Quarterly Shareholder Update – December 2020

Dear Shareholder,

The December quarter saw a significant event with the FDA approval of Bronchitol and a consequent US$7m payment from our US partner Chiesi. I described this achievement as transformative for the Pharmaxis business as it triggers cash milestone payments and increased sales from our Sydney factory that will result in the mannitol respiratory business (Aridol and Bronchitol) becoming cash flow positive in 2021 and beyond. The FDA approval is further validation of the company's strategy and its partnership with Chiesi, and makes available a much-needed new medication for US cystic fibrosis patients.

There are further changes to the mannitol respiratory business planned as we seek to further reduce costs and reconfigure distributor relationships so we can capitalise on profitable manufacturing. In the meantime, the FDA approval allows us to focus our energy and resources on the drug discovery business. Following are some highlights from the last quarter:

- **PXS-5505 in final planning stage for international phase 1c/2 study**
  Following the positive FDA feedback on our IND submission in August Pharmaxis finalised the preparations for a myelofibrosis study and which is on track to start recruitment this March quarter. This study which is due to conclude by the end of 2022 will generate safety and efficacy data in this deadly disease where patients have limited treatment options.
- **PXS-5505 continues to shine in oncology pre-clinical studies**
  There has been a lot of research conducted historically into the role of the lysyl oxidase enzymes in a wide variety of cancers. The availability of Pharmaxis pan-LOX inhibitor PXS-5505 which inhibits all lysyl oxidases has stimulated independent scientific and clinical opinion leaders to propose new studies to prove the hypothesis that inhibiting lysyl oxidases will improve treatment outcomes with existing chemotherapies. That work continues into 2021 and we look forward to reporting on it as the peer reviewed publications appear.
- **Returned Boehringer Ingelheim drug PXS-4728A shows promise in neuro degenerative disease**
  Boehringer's decision to return our SSAO inhibitor was due to its off target inhibition of MAOB in the brain and the potential drug interactions that could flow from that. Following an extensive review of the Boehringer data sets Pharmaxis has identified a potential opportunity in neurodegenerative diseases where the joint inhibition of SSAO and MAOB enzymes would be neuroprotective. We have held initial discussions with key scientific and clinical opinion leaders in neurology and identified a number of potential indications. We are now exploring the clinical development path and commercial interest in this therapeutic area.
- **PXS-5382 LOXL2 inhibitor partnering**
  Our commercial partnering efforts with a number of companies slowed in the last quarter over concerns about value and the right choice of clinical development path. We're refocussing our strategy here and whilst NASH has been hit by a number of late stage clinical failures, we see promise for our pipeline drug PXS-5382 in a number of orphan renal diseases where some ground breaking pre-clinical work with PXS-5382 is awaiting publication. We are in discussions with our external scientific advisors on clinical development paths and I took the opportunity to check on commercial interest in kidney disease with a number of companies at the annual JP Morgan conference in January. I see some potential here that we will be following up.

Sincerely,

Gary Phillips - Chief Executive Officer

Products and Pipeline at a glance

Disease/target	Drug	Status
Cystic fibrosis	Bronchitol	Approved
Asthma	Aridol	Approved
Inflammation (AOC3 inhibitor)	PXS-4728	Phase 2
Myelofibrosis (oral pan-LOX inhibitor)	PXS-5505	Phase 1c/2 commencing
Other cancers (oral pan-LOX inhibitor)	PXS-5505	Phase 1
LOXL2 inhibitor	PXS-5382	Phase 1 completed
Topical pan-LOX inhibitor	PXS-6302	Phase 1 commencing
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, despite a significant reduction in international freight routes.

The effect on sales is discussed below. Overall we are seeing an increase in sales compared to earlier in the year, with large variances between markets/countries.

Importantly, there has not been to date any impact of COVID-19 on the various clinical trials in which the Company has been involved, and the upcoming phase 1c/2a trial in myelofibrosis is proceeding for the dosing of its first patient 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 focussed on the rare bone cancer myelofibrosis. The ant-fibrotic drug PXS-5505 was developed from the Company's amine oxidase chemistry platform, and is scheduled to enter into a 6-month phase 1c/2 clinical trial in 2021.

PXS-5505 has completed long-term toxicity studies and Phase 1a and 1b clinical trials demonstrating a well-tolerated drug that effectively inhibits all enzymes in the lysyl oxidase family that are involved in fibrosis.

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 and in August 2020 the FDA gave Pharmaxis the green light to proceed with a phase 1c/2a clinical trial for the treatment of myelofibrosis in adults.

The clinical trial protocol incorporates a one-month dose escalation phase followed by six months' treatment in an open label study of patients who are not on a JAK inhibitor. The company is well advanced in its preparations to start this study and expects patient recruitment in Q1 2021. The study is expected to conclude in 2022.

While Pharmaxis' primary focus is the development of PXS-5505 for myelofibrosis, the drug also has potential in several other cancers including liver and pancreatic cancers 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.

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

In quarter 3 of 2020 Boehringer Ingelheim advised it would terminate the 2015 agreement with Pharmaxis under which Pharmaxis had received a total of A$83 million in upfront and milestone payments.

Boehringer had completed a significant amount of development of PXS-4728 over its five year program including two phase 2a trials, one in NASH (fatty liver) and the other in diabetic retinopathy. The main reason stated by Boehringer for the return of the drug was the risk of dose dependent drug interactions of the compound in patients identified in a Phase 1 safety study. The concern centred on the unexpected finding that PXS-4728 inhibits the enzyme MAOB in the brain and could lead to some drug interactions.

Based on recent publications SSAO remains an important clinical target. Pharmaxis is currently reviewing the extensive data generated by Boehringer over its five year development program to evaluate potential opportunities in other indications that already have supportive pre-clinical data, where the risk of drug interactions are of less concern and where the inhibition of MAOB in the brain is a desired clinical effect. For example, MAOB is the target of a number of successful Parkinson's disease drugs and neuro inflammation is a hallmark of several neuro degenerative diseases including Parkinson's, Alzheimer's, Huntington's and some sleep disorders that are precursors to these diseases. Current data suggest that the early intervention with a dual SSAO/MAO-B inhibitor should be neuroprotective. Pharmaxis expects to have completed its review, including discussions with key opinion leaders and relevant pharma companies in the first half of 2021.

LOXL2 inhibitor program (PXS-5382)

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

The Pharmaxis drug discovery group developed two small molecule inhibitors to the LOXL2 enzyme that have completed phase 1 clinical trials and 3-month toxicology studies (PXS-5382 and PXS-5338) and currently focusses on PXS-5382 as an anti-fibrotic drug for systemic fibrosis.

Since the conclusion of phase 1 studies in 2019 the Company has engaged in scientific and commercial discussions with a large number of pharma and biotech companies to enable the drug to enter the clinic in phase 2 trials. Despite strong initial interest, the attractiveness of liver disease NASH as an indication has waned due to a number of late stage clinical failures in NASH with anti-fibrotic drugs and lingering concern over failed clinical studies with Gilead's LOXL2 antibody simtuzumab in a number of indications.

The company has in the meantime conducted head to head studies with the LOXL2 antibody in an agreement with Gilead to demonstrate the superiority of PXS-5382 and provide a rationale for Simtuzumab's failure in clinical trials. That work will be published shortly along with some ground breaking research highlighting the role of LOXL2 in kidney fibrosis where there remains a large unmet need to tackle both widespread and orphan diseases. In conjunction with these researchers and its scientific advisors Pharmaxis is developing alternative clinical development paths and is focusing current and future partnering discussions on companies interested in pursuing renal disease. We have been encouraged by pharma interest at the recent JP Morgan healthcare conference.

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 lead candidate has been selected (PXS-6302) and has completed pre-clinical development including initial stability studies of the topical formulation.

The Company is well advanced in discussions with an Australian based hospital burns units that is interested in commencing a series of investigator initiated clinical studies to assess the safety and initial efficacy of this drug in burns related scars and pre-existing scars. The phase 1 study is

currently scheduled to commence in the first quarter of the 2021 calendar year.

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. On 30 October 2020 the US Food and Drug Administration (FDA) approved Bronchitol® (mannitol) as add-on maintenance therapy to improve pulmonary function in cystic fibrosis (CF) patients 18 years of age and older. Chiesi plans to launch Bronchitol in the first half of 2021.

Consequently, a US$7 million (A$10 million) milestone was paid by Chiesi to Pharmaxis during the quarter with a further US$3 million (~A$4 million) payable on shipment by Pharmaxis of commercial launch stock scheduled for the first quarter of 2021.

Pharmaxis expects Bronchitol sales in the US market to contribute strongly to the product's global sales and profit growth from its launch making the Pharmaxis mannitol business 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 in Switzerland via an exclusive distributor.

Other territories

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

Bronchitol sales

Bronchitol sales for the quarter and half year ended 31 December 2020 and 31 December 2019 are as follows:

$'000	Quarter		Half year	
	2020	**2019**	**2020**	**2019**
Australia	297	273	544	571
Western Europe	103	28	120	862
Eastern Europe	137	51	167	147
Russia	1,365	588	1,365	588
Total	**$1,902**	**$940**	**$2,196**	**$2,168**

A large order (A$1.365 million) was shipped to Russia during the quarter with a further order expected before the end of the financial year.

In Western Europe, despite disruptions caused by the COVID-19 pandemic, in-market unit sales of Bronchitol by Chiesi in the UK, Germany, Italy and the Nordics for the three months ended 31 December 2020 were only 2% less than 2019 while sales for the 12 months ended 31 December 2020 increased 2.5% over 2019.

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. Large orders were shipped to Chiesi in the June quarter of 2020 and no orders were shipped in the current period.

In Australia where Pharmaxis sells directly to clinics, unit sales were 21% higher than the prior (September) quarter, and unit sales for the 2020 calendar year were 12% higher than 2019.

The COVID-19 global pandemic has not to date impacted purchasing of Bronchitol by our international distributors.

The Company continues to monitor the situation.

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. In Australia unit sales decreased 7% for the December quarter compared to 2019 and 25% for the 2020 calendar year compared to 2019. Unit sales for the December 2020 quarter were 27% above the September 2020 quarter.

In Europe unit sales decreased 42% for the December quarter compared to 2019 and 443% for the 2020 calendar year compared to 2019. Unit sales for the December 2020 quarter were 54% above the September 2020 quarter.

The Company continues to monitor the situation.

Aridol sales for the quarter and half year ended 31 December 2020 and 31 December 2019 are as follows:

$'000	Quarter		Half year	
	2020	2019	2020	2019
Australia	115	117	201	260
Europe	139	246	241	502
USA & Canada	98	72	98	72
South Korea	170	172	350	257
Total	**$522**	**$607**	**$890**	**$1,091**

Corporate

Recent media interviews with Pharmaxis CEO Gary Phillips

- Proactive Investors: *"Pharmaxis CEO says US FDA approval for Bronchitol® is 'big day in company's history'"* (2 November 2020)
- BioPharmaDispatch: *"Pharmaxis CEO says approval shows the importance of 'resilience'"* (3 November 2020)
- Pharmaxis Presents at *Proactive CEO Investor Session* (10 November 2020)
- Ticker News: *Cystic Fibrosis Breakthrough* (10 November 2020)

Pharmaxis Investment Summary

Pharmaxis recently published an investment summary - available on the company website.

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Financials

Key financial metrics

This quarter we introduce new format key financial metrics to better reflect the focus of the business:

	Three months ended		Six months ended	
	2020	**2019**	**2020**	**2019**
Segment results – adjusted EBITDA				
New drug development				
Oral Pan-LOX (external costs)	(545)	(939)	(1,323)	(1,400)
Other program external costs (net of grants)	(478)	(481)	(775)	(1,078)
Employee costs	(875)	(717)	(1,799)	(1,529)
Overhead	(145)	(118)	(238)	(281)
R&D tax credit			148	259
EBITDA	**(2,043)**	**(2,255)**	**(3,987)**	**(4,029)**
Mannitol respiratory business				
Sales	2,424	1,547	3,086	3,259
Other income	9,956	6	10,098	10
	12,380	1,553	13,184	3,269
Expenses – employee costs	(1,527)	(1,518)	(2,914)	(3,037)
Expenses – manufacturing purchases	(1,100)	(388)	(1,172)	(746)
Expenses – other	(1,164)	(774)	(2,374)	(1,755)
EBITDA	**8,589**	**(1,127)**	**6,724**	**(2,269)**
Corporate – EBITDA	**(1,163)**	**(902)**	**(2,024)**	**(1,701)**
Total Adjusted EBITDA	**5,382**	**(4,284)**	**713**	**(7,999)**
Net profit(loss)	**5,026**	**(4,569)**	**46**	**(10,319)**
Statement of cash flows				
Cash inflow/ (outflow) from:				
Operations	9,414	3,417	5,048	(3,692)
Investing activities	(181)	(130)	(281)	(328)
Financing activities	(640)	(620)	(1,282)	(1,240)
Total cash generated/(used)	**8,593**	**2,667**	**3,485**	**(5,260)**
Cash at bank	**18,249**	**25,864**	**18,249**	**25,864**

Highlights

- New drug development
  - Oral pan-LOX expenditure in the quarter and half relates to the phase 1c/2a clinical trial that is due to commence patient dosing in the first quarter of 2021. 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 quarter and half include the 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, scheduled to progress to investigator initiated clinical trials in the first quarter of 2021.
    - The increase in employee costs includes the re-assignment of staff from the mannitol business unit where they supported the approval of Bronchitol in several territories including the US, to support the new clinical programs in drug development.
- Mannitol respiratory business
    - See above for detail and commentary in relation to Bronchitol and Aridol sales for the quarter.
    - Other income includes the US$7 million (A$10.0m) milestone received from Chiesi subsequent to the approval of Bronchitol in the United States on 30 October 2020 and a $100,000 milestone received in the September quarter in relation to approval of Bronchitol in Brazil.
    - The increase in expenses for both the quarter and half reflect increased third party support of a routine European safety audit and increased manufacturing activity associated with a large Russian order shipped during the quarter and the upcoming US launch of Bronchitol for which the Company has been preparing at risk.
- Corporate
    - The increase in net expenses includes net foreign exchange losses of $212,000 for the quarter (2019: $7,000) and $156,000 for the six months (2019: $31,000) relating to foreign currency cash, receivables and payables. Foreign currency gains on losses on balances held to fund the myelofibrosis clinical trial (a US$ denominated contract) are excluded from segment reporting and form part of the reconciliation to net profit(loss).
- 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. These will be detailed in the Company's half year report.
- Cash
    - The Company finished the quarter and half with $18 million in cash.
    - The Company is entitled to receive a milestone of US$3 million (~A$4 million) on shipment of Bronchitol launch stock, scheduled for the first quarter of 2021).
    - In addition to the Chiesi A$9 million milestone, during the quarter and half the Company received its 2020 R&D tax incentive of $5.0 million.

Additional Financial Information

Income statements and summary balance sheets are provided below. Additional financial information will be included in the Company's half year report due to be released on 11 February 2021.

Income statements

A$'000	Three months ended		Six months ended	
(unaudited)	**31-Dec-20**	**31-Dec-19**	**31-Dec-20**	**31-Dec-19**
Revenue				
Revenue from sale of goods	2,425	1,548	3,086	3,259
Chiesi US FDA approval milestone	9,949	-	9,949	
Interest	15	101	36	230
R&D tax incentive			148	259
Other	245	142	468	273
Total revenue	**$12,634**	**$1,791**	**$13,687**	**$4,021**
Expenses				
Employee costs	(3,166)	(2,968)	(6,200)	(6,005)
Administration & corporate	(691)	(641)	(1,220)	(1,153)
Rent, occupancy & utilities	(280)	(255)	(524)	(483)
Clinical trials	(620)	(945)	(1,279)	(1,069)
Drug development	(502)	(377)	(917)	(1,311)
Sales, marketing & distribution	(392)	(347)	(747)	(668)
Safety, medical and regulatory affairs	(420)	(153)	(977)	(487)
Manufacturing purchases and changes in inventory	(1,101)	(387)	(1,172)	(746)
Other	(79)	(156)	(126)	(374)
Depreciation & amortisation	(785)	(808)	(1,589)	(1,616)
Foreign currency exchange gains & losses	550	833	(1,362)	(121)
Finance costs	(122)	(150)	(252)	(307)
Total expenses	**(7,608)**	**(6,354)**	**(13,641)**	**(14,340)**
Net profit (loss) before tax	**5,026**	**(4,563)**	**46**	**(10,319)**
Income tax credit/(expense)				
Net profit (loss) after tax	**$5,026**	**($4,563)**	**$46**	**($10,319)**

Summary balance sheets

A$'000 (unaudited)	31-Dec-20	30-June-20
Assets		
Cash	18,249	14,764
R&D tax incentive – received October		4,900
Accounts receivable	1,494	1,459
Inventory	2,691	2,630
PP&E	7,512	8,906
Other	2,848	2,757
	$32,794	**$35,416**
Liabilities		
Accounts payable and accrued expenses	3,070	2,765
Lease liability (Frenchs Forest facility)	7,259	8,154
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	18,786	21,200
Other liabilities	1,791	1,866
	$30,906	**$33,985**
Net Assets	**$1,888**	**$1,431**