



# Annual General Meeting

21 November 2019



# Presentation by Chief Executive Officer

Gary Phillips

# 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), \$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)

# 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

# A broad pipeline with multiple opportunities

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Approval	Marketed by		
<b>Mannitol business</b>											
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	
<b>Drug development</b>											
<b>Clinical</b>											
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									
<b>Preclinical</b>											
Topical LOX	Anti-fibrotic: scarring	Effective in scarring models. Commence phase 1 CY 2020									

# Highlights of the past year

## 2019 Progress Positions Pharmaxis for Pivotal 2020

### New Drug Development

- BI acquired drug
  - NASH phase 2a study completed
  - DR phase 2a > 50% recruited
- LOXL2 program completes phase 1 and 3 month tox
- System LOX program completes phase 1a, starts phase 1b; positive preclinical studies in myelofibrosis
- Topical LOX prepares for phase 1

### Mannitol Business

- Bronchitol in-market sales increase in EU (17%) & Aust (12%)
- FDA approves Frenchs Forest factory
- Aridol relaunched in US, approved in Canada. Sales up 55%
- Bronchitol reimbursed in Russia and commercial sales commence
- FDA clarifies Bronchitol approval requirements

### Corporate

- \$24m share placement
- UK specialist biotech investor Arix joins share register
- Ed Rayner joins board
- R&D day – presentations by Boehringer Ingelheim and the Garvan
- Pharmaxis recognised as a top innovator company in Asia Pacific
- Pharmaxis receives \$6.2m R&D tax incentive

# Boehringer Ingelheim deal

## Deal summary

### First indication (NASH)

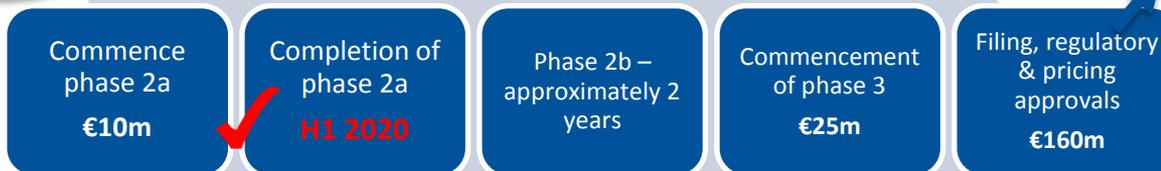


**PLUS earn-out payments on annual net sales**

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

Upfront (2015)  
€29m

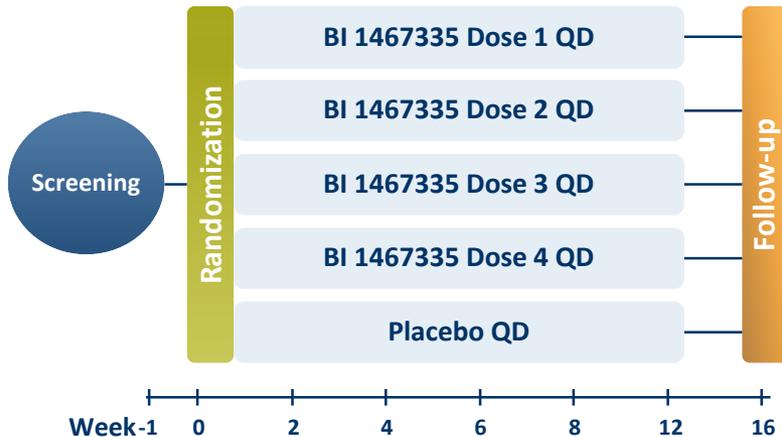
### Second indication (diabetic retinopathy)



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

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

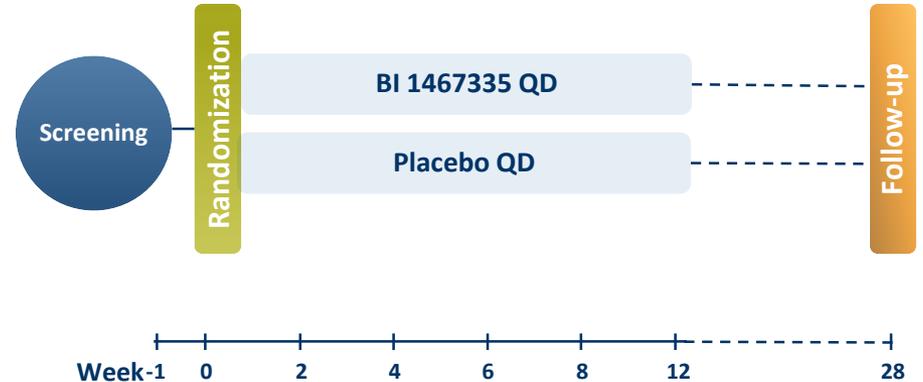


## Phase IIa in NASH patients

*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 June 2019
- **Study to report Q4 2019**

BI phase 1 safety studies: 6 completed, 2 ongoing



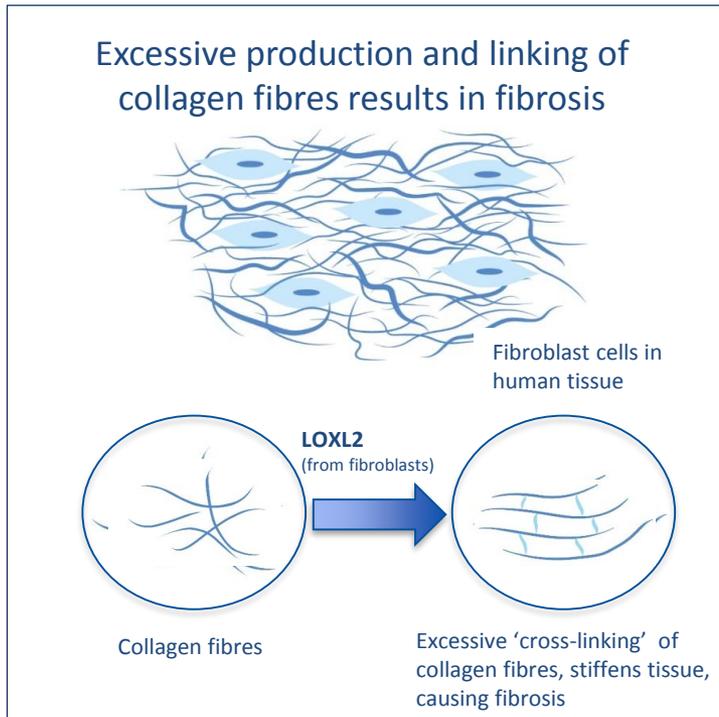
## Phase IIa in Diabetic Retinopathy patients

*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; \$35b<sup>1</sup>
- Pulmonary fibrosis (IPF); \$3.5b<sup>2</sup>
- Kidney fibrosis
- Cardiac fibrosis

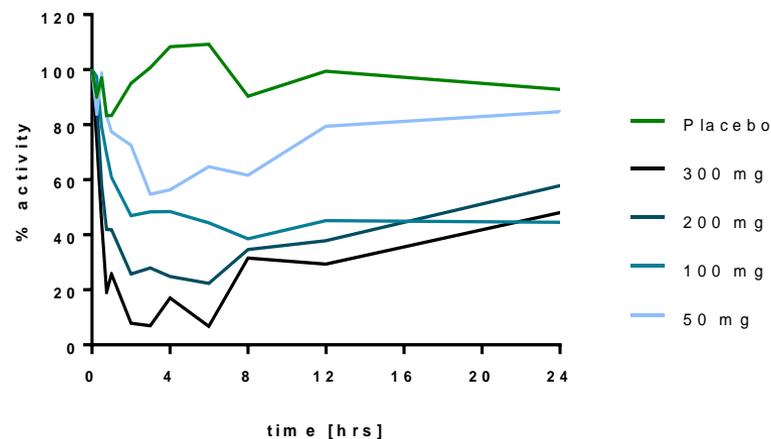
Significant  
market  
opportunity

# Systemic LOX inhibitor

Opportunities to fast track into patient proof of clinical efficacy studies

Program	Systemic LOX
Indication	Severe fibrotic indications <ul style="list-style-type: none"> <li>myelofibrosis</li> <li>pancreatic cancer</li> </ul>
Status	<ul style="list-style-type: none"> <li>Phase 1a completed</li> <li>Effective in animal models of myelofibrosis and other acute fibrotic diseases</li> <li>2018 patent priority date</li> </ul>
Next steps	<ul style="list-style-type: none"> <li>Complete 3 month tox studies</li> <li>Complete Phase 1b (Multiple Ascending Dose)</li> <li>Commence phase 1c/2 study in myelofibrosis and or pancreatic cancer patients by H1 2020</li> </ul>

## Phase 1a - Single Ascending Dose



*Dose dependent reduction in LOX activity in plasma*

- Good safety profile - cleared to proceed to Multiple Ascending Dose stage
- Significant 24 hour inhibition of LOX enzymes from single once a day dose

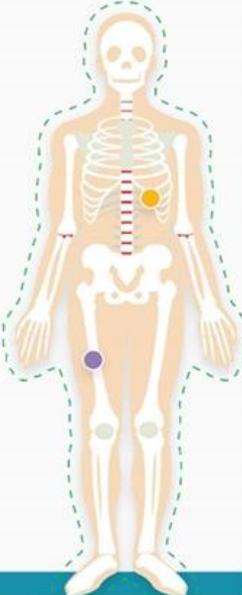
# Myelofibrosis background

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

## 3 PRIMARY WAYS MF AFFECTS PEOPLE

From the outside, many people with myelofibrosis look the same as their healthy friends and family. But inside, patients are often experiencing:

- 1 ENLARGED SPLEEN (SPLENOMEGALY):**  
abdominal pain, a feeling of early fullness, bloating and high pressure in the liver vasculature<sup>9</sup>
- 2 IMPAIRED BLOOD CELL PRODUCTION:**  
reduced platelets (thrombocytopenia), red blood cells (anemia), and white blood cells (leukopenia)<sup>9</sup>
- 3 CONSTITUTIONAL SYMPTOMS:**  
fatigue/tiredness, night sweats, itching, bone pain, inactivity, concentration problems, fever, and weight loss<sup>9</sup>



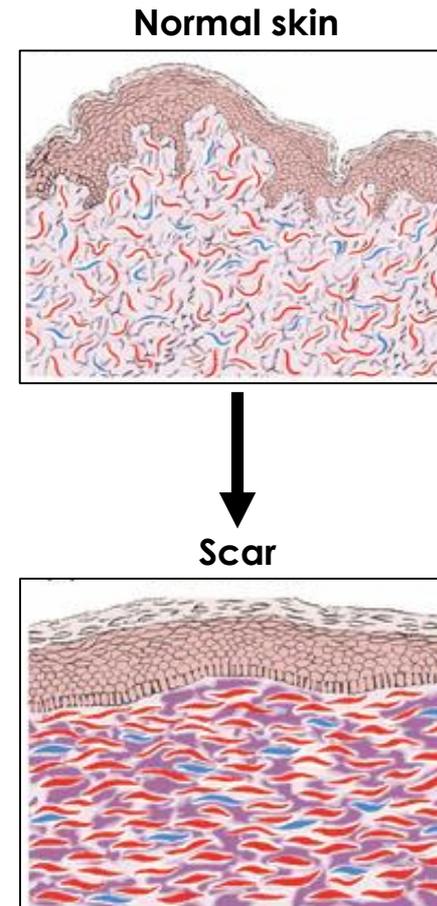
Source: MPN Research Foundation

- **5k** US patients diagnosed per year
- **16k** total US patients
- **5 Years** Median survival
- **US\$1b+** market
- Current treatments target JAK1/2 enzymes
  - Improve constitutional symptoms (e.g. fevers, weight loss) reduce spleen size and bone pain
  - Don't impact bone marrow histopathology and anaemia
- Limited pipeline of drugs in development
  - 2<sup>nd</sup> generation JAK inhibitors
  - Few anti fibrotic drugs
- LOX an important target in myelofibrosis
  - The role of the extracellular matrix and LOX in primary myelofibrosis highlighted in *Blood Cancer Journal* review article
  - <http://dx.doi.org/10.1038/bcj.2017.6>

# Topical LOX inhibitor

Opportunities to fast track into patient proof of clinical efficacy studies

Program	Topical LOX
Indication	<ul style="list-style-type: none"><li>▪ Scar revision</li><li>▪ Keloid scarring</li><li>▪ Burns</li></ul>
Status	<ul style="list-style-type: none"><li>▪ Limited competition</li><li>▪ Lead candidate selected</li><li>▪ Improves scar appearance and function in animal models</li><li>▪ Strong academic and clinical advocacy</li><li>▪ Short term tox studies completed successfully</li></ul>
Next steps	<ul style="list-style-type: none"><li>▪ Complete 3 month tox studies</li><li>▪ Ready to commence proof of concept study in patients 2020</li></ul>



# 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 – 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 H1 CY 2020

# Expectations for 2020

## New Drug Development

- BI acquired drug
  - NASH phase 2a study report and commercial assessment to move to phase 2b - Q4 19
  - DR phase 2a study to report and commercial assessment - mid 2020
- LOXL2 program partnering to conclude
- Systemic LOX program complete phase 1, commence trial in cancer patients
- Topical LOX program to complete 3 month tox; commence phase 1 in healthy volunteers with scars

## Mannitol Business

- US FDA to complete review H1 2020 - if approved
  - launch milestone US\$10m (H1)
  - US sales commence (H2)
- Sales growth in existing and new territories expected (including Russia Russia)
- => Business unit transitions to profitability

## Corporate

- R&D day – currently scheduled H1 2020



# Financial Overview

David McGarvey CFO

# Financials – highlights

30 June 2019

A\$'000	2019	2018	2017	2016
<b>Income Statements</b>				
Sales revenue	5,676	6,094	4,823	6,135
Other revenue	7,404	44,739	13,178	12,885
<b>Total revenue</b>	<b>13,080</b>	<b>50,833</b>	<b>18,001</b>	<b>19,020</b>
Expenses	(33,138)	(44,413)	(36,437)	(35,476)
<b>Net profit (loss) after tax</b>	<b>(20,058)</b>	<b>6,428</b>	<b>(18,346)</b>	<b>(16,463)</b>
<b>Segment results - adjusted EBITDA</b>				
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)
	<b>(15,651)</b>	<b>(11,519)</b>	<b>(15,231)</b>	<b>(14,841)</b>
<b>Cash flow</b>				
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)
	<b>51</b>	<b>9,569</b>	<b>(17,706)</b>	<b>(15,084)</b>
<b>Cash at bank</b>	<b>31,124</b>	<b>31,073</b>	<b>21,504</b>	<b>39,209</b>

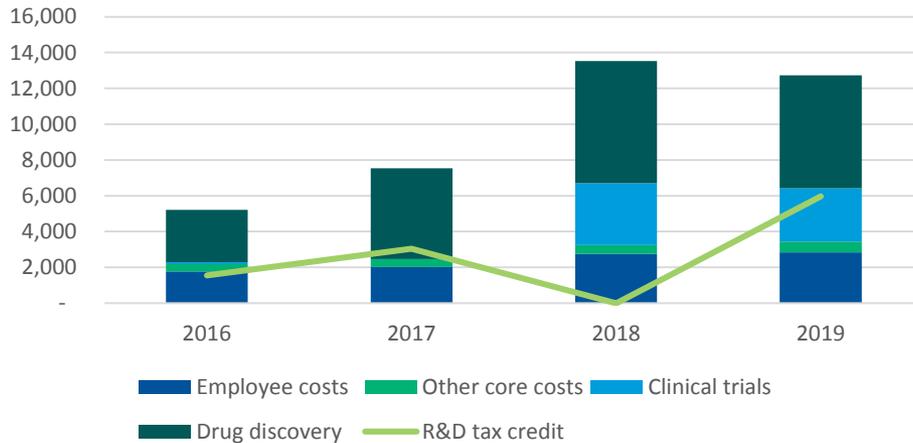
- Refer to following individual segment slides for commentary on financial results
- For additional financial information and commentary refer to 2019 Annual Report and the June 2019 Quarterly Shareholder Update
- Cash flow investing activities relate to drug discovery capability, manufacturing upgrades and patent applications
- Cash flow financing activities – predominantly finance lease over facility at Frenchs Forest. In 2019 includes \$24m placement
- Closing cash of \$31m
- R&D tax credit of \$6.2m received October 2019

Refer to and June 2019 Quarterly Shareholder Update for additional financial information

# New Drug Development

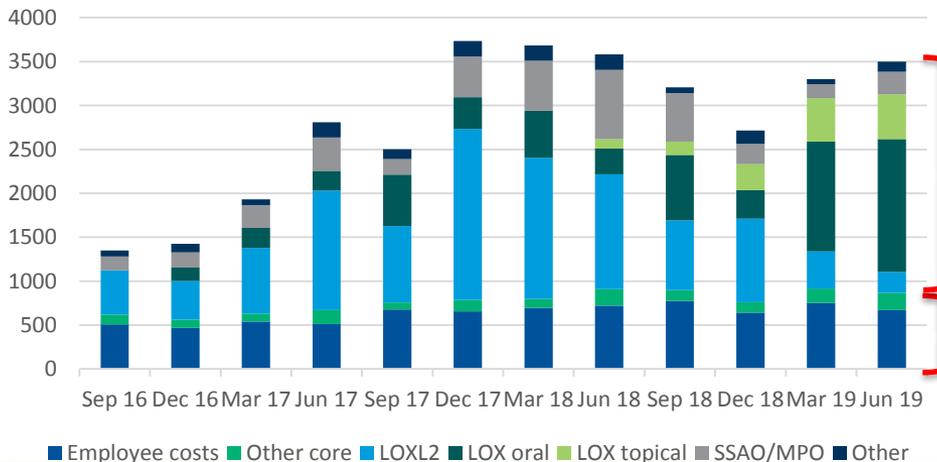
## Expenses (financial years ended 30 June)

New Drug Development Expenses



- Employee and other core costs are stable and a small percentage of total drug development expenditure
- Drug discovery and clinical trial costs are the major component of expenditure
  - these are external costs
  - vary on a project by project basis as drugs progress through development – see bottom graph
- 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.

New Drug Development Expenses - Quarterly

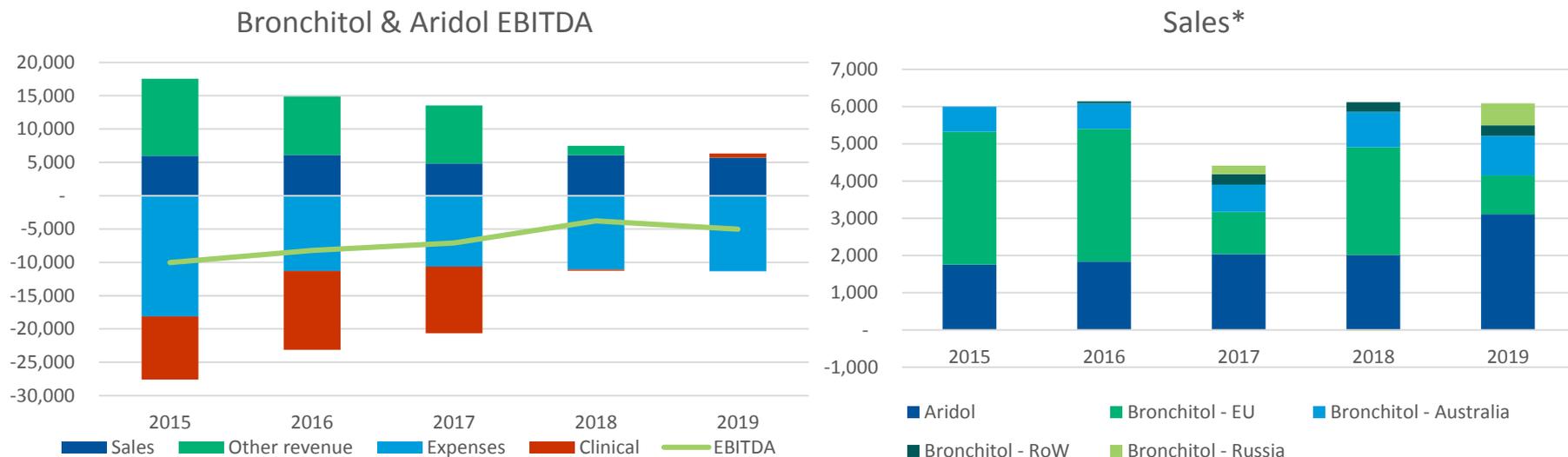


Preclinical development and clinical trial costs incurred to progress drug candidates from successful research programs.

Core fixed operating costs

# Mannitol Business (Bronchitol & Aridol)

## Segment profitability & sales analysis (financial years ended 30 June)



### Path to profitability: increase revenue to leverage cost base

- Core cost base relatively fixed vs sales volume
- Reimbursement of Bronchitol in Russia achieved 1 January 2019. (Sales in 2019 reduced by credit note of \$411k in relation to price change and expired inventory held by distributor – one off.)
- US approval – Subject to FDA approval (~Q1 CY 2020), launch milestone of US\$10m and sales commence in Q2 CY 2020
- Other Bronchitol sales growth opportunities
  - Chiesi territory expanded to include Greece, Nordic
  - EffRx appointed as Swiss distributor in June 2019
- Aridol: US launch Dec 18; Canada – approval in June 2019, launch H2 2019
- FY 2019 includes reimbursement of CF303 clinical trial costs

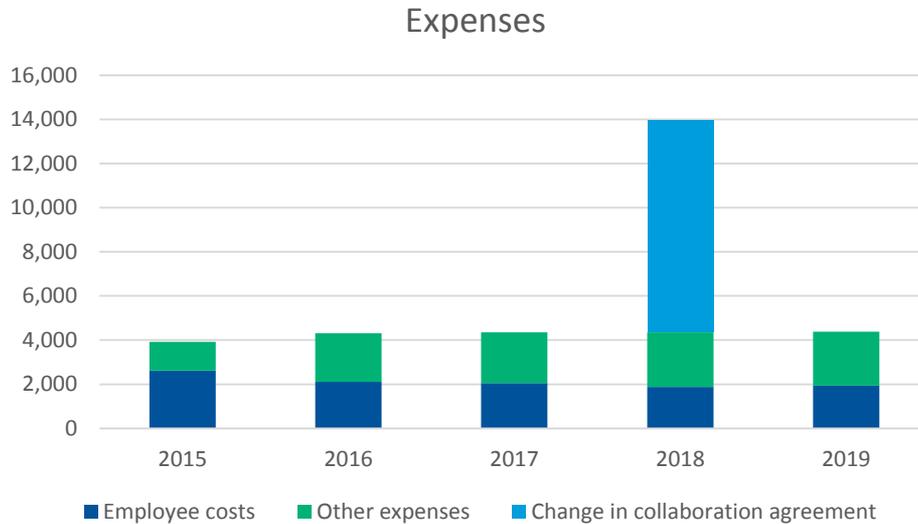
### Revenue\*

- FY 2015: Direct to pharmacy until June 15 (ie all sales revenue to PXS)
- FY 2016: EU sales via distributors at lower margin (~50%) to PXS. Chiesi builds inventory levels
- FY 2017: First sale to Russia
- FY 2018: Growth in EU (Chiesi) & Australia (expanded PBS coverage)
- FY 2019:
  - Aridol includes US relaunch.
  - Major EU distributor order moved from H1 to H2 CY 2019
- Other revenue in all years is predominantly reimbursement of clinical trial costs by US partner

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

# Corporate

## Expenses (financial years ended 30 June)

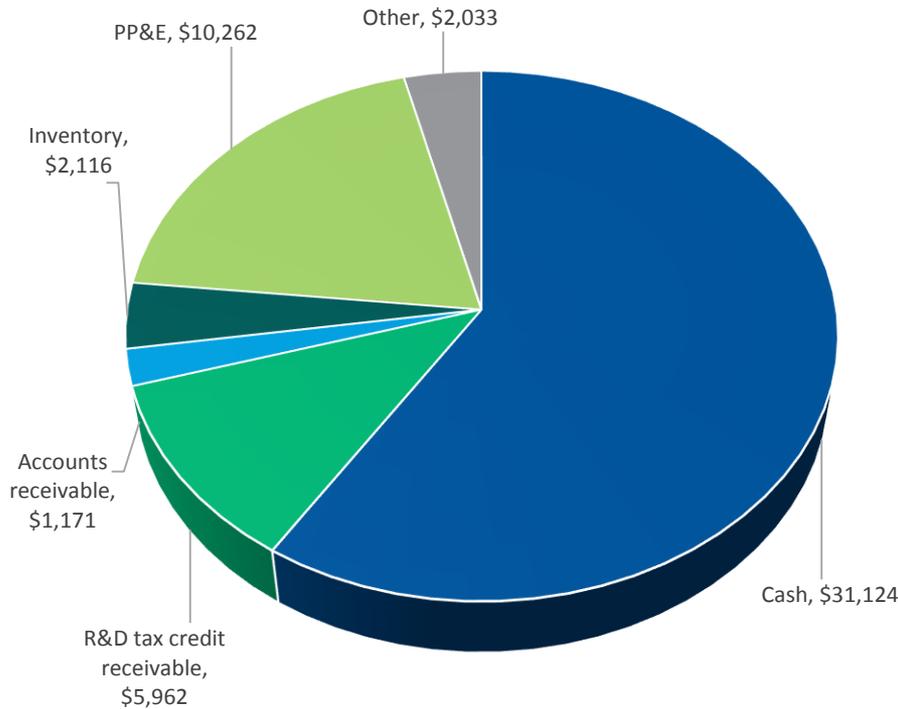


- Employee and other costs stable
- One-off expense in H1 FY 2018 to change collaboration agreement with Synairgen

# Balance sheet

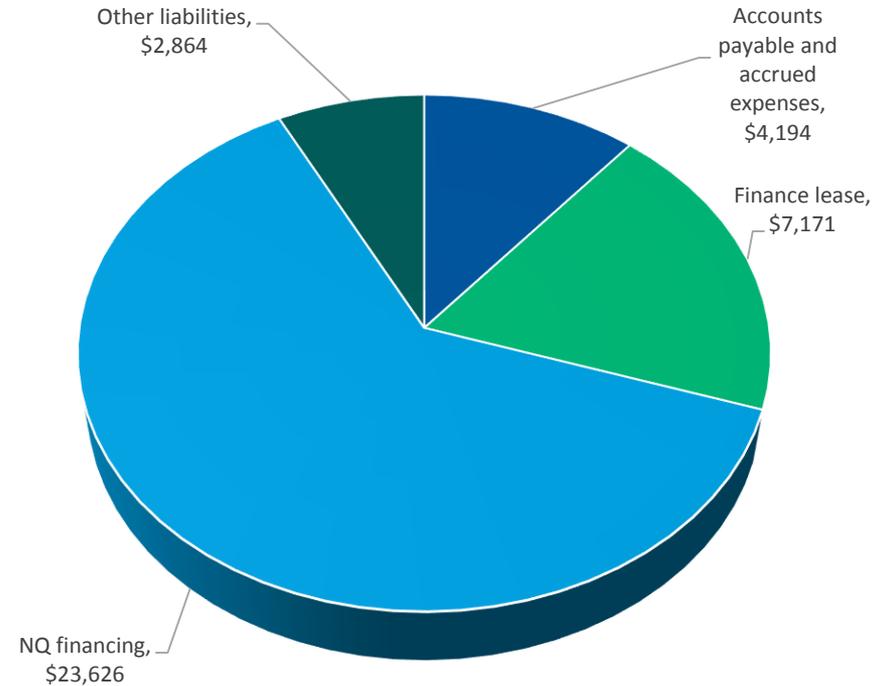
30 June 2019

## Assets (\$52.7m)



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

## Liabilities (\$37.9m)



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

# Shareholders & trading



Financial Information	
ASX Code	PXS
Market Cap <sup>1</sup>	\$95m
Shares on Issue <sup>1</sup>	395m
Employee Options <sup>1</sup>	19m
Liquidity (turnover last 12 months) <sup>1</sup>	45m shares
Share price <sup>1</sup>	\$0.24
Analyst valuation <sup>2</sup>	\$0.54
Cash Balance (30 June 19)	A\$31m

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%
<b>Total Institutional Ownership</b>	<b>58%</b>

1. As at 20 November 2019

2. Bell Potter Securities Research 24 June 2019