Quarterly Shareholder Update – September 2020

Dear Shareholder,

I reported in the June Quarter some important developments such as the filing of an investigational new drug (IND) application for our myelofibrosis drug along with a significant amount of activity to reshape the Pharmaxis business for the future. We have continued that momentum and despite altered working conditions to conform to local COVID-19 restrictions have delivered on a number of key objectives for lead asset PXS-5505 while progressing work on the rest of our pipeline.

To summarise:

- **FDA grants permission to progress our myelofibrosis drug, PXS-5505, into a phase 1c/2 study**
  Our IND application was a comprehensive document including over 20,000 pages of reports on both pre-clinical and clinical testing to support approval for a phase 1c/2 study in the rare bone marrow cancer myelofibrosis. Following the positive FDA feedback in August we have moved quickly to secure an experienced contract research organisation, Parexel, to conduct the study which will start recruiting in Q1 2021.
- **FDA grants Pharmaxis orphan drug designation for PXS-5505 in myelofibrosis**
  The IND is important because it shows the FDA is satisfied there is a sufficient body of evidence in the program to support its use in myelofibrosis.
- **Pharmaxis awarded $1m Australian Government funding for pre-clinical drug**
  In September we were awarded $1 million funding from the Biomedical Translation Bridge (BTB) program to significantly advance work on the Company's drug discovery for the treatment of the devastating genetic disorder Duchenne Muscular Dystrophy (DMD), an orphan disease that affects many young boys and their families around the world. This is a matched funding grant that will take us all the way to the start of phase 1 trials for this exciting new drug candidate and is an example of our strategy to accelerate drug discovery with the use of non-dilutive funds until we can further define the commercial opportunity.

The quarter also saw a setback for an earlier program that we had partnered with Boehringer Ingelheim. Boehringer raised some potential drug interaction issues in deciding not to progress the drug (PXS-4728) in either NASH or diabetic retinopathy and terminated their agreement with us. Whilst this is disappointing it is worth noting that we received $83m from Boehringer over the life of their program and we now have the right to the IP as well as the considerable bank of data generated by Boehringer at no charge. Pharmaxis is conducting a thorough review to establish whether there are other indications where the drug-drug interaction reported by Boehringer would not prevent its development.

Bronchitol US FDA review

For the coming quarter we will be preparing to start the myelofibrosis study but there will also be significant focus on the outcome of the Bronchitol NDA filed by our US licensee Chiesi. The US Food and Drug Administration (FDA) have advised it has a 'Goal Action Date' of 1 November 2020 which is very close to the publication date of this shareholder update. We have US$10m milestone payments attached to the approval and supply of launch stock to Chiesi.

We plan to provide a comprehensive update to the market as soon as the FDA decision is received.

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 ready
Other cancers (oral pan-LOX inhibitor)	PXS-5505	Phase 1
LOXL2 inhibitor	PXS-5382; PXS-5338	Phase 1 completed
Topical pan-LOX inhibitor	PXS-6302	Phase 1 ready
Duchenne Muscular Dystrophy (combination 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.

Importantly, there has been no impact of COVID-19 on the various clinical trials in which the Company has been involved, although the planning for our upcoming phase 1c/2a trial in myelofibrosis has required agility on the part of our contract research organisation to identify countries and sites where clinical trials are still proceeding.

While employees are now able to spend more days in the office and labs with social distancing and enhanced cleaning protocols the option of working from home remains in place for those who can.

The Company continues to monitor the situation.

Drug discovery

Oral pan-LOX inhibitor program

Pharmaxis is progressing two pan lysyl oxidase (pan-LOX) programs from its amine oxidase chemistry platform, both of which are planned to enter into phase 1c/2 clinical trials in 2021.

The most advanced LOX program has developed an oral drug (PXS-5505) that has successfully cleared pre-clinical safety studies including 6-month toxicity and has shown significant reductions in fibrosis in in-vivo models of myelofibrosis (MF), pancreatic cancer and other cancers as well as in models of systemic sclerosis. PXS-5505 has shown to be well tolerated in Phase 1 single and multiple ascending dose studies that reported earlier in the year with an excellent pharmacokinetic and pharmacodynamic profile.

PXS-5505 works by inhibiting all of the lysyl oxidase family members that are involved in the bone marrow fibrosis that characterises myelofibrosis. It is hoped that this will have beneficial effects on blood cell production and consequently other aspects of this fatal disease which has a very high unmet medical need. Pharmaxis strategy for PXS-5505 is to follow this first phase 1c/2 study as a monotherapy with further studies to include myelofibrosis patients being treated with JAK inhibitors which are the existing standard of care for many patients.

In July the US Food and Drug Administration (FDA) granted Pharmaxis orphan-drug designation for PXS-5505 for the treatment of myelofibrosis.

In August the FDA advised it had completed a safety review of the company's Investigational New Drug (IND) application for PXS-5505 and gave Pharmaxis permission to proceed with a phase 1c/2 clinical trial for the treatment of myelofibrosis in adults. The IND application was a significant body of work containing over 20,000 pages of reports on the phase 1 studies in healthy volunteers, numerous individual pre-clinical studies and details of the manufacture of the drug substance and drug product to be used in human clinical trials.

Pharmaxis has appointed international clinical research organisation Parexel International (IRL) Limited to manage the phase 1/2 clinical trial. Parexel has identified sites in South Korea and Australia where the current COVID-19 pandemic will not prevent recruitment of the initial stage of the trial.

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 chemotherapy. 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.

Boehringer Ingelheim discontinues development of BI 1467335

BI 1467335 (PXS-4728) was acquired by Boehringer Ingelheim in 2015 with an upfront payment to Pharmaxis of $41 million. Subsequent payments on the commencement of two clinical trials by Boehringer in two indications brought the total receipts by Pharmaxis to A$83 million.

During the quarter Boehringer advised the discontinuation of the development of anti-inflammatory AOC3 inhibitor BI 1467335 for the treatment of patients with moderate-severe non-proliferative diabetic retinopathy (NPDR) after receiving results from a phase 2a clinical trial in patients with moderate-severe NPDR. BI 1467335 met its primary endpoint in ocular safety with the treatment being well tolerated. Boehringer decided not to further develop BI 1467335 in this indication based on the lack of a clear efficacy signal and risk of dose dependent drug interactions of the compound in NPDR patients identified in another Phase 1 study. Consequently, Boehringer advised that it would terminate the agreement with Pharmaxis.

Based on recent publications AOC3 remains an important clinical target. Pharmaxis has commenced a review of 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 and where the risk of drug interactions are of less concern. Pharmaxis expects to have completed its review in the first half of 2021.

LOXL2 inhibitor program

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).

As reported last quarter, the Company completed an additional small phase 1 study in response to questions from pharma companies interested in the program, demonstrating an improved pharmacokinetic profile in a number of different dosing regimens.

Pharmaxis is currently pursuing a number of different partnering options to enable the drug to enter the clinic in phase 2 trials. While the process has taken longer than originally expected, and much of the industry focus has been on the Covid-19 pandemic, the Company continues to have discussions with a number of potential partners. Pharmaxis will provide more information when the process concludes.

Topical pan-LOX inhibitor program

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.

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

The Company has ongoing 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 before the end of the 2020 calendar year.

Pharmaxis awarded $1m Australian Government funding to progress Duchenne Muscular Dystrophy drug into the clinic

In September Pharmaxis announced it had been awarded $1 million funding from the Biomedical Translation Bridge (BTB) program to significantly advance work on the Company's drug discovery for the treatment of the devastating genetic disorder Duchenne Muscular Dystrophy (DMD), a genetic disorder affecting thousands of Australians.

Pharmaxis was selected following a highly competitive review conducted by an independent expert evaluation committee as part of the BTB program, which is administered by MTPConnect. The Australian government matched funding will allow the company to take one of its pipeline drugs (PXS-4699) through to the commencement of human clinical trials. A planned comprehensive program of pre-clinical studies will build on the pioneering work already conducted on Pharmaxis compounds by independent international researchers focused on DMD. PXS-4699 is a dual amine oxidase inhibitor which is expected to protect muscle and reduce inflammation as well as organ fibrosis in DMD. It is hoped this could result in better daily functioning for patients, improved quality of life and longer life expectancy.

Mannitol 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 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.

United States

The Company's US partner Chiesi Group is responsible for the commercialisation of Bronchitol in the United States. Subsequent to Chiesi filing a response to the Food and Drug Administration (FDA) in the June quarter of 2020 the FDA advised a Goal Action Date of 1 November 2020 in relation to the FDA's review of Bronchitol for the US.

During the quarter Pharmaxis negotiated an accelerated timeline on payment of the initial tranche of a US$10 million Bronchitol launch milestone. US$7 million of the milestone is now payable by Chiesi upon US approval of Bronchitol by the FDA. A further US$3 million remains payable on shipment by Pharmaxis of commercial launch stock, scheduled for the first quarter of 2021. Subject to approval, Pharmaxis will also receive mid to high teen percentage royalties and will be the exclusive supplier of Bronchitol for the US market - on a cost-plus basis.

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 ended 30 September 2020 and 30 September 2019 are as follows:

$'000	Three months	
	2020	**2019**
Australia	246	297
Western Europe	17	835
Russia & Eastern Europe	31	96
Total	**$294**	**$1,228**

Pharmaxis Bronchitol distributors typically order on a six monthly basis. Pharmaxis ex-factory sales for the current quarter reflect the buying patterns of its international distributors with no orders

shipped to our EU or Russian distributors. Despite disruptions caused by the COVID-19 pandemic, in-market unit sales of Bronchitol by Chiesi in the UK, Germany and Italy for the three ended 30 September 2020 were at the same level as 2019. Sales for the 12 months ended 30 September 2020 increased 2% over 2019.

In Australia where Pharmaxis sells directly to clinics, unit sales were 11% lower than the prior quarter, although unit sales for the first nine months of the 2020 calendar year are 13% above the same period in 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

At the beginning of the COVID-19 pandemic a number of countries, including those where Aridol is sold, respiratory specialists were advised 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 39% for the September quarter compared to 2019 and 52% for the nine months of the 2020 calendar year to date compared to 2019. In Europe unit sales decreased 59% for the September quarter compared to 2019 and 66% for the nine months of the 2020 calendar year to date compared to 2019. We have been advised that the limitation on lung function testing has been eased in a number of markets. The Company continues to monitor the situation.

Aridol sales

Aridol sales for the quarter ended 30 September 2020 and 30 September 2019 are as below.

$'000	Three months	
	2020	**2019**
Australia	85	141
Europe	102	256
USA	-	-
South Korea	180	86
Total	**$367**	**$483**

Corporate

2020 Annual General Meeting

The 2020 Annual General Meeting of Pharmaxis Ltd will be a virtual meeting, and is to be conducted online at 10.00am on 4 November 2020.

If you choose to participate online on the day of the meeting you will be able to view a live webcast of the meeting, ask the Directors questions and submit your votes in real time.

The notice of meeting, proxy form and information on how to participate in the virtual meeting were sent to shareholders on 2 October 2020.

Non-personalised information can be found on the Pharmaxis website.

Resignation of Edward Rayner

In August the Company announced that non-executive director Mr Edward Rayner had resigned from the Board. Ed joined the Pharmaxis Board in conjunction with a share placement to UK based Arix Bioscience plc in September 2018. At the time he was the investment director leading the Arix investment. Mr Rayner has recently commenced a new executive position with a UK life science technology company, having left Arix earlier this year.

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Financials

Key financial metrics

A$'000	Three months ended	
(unaudited)	**30-Sep-20**	**30-Sep-19**
Income statements		
Sales of Bronchitol & Aridol	661	1,711
Total revenue	1,053	2,237
Total expenses	(6,033)	(7,986)
Net profit (loss) after tax	**(4,980)**	**(5,750)**
Segment results – adjusted EBITDA		
Bronchitol & Aridol	(1,865)	(1,142)
New drug development	(1,943)	(1,774)
Corporate	(860)	(799)
Total	**(4,668)**	**(3,715)**
Statement of cash flows		
Cash inflow/ (outflow) from:		
Operations	(4,366)	(7,109)
Investing activities	(100)	(198)
Financing activities	(642)	(620)
Total cash generated/(used)	**(5,108)**	**(7,927)**
Cash at bank	**9,656**	**23,197**

Highlights

- Revenue
  - See above for detail and commentary on Bronchitol and Aridol sales for the quarter.
- Expenses
  - When unrealised foreign exchange rate gains and losses are excluded, total expenses for the quarter were marginally lower than the prior period. ($6.8 million compared to $7.1 million for the prior period).
- Net loss
  - Net loss for the period of $5.0 million compares to $5.8 million for the prior period.
- Cash
  - The Company finished the quarter with $10 million in cash.
  - On 14 October the Company received its 2020 R&D tax incentive of $5.0 million.

Segment information

A$'000	Segment information - three months ended							
(unaudited)	30-Sep-20				30-Sep-19			
Income statements	Mannitol Business	New Drug Development	Corporate	Total	Mannitol Business	New Drug Development	Corporate	Total
Revenue								
Sale of Bronchitol	294			294	1,228			1,228
Sale of Aridol	367			367	483			483
	661			**661**	**1,711**			**1,711**
Tax credit		148		148		259		259
Other revenue	137			137				
Other revenue	5		80	85	5		131	136
	803	**148**	**80**	**1,031**	**1,716**	**259**	**131**	**2,106**
Expenses								
Employee costs	(1,385)	(924)	(539)	(2,848)	(1,519)	(805)	(442)	(2,766)
Clinical trials		(659)		(659)		(124)		(124)
Drug discovery		(415)		(415)		(937)		(937)
Other expenses	(1,283)	(93)	(401)	(1,777)	(1,339)	(167)	(488)	(1,994)
Total expenses	(2,668)	(2,091)	(940)	(5,699)	(2,858)	(2,033)	(930)	(5,821)
Adjusted EBITDA	**($1,865)**	**($1,943)**	**($860)**	**($4,668)**	**($1,142)**	**($1,774)**	**($799)**	**($3,715)**

Commentary for the quarter

- Mannitol Business:
  - Sales of Bronchitol and Aridol are detailed and discussed in the commentary above.
  - Other revenue includes a US$100,000 milestone in relation to approval of Bronchitol in Brazil.
  - Expenses of the Mannitol business are relatively fixed.
  - As such, the $903,000 decrease in revenue compared to 2019 resulted in $723,000 decrease in EBITDA.
  - The reduction in employee expenses for the quarter reflects redeployment of clinical and regulatory resources to New Drug Development in support of the upcoming clinical program in myelofibrosis.
- New Drug Development:
  - The increase in employee expenses reflects redeployment of clinical and regulatory resources from the mannitol business.
  - Clinical trial expenses relate to preparation for the phase 1c/2a trial for the oral pan-LOX in myelofibrosis.
  - Drug discovery expenses include small investments on oral pan-LOX and SSAO combo programs. Prior period expenses related to the oral and topical pan-LOX programs.
- Corporate
  - Overall corporate expenses were consistent with the prior period.

Income statements

A$'000	Three months ended	
(unaudited)	**30-Sep-20**	**30-Sep-19**
Revenue		
Revenue from sale of goods	661	1,711
Interest	21	129
R&D tax incentive	148	259
Other	223	131
Total revenue	**$1,053**	**$2,237**
Expenses		
Employee costs	(3,034)	(3,037)
Administration & corporate	(529)	(512)
Rent, occupancy & utilities	(244)	(228)
Clinical trials	(659)	(124)
Drug development	(415)	(934)
Sales, marketing & distribution	(355)	(321)
Safety, medical and regulatory affairs	(557)	(334)
Purchases and changes in inventories	(71)	(359)
Other	(47)	(218)
Depreciation & amortisation	(804)	(808)
Foreign currency exchange gains & losses	812	(954)
Finance costs	(130)	(157)
Total expenses	**(6,033)**	**(7,986)**
Net profit (loss) before tax	**(4,980)**	**(5,750)**
Income tax (expense)	-	-
Net profit (loss) after tax	**($4,980)**	**($5,750)**

Summary balance sheets

A$'000 (unaudited)	30-Sep-20	30-June-20
Assets		
Cash	9,656	14,764
R&D tax incentive – received October	5,048	4,900
Accounts receivable	499	1,459
Inventory	2,955	2,630
PP&E	8,163	8,906
Other	2,421	2,757
	$28,742	**$35,416**
Liabilities		
Accounts payable and accrued expenses	1,943	2,765
Lease liability (Frenchs Forest facility)	7,710	8,154
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	20,345	21,200
Other liabilities	2,106	1,866
	$32,104	**$33,985**