Quarterly Shareholder Update – September 2019

Dear Shareholder,

It's been a busy period of development in the Pharmaxis product pipeline and this has generated significant news flow for investors and shareholders to consider.

A year ago, we held a research update for investors at our Sydney headquarters. Boehringer Ingelheim sent two senior executives to present their development plan for the anti-inflammatory AOC3 inhibitor Boehringer acquired from Pharmaxis in 2015. It is both satisfying and exciting to consider that now one year on, we will see the first results from a phase 2 study in NASH patients report in the December quarter. We have become accustomed to the professional way in which Boehringer conduct its business and expect that their next report will provide a comprehensive review of all the studies conducted to date and a decision on whether to advance the drug into a phase 2b study. No cash milestone is attached to this step but a decision to move forward would obviously be very positive and significantly increases the probability of the deal concluded in 2015 paying out in the future.

I wrote in the June quarterly shareholder update that the commercial stage of partnering discussions for the Pharmaxis LOXL2 inhibitor program had been extended due to a number of changes in the market. Confidentiality is fundamental to securing the right outcome for our shareholders and whilst we will not provide any updates until we reach a conclusion to the process, we have recently released additional information regarding the ongoing scientific work we have been conducting. We have used our proprietary expertise in highly sensitive LOXL2 assays to evaluate and design new studies that address partner questions and ensure that the program remains relevant and competitive in the ever changing therapeutic development race to find new treatments for patients with NASH or IPF. You can find more information on these studies in this update, as well as in recent press releases and my presentation to the 2019 AusBiotech conference which are available on the Pharmaxis website.

The timeline for the FDA review of Bronchitol for adult cystic fibrosis patients has been extended by a month and therefore puts our expectation for an FDA decision into Q2 2020. Overall, I consider this to be good news due to the greater clarity we now have from the FDA. The engagement between the sponsor and the FDA after receipt of a complete response letter (CRL) is an iterative one with the company presenting its plans to address the concerns raised in the CRL and then the FDA having an opportunity to further clarify its expectations. Our US licensee Chiesi has received that feedback and is now proceeding with a revised human factor study with the confidence that the planned study design meets FDA expectations. The study design also includes a pilot phase to ensure that any unforeseen complications are ironed out before the study commences. Pharmaxis has commenced preparations to produce the Bronchitol launch stock for the US and of course we look forward to the moment when we will earn the US$10m milestone payment due upon its delivery.

Lastly, we have advanced our anti-fibrotic cancer drug PXS-5505 into the second stage of phase 1 human studies. We decided to complete the 13 week tox studies to further de-risk the drug before investing in multiple ascending dose stage of the phase 1 study where patients are dosed daily for 14 days. We now expect to see results in Q1 2020. The results seen in the first stage of the phase 1 studies were excellent, with PXS-5505 showing good pharmacokinetics and significant inhibition of all the LOX family of enzymes. In line with our strategy of retaining this asset until we have attained the value enhancing step of clinical proof

of concept in a disease with high unmet need, we plan to meet with the FDA early next year to discuss our trial plans for myelofibrosis or pancreatic cancer.

So – a busy quarter behind us and a pivotal one ahead.

Sincerely,

Gary Phillips – Chief Executive Officer

Drug discovery

Boehringer Ingelheim development of BI 1467335 – phase 2a clinical trial in NASH to report Q4 2019

Boehringer Ingelheim is developing BI 1467335 (formerly known as PXS-4728A), a drug it acquired from Pharmaxis in 2015, for two indications – the liver disease Non-Alcoholic Steatohepatitis (NASH) and the eye disease diabetic retinopathy (DR). Boehringer initiated phase 2a proof of clinical principle trials for NASH in August 2017 and for DR in January 2018. The achievement of these development milestones resulted in Pharmaxis receiving a total of €28 million (A$42 million) in the 2018 financial year.

In June, Boehringer advised that its phase 2(a) proof of clinical principle trial for NASH had been completed with the last patient dosed. Boehringer expects to report the results from this study to Pharmaxis in quarter 4 this year after it has completed an internal post study analysis as well as an internal review of the future NASH development program for BI 1467335 based on the phase 2a results and its extensive preclinical work and phase 1 safety studies (6 completed, 2 ongoing).

Non-alcoholic fatty liver disease (NAFLD), the most common liver disorder in Western industrialised nations and its more serious form NASH, is highly prevalent amongst patients with type 2 diabetes. NASH is a major cause of liver fibrosis and cirrhosis and is an area of high unmet medical need with no treatments currently available. The high prevalence of type 2 diabetes and obesity is expected to make NASH one of the most common causes of advanced liver disorders in coming decades. Some 25% of the general adult population in the world has NAFLD and the prevalence of NASH has been found to range from 1.5% to 6.5% in current research, a number twice as high as 20 years ago.

The phase 2a NASH trial is a multi-centre, double-blind design in 114 patients with clinical evidence of NASH. The trial is being conducted in nine countries across North America and Europe. The primary objectives are to establish proof of clinical principle, investigate suitable dosing, and to evaluate the safety of BI 1467335. Patients have been randomised to either one of four dosages of BI 1467335 or to placebo for a 12-week treatment period followed by a 4-week observation period. A subsequent Phase 2b study will seek to confirm and extend these findings.

Diabetic retinopathy is the leading cause of vision-loss in adults. Of an estimated 285 million people with diabetes mellitus worldwide, approximately one third have signs of DR and of these, a further one third is vision-threatening. The DR trial is scheduled to report mid 2020.

Boehringer has total responsibility for the development program of BI 1467335 and Pharmaxis receives payments upon achievement of certain development milestones. The total development milestones in the deal (€419m /A$625m), would be payable to Pharmaxis should both indications be approved.

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 which have completed phase 1 clinical trials and 3-month toxicology studies. In January 2019, Pharmaxis announced that for both compounds, doses that resulted in 85% or greater inhibition of the target enzyme in the phase 1 studies were below the human equivalent No Observed Adverse Effect Level doses in all toxicity studies and thus demonstrated an adequate safety margin to start phase 2 studies of at least 3 months in length.

The excellent pharmacokinetic parameters and the significant and long lasting inhibition of the target LOXL2 enzyme has demonstrated that these compounds are best-in-class.

During the quarter, Pharmaxis presented data at scientific conferences and in scientific publications of its unique proprietary assays that for the first time allow for the concentration and activity of members of the LOX enzyme family to be measured in serum and tissue, even at very low levels. These data demonstrate that LOXL2 in particular has a relatively fast turnover in human serum and suggests that an effective drug will need to both penetrate fibrotic tissue and be

present in concentrations high enough to inhibit LOXL2 at all times, criteria which only Pharmaxis' inhibitors fulfil. Pharmaxis has used this unique assay technology in the development and assessment of all its current LOX and LOXL2 inhibitor programs.

Over the course of this year, Pharmaxis has been able to reanalyse samples from existing studies and design new pre-clinical studies that have clearly shown the link between LOXL2 inhibition in diseased organs, a reduction in collagen crosslinking which is the cause of fibrosis and clinical effect as measured by the area of fibrosis. This data has energised the ongoing partnering process by underlining the relevance of LOXL2 and the superiority of our compounds particularly when compared to a large pharma program targeting LOXL2 that had previously failed in the clinic. The large pharma did not have an assay to confirm adequate target inhibition of LOXL2.

Pharmaxis is currently pursuing a number of different partnering options to enable this drug to enter the clinic in phase 2 trials and will provide more information when the process concludes.

Systemic LOX inhibitor program

In addition to the SSAO inhibitor (BI 1467335) and the LOXL2 inhibitors, Pharmaxis is progressing two lysyl oxidase (LOX) programs from its amine oxidase chemistry platform, both of which are planned to partner after phase 2 clinical trials.

The most advanced LOX program has developed an oral once-a-day drug that inhibits all lysyl oxidase family members (LOX, LOXL1, 2, 3 & 4).

The compound has shown significant reductions in fibrosis in in-vivo models of kidney, liver and lung fibrosis, myelofibrosis as well as pancreatic cancer. It is suited to the treatment of severe fibrosis as well as cancer with prominent stroma (connective tissue) or fibrotic metastatic niches.

During the quarter, Pharmaxis announced positive results from the clinical Phase 1A study. The double-blind placebo-controlled study commenced in February 2019 and consists of two stages. The first single ascending dose stage (phase 1A) was conducted in 40 healthy subjects divided into five groups with each taking a different single oral dose or placebo. The drug was well tolerated and no safety signals were identified during the study. Importantly for potential clinical benefit, the data showed a drug with good pharmacokinetics and a dose related inhibition of members of the LOX family with the upper doses causing significant inhibition for a full 24 hours after a single application.

The second multiple ascending dose stage (phase 1B) commenced in October and is due to report in Q1 2020.

During the quarter, a scientific publication reported that Pharmaxis compounds had significantly decreased the bone marrow fibrotic burden in two different models of primary myelofibrosis (PMF) which is a chronic myeloproliferative cancer with a poor prognosis and limited therapeutic options available. The authors of the publication noted that LOX is an enzyme vital for collagen cross-linking and extracellular matrix stiffening and has been found to be upregulated in PMF. They concluded on the basis of their multiple studies that the Pharmaxis compounds appear to be promising new candidates for the treatment of fibrosis in PMF.

Pharmaxis is very encouraged by the feedback we have received from both academic and clinical thought leaders about the use of its LOX inhibitor in patients with PMF, as an adjunct to existing standard of care and as a monotherapy.

After successful completion of the phase 1 study, Pharmaxis will have all the data required to support the commencement of clinical proof of concept studies in either myelofibrosis or pancreatic cancer. The Company plans to discuss the program with the FDA prior to filing an IND that supports entering phase 2 studies in H2 2020.

Topical LOX inhibitor program

The Company's other LOX program 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 and is currently in pre-clinical development including initial stability of the topical formulation, ongoing evaluation in various disease models of scarring and tox studies. Three month tox studies commenced this quarter.

The program aims to commence phase 1 studies in 2020 and is planning to conduct the trial in

healthy volunteers with scarring so as to be able to simultaneously test clinical efficacy.

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 (Chiesi) is responsible for the commercialisation of Bronchitol in the United States.

Following a positive recommendation from an FDA convened Pulmonary-Allergy Drugs Advisory Committee meeting in May 2019, Chiesi received a complete response letter from the FDA on 19 June 2019.

The FDA requires Chiesi to revise the product packaging and user instructions for Bronchitol and then conduct a human factor study (HFS) demonstrating that the revisions would enable healthcare professionals to properly administer the mannitol tolerance test – an initial test to ensure patients hypersensitive to mannitol are not prescribed Bronchitol. During the quarter, Chiesi submitted a protocol for the HFS to ensure the FDA's requirements were fully incorporated in the study. Chiesi subsequently received the FDA's advice to increase the size of the HFS as well as other recommendations that have now been incorporated into the final study design.

Based on this feedback, Chiesi has added an additional month to its timetable. The FDA review of the Bronchitol NDA is therefore now expected to be completed in the second quarter of 2020. While the timetable has extended by a month, importantly the FDA advice sought and received by Chiesi ensures the HFS is conducted in accordance with the FDA's expectations.

Subject to approval, Pharmaxis will receive a US$10 million milestone payment on the commercial launch of Bronchitol in the US, mid to high teen percentage royalties and will be the exclusive supplier of Bronchitol for the US market.

The 2019 North American Cystic Fibrosis Conference includes a poster presentation titled "Inhaled Dry Powder Mannitol Improves Lung Function in Adults with Cystic Fibrosis – an Integrated Analysis". The presentation analyses the combined results of the three phase 3 studies of Bronchitol for adult patients using the same statistical methods pre-planned for the final study (CF303). The presentation concludes "Previous studies have demonstrated that DPM improves mucociliary and airway clearance. In these current studies, DPM (Bronchitol) demonstrated sustained and significant improvements in lung function in addition to standard of care in adults with CF. The safety profile of DPM (Bronchitol) in adults has been well-characterized. This phase 3 study program supports the clinical benefit of DPM (Bronchitol) in the management of adult patients with CF."

Western Europe

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

Pharmaxis also markets Bronchitol in Austria via its German based logistics provider and in Spain and Switzerland via exclusive distributors.

Other territories

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

Bronchitol sales

Bronchitol sales for the three months ended 30 September 2019 and 30 September 2018 are as follows:

$'000	Three months	
	2019	2018
Australia	297	249
Western Europe	835	36
Russia & Eastern Europe	96	139
Total	$1,228	$900

The increase in sales to Western Europe relate to orders shipped to Chiesi.

Pharmaxis Bronchitol distributors typically order on a six monthly basis.

Aridol

Aridol was relaunched in the US in December 2018 by Pharmaxis' exclusive distributer in North America, Methapharm Inc., who are experts in the specialist respiratory diagnostic market.

Subsequent to approval for Aridol from Canadian regulatory authorities in June 2019, the Company expects the product launch by Methapharm to occur in the second half of 2019.

Aridol sales

Aridol sales for the three months ended 30 September 2019 and 30 September 2018 are as follows:

$'000	Three months	
	2019	2018
Australia	141	118
Europe	256	203
USA	-	-
South Korea	86	154
Total	$483	$475

Following two large orders in the previous financial year, there were no US Aridol orders during the quarter from the Company's US distributor.

Corporate

Pharmaxis at AusBiotech

Pharmaxis CEO Gary Phillips presented at the 2019 annual AusBiotech conference. Together with three other CEOs of ASX listed companies, Mr Phillips spoke to the topic "Anti-fibrotic drugs: a hot area for development". A copy of Mr Phillips' presentation is available on the Pharmaxis website.

2019 Annual General Meeting

The 2019 Annual General Meeting will be held on 21 November 2019 at the offices of Computershare, Level 3, 60 Carrington Street, Sydney NSW 2000 at 2.30 pm (Sydney time). The Notice of Meeting and Proxy Voting Form were distributed to shareholders on 18 October. The formal part of the Meeting will cover consideration of the Company's financial statements and remuneration report, the re-election of a non-executive director, the grant of securities to the Chief Executive Officer and a minor amendment to the Pharmaxis constitution.

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Financials

Key financial metrics

A$'000	Three months ended	
(unaudited)	**30-Sep-19**	**30-Sep-18**
Income statements		
Sales of Bronchitol & Aridol	1,711	900
Total revenue	2,237	1,231
Total expenses	(7,986)	(8,765)
Net profit (loss) after tax	**(5,750)**	**(7,533)**
Segment results – adjusted EBITDA		
Bronchitol & Aridol	(1,142)	(1,837)
New drug development	(1,774)	(3,209)
Corporate	(799)	(1,110)
Total	**(3,715)**	**(6,156)**
Statement of cash flows		
Cash inflow/ (outflow) from:		
Operations	(7,109)	(6,209)
Investing activities	(198)	(333)
Financing activities	(620)	22,227
Total cash generated/(used)	**(7,927)**	**15,685**
Cash at bank	**23,197**	**46,758**

Highlights

- Revenue
  - See above for detail and commentary on Bronchitol and Aridol sales.
  - The Company has not booked an estimated R&D tax incentive of $0.9 million in relation to quarter as it expects revenue for the financial year to be above the $20 million cap after which the incentive is not payable.
- Expenses
  - The reduction in total expenses compared to the prior quarter is primarily due to lower clinical trial and drug discovery expenditure.
- Cash
  - The Company finished the quarter with $23 million in cash. In October, the Company received the 2019 R&D tax incentive of $6.2 million.
  - Operational cash flows for the quarter included the annual FDA Aridol product fee (A$450,000) half of which is reimbursed by the Company's US distributor in the December quarter.

Segment information

A$'000	Segment information - three months ended							
(unaudited)	30-September-19				30-September-18			
Income statements	Bronchitol & Aridol	New drug development	Corporate	Total	Bronchitol & Aridol	New drug development	Corporate	Total
Revenue								
Sale of Bronchitol	1,228			1,228	425	-	-	425
Sale of Aridol	483			483	475	-	-	475
	1,711			**1,711**	**900**	**-**	**-**	**900**
Milestones from sale of drug					-	**-**	-	-
Tax credit		259		259	-	-	-	-
Other revenue	5		131	136	9	-	124	133
	1,716	**259**	**131**	**2,106**	**909**	**-**	**124**	**1,033**
Expenses								
Employee costs	(1,519)	(805)	(442)	(2,766)	(1,397)	(774)	(559)	(2,730)
Clinical trials		(124)		(124)	-	(636)	-	(636)
Drug discovery		(937)		(937)	-	(1,675)	-	(1,675)
Other expenses	(1,339)	(167)	(488)	(1,994)	(1,361)	(124)	(663)	(2,148)
Total expenses	(2,858)	(2,033)	(930)	(5,821)	(2,758)	(3,209)	(1,222)	(7,189)
Adjusted EBITDA	**($1,142)**	**($1,774)**	**($799)**	**($3,715)**	**($1,849)**	**($3,209)**	**($1,098)**	**($6,156)**

Commentary for the quarter

- Bronchitol & Aridol:
  - Sales of Bronchitol and Aridol are discussed in commentary above.
  - Expenses for the quarter were consistent with the prior period.
- New drug development:
  - After completion of the 2019 tax return, the Company booked an additional R&D tax incentive of $0.3 million in respect of the 2019 financial year. The total 2019 incentive of $6.2 million was received in October 2019.
  - Clinical trial expenses include the phase 1 trial for the LOX oral program that commenced in the March quarter of 2019. In 2018, the clinical trial expenses related to the phase 1 trials conducted in the LOXL2 program which completed in the December 2018 quarter.
  - Drug discovery expenses include work on the Systemic LOX program ($419,000 for the quarter; $744,000 in 2018) and the Topical LOX topical program ($193,000 for the quarter; $153,000 in 2018).

Income statements

A$'000	Three months ended	
(unaudited)	**30-Sep-19**	**30-Sep-18**
Revenue		
Revenue from sale of goods	1,711	900
Interest	129	199
R&D tax incentive	259	-
Other	131	133
Total revenue	**$2,237**	**$1,232**
Expenses		
Employee costs	(3,037)	(3,071)
Administration & corporate	(512)	(574)
Rent, occupancy & utilities	(228)	(330)
Clinical trials	(124)	(636)
Drug development	(934)	(1,675)
Sales, marketing & distribution	(321)	(253)
Safety, medical and regulatory affairs	(334)	(311)
Manufacturing purchases	(414)	(340)
Other	(163)	(361)
Depreciation & amortisation	(808)	(641)
Foreign currency exchange gains & losses	(954)	(698)
Finance costs	(157)	125
Total expenses	**(7,986)**	**(8,765)**
Net profit (loss) before tax	**(5,750)**	**(7,533)**
Income tax (expense)	-	-
Net profit (loss) after tax	**($5,750)**	**($7,533)**

Summary balance sheets

A$'000		
(unaudited)	**30-Sep-19**	**30-Jun-19**
Assets		
Cash	23,197	31,124
R&D tax credit (received October 2019)	6,221	5,962
Accounts receivable	2,112	1,171
Inventory	2,190	2,116
PP&E	12,133	10,262
Other	2,362	2,033
	$48,215	**$52,668**
Liabilities		
Accounts payable and accrued expenses	1,952	4,194
Lease liability (Frenchs Forest facility)	9,402	7,171
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	24,492	23,626
Other liabilities	2,779	2,863
	$38,625	**$37,854**
Net Assets	**$9,590**	**$14,814**