

Quarterly Shareholder Update – March 2021



Dear Shareholder,

2021 is all about achieving milestones that deliver a streamlined business model geared to unlock the potential in our pipeline of clinical stage assets.

The significant achievements of 2020 that saw the FDA approve Bronchitol and grant an IND for our myelofibrosis drug PXS-5505, provide the foundations for future success and the March quarter saw us get some early and significant runs on the board.

- **PXS-5505 starts international phase 1c/2 study in myelofibrosis**

We have opened clinical trial sites in Australia and South Korea; two countries where COVID -19 is not significantly affecting recruitment and are already planning to add more sites in the US in anticipation of the phase 2 dose expansion phase starting in the second half of 2021. This study which is due to conclude by the end of 2022 will generate safety and efficacy data in a deadly disease where patients have limited treatment options.

- **PXS-6302 starts phase 1/1c study in skin scarring**

A major step forward in our collaboration with Prof Fiona Wood's research group at the University of Western Australia (UWA) in Perth. The promise shown by PXS-6302 in pre-clinical studies was such that we are now launching into human studies. The healthy volunteer safety study started recruiting this last quarter and after it reports later this year the UWA will transition into a study in patients with scars. Professor Wood's vision is to achieve scarless wound healing – something that would have huge benefits for patients and significant commercial value.

- **Bronchitol delivers significant non-dilutive cash as mannitol respiratory business moves into profit**

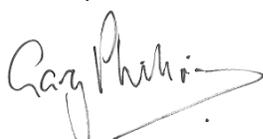
The US\$3m milestone paid by Chiesi in Q1 21 following shipping of the Bronchitol launch stock plus the sale of Russian market rights for ~A\$2m comes hard on the heels of the US\$7m received in Q4 20 for the FDA approval. The sale of Russian rights also cuts ~A\$1m of annual costs out of our business. Expect further initiatives currently underway to generate non-dilutive cash and reduce operating expenses to be announced as they are completed.

- **Capital Raise strengthens balance sheet as Pharmaxis targets the delivery of clinical results in myelofibrosis and skin scarring by end of 2022**

In April we added just over \$4m to our cash reserves via a placement priced at a small premium to the market and saw a new shareholder Karst Peak Capital join our register with an 8.9% holding. Our other major shareholders including BVF Partners supported the raise pro rata. This, together with our other initiatives, strengthens the Pharmaxis balance sheet as we look to deliver results from the clinical studies for PXS-5505 and PXS-6302, results that if positive should see a significant re-rating of our market valuation.

So – a solid first quarter and a clear focus for the months ahead: drive recruitment in our clinical studies whilst continuing to deliver non-dilutive cash and cost savings from our business.

Sincerely,



Gary Phillips - Chief Executive Officer

Products and Pipeline at a glance

Disease/target	Drug	Status
Cystic fibrosis	Bronchitol	Approved
Asthma	Aridol	Approved
Neuro inflammation (SSAO inhibitor)	PXS-4728	Phase 2
Myelofibrosis (oral pan-LOX inhibitor)	PXS-5505	Phase 1c/2 commenced
Other cancers (oral pan-LOX inhibitor)	PXS-5505	Phase 1
Scarring (Topical pan-LOX inhibitor)	PXS-6302	Phase 1 commenced
Chronic fibrotic diseases (LOXL2 inhibitor)	PXS-5382	Phase 1 completed
Duchenne Muscular Dystrophy (dual SSAO/MAOB inhibitor)	PXS-4699	Pre-clinical

Impact of COVID-19

Pharmaxis' response to the COVID-19 global pandemic has been outlined in quarterly shareholder updates, where we have described the precautions the Company is taking to protect employees and to continue manufacturing and supply of its approved respiratory products.

The Company has continued an uninterrupted supply to local and global customers.

The effect on sales is discussed below. Overall, there are large variances in the impact of COVID between markets/countries, and while we are seeing a recovery of Aridol sales in countries where COVID-19 is brought under control, Bronchitol continues to lag pre-COVID-19 sales levels. We are working with our commercial partners to better understand and respond on a country by country basis.

Importantly, there has not been to date any significant impact of COVID-19 on the various clinical trials in which the Company has been and is now involved with the 1c/2a trial in myelofibrosis and the phase 1c trial in scarring

both dosing their first patients in the first quarter of 2021.

Drug discovery

Oral pan-LOX inhibitor program (PXS-5505)

Pharmaxis' primary drug development initiative is its pan-Lysyl Oxidase (pan-LOX) inhibitor program focused on the rare bone cancer myelofibrosis. PXS-5505 is an orally taken drug that inhibits the lysyl oxidase family of enzymes and was developed from the Company's amine oxidase chemistry platform. In pre-clinical models of myelofibrosis PXS-5505 reversed the bone marrow fibrosis that drives morbidity and mortality in myelofibrosis and reduced many of the abnormalities associated with this disease.

During the quarter the first patient was enrolled in a phase 1c/2a trial cleared by the FDA under the Investigational New Drug (IND) scheme. The study aims to demonstrate that PXS-5505 is safe and effective as a monotherapy in myelofibrosis patients who are intolerant, unresponsive or ineligible for treatment with approved JAK inhibitor drugs.

Pharmaxis has completed site initiation at several Australian and South Korean hospitals. The initial dose escalation phase of the study aims to select the optimum dose of PXS-5505 and will recruit up to 18 patients. It is expected to conclude and report in H2 2021 and will be followed by a six-month dose expansion phase (24 patients) to evaluate safety and efficacy. Sites in other countries including the USA will be added for the dose expansion phase.

Read the media release [here](#).

Myelofibrosis is a cancer with a poor prognosis and limited therapeutic options. Pharmaxis believes that the current treatments can be augmented by use of a pan-LOX inhibitor and the combination should be disease modifying in a market that is conservatively worth US\$1 billion per annum.

PXS-5505 was granted Orphan Drug Designation by the US Food and Drug Administration (FDA) in July 2020.

While Pharmaxis' primary focus is the development of PXS-5505 for myelofibrosis, the

drug also has potential in several other cancers including myelodysplastic syndrome, liver and pancreatic cancers, melanoma and glioblastoma, where it aims to breakdown the fibrotic tissue in the tumour and enhance the effect of existing chemotherapies. Pharmaxis has a number of scientific collaborations with centres of excellence across the world who have shown interest in PXS-5505. The Company aims to support these and encourage the use of PXS-5505 in independent investigator initiated clinical studies.

Topical pan-LOX inhibitor program (PXS-6302)

The Company has a second pan-LOX program that has developed a drug for topical application with the potential for use in scar revision, keloid scarring and scarring from burn wounds.

The Pharmaxis discovery, PXS-6302, has shown promising pre-clinical results in inhibiting the enzymes that play a critical role in the development of scar tissue.

At the end of the quarter the Company announced the commencement of a phase 1 trial being conducted by a group of researchers from the University of Western Australia (UWA) led by Professor Fiona Wood AM, and the Fiona Stanley Hospital. The clinical trial will first determine the safety and tolerability of PXS-6302 in healthy volunteers, and then proceed to trials in patients with scarring subsequent to burn injury and also established scars.

Read the media release [here](#).

SSAO inhibitor program (previously partnered with Boehringer Ingelheim) (PXS-4728)

The PXS-4728 development program undertaken by Boehringer Ingelheim (BI) from 2015 to 2020 was returned to Pharmaxis during the quarter, including the extensive preclinical, clinical, safety and regulatory work they carried out. Further analysis of the data package by Pharmaxis scientists has uncovered some potential in neuro inflammatory diseases where the clinical benefits would not be impacted by the BI findings that caused BI to discontinue development. Pharmaxis is in discussion with independent investigators and potential partners in relation to neuro inflammatory indications, study protocol design and funding options including grants.

LOXL2 inhibitor program (PXS-5382)

The Lysyl Oxidase Like 2 (LOXL2) enzyme is fundamental to the fibrotic cascade that follows chronic inflammation in kidney fibrosis, the liver disease NASH, cardiac fibrosis and idiopathic pulmonary fibrosis (IPF) and it also plays a role in some cancers.

The Pharmaxis drug discovery group developed a small molecule inhibitor to the LOXL2 enzyme (PXS-5382) that has completed phase 1 clinical trials and 3-month toxicology studies.

Pharmaxis is currently pursuing a number of different options to enable PXS-5382 to enter the clinic in phase 2 trials in a chronic kidney disease. The Company continues to have discussions with potential partners and independent investigators in relation to study protocol design and funding options including grants.

Mannitol respiratory business

Bronchitol and Aridol

Bronchitol® (mannitol) is an inhaled dry powder for the treatment of cystic fibrosis (CF) and has been the subject of three large scale global clinical trials conducted by Pharmaxis. The product is approved and marketed in the United States, Australia, Europe, Russia and several other countries.

Aridol® is an innovative lung function test designed to help doctors diagnose and manage asthma. Aridol is approved for sale in Australia, major European countries, the United States, Canada and South Korea.

Bronchitol

United States

The Company's US partner Chiesi Group is responsible for the commercialisation of Bronchitol in the United States. Subsequent to the approval of Bronchitol on 30 October 2020 by the US Food and Drug Administration (FDA), Chiesi announced the commercial availability of Bronchitol in the second half of the March quarter.

Read the media release [here](#).

A total of US\$10 million (A\$13.8 million) in milestones has now been paid by Chiesi to Pharmaxis in relation to the approval and shipment by Pharmaxis of launch stock – US\$3 million (A\$3.9 million) in the March quarter.

Pharmaxis expects Bronchitol sales in the US market to contribute strongly to the product's global sales and profit growth from its launch. The Pharmaxis mannitol business is forecast to be cash flow positive from FY 2021.

Pharmaxis will earn high teen percentage of Chiesi net sales and will be the exclusive supplier of Bronchitol for the US market - on a long term, cost-plus basis. Three sales milestones totalling US\$15m are also payable on achieving annual sales thresholds.

The additional volume of Bronchitol that Pharmaxis will produce to supply the US, on top of Australia and 17 other international markets, will greatly increase factory capacity utilisation and radically improve the unit cost of goods.

Western Europe

In the EU, Chiesi is the Pharmaxis exclusive Bronchitol distributor for the markets of the UK, Ireland, Germany, Italy, Norway, Sweden, Finland, Denmark, Cyprus, Spain and Greece.

Pharmaxis also markets Bronchitol in Austria via its German based logistics provider and plans to market in Switzerland via an exclusive distributor that is currently applying for approval.

Russia

Russia represents a valuable Bronchitol market for Pharmaxis, now established as a fast-growing business bringing a new drug to Russian cystic fibrosis patients.

Subsequent to quarter end Pharmaxis announced the sale of Bronchitol distribution rights in Russia to GEN İlaç ve Sağlık Ürünleri San. ve Tic. A.Ş. (GEN), a trusted Pharmaxis business partner in other territories for more than seven years. GEN will take on full responsibility for Bronchitol within Russia. Pharmaxis will continue to manufacture and export Bronchitol to Russia from its factory in Sydney.

The sale of the distribution rights for Bronchitol in Russia is effective May 1st 2021.

Pharmaxis will receive a €1.25 million (~A\$2m) distributor appointment fee, seventy percent of which is now payable with the remaining thirty percent due in twelve months. Importantly, Pharmaxis will reduce selling, marketing and regulatory expenses by a total of approximately A\$1 million per annum as a result of the transfer of commercial and product responsibilities to GEN.

Read the media release [here](#).

Other territories

Bronchitol is sold in Australia by Pharmaxis and in Turkey, the Czech Republic and Hungary by specialist distributors.

Bronchitol sales

Bronchitol sales for the three and nine months ended 31 March 2021 and 31 March 2020 are as follows:

	Quarter		Half year	
	2021	2020	2021	2020
Australia	206	423	750	994
Western Europe	135	163	255	1,025
Eastern Europe	254	77	421	223
Russia	-	(33)	1,365	556
United States	839	-	839	-
Total	\$1,434	\$630	\$3,630	\$2,798

Pharmaxis supplied stock to the US ahead of the commercial launch by Chiesi in March. Product sold to the US is invoiced in two stages – the first when the product is supplied to Chiesi and the second when the product is sold by Chiesi into its distribution chain. The two invoicing stages are approximately equal in value.

Pharmaxis Bronchitol distributors typically order on a six monthly basis. Pharmaxis ex-factory sales for the current quarter and half year reflect the buying patterns of its international distributors. Larger orders for Western Europe and Russia are forecast in the June 2021 quarter.

In Western Europe, disruptions caused by the COVID-19 pandemic continue to impact in-market

unit sales of Bronchitol by Chiesi in the UK, Germany, Italy and the Nordics. For the March 2021 quarter, in-market unit sales were 46% lower than the March 2020 quarter and 33% lower than the December 2020 quarter. However, for the twelve months ended March 2021 unit sales were 11% lower than the twelve months ended March 2020.

In Australia where Pharmaxis sells directly to clinics, in-market unit sales for the March 2021 quarter were 51% lower than the March 2020 quarter and 31% lower than the December 2020 quarter. For the twelve months ended March 2021 unit sales 22% lower than the twelve months ended March 2020.

The Company continues to monitor the situation whilst working with our commercial partners to better understand and respond on a country by country basis.

Aridol sales

At the beginning of the COVID-19 pandemic a number of countries, including those where Aridol is sold, provided advice to respiratory specialists to limit all lung function testing to more severe cases due to health risks arising from patients exhaling multiple times with force as part of the test. In the markets where Pharmaxis sells Aridol directly to lung function testing laboratories (Australia and Europe) sales have reduced on a state and country basis consistent with the impact of the pandemic and this impact continues, particularly in Europe.

In Australia sales for the March 2021 quarter were marginally lower than the March 2020 quarter. Looking over a longer time period, sales for the nine months ended March 2021 were 18% lower than the nine months ended March 2020.

In Europe the impact of COVID19 is greater. Sales for the March 2021 quarter were 47% lower than the quarter March 2020, but pleasingly 30% higher than the December 2020 quarter. Sales for the nine months ended March 2021 were 50% lower than sales for the nine months ended March 2020.

The Company continues to monitor the situation.

Aridol sales for the three and nine months ended 31 March 2021 and 31 March 2020 are as follows:

\$'000	Three months		Nine months	
	2021	2020	2021	2020
Australia	109	118	310	377
Europe	181	339	422	841
USA & Canada	-	-	98	73
South Korea	-	-	350	257
Total	\$290	\$457	\$1,180	\$1,548

Corporate

Pharmaxis completes \$4.4 million placement

Subsequent to the end of the quarter Pharmaxis completed a \$4.4 million placement to institutional investors, issuing approximately 54.6m shares within the Company's 15% placement capacity under ASX Listing Rule 7.1. The placement issue price of A\$0.08 represented a 1.3% premium to last closing price on 12 April 2021.

The purpose of the raising was to strengthen the balance sheet as the Company conducts a phase 1/2 study in myelofibrosis with its lead drug PXS-5505 and a phase 1c study in patients with problematic skin scarring with its topical drug PXS-6302.

The placement received strong support from Hong Kong and Sydney based Karst Peak Capital Limited which invested A\$3.2m, giving it a 8.9% shareholding. Karst Peak is an investment management firm focusing on equity investments in listed companies in the consumer, healthcare, and technology sectors in Asia and Australasia.

Both of the Company's substantial shareholders, BVF Partners LP and D&A Income Limited invested to maintain their shareholdings in Pharmaxis of 19.5% and 7% respectively.

Due to the Board's desire to limit the size of the raising, and the issue price of the raising being at market, a retail issue was not pursued.

Read the Media Release [here](#).

Recent interviews and articles

Pharmaxis has featured in a series of positive investor news reports focused on recent announcements.

- Biotech Daily: Dr Boreham's Crucible: "Why is Pharmaxis' valuation so 'pitiful'? Karst Peak doesn't know either". Read the article [here](#).
- Proactive: "Pharmaxis CEO talks \$4.4m fundraise and sale of distribution rights in Russia for Bronchitol." Watch the interview [here](#).
- The Sentiment: Tim Boreham examines our expertise in fibrotic diseases and how it is being applied to find a new treatment for myelofibrosis - a potential billion dollar-plus market. Read the article [here](#).
- Proactive: "Pharmaxis CEO Gary Phillips details world-first clinical trial of treatment to prevent

wound and burn scars". Watch the interview [here](#).

- Proactive: "Pharmaxis begins exporting its cystic fibrosis drug Bronchitol® to the USA" Watch the interview [here](#).

Pharmaxis investment summary

Pharmaxis recently published an updated investment summary - available on the company [website](#).

Pharmaxis investor presentation

View the current investor presentation [here](#).

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Financials

Key financial metrics

	A\$'000	Three months ended		Nine months ended	
	(unaudited)	2021	2020	2021	2020
Segment results – adjusted EBITDA					
New drug development					
Oral pan-LOX (external costs)		(582)	(948)	(1,905)	(2,348)
Topical pan-LOX (external costs)		(188)	(518)	(233)	(858)
Other program external costs (net of grants)		(291)	(616)	(1,021)	(1,354)
Employee costs		(741)	(943)	(2,540)	(2,472)
Overhead		(14)	(70)	(252)	(351)
R&D tax credit		-	-	148	259
EBITDA		(1,816)	(3,095)	(5,803)	(7,124)
Mannitol respiratory business					
Sales		1,724	1,087	4,810	4,346
Other income		3,899	5	13,997	15
		5,623	1,092	18,807	4,361
Expenses – employee costs		(1,268)	(1,516)	(4,182)	(4,553)
Expenses – manufacturing purchases		(417)	(79)	(1,589)	(825)
Expenses – other		(979)	(961)	(3,353)	(2,716)
EBITDA		2,959	(1,464)	9,683	(3,733)
Corporate – EBITDA		(777)	(582)	(2,801)	(2,283)
Total Adjusted EBITDA		\$366	(\$5,141)	\$1,079	(\$13,140)
Net profit(loss)		(\$951)	(\$9,539)	(\$905)	(\$19,858)
Statement of cash flows					
Cash inflow/ (outflow) from:					
Operations		(1,296)	(4,863)	3,752	(8,555)
Investing activities		(152)	(130)	(433)	(458)
Financing activities		(636)	(620)	(1,918)	(1,860)
Total cash generated/(used)		(\$2,084)	(\$5,613)	\$1,401	(\$10,873)
Cash at bank		\$16,165	\$20,251	\$16,165	\$20,251

Highlights

- New drug development
 - Oral pan-LOX expenditure in the three and nine months relates to the phase 1c/2a clinical trial in myelofibrosis that commenced patient dosing during the first quarter, and a small amount in support of pre-clinical work by a European university in relation to the effectiveness of PXS-5505 in models of myelodysplastic syndrome. Prior period expenditures relate to the phase 1a/b and longer term tox studies completed in order to proceed to phase 1c/2a.
 - Other program external costs in the three and nine months includes pre-clinical and preliminary phase 1 clinical trial costs in relation to the topical pan-LOX program targeting scarring, and

preclinical work on the SSAO/MAOB program targeting Duchenne Muscular Dystrophy, co-funded by the Biomedical Translation Bridge (BTB) program from September 2020. Prior period expenditure also includes preclinical development of the Company's topical pan-LOX inhibitor, and a small phase 1 trial of the Company's LOXL2 inhibitor.

- Mannitol respiratory business
 - See above for detail and commentary in relation to Bronchitol and Aridol sales for the quarter.
 - Other income includes the milestones received from Chiesi subsequent to the approval of Bronchitol in the United States on 30 October 2020 (US\$3 million for the quarter and US\$10 million for the nine months), and a US\$100,000 milestone received in the September quarter in relation to approval of Bronchitol in Brazil.
 - The increase in expenses for both the nine months reflect increased third party support of a routine European safety audit and increased manufacturing activity associated with the US launch of Bronchitol.
- Corporate
 - The increase in net expenses includes net foreign exchange losses and legal and consultant costs associated with business development activities.
- Net profit (loss)
 - The difference between total adjusted EBITDA and net profit(loss) primarily relates to non-cash items (depreciation, amortization, share based payment expense) and foreign exchange rate gains and losses.
- Cash
 - The Company finished the quarter and half with \$16 million in cash.
 - In April the Company completed an institutional placement raising \$4.1 million net of expenses
 - In April the Company also announced the sale of Russian Bronchitol distribution rights for ~A\$2 million to be received 70% immediately and 30% in twelve months time. As a result of the sale the Company is able to reduce its sales and employees operating expenses by approximately A\$1 million per annum.

Additional Financial Information

Income statements and summary balance sheets are provided below.

Income statements

	A\$'000	Three months ended		Nine months ended	
	(unaudited)	31-Mar-21	31-Mar-20	31-Mar-21	31-Mar-20
Revenue					
Revenue from sale of goods		1,724	1,087	4,810	4,346
Chiesi US FDA approval milestone		4,033		13,982	
Interest		8	86	44	316
R&D tax incentive		0		148	259
Other		152	110	620	383
Total revenue		\$5,917	\$1,283	\$19,604	\$5,304
Expenses					
Employee costs		(2,617)	(3,040)	(8,817)	(9,045)
Administration & corporate		(621)	(456)	(1,841)	(1,609)
Rent, occupancy & utilities		(264)	(263)	(788)	(746)
Clinical trials		(616)	(1,024)	(1,895)	(2,093)
Drug development		(519)	(1,056)	(1,436)	(2,367)
Sales, marketing & distribution		(350)	(387)	(1,097)	(1,055)
Safety, medical and regulatory affairs		(285)	(188)	(1,262)	(675)
Manufacturing purchases and changes in inventory		(417)	(80)	(1,589)	(826)
Other		(82)	(128)	(208)	(502)
Depreciation & amortisation		(786)	(808)	(2,375)	(2,424)
Foreign currency exchange gains & losses		(196)	(3,249)	1,166	(3,370)
Finance costs		(115)	(143)	(367)	(450)
Total expenses		(6,868)	(10,822)	(20,509)	(25,162)
Net profit (loss) before tax		(\$951)	(\$9,539)	(\$905)	(\$19,858)
Income tax credit/(expense)					
Net profit (loss) after tax		(\$951)	(\$9,539)	(\$905)	(\$19,858)

Summary balance sheets

A\$'000 (unaudited)	31-Mar-21	30-June-20
Assets		
Cash	16,165	14,764
R&D tax incentive – received October		4,900
Accounts receivable	2,048	1,459
Inventory	2,681	2,630
PP&E	6,812	8,906
Other	2,925	2,757
	\$30,631	\$35,416
Liabilities		
Accounts payable and accrued expenses	2,053	2,765
Lease liability (Frenchs Forest facility)	6,800	8,154
Financing agreement (not repayable other than as a % of US Bronchitol revenue)	18,974	21,200
Other liabilities	1,690	1,866
	\$29,517	\$33,985
Net Assets	\$1,114	\$1,431