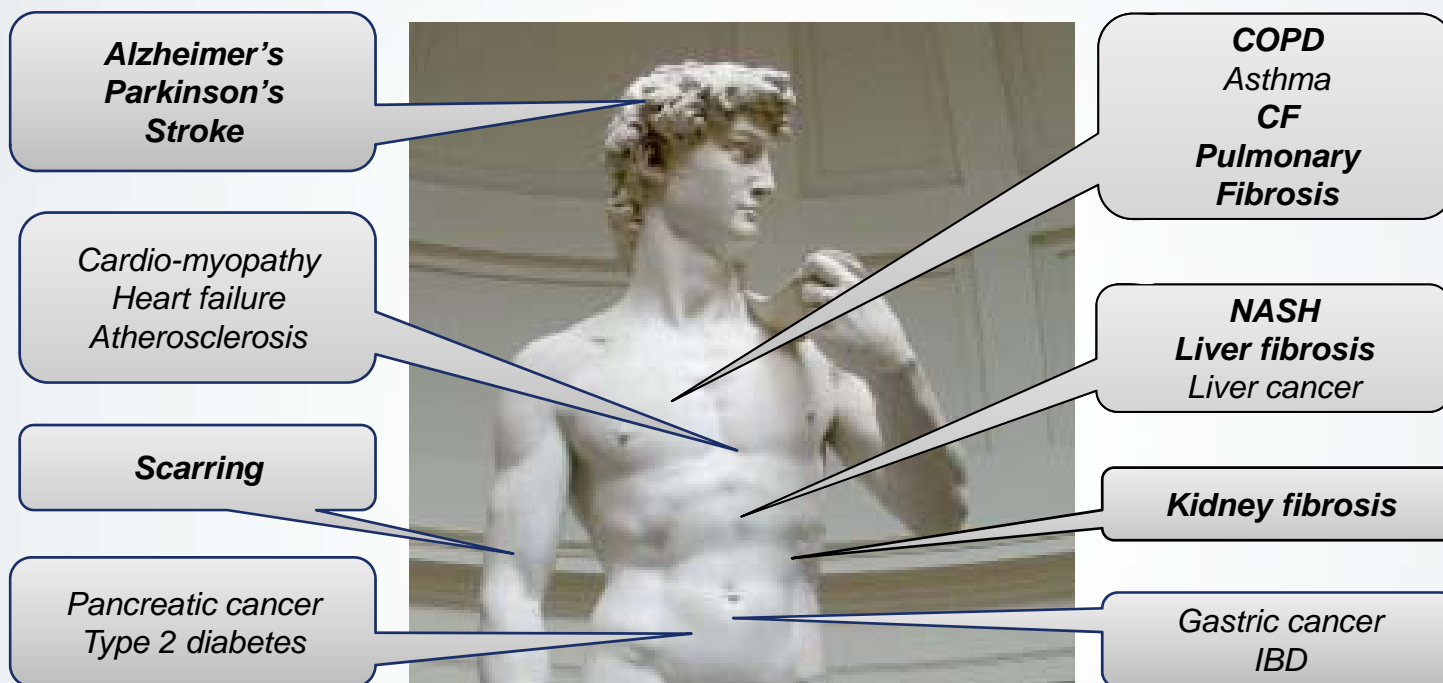


Financial strength

- ❑ \$54m cash balance at June 2015
- ❑ Significant value milestones from existing partner deals within reach
- ❑ Growing institutional presence on share register - >45%

Our therapeutic focus

the inhibition of amine oxidase enzymes has broad potential application



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

Pharmaxis drug discovery strategy

Building a biotech powerhouse in fibrosis and inflammation

Strategy

Drug discovery:

- ❑ Build a biotech powerhouse in fibrosis and inflammation
 - Prioritise validated targets
 - Multiple drugs from in-house amine oxidase chemistry platform
 - Develop to phase 1 or 2

Partnering:

- ❑ Create value via
 - 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



Achievements to date

Drug discovery:

- ❑ First in class NASH drug taken to phase 1
- ❑ Two further candidates in lead optimisation phase
- ❑ One lead candidate moving to preclinical

Partnering:

- ❑ In house BD expertise achieves valuable deal with Boehringer Ingelheim - A\$39m upfront, total potential > A\$750m
- ❑ Collaboration with Synairgen Research plc for early stage fibrosis program to widen spread of indications, enhance time to value inflection and spread risk

SSAO for NASH



SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

PXS 4728A

- ❑ Mechanism based inhibitor of SSAO
- ❑ Development status:
 - Pharmaxis discovery – patent filed 2012
 - Effective in pre clinical models of NASH and airway inflammation
 - Phase 1 study reported
 - orally bioavailable
 - long lasting inhibition after single dose
 - progressive dose response
- ❑ Competitors:
 - Genefit – GF505 in Phase 2b NASH
 - Intercept - OCA (FXR agonist) in Phase 2b NASH
 - Gilead – FXR agonist in pre clinical

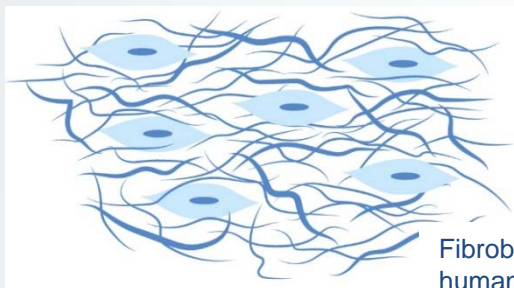
Boehringer Ingelheim

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

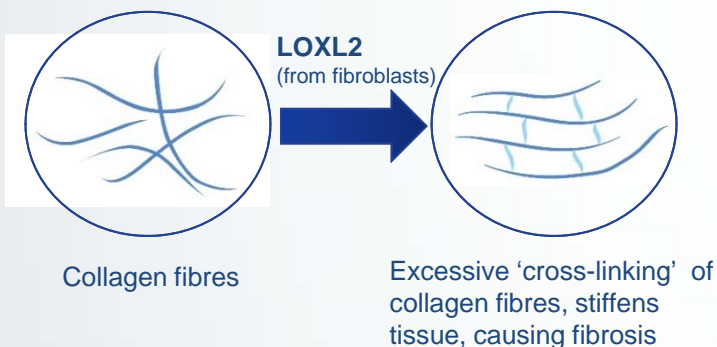
LOXL2 inhibition for NASH & other fibrotic diseases

an attractive target and development program

Excessive production and linking of collagen fibres results in fibrosis



Fibroblast cells in human tissue



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 (IPF)
- ❑ 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

LOXL2 for pulmonary fibrosis



Collaboration with Synairgen

Idiopathic Pulmonary Fibrosis (IPF)

- ❑ IPF primarily affects people over the age of 50
- ❑ 5,000 patients have IPF in Australia
- ❑ 100,000 people with IPF in the US
- ❑ Prognosis is worse than that of many cancers
- ❑ Two drugs approved recently
 - Nintedanib (Boehringer Ingelheim)
 - Pirfenidone (Roche)
- ❑ Need for new therapies
- ❑ Current products expected to produce global revenues > \$1.1 billion by 2017

Synairgen collaboration

- ❑ Access to
 - Synairgen's strength in fibrosis biology and respiratory clinical development - BioBank human tissue models technology platform
 - expertise at University of Southampton
- ❑ Faster time to value appreciation and partnering points of phase 1 or 2a
- ❑ Synairgen to fund pre clinical tox and phase 1
- ❑ Shares risk and reward based on investment in program
- ❑ Allows PXS to pursue further indications in parallel

Pharmaxis opportunities

Building a biotech powerhouse in fibrosis and inflammation



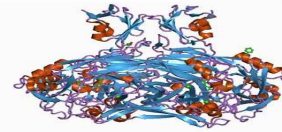
SSAO program for NASH (fatty liver)

- ❑ NASH: US\$3.5B market by 2025
- ❑ PXS SSAO inhibitor of NASH successfully taken to phase 1
- ❑ Acquired by BI for A\$39m upfront, total >A\$750m
- ❑ BI to develop for NASH and other inflammatory indications (eg. kidney fibrosis, COPD)
- ❑ Next milestone: start of phase 2 ~end CY 2016



LOXL2 program for pulmonary fibrosis

- ❑ Pulmonary fibrosis: market >\$1B
- ❑ Collaborate to phase 1 or 2 then seek partner
- ❑ Revenue share for phase 1 partnering deal: 50/50
- ❑ Next milestone – commencement of formal preclinical program ~ beginning CY 2016



LOXL2 for NASH and other diseases

- ❑ Big pharma interest in NASH, LOXL2 and PXS approach
- ❑ Complimentary to SSAO program acquired by BI
- ❑ Next milestone – commencement of formal preclinical program ~ beginning CY 2016



Bronchitol FOR CF in US

- ❑ US is largest CF market
- ❑ Partnered - Chiesi
- ❑ Chiesi funding CF303 to a cap of US\$22m
- ❑ \$25m milestone payments on launch and sales thresholds
- ❑ High mid teens royalty% on in-market sales
- ❑ Mid teens % uplift on COGs

Major upcoming milestones

Cash funds (\$54m at 30 June) sufficient to reach near term valuable milestones

Calendar years

2015

2016

2017



☐ **PXS4728A Phase 2 commences**



- ☐ CF303 fully recruited
- ☐ CF204 (paediatric) reports

- ☐ CF303 – last patient completes trial
- ☐ CF303 – reports

- ☐ FDA decision on Bronchitol approval in US
- ☐ **Bronchitol US launch**

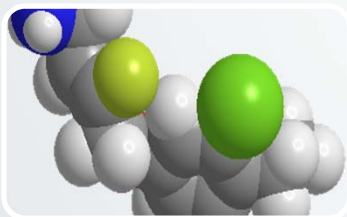


- ☐ Lead candidate for IPF identified

- ☐ 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**

Pharmaxis product portfolio

Product	Indication	Status
<i>LOXL2 inhibitor</i>	<i>NASH (fatty liver disease), Liver & kidney fibrosis</i>	<i>Lead optimisation</i>
<i>LOXL2 inhibitor</i>	<i>Idiopathic pulmonary fibrosis</i>	<i>Lead optimisation; collaboration with Synairgen</i>
LOX/LOXL2 inhibitor	Fibrosis, cancer	Exploratory
<i>SSAO inhibitor</i>	<i>NASH</i>	<i>Successful phase 1 study reported; sold to Boehringer</i>
SSAO/MAOB inhibitor	Neuro inflammation; Alzheimer's, MS, etc.	Lead candidate selected
<i>SSAO/MPO inhibitor</i>	<i>Respiratory inflammation; Asthma, COPD</i>	<i>Exploratory</i>
Orbital	Dry powder inhalation device	Phase 1 – seeking partner
ASM8	Asthma	Phase 2 - seeking partner
Bronchitol US	Cystic Fibrosis	Partner: Chiesi, funding phase 3 study - currently underway
Bronchitol EU	Cystic Fibrosis	Partner: Chiesi (UK & Germany) - marketed
Bronchitol rest of world	Cystic Fibrosis	Marketed: Australia, CEE Approval pending; Brazil, Russia
Aridol	Asthma diagnosis	Marketed: Australia, EU, Korea

**amine
oxidase
chemistry
platform**