



Investor Presentation

Gary Phillips CEO

May 2020

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.

Summary

A global leader in drug development for fibrotic & inflammatory diseases

1. Exciting product pipeline with multiple near term opportunities

- Best in class anti fibrotic LOXL-2 inhibitor program for the treatment of chronic fibrotic diseases; e.g. NASH and IPF
 - Completed phase 1 safety trials; Commercial partnering in progress
- First in class anti cancer oral pan-LOX inhibitor program for the treatment of cancers that have a significant fibrotic element
 - Phase 1 and six month toxicity studies complete;
 - To commence phase 2 clinical trial in myelofibrosis in H2 2020
- First in class topical pan-LOX inhibitor program targeting skin scar revision
 - To commence phase 1 trials in H2 2020
- Anti inflammatory AOC3 inhibitor (BI1467335) sold to Boehringer Ingelheim (BI) in 2015 in development for diabetic retinopathy
 - \$83m received to date from BI;
 - Diabetic retinopathy deal >\$380m+ in development milestones plus royalties;
- Mannitol business unit (Bronchitol and Aridol) nearing breakeven revenues
 - US FDA approval expected mid 2020; US\$10m launch milestone payment Q3 2020

2. Management team with significant international experience in drug development, commercialisation and partnering

- Big Pharma validation of science and commercial acumen from existing deals with BI and Chiesi

3. A\$20m cash (Mar 20)

4. Specialist US, UK and Australian institutional biotech investors on the share register

5. Numerous catalysts over next 12 months including two cash generating events (LOXL2 partnering & Bronchitol US)

A broad pipeline with multiple opportunities

Drug	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Approval	Marketed by	
Mannitol business										
Bronchitol® US	Cystic fibrosis	FDA expected to complete review of NDA mid 2020. Subject to FDA approval, US partner Chiesi will launch commercially in the US in H2 2020.								
Bronchitol Rest of world	Cystic fibrosis	Bronchitol is currently sold in the UK, Germany, Italy, Greece & Nordic countries 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, Canada and a number of European countries. Canadian launch was H2 2019.							Direct & Dist	
Drug development										
Clinical										
AOC3	Diabetic retinopathy	BI1467335; Boehringer commenced dosing a Phase 2a trial in January 2018. Last patient recruited Q4 2019. To report H2 2020.								
LOXL-2	Chronic fibrosis - NASH, IPF, CKD	PXS-5382/PXS5338; Phase 1 trials in 2 compounds complete. Commercial partnering process in progress.								
Oral pan-LOX	Cancer Acute fibrosis	PXS5505; Completed phase 1 clinical plus long term tox studies. To commence phase 2 in myelofibrosis in H2 2020								
Preclinical										
Topical pan-LOX	Scarring Skin fibrosis	PXS 6302; Effective in scarring models. Completing pre-clinical studies. To commence phase 1 CY 2020								

Experienced senior management team

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



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



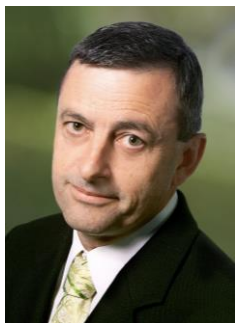
David McGarvey – CFO

- 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



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



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

Non Executive Directors

- **Malcolm McComas – Chair**
 - former investment banker
 - former MD Citi Group
- **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

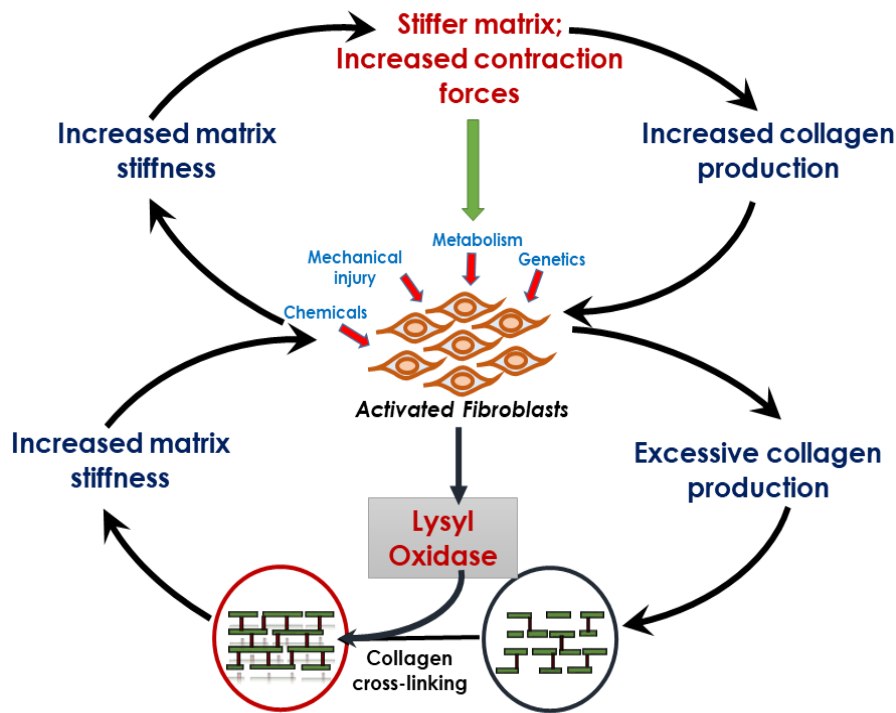
Key catalysts targeted for 2020

Pharmaxis value driving events

- **LOXL2 anti fibrotic program**
 - Partnering process ongoing
- **Pan-LOX cancer program**
 - Phase 2a study in myelofibrosis to commence H2 2020
- **Boehringer Ingelheim acquired AOC3 inhibitor to report clinical proof of concept**
 - Phase 2a diabetic retinopathy study in 79 patients for 3 months - fully recruited
 - Clinical and commercial assessment due from BI - H2 2020
- **Mannitol Business (Aridol & Bronchitol) to turn profitable in CY 2020**
 - US - FDA to complete review mid 2020; if approved - launch milestone US\$10m Q4
 - Sales growth in existing and new territories
 - Transformational impact on cash burn

Role of lysyl oxidase enzymes in genesis of fibrotic tissue

Impact of LOX enzymes on fibrosis



Therapeutic benefits of LOX inhibition

■ Anti-fibrotic effects

- Reduces cross-linking of extracellular matrix proteins (collagen, elastin)
- Tissue stiffness will be diminished
- Cellular stress will be reduced

■ Anti-metastatic effects

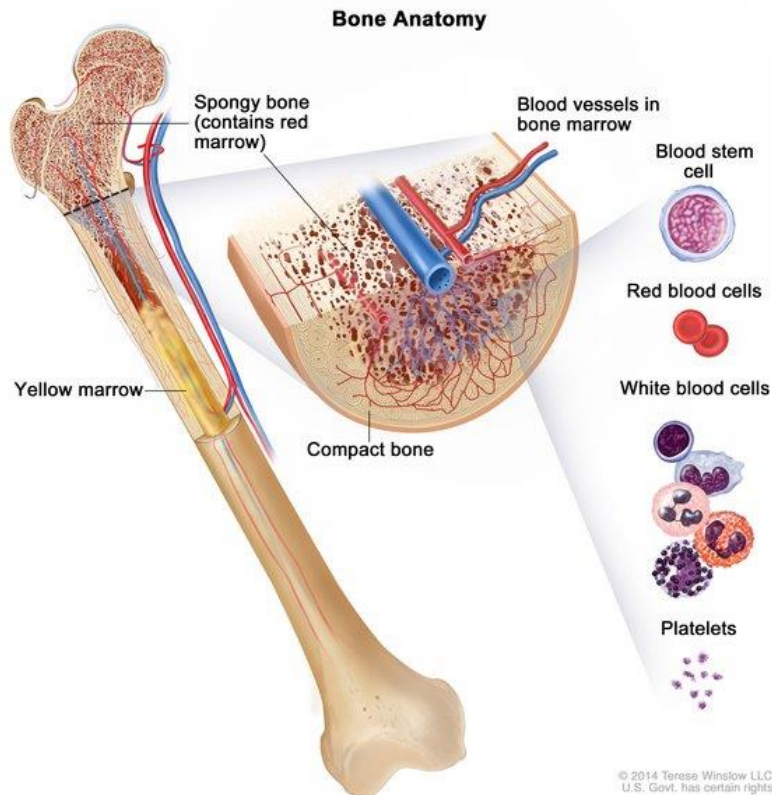
- Inhibition of lysyl oxidase reduces fibrotic microenvironment required for metastases

■ Adjuvant therapy for cytotoxic drugs and checkpoint inhibitors.

- Lysyl oxidase inhibition is downstream of every fibrotic pathway and hence complementary to any standard of care
- Inhibition of LOX loosens stiff stroma of primary tumours to improve access of anti-cancer drugs e.g. checkpoint inhibitors

Myelofibrosis background

A rare type of bone marrow cancer that disrupts your body's normal production of blood cells



Primary Myelofibrosis is caused by a build up of scar tissue (fibrosis) in bone marrow reducing the production of blood cells:

- Reduced red blood cells extreme tiredness (fatigue) or shortness of breath
- Reduced white blood cells can lead to an increased number of infections
- Reduced platelets can cause easy bleeding or bruising
- Spleen increases blood cell production and becomes enlarged
- Other common symptoms include fever, night sweats, and bone pain

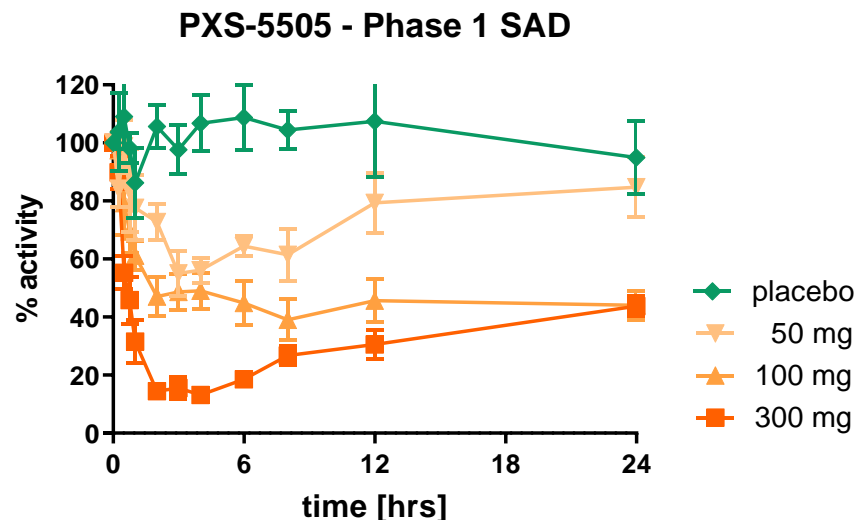
Key facts

- Effects 1 in 500,000 people worldwide
 - 5k US patients diagnosed per year
 - 16k total US patients
- 5 Years Median survival
- Age of onset 50 – 80
- US\$1b+ market

Pan-LOX cancer program

First in class drug with opportunities to fast track into phase 2 studies

Program	Pan-LOX inhibitor
Indications	<ul style="list-style-type: none"> Primary myelofibrosis Pancreatic cancer Oral cancer Severe fibrotic indications
Status	<ul style="list-style-type: none"> Phase 1 studies complete 6 month tox studies complete Effective in animal models of myelofibrosis and other acute fibrotic diseases 2018 patent priority date
Next steps	<ul style="list-style-type: none"> File IND application Contract Clinical CRO Commence phase 2 study in myelofibrosis Q4 20 Identify further potential indications through academic and industry collaborations

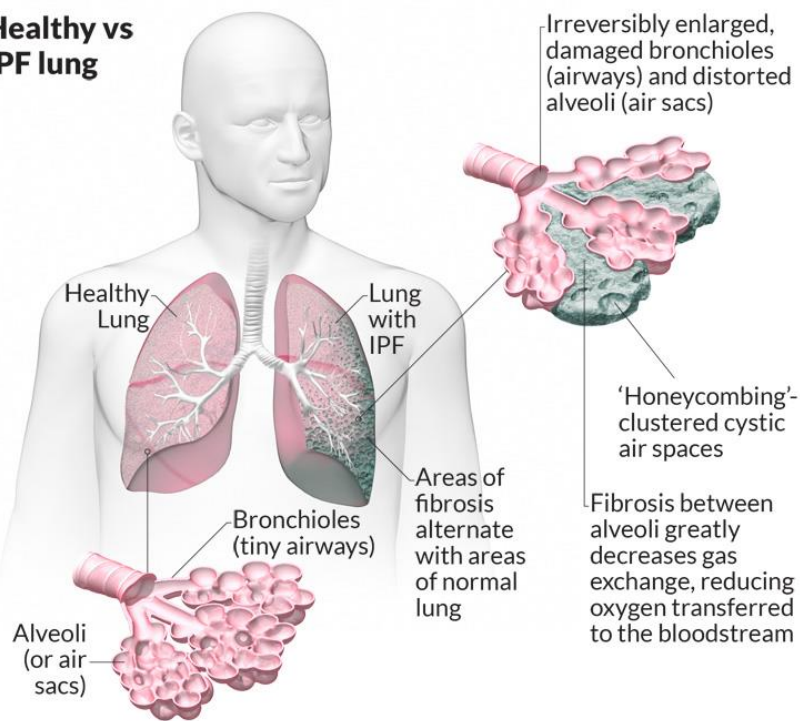


- Good safety profile with 6 month tox studies complete
- Dose dependant 24 hour inhibition of LOX enzymes from single once a day dose

LOXL2 anti fibrotic program in partnering process

for NASH, IPF & other high value fibrotic diseases

Healthy vs IPF lung



Potential indications / market size:

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

Significant market opportunity

■ LOXL2 and fibrosis

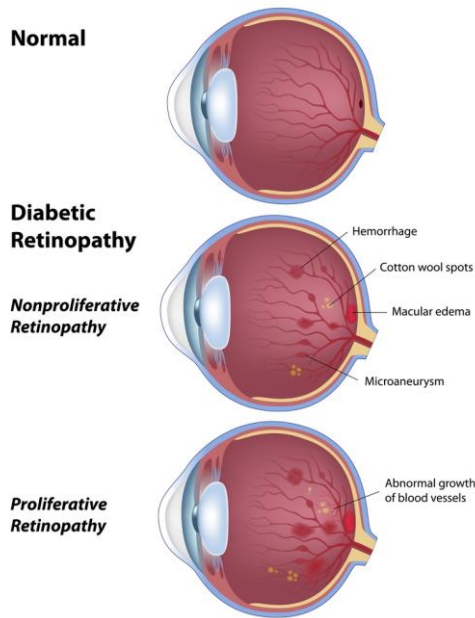
- LOX family of enzymes catalyse the final step in the fibrotic disease process
- Clear association of increased levels of serum LOXL2 with disease progression in IPF, NASH and cardiac fibrosis

■ 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 in phase 1 studies
- 13 week tox studies (2 species) for both compounds
- Only known drug in clinical development to also 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

Boehringer AOC3 program in diabetic retinopathy

Once a day oral treatment for one of the most significant complications of diabetes



Phase 2a in Diabetic Retinopathy patients

ClinicalTrials.gov Identifier: NCT03238963

- Safety, Tolerability, PD, and PK with and without treatment
- Secondary efficacy endpoints
- Recruitment completed (N=79 from Europe and US)
- 12 week treatment compared to placebo with a 12 week follow up period in patients with non-proliferative diabetic retinopathy
- Estimated study completion May 2020
- Boehringer decision to progress 2H 2020

- Affects one third of diabetic patients world wide (~95 million people)
- No approved treatments for early stage disease
- AOC3 is an enzyme associated with inflammation and oxidative stress expressed in human eye
- First in class anti inflammatory AOC3 inhibitor for DR with peak sales potential of ~US\$800m [Analyst's estimate]

Boehringer Ingelheim deal



Deal summary

History



Opportunity



PLUS

- Earn-out payments on annual net sales
 - Tiered percentages increasing from high single digits
 - Plus sales milestones

- No further investment required from Pharmaxis
- Commercial go/no go for phase 2b in DR expected H2 2020
- Start of Phase 3 milestones ~A\$60m

Mannitol business – profitable from 2020

Driven by existing market growth plus market entry of Bronchitol into US



- Two mannitol based products from Sydney factory; FDA, TGA, EU approved
 - Aridol (Asthma Diagnostic)
 - Bronchitol (Cystic Fibrosis)
- Strong 2019 sales and healthy order book for both drugs
 - Bronchitol EU FY 19 in-market sales +17%
 - Bronchitol Australia FY 19 in-market sales +12%
 - Aridol global sales FY 19 +55%
- Increasing rate of profitability on growing sales as factory increases capacity utilisation

The US Market Tipping Point

- FDA issued a Complete Response Letter (CRL) in June 2019
 - Details all of the remaining matters to be addressed before Bronchitol® can be approved
 - Main requirements in CRL are that Chiesi:
 - Revise the product packaging and user instructions
 - Conduct a human factor study (HFS) demonstrating healthcare professionals can properly administer the mannitol tolerance test.
- Expected timing
 - Design HFS ✓
 - FDA review of HFS ✓
 - Completion of HFS – Q1 2020 ✓
 - File HFS and other requested information with FDA – Q2 2020
 - FDA completes review – 60 days from filing
- US sales commence in H2 CY 2020 and turn business cash flow positive.
- Launch milestone US\$10m in H2 2020

Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap ¹	\$36m
Shares on Issue	395m
Employee Options	19m
Liquidity (turnover last 12 months) ¹	77m shares
Share price ¹	\$0.091
Analyst valuation ²	\$0.26
Cash balance (31 March 2020)	A\$20m

Institutional Ownership	31 Mar 20
BVF Partners (US)	20%
Arix Bioscience (UK)	11%
Australian Ethical	7%
D&A Income Limited	7%
Other Institutions	8%
Total Institutional Ownership	53%

1. As at 30 April 2020

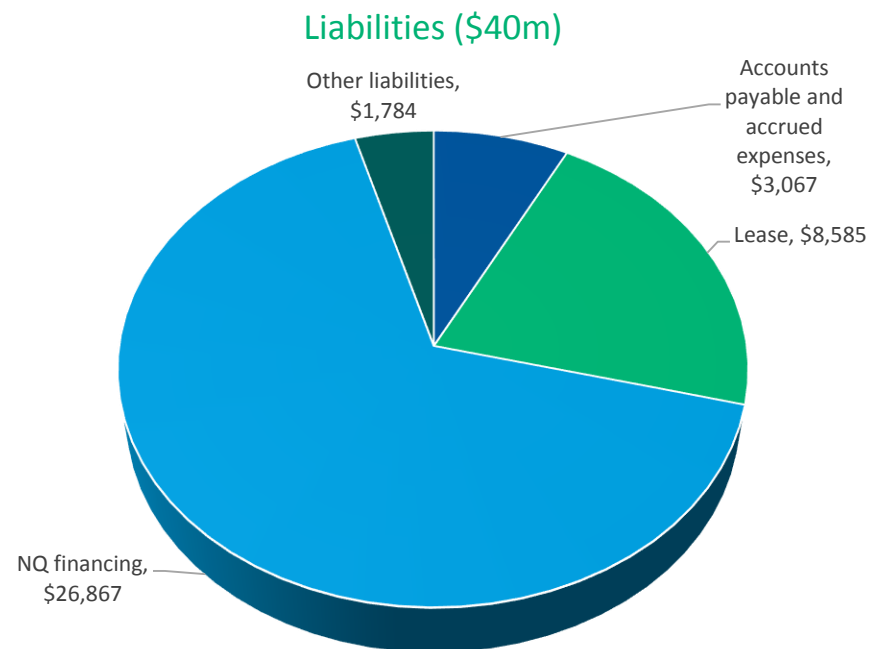
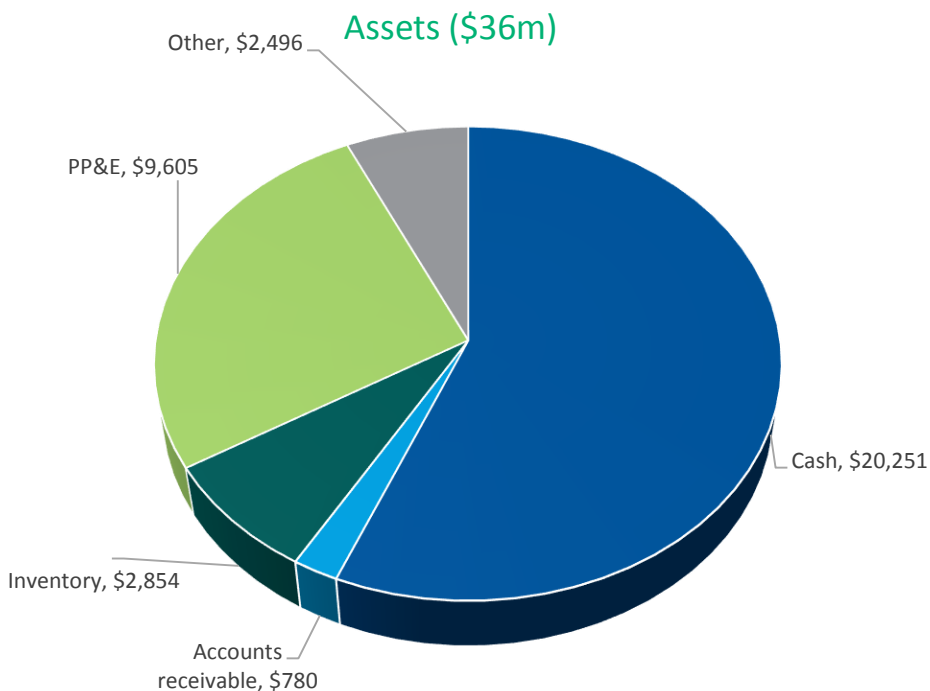
2. Bell Potter Securities Research 13 February 2020

Financials – highlights

	Nine months ended 31 Mar		Financial Years ended 30 June			
A\$'000	2020	2019	2019	2018	2017	2016
Income Statements						
Sales revenue	4,346	4,171	5,676	6,094	4,823	6,135
Other revenue	958	1,112	7,404	44,739	13,178	12,885
Total revenue	5,304	5,283	13,080	50,833	18,001	19,020
Expenses	(25,162)	(24,199)	(33,138)	(44,413)	(36,437)	(35,476)
Net profit (loss) after tax	(19,858)	(18,916)	(20,058)	6,428	(18,346)	(16,463)
Segment results - adjusted EBITDA						
Mannitol business (Bronchitol & Aridol)	(3,733)	(3,194)	(5,013)	(3,786)	(7,100)	(8,228)
New drug development	(7,124)	(9,226)	(6,764)	28,771	(4,114)	(2,625)
Corporate	(2,283)	(3,045)	(3,874)	(13,466)	(4,017)	(3,988)
	(13,140)	(15,465)	(15,651)	(11,519)	(15,231)	(14,841)
Cash flow						
Operations	(4,863)	(6,114)	(19,798)	12,206	(15,262)	(11,989)
Investing activities	(130)	(295)	(981)	(884)	(723)	(1,381)
Financing activities	(620)	(465)	20,830	(1,753)	(1,721)	(1,714)
	(5,613)	(6,874)	51	9,569	(17,706)	(15,084)
Cash at bank	20,251	35,129	31,124	31,073	21,504	39,209

Balance sheet

31 March 2020



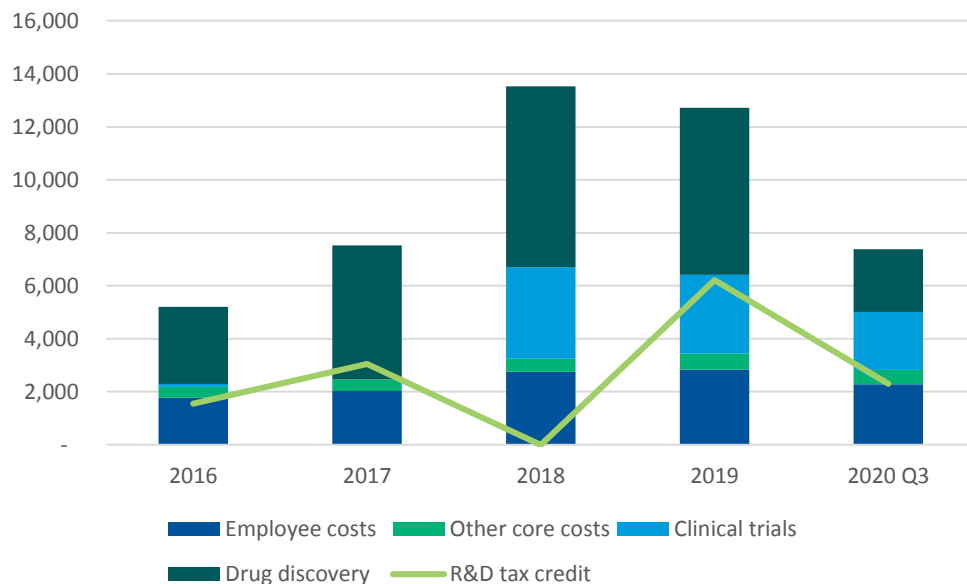
Notes to Liabilities

- Lease over 20 Rodborough Rd (to 2024)
- NovaQuest financing – not repayable other than as % of US & EU Bronchitol revenue – up to 7 years

New drug development

Expenditure and R&D tax incentive funding

New Drug Development Expenses



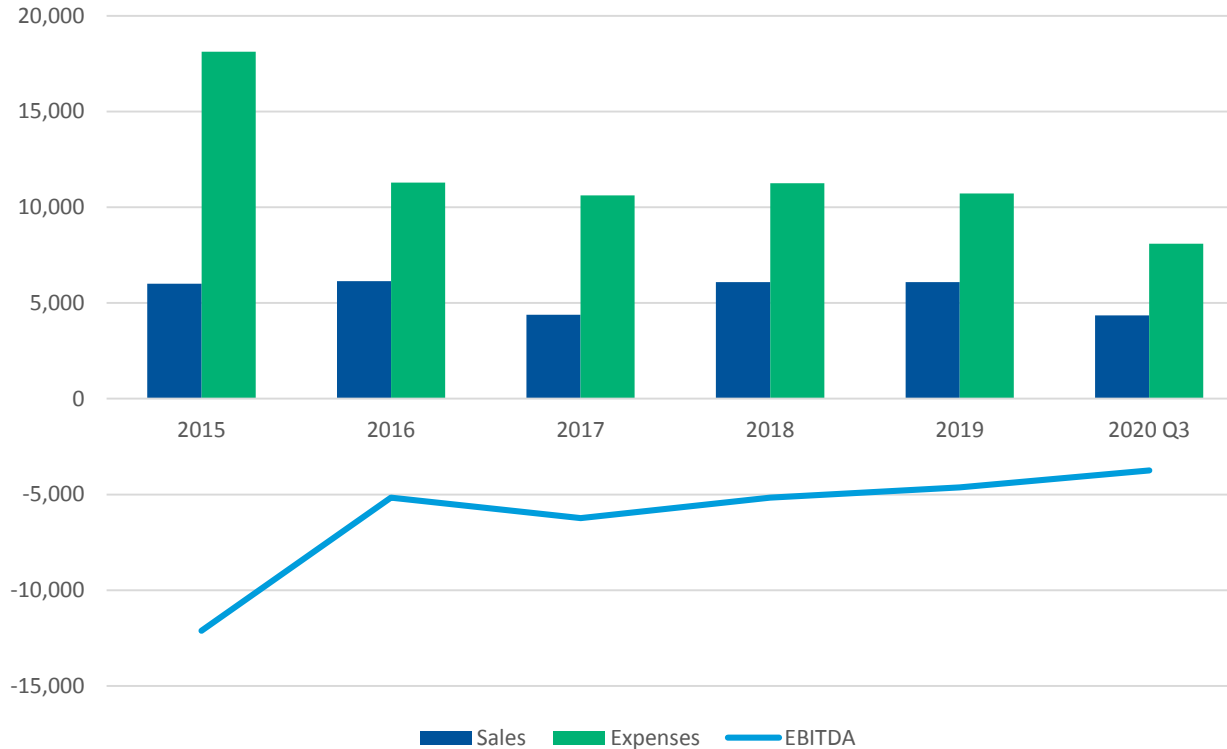
- R&D tax incentive:
 - Eligible R&D expenditure receives an R&D tax incentive (cash) of approximately 43%.
 - Eligibility is subject to total company revenue being less than \$20 million.
 - In 2018 revenue exceeded \$20m, 2020 YTD incentive is illustrative.
 - PXS tax incentive has averaged 37% of total R&D expenditure.

- Employee & other core costs net of tax incentive is currently ~\$1.6m pa

- Drug discovery and clinical trial expenses:
 - External expenditure related to development of specific projects
 - Drug discovery expenditure includes preclinical development for 5 compounds (two LOXL2 compounds, Anti-cancer oral LOX, topical LOX, and SSAO combo).
 - Clinical trial expenditure includes phase 1 clinical trials for LOXL2 (2 compounds) and Anti-cancer oral LOX.

Mannitol business segment

Financial results excluding clinical trial related revenue and costs



1. Subsequent to the end of the quarter the Company has invoiced over \$1m in distributor orders.
2. Sales adjusted for Russian credit note in 2019 in relation to sale made in 2017

A decorative graphic on the left side of the slide consists of several overlapping hexagons in various shades of blue, creating a molecular or crystalline structure.

Pharmaxis Ltd
20 Rodborough Road
Frenchs Forest NSW 2086
Australia
T: +61 2 9454 7200
www.pharmaxis.com.au

Gary Phillips
Chief Executive Officer
gary.phillips@pharmaxis.com.au

David McGarvey
Chief Financial Officer
david.mcgarvey@pharmaxis.com.au