



2020 AGM| 4 November 2020 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. 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 developing or partnering any of the 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.

## **Executive Summary**

Transformative impact of FDA approval on Pharmaxis operations

**FDA approves** Bronchitol for adult Cystic Fibrosis patients triggering approval and launch **milestones totaling US\$10m** 

Access to large US market **transformative** with mannitol business cash flow positive from FY 2021 onwards

Cash position strengthened\* and increased sales provide **cash runway to H2 2022** with potential cost savings and further opportunities to extend

pharmaxis

Best in class LOXL2 inhibitor in partnering discussions for entry into phase 2 trials for chronic fibrotic disease

Breakthrough clinical program launched for PXS-5505 with disease modifying potential in Myelofibrosis. Phase 2 trial funded to conclusion, begins Q1 2021

Global scientific and clinical collaborations to **extend value** of PXS-5505 into other myeloproliferative diseases and cancer indications

Strong pipeline of assets supported by funding and collaborations with industry, government and academia until commercial opportunity is clear

# Experienced Scientific Leadership Team

Significant experience in drug development, commercialisation and partnering

### In senior management



### 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-Plank Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany





### **Gary Phillips – CEO and Managing Director**

- 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



### **Dieter Hamprecht – Head of Chemistry**

- more than 20 years experience with small molecule and peptide experience, contributed to greater than 10 drug candidates brought to development and co-inventor of 50 patent families, co-author of 30+ scientific publications
- previously Managing Director Boehringer Ingelheim's research group in Milan
- senior medicinal chemistry positions at GSK



### Kathleen Metters - Non Executive Director

- former Senior Vice President and Head of Worldwide Basic Research for Merck
   & Co. with oversight of all the company's global research projects.
- in a subsequent role at Merck &Co she led work on External Discovery and Preclinical Sciences
- former CEO of biopharmaceutical company Lycera Corp



#### **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



### Neil Graham - Non Executive Director

- former VP of immunology and inflammation responsible for strategic program direction overseeing pipeline development and clinical programs at Regeneron (REGN:US)
- former SVP program and portfolio management at Vertex Pharmaceuticals
- former Chief Medical Officer at Trimeris Inc and Tibotec Pharmaceuticals

# FDA approves Bronchitol for Cystic Fibrosis

US Marketing Authorisation granted on 30 October, 2020

FDA approval as "add-on maintenance therapy to improve pulmonary function in adult patients 18 years of age and older with cystic fibrosis"

US CF market >65% of global market

 US market doubles global cystic fibrosis patient opportunity with attractive pricing



### **Chiesi US License**

- Chiesi approval /launch milestone payments US\$10m
- US sales commence in H1 CY 2021
- High teens royalty + long term supply contract - ~20% of Chiesi US Bronchitol net sales flow directly to Pharmaxis bottom line
- Three sales milestones totaling US\$15m payable on achieving annual sales thresholds

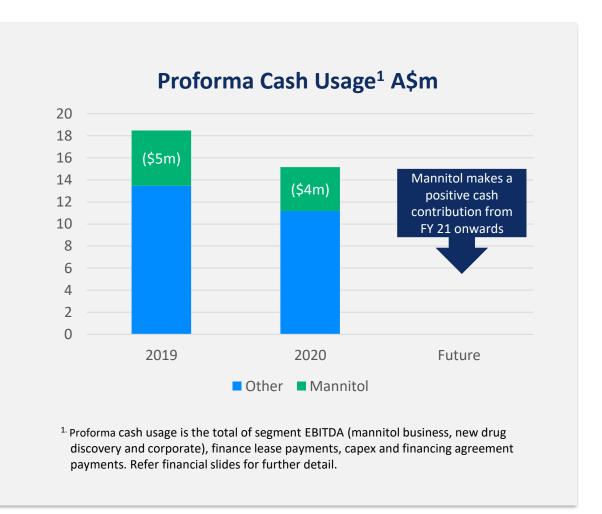
# Strengthened cash position

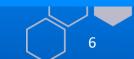
### Further opportunities to extend cash runway ahead

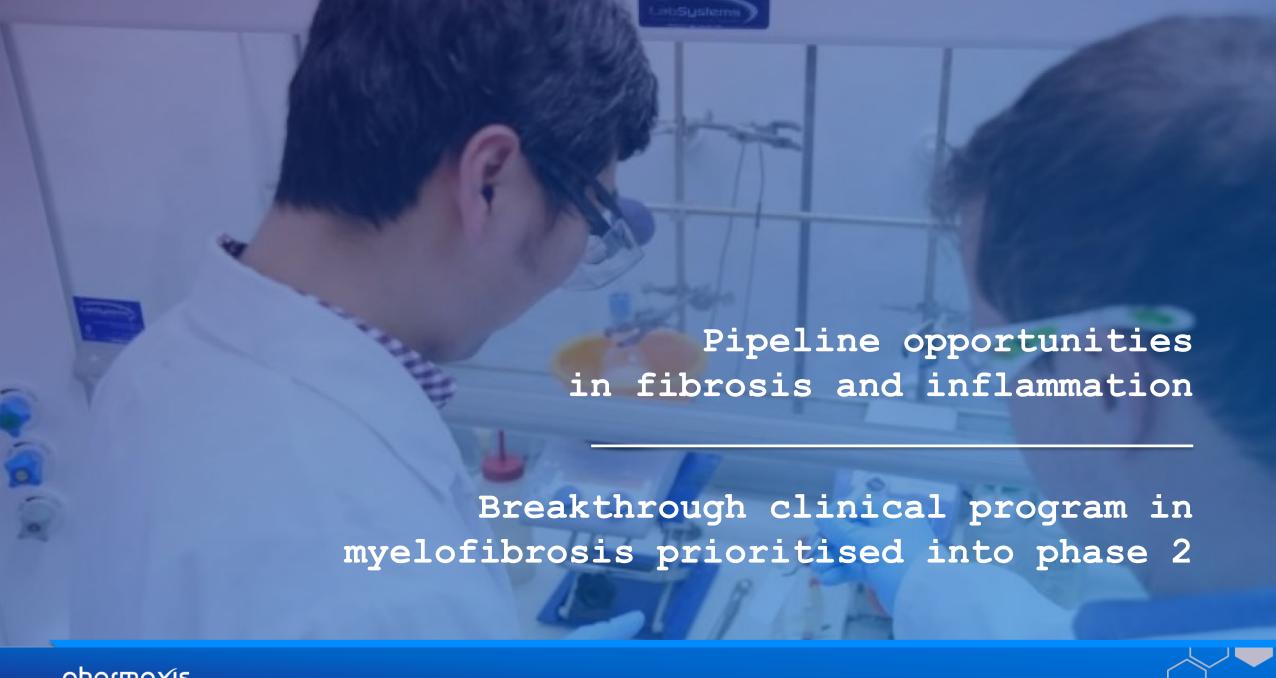
- June 20 proforma cash balance of A\$34m
  - Cash June 2020: A\$20m (includes \$5m R&D tax credit received Oct 2020)
  - Chiesi milestone payments ~A\$14m
     (US approval US\$7m and launch stock US\$3m)
- Mannitol business to go from cash burn (FY 20: A\$4m) to cash flow positive from FY 21 onwards (FY 26: A\$10m+)

Year	2019	2020	From 2021 E
EBITDA (A\$m)*	(\$5.0)	(\$4.0)	Cash Flow Positive

- Further opportunities to extend cash runway
  - Mannitol potential cost savings
  - Distribution license fees from additional Aridol and Bronchitol territories
  - Pipeline supported by grants and R&D tax credit (~A\$5m 2020)
  - Partnering deals with pipeline assets (eg LOXL2)







# First in class PXS-5505 fast tracked into the clinic

Novel anti fibrotic approach with broad applications in difficult to treat cancers



# Myelofibrosis: Orphan Disease with high unmet need forecast to exceed US\$1b

- Drug with disease modifying potential patented 2018
- Long term tox and phase 1 studies completed 1H 2020
- FDA orphan status granted July 2020
- IND approved August 2020
- Fully funded phase 1/2a proof of concept myelofibrosis study starts Q1 21



# Adjunct to best standard of care in multiple cancers

- Pan-LOX inhibition synergistic with current standard of care and pharma development pipeline in many stromal cancers
- Academic and clinical interest to explore independent grant funded studies; eg Myeloproliferative disorders, liver carcinoma, pancreatic cancer, etc
- International studies facilitated by IND approval and availability of drug product

# Myelofibrosis - other programs

PXS-5505 unique mechanism of action promises disease modification and good tolerability

Company	Market cap <sup>(1)</sup>	Bourse	Asset	Description	Clinical phase
Constellation PHARMACEUTICALS	\$1.1bn	Nasdaq	CPI-0610	BET inhibitor	Phase 2 data
KARTOS THERAPEUTICS	\$0.7bn <sup>(2)</sup>	n.a. – private	KRT-232	MDM2 antagonist	Phase 2
geron	\$0.5bn	Nasdaq	Imetelstat	Telomerase inhibitor	Phase 2 data
phormoxis	\$23.6m (A\$32.9m)	ASX	PXS-5505	Pan-LOX inhibitor	Phase 2 ready

Existing pipeline in development all have challenging safety / side effect profiles

PXS-5505 mechanism of action expected to deliver additional efficacy on top of existing standard of care and/or known pipeline drugs without adding to tolerability issues

PXS-5505 unique mechanism of action with expected good efficacy AND tolerability

# Pipeline opportunities in fibrosis and inflammation

Funding of PXS-5505 prioritised

Product Candidate	Indications	Pre- clinical	Phase 1	Phase 2	Next Steps	
Pan-LOX; PXS-5505	Myelofibrosis	MF	-101		<ul> <li>Phase 2 commencing Q1 2021</li> </ul>	
Pan-LOX; PXS-5505	Liver and pancreatic cancer				<ul> <li>Protocol and funding discussions v independent investigators</li> </ul>	with
LOXL-2; PXS-5382	Anti fibrotic CKD / IPF / NASH				<ul> <li>Partnering discussions for phase 2 commencement</li> </ul>	
Pan-LOX; PXS-6302	Anti scarring; Burns, established scars		l		<ul> <li>Phase 1 IIS* commencing Q4 2020</li> <li>IIS patient studies in burns and established scars 2H 2021</li> </ul>	)
SSAO/MAOB; PXS-4699	Anti inflammatory Muscular Dystrophy				<ul> <li>\$1m matched funding grant</li> <li>DMD TACT committee Q2 2021</li> <li>Advance to the clinic H1 2022</li> </ul>	
SSAO/MPO; PXS-5370	Anti inflammatory Multiple indications				Grant identification in process	
SSAO; PXS-4728A	Anti inflammatory Neuro inflammation				<ul> <li>Evaluate Boehringer data package opportunity to repurpose</li> </ul>	and

# Anticipated news flow: 2020 - 2021

Transformative impact of FDA approval on Pharmaxis operations

### Q4 2020

- FDA approval for Bronchitol to treat adult cystic fibrosis patients on October 30, 2020
- Chiesi US\$7m milestone due December 2020

### H1 2021

- Breakthrough drug PXS-5505 phase 1c/2a myelofibrosis study commences recruitment
- Chiesi pay US\$3m milestone on Pharmaxis shipment of US launch
- Cash receipts from sale of US Bronchitol launch stock
- Mannitol business simplification completed realising annual cost savings
- Best in Class LOXL2 inhibitor partnering

### H<sub>2</sub> 2021

- First collaborations to progress PXS-5505 into clinical trials in other myeloproliferative diseases and/or cancer indications
- Cash receipts from royalties on US Bronchitol sales commence
- LOX topical drug enters independent investigator patient studies
- Feedback from global advisory committee on development fast tracking for Duchenne muscular dystrophy clinical trials.





2020 AGM| November 2020 David McGarvey CFO

# Financials

### Cash

2020	2019	Notes
14,764	31,124	
5,048	5,962	Received \$5,048 on 14 October 2020
~14,000	-	US\$7m on approval (Q4 2020); US\$3m supply launch stock (Q1 21)
~\$33,812	\$37,086	<del></del>
(13,284)	(19,798)	2019 R&D tax credit received 2020
(574)	(981)	PP&E and patents
(2,232)	(1,593)	Frenchs Forest facility lease liability
(270)	(254)	Novaquest obligation - mid single digit % of Chiesi US sales for 7 years from launch
-	22,677	
(\$16,360)	\$51	
	14,764 5,048 ~14,000 <b>~\$33,812</b> (13,284) (574) (2,232) (270)	14,764 31,124 5,048 5,962 ~14,000 - \$33,812 \$37,086 (19,798) (574) (981) (2,232) (1,593) (270) (254) - 22,677

<sup>1.</sup> Lease over 20 Rodborough Rd (to 2024) – total liability at 30 Jun 2020: \$8.2 million

<sup>2.</sup> NovaQuest financing – not repayable other than as % of US & EU Bronchitol revenue – up to 7 years

# **Financials**

## Income statement highlights

Financial years ended 30 June (A\$'000)	2020	2019	Notes
Mannitol business			
Sales & other revenue	7,047	5,703	
Expenses <sup>1</sup>	(11,122)	(11,337)	Manufacturing costs relatively fixed
Mannitol EBITDA	(4,075)	(5,634)	
New drug development			
Clinical	(2,730)	(2,975)	
Drug discovery	(3,709)	(6,308)	
Employee & other	(3,833)	(3,443)	
R&D tax incentive	5,159	5,962	43.5% of eligible expenses including employee and other costs
Drug development EBITDA	(5,113)	(6,764)	
Corporate EBITDA	(2,990)	(3,874)	
Total adjusted EBITDA	(12,178)	(16,272)	
Bronchitol clinical trial refunds	98	621	Excludes clinical trial refunds from prior years
Interest income & finance lease charges	(221)	324	
Other reconciling items	(1,642)	(4,731)	
Profit before & after tax	(13,943)	(20,058)	

# Shareholders & trading



Financial Information	3 Nov 20
ASX Code	PXS
Market Cap	A\$42m
Shares on Issue	397m
Employee Options	19m
Liquidity (turnover last 12 months)	276m shares
Share price	A\$0.105
Proforma cash balance (30 June 2020)	A\$34m

Institutional Ownership	3 Nov 20
BVF Partners (US)	20%
D&A Income Limited	7%
Other Institutions	9%
Total Institutional Ownership	36%