



For personal use only

Investor Presentation

Gary Phillips CEO

November 2019

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 fibrosis & inflammation

1. Exciting product pipeline with multiple near term opportunities

- Lead drug (BI1467335) sold to Boehringer Ingelheim (BI) in 2015 in development for two disease indications
 - Total deal >\$600m+ in development milestones plus royalties; \$83m received to date from BI
- Anti fibrotic LOXL-2 program for the treatment of diseases including NASH and IPF completed phase 1 safety trials
 - Commercial partnering in progress
- Bronchitol and Aridol (Mannitol business) business unit nearing breakeven revenues
 - US FDA approval expected H1 2020
- Two further anti fibrotic programs in late stage pre clinical / phase 1
 - Patient proof of clinical efficacy trials due to start in 2020; myelofibrosis & skin scar revision

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. Strong balance sheet - A\$23m cash (9/19) plus \$6m R&D Tax Incentive received October 2019

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

	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 in Q1 2020. Subject to FDA approval, US partner Chiesi will launch commercially in the US in 2020.								
Bronchitol RoW	Cystic fibrosis	Bronchitol is currently sold in the UK, Germany, Italy, Greece & Nordic 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 and a number of European countries. Canadian approval received June 2019.							Direct & Dist	
Drug development										
<u>Clinical</u>										
AOC3	NASH	Sold to Boehringer Ingelheim in May 2015. Phase 2a trial completed June 2019 – to report Q4 CY 2019. PXS has received payments of A\$68m to date.								
AOC3	Diabetic retinopathy	Boehringer commenced dosing a Phase 2a trial in January 2018. PXS received A\$15m to date.								
LOXL-2	NASH, fibrosis - liver, lung, kidney, heart	Phase 1 trials in 2 compounds complete. Commercial partnering process commenced.								
Systemic LOX	Anti-fibrotic: cancer	Completed phase 1a SAD. To complete phase 1b MAD Q1 2020								
<u>Preclinical</u>										
Topical LOX	Anti-fibrotic: scarring	Effective in scarring models. Commence phase 1 CY 2020								

For personal use only

Experienced senior management team

Significant experience in drug development, commercialisation and partnering

For personal use only



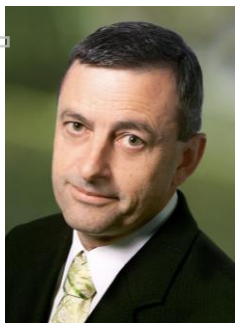
Gary Phillips – CEO

- 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 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 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



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

Non Executive Directors

- Malcolm McComas – Chair**
 - former investment banker
 - former MD Citi Group
- Kathleen Metters**
 - former head of worldwide basic research at Merck
 - former CEO of biopharmaceutical company Lycera Corp
- Will Delaat**
 - former CEO of Merck Australia
 - former chair of Medicines Australia
- Edward Rayner**
 - over 20 years' experience in global capital markets

Key catalysts targeted for 2019/2020

Pharmaxis value driving events

1. Boehringer Ingelheim acquired AOC3 inhibitor to report clinical proof of concept

- Phase 2a NASH study in 114 patients for 3 months - last patient last visit complete. Phase 2a clinical trial result and commercial assessment to progress to Phase 2b due from BI - Q4 2019
- Phase 2a diabetic retinopathy study in 100 patients for 3 months - >50% recruited. Clinical and commercial assessment due from BI - mid 2020

2. LOXL2 anti fibrotic program

- Partnering process to conclude - H2 2019

3. Mannitol Business (Aridol & Bronchitol) to turn profitable in CY 2020

- US - FDA to complete review H1 2020; if approved - launch milestone US\$10m
- Sales growth in existing and new territories

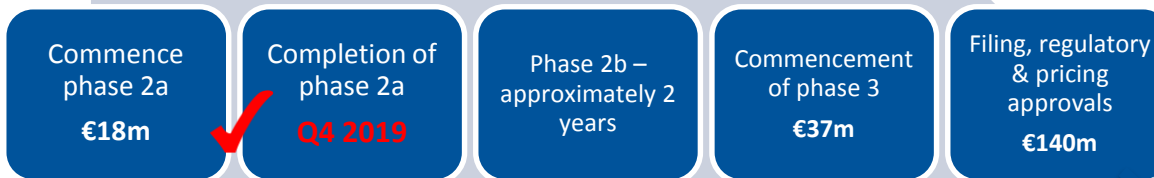
Boehringer Ingelheim deal



Deal summary

For personal use only

First indication (NASH)

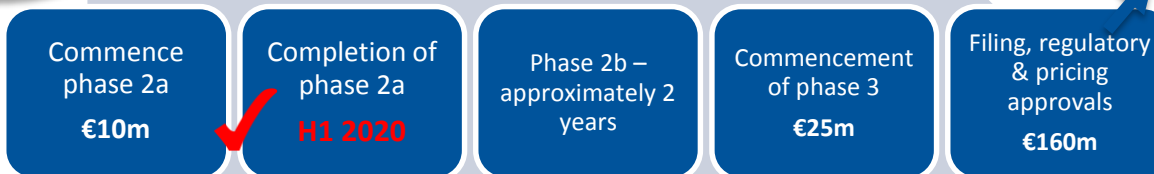


PLUS earn-out payments on annual net sales

- Tiered percentages increasing from high single digits
- Plus sales milestones

Upfront (2015) €29m

Second indication (diabetic retinopathy)



Total Potential Milestones €419 (~A\$625m)

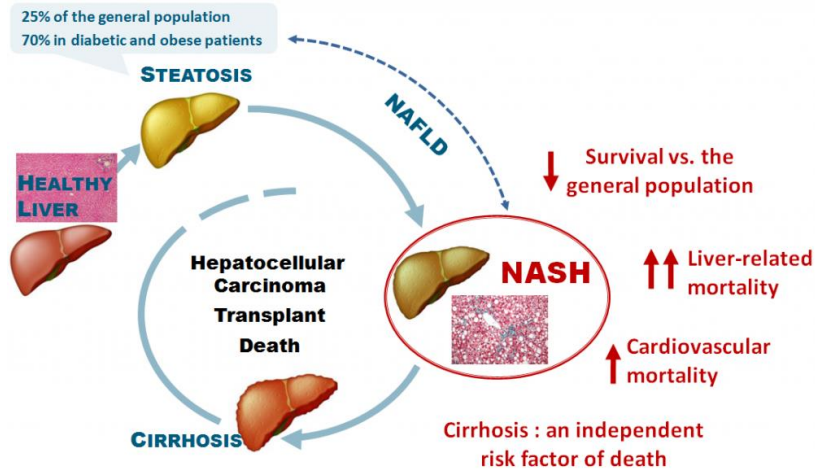
- €57m (A\$83m) already received
- No further investment required from Pharmaxis
- Commercial go/no go for phase 2b in NASH expected Q4 2019
- Start of Phase 3 milestones ~A\$100m

Pharmaxis drug a key player in race for NASH treatment

First in class drug with complementary anti inflammatory mechanism of action

For personal use only

NASH



- Expected to become leading cause of liver transplant by 2020
- No approved treatments
- Deutsche Bank estimate market size of US\$35b by 2025
- First in class anti inflammatory AOC3 inhibitor for NASH with peak sales potential of ~US\$2b [Analyst's estimate]

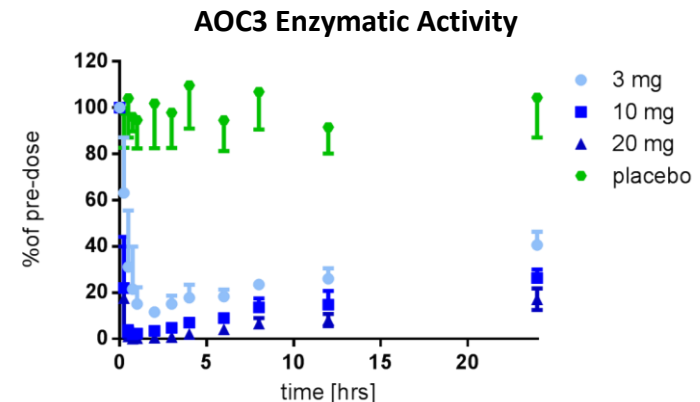
Drug BI 1467335

■ Why target AOC3?

- AOC3 is an enzyme associated with inflammation and oxidative stress
- AOC3 is strongly involved with the development of fatty liver disease
- AOC3 levels are positively correlated with liver fibrosis and cirrhosis

■ Clinical characteristics of BI 1467335

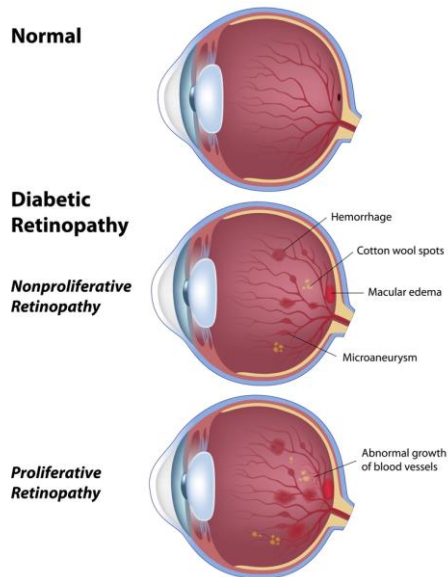
- Good oral bioavailability and short half-life
- No adverse events were found in phase 1 studies
- Long lasting AOC3 enzyme inhibition after a single low dose



Potential break through treatment in diabetic retinopathy

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

Diabetic Retinopathy



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

Drug BI 1467335

Normal vision



Vision with Advanced, Untreated PDR



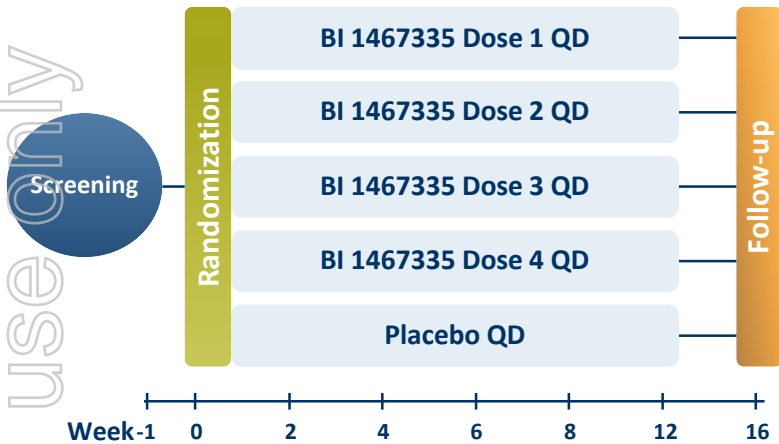
■ Why target AOC3?

- AOC3 is an enzyme associated with inflammation and oxidative stress
- AOC3 is expressed in human eye
- Inhibition of AOC3 in animal models effective in diabetic retinopathy

■ Competitive profile of BI 1467335

- AOC3 enzyme inhibition is a unique mechanism of action and BI 1467335 is a first in class drug
- Once a day oral drug can treat both eyes simultaneously compared to existing therapies which utilise laser treatment or intra ocular injections into each eye

BI 1467335 in two “proof of clinical concept” Phase IIa trials

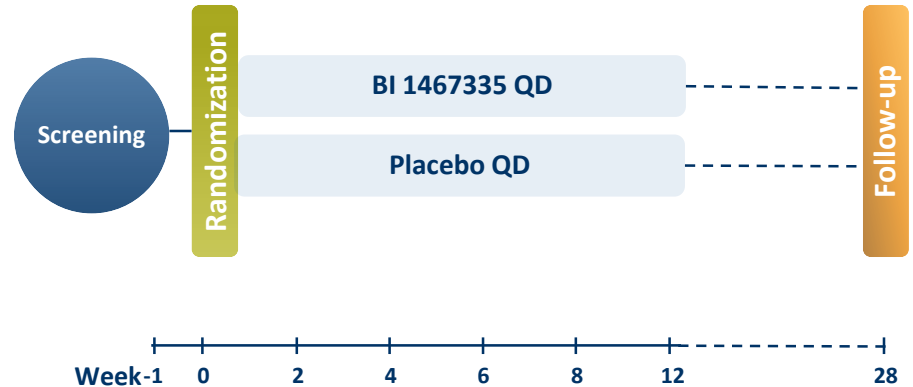


Phase IIa in NASH patients

ClinicalTrials.gov Identifier: NCT03166735

- Safety, Tolerability, PD, and PK in four doses
- Secondary efficacy endpoints
- N=114 from Europe and North America
- 12 week treatment period compared to placebo in patients with clinical evidence of NASH.
- Study completed dosing June 2019
- **Study to report Q4 2019**

BI phase 1 safety studies: 6 completed, 2 ongoing



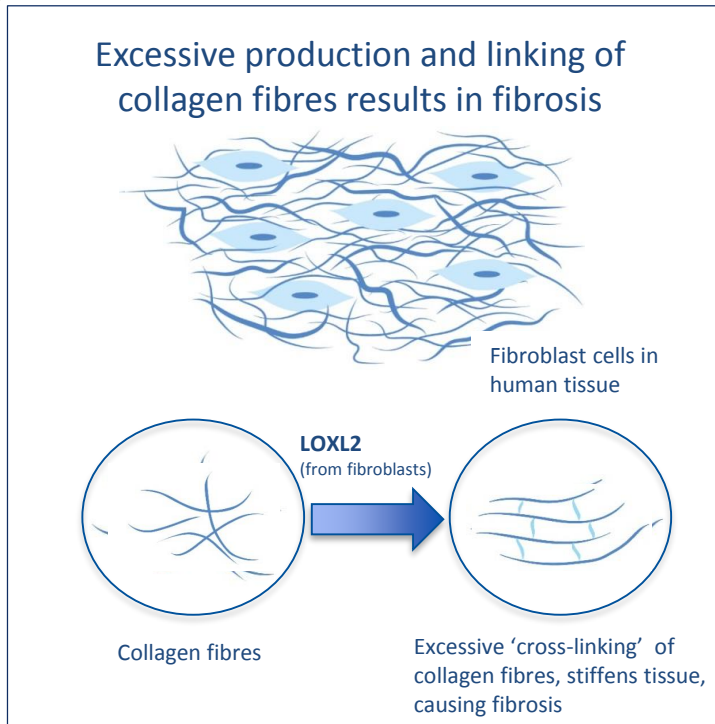
Phase IIa in Diabetic Retinopathy patients

ClinicalTrials.gov Identifier: NCT03238963

- Safety, Tolerability, PD, and PK with and without treatment
- Secondary efficacy endpoints
- N=100 from Europe and US
- 12 week treatment compared to placebo with a 12 week follow up period in patients with non-proliferative diabetic retinopathy
- > 50% recruited
- **Estimated study completion May 2020**

LOXL2 inhibition program in partnering process

for NASH, IPF & other high value fibrotic diseases



■ 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

Potential indications / market size:

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

Significant
market
opportunity

Mannitol business – profitable from 2020

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

For personal use only



- 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 – Q1 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 mid 2020

Financials – highlights

30 June 2019

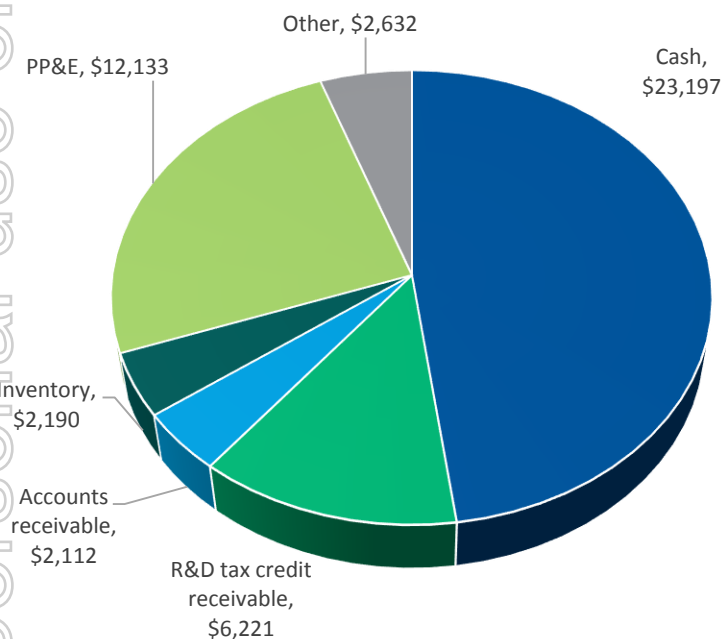
	A\$'000	2019	2018	2017	2016
Income Statements					
Sales revenue		5,676	6,094	4,823	6,135
Other revenue		7,404	44,739	13,178	12,885
Total revenue		13,080	50,833	18,001	19,020
Expenses		(33,138)	(44,413)	(36,437)	(35,476)
Net profit (loss) after tax		(20,058)	6,428	(18,346)	(16,463)
Segment results - adjusted EBITDA					
Mannitol (Bronchitol & Aridol)		(5,013)	(3,786)	(7,100)	(8,228)
New drug development		(6,764)	28,771	(4,114)	(2,625)
Corporate		(3,874)	(13,466)	(4,017)	(3,988)
		(15,651)	(11,519)	(15,231)	(14,841)
Cash flow					
Operations		(19,798)	12,206	(15,262)	(11,989)
Investing activities		(981)	(884)	(723)	(1,381)
Financing activities		20,830	(1,753)	(1,721)	(1,714)
		51	9,569	(17,706)	(15,084)
Cash at bank		31,124	31,073	21,504	39,209

Refer to and Quarterly Shareholder Updates and 2019 Annual Report for additional financial information

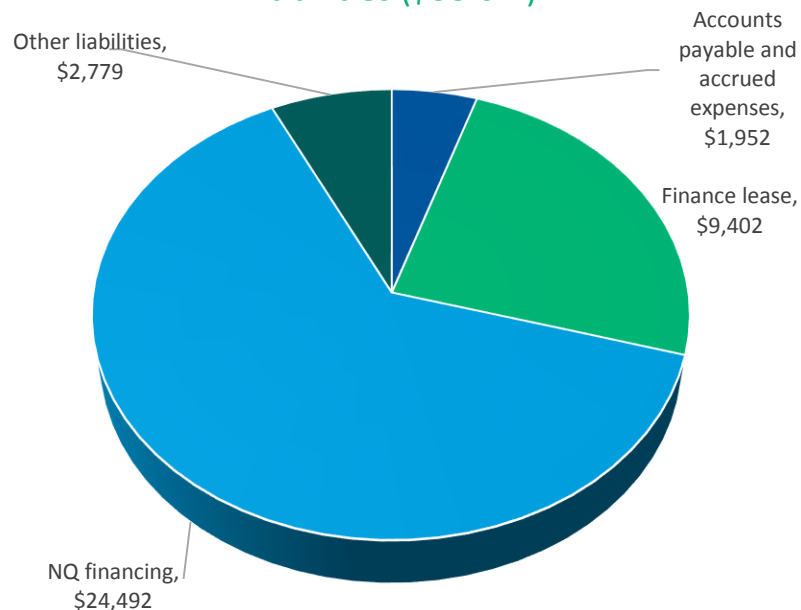
Balance sheet

30 September 2019

Assets (\$48.2m)



Liabilities (\$38.6m)



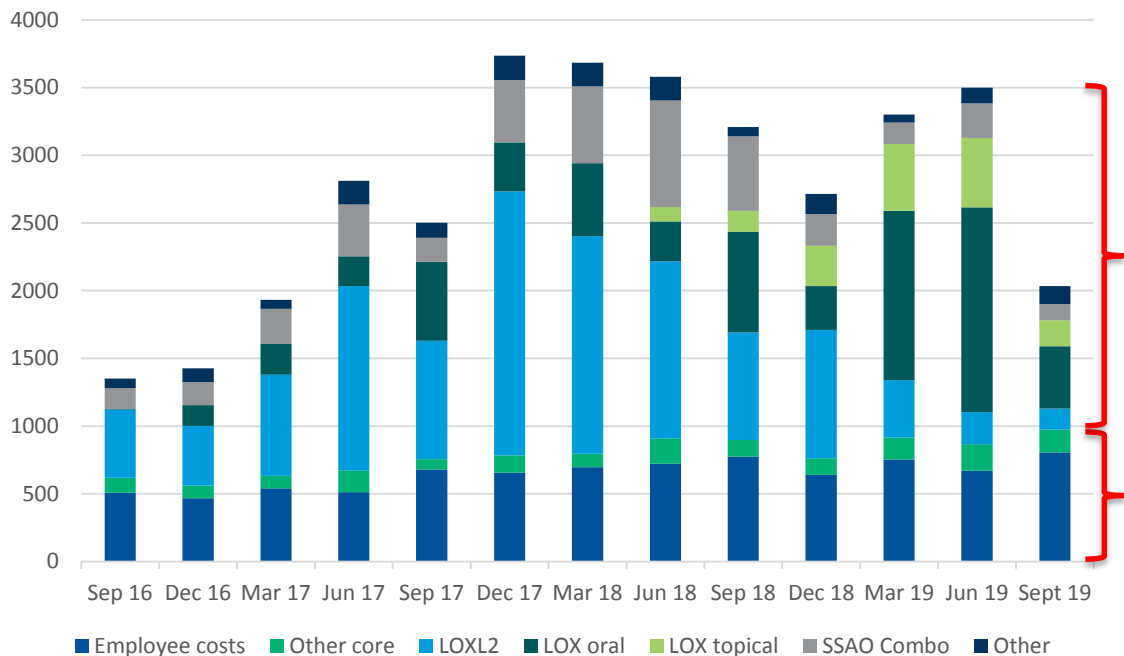
2019 R&D tax credit of \$6.2m received October 2019

- Finance 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 by quarter

New Drug Development Expenses - Quarterly



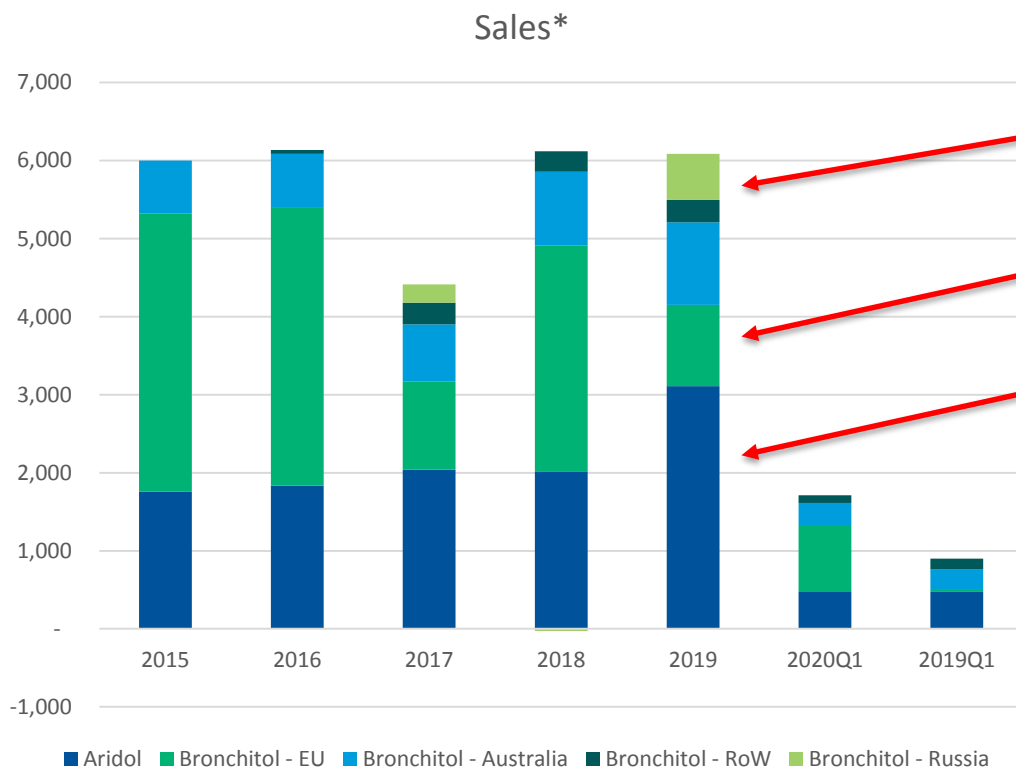
Preclinical development and clinical trial costs incurred to progress drug candidates from successful research programs.

Core fixed operating costs

Pharmaxis new drug development expenditure is eligible for an R&D tax incentive (cash) of approximately 40%, subject to total company revenue being less than \$20 million.

Mannitol business segment

Sales by financial year



First Russian order after reimbursement achieved 1 Jan 2019*

Bronchitol EU is mainly sold by Chiesi – sales reflect Chiesi ordering – in market sales by Chiesi increased 17% in FY 2019.

Includes relaunch of Aridol in US

* Sales adjusted for Russian credit note in 2019 re sale made in 2017

Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap ¹	\$85m
Shares on Issue	395m
Employee Options	20m
Liquidity (turnover last 12 months) ¹	45m shares
Share price ¹	\$0.215
Analyst valuation ²	\$0.54
Pro forma cash balance (30 Sept 2019) ³	A\$29m

Institutional Ownership	30 Sept 19
BVF Partners (US)	20%
Arix Bioscience (UK)	11%
Australian Ethical	8%
D&A Income Limited	7%
Allan Gray	5%
Other Institutions	7%
Total Institutional Ownership	58%

1. As at 19 November 2019
2. Bell Potter Securities Research 24 June 2019
3. Includes \$6.2m R&D Tax Incentive received October 2019

For personal use only

pharmaxis

For personal use only

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