Quarterly Shareholder Update – March 2019

Dear Shareholder,

In the December Quarterly update, I focused my editorial on the Management and Board priorities of partnering our LOXL2 program and the upcoming result from the Boehringer Ingelheim NASH phase 2 trial with the AOC3 inhibitor program acquired from Pharmaxis in 2015. One quarter into 2019 and the view from the CEO's chair looks very much the same in terms of events that will drive short term shareholder value but with our systemic pan LOX inhibitor for pancreatic cancer now in phase 1 trials and the FDA about to hold a public advisory committee meeting on the Bronchitol NDA, there are certainly other important events and news flow that should keep Pharmaxis at the forefront of investors' minds.

We believe our LOXL2 program has an important role to play in treating both the liver disease NASH and idiopathic pulmonary fibrosis. We have commenced the commercial phase of this program. This process involves balancing the clinical development plans that potential partners are willing to commit to with the cash value of any deal and the development milestones on offer. As for all partnering programs at this stage of development, it is a complex and multi-faceted process which is also confidential in nature. We will therefore not provide any updates until we reach a conclusion.

The Boehringer NASH study finished recruiting in this last quarter and it was pleasing to see the prominence that this program was given at Boehringer's annual research update. A number of potential NASH treatments have produced disappointing results in clinical trials in the last year and whilst this illustrates the difficulty in treating this disease, it also highlights the need for new and unique mechanisms of action; like the anti-inflammatory Boehringer AOC3 inhibitor which is a first in class drug. Boehringer announced that it expects to report the results from this study in September or October this year after completing both the study and internal post study analysis.

As noted above, Pharmaxis continues to make progress on other fronts and I was very pleased to report earlier this quarter that our systemic pan LOX inhibitor for pancreatic cancer has commenced its first human clinical trials. This first trial is evaluating safety, pharmacokinetics and target engagement in healthy volunteers and is a vital prelude to commencement of our first study in pancreatic cancer patients which we now expect to be early next year.

In more immediate news, the FDA is holding a Pulmonary-Allergy Drug Advisory Committee meeting on 8th May to discuss the Bronchitol NDA that was resubmitted in December 2018. It was a negative vote by this committee in 2013 that lead to the FDA requesting a further study. The results from that study, CF303, will be the main focus of the review and Pharmaxis team members have been fully engaged this past quarter supporting our US license partner Chiesi who are responsible for the NDA. Pharmaxis will take an active role in the advisory committee meeting. The meeting is held in public and the results of the voting will be available immediately after the meeting concludes.

Sincerely,

Gary Phillips – Chief Executive Officer

Drug discovery

Boehringer Ingelheim development of BI 1467335 (formerly known as PXS-4728A)

Boehringer Ingelheim is developing BI 1467335, 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 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 February 2019 Pharmaxis announced completion of enrollment of Boehringer's phase 2a clinical trial in NASH.

Non-alcoholic fatty liver disease (NAFLD), the most common liver disorder in Western industrialized 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. 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 6.45% 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. At its recent annual press briefing Boehringer advised it expects to report data in September or October. 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 complete early in the first half of 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.

A global project manager from Boehringer presented at the Pharmaxis Investor Research Briefing held in November 2018 – the presentation was recorded and is available here.

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 now 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 therefore demonstrated an adequate safety margin to start phase 2 studies of up to 3 months in length.

The Pharmaxis LOXL2 program is one of the very few truly anti-fibrotic mechanisms in clinical development. The excellent pharmacokinetic parameters and the significant and long lasting inhibition of the target LOXL2 enzyme showed that these compounds are best-in-class. Subsequent to completion of the data package including the phase 1 trials and the three month tox studies, the program has entered the commercialisation stage.

LOX oral inhibitor program

In addition to the SSAO inhibitor (BI 1467335) and the LOXL2 program, 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 successfully cleared pre-clinical safety and toxicity studies in the third quarter of 2018 and has shown significant reductions in fibrosis in in-vivo models of kidney fibrosis, lung fibrosis, myelofibrosis and pancreatic cancer. It is suited to the treatment of severe fibrosis as well as cancer with prominent stroma (connective tissue) or fibrotic metastatic niches.

In February 2019 Pharmaxis announced dosing of the first subject in a Phase 1 clinical trial. The double-blind placebo controlled study consists of two stages. The first single ascending dose stage (SAD) will be conducted in 40 healthy subjects divided into five groups with each taking a different single dose or placebo. The second multiple ascending dose stage (MAD) will be conducted in 16 healthy subjects divided into two groups with each group receiving a different dose or placebo for 14 days. The SAD stage of the clinical trial is due to report in June 2019 and the MAD stage will report later in the year.

The compound is currently undergoing three month tox studies, and additional animal models of pancreatic cancer and myelofibrosis with the aim of being ready to commence phase 1c/2 study in pancreatic cancer patients by early 2020.

LOX topical 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.

The program aims to commence phase 1 studies in early 2020 and is planning to conduct the trial in healthy volunteers with scarring so as to be able to simultaneously test clinical efficacy.

Bronchitol and Aridol

Bronchitol® 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 Europe, Russia, Australia 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 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 resubmission of the Bronchitol New Drug Application (NDA) to the US Food and Drug Administration (FDA) by Chiesi in December 2018, the FDA recently advised it will convene a Pulmonary-Allergy Drugs Advisory Committee (PADAC) meeting on 8 May 2019 (US time) to make recommendations on the use of Bronchitol® for adult cystic fibrosis patients in the United States.

PADAC reviews and evaluates available data concerning the safety and effectiveness of marketed and investigational human drug products for use in the treatment of pulmonary disease and diseases with allergic and/or immunologic mechanisms and makes appropriate recommendations to the Commissioner of Food and Drugs.

The Committee consists of a core of 11 voting members including the Chair. Members and the Chair are selected by the Commissioner from among authorities knowledgeable in the fields of pulmonary medicine, allergy, clinical immunology, and epidemiology or statistics.

The specific questions to be asked of the Committee will be advised by the FDA closer to the meeting date.

Pharmaxis is supporting Chiesi in its preparations for the PADAC meeting.

The NDA resubmission responds to the matters raised by the FDA in its Complete Response Letter issued in March 2013 and includes the results of the phase 3 clinical trial conducted after consultation with the FDA. Pharmaxis expects the FDA review process to take between six and twelve months from the resubmission date.

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.

Western Europe

In the EU, Chiesi is the Pharmaxis exclusive Bronchitol distributor for the markets of the UK, Ireland, Germany and Italy.

Pharmaxis also markets Bronchitol in Austria, Denmark and Sweden via its German based logistics provider, and Spain via an exclusive distributor.

Other territories

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

Russia represents a potential significant opportunity for Bronchitol which was approved for both adult and paediatric CF patients in 2016. Pharmaxis has navigated the process to have Bronchitol reimbursed nationally and from 1 January 2019 national reimbursement was granted by the Russian Ministry of Health.

Bronchitol sales

Bronchitol sales for the three and nine months ended 31 March 2019 and 31 March 2018 are as follows:

$'000	Three months		Nine months	
	2019	2018	2019	2018
Australia	239	287	782	655
Western Europe	936	963	1,017	2,007
Russia & Eastern Europe	125	72	158	131
Total	**$1,300**	**$1,322**	**$1,957**	**$2,793**

The increase in Australian sales for the nine months reflects the widened government reimbursement for Bronchitol granted on 1st January 2018.

During the current quarter the Company made sales to Chiesi for the UK, German and Italian markets.

Sales to Russia & Eastern Europe related to the Turkish market place. The next order for Russia is now expected to ship in the June quarter.

Pharmaxis distributors typically order Bronchitol 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.

In June 2018 Pharmaxis filed an approval submission to Canadian authorities. The Company expects the approval process to conclude in mid-2019.

Aridol sales

Aridol sales for the three and nine months ended 31 March 2019 and 31 March 2018 are as follows:

$'000	Quarter		Nine months	
	2019	2018	2019	2018
Australia	105	97	340	309
Europe	277	243	733	656
USA	-	-	659	-
South Korea	252	81	482	436
Total	**$634**	**$421**	**$2,214**	**$1,401**

Corporate

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Financials

Key financial metrics

A$'000	Three months ended		Nine months ended	
(unaudited)	**31-Mar-19**	**31-Mar-18**	**31-Mar-19**	**31-Mar-18**
Income statements				
Sales of Bronchitol & Aridol	1,934	1,743	4,171	4,194
Milestones from sale of drug	-	15,239	-	42,130
Total revenue	2,333	17,276	5,283	48,620
Total expenses	(8,662)	(9,122)	(24,199)	(34,554)
Net profit (loss) after tax	**(6,329)**	**(8,154)**	**(18,916)**	**14,074**
Segment results – adjusted EBITDA				
Bronchitol & Aridol	(1,405)	(1,141)	(3,194)	(2,588)
New drug development	(3,303)	11,581	(9,226)	32,453
Corporate	(947)	(884)	(3,045)	(12,519)
Total	**(5,655)**	**9,556**	**(15,599)**	**17,346**
Statement of cash flows				
Cash inflow/ (outflow) from:				
Operations	(6,114)	10,279	(16,394)	14,918
Investing activities	(295)	(369)	(857)	(604)
Financing activities	(465)	(447)	21,307	(1,310)
Total cash generated/(used)	**(6,874)**	**9,463**	**4,056**	**13,004**
Cash at bank	**35,129**	**34,508**	**35,129**	**34,508**

Highlights

- Revenue
  - Sales for the quarter include shipments to Chiesi for the German, UK and Italian markets.
  - Sales for the nine months include the relaunch of Aridol in the US.
  - In comparing the revenue with the prior comparable periods please note the milestone from sale of drug in the March 2018 quarter related to a milestone received from Boehringer Ingelheim on the commencement of a phase 2a clinical trial in diabetic retinopathy.
- Expenses
  - Compared to the prior quarter, reduced drug development expenses, clinical trials expenses and reduced unrealised foreign currency exchange losses were partly offset by increased overhead costs transferred from inventory.
  - Total expenses for the comparable nine months included $9.6 million of costs incurred in the December quarter of 2017 associated with changes to the collaboration agreement with Synairgen.
- Cash
  - Investing activities for the quarter included $212,000 to acquire new research equipment and software for drug discovery.

Segment information

A$'000	Segment information - three months ended							
(unaudited)	31-Mar-19				31-Mar-18			
Income statements	Bronchitol & Aridol	New drug development	Corporate	Total	Bronchitol & Aridol	New drug development	Corporate	Total
Revenue								
Sale of Bronchitol	1,300			1,300	1,322			1,322
Sale of Aridol	634			634	421			421
	1,934			**1,934**	**1,743**			**1,743**
Milestones from sale of drug	-	-	-	-	-	15,239	0	15,239
Tax credit	-	-	-	-	-	-	-	-
Other revenue	6	-	128	134	5	-	120	125
	1,940	**-**	**128**	**2,068**	**1,748**	**15,239**	**120**	**17,107**
Expenses								
Employee costs	(1,566)	(752)	(465)	(2,783)	(1,499)	(696)	(445)	(2,640)
Clinical trials	-	(795)	-	(795)	(24)	(1,076)	-	(1,100)
Drug discovery	-	(1,591)	-	(1,591)	-	(1,788)	-	(1,788)
Other expenses	(1,779)	(165)	(610)	(2,554)	(1,366)	(98)	(559)	(2,023)
Total expenses	(3,345)	(3,303)	(1,075)	(7,723)	(2,889)	(3,658)	(1,004)	(7,551)
Adjusted EBITDA	**($1,405)**	**($3,303)**	**($947)**	**($5,655)**	**($1,141)**	**$11,581**	**($884)**	**$9,556**

Commentary for the quarter

- Bronchitol & Aridol:
  - Sales of Bronchitol and Aridol are discussed in commentary above.
  - Other expenses for the quarter included an expense of $753,000 ($9,000 credit in the comparative quarter) representing the net transfer of manufacturing labour and overhead to and from inventory as product is first manufactured and then subsequently sold to distributors and customers.
- New drug development:
  - The Company does not expect to qualify for an R&D tax credit in 2019 due to total revenue for the year expected to exceed the $20 million cap.
  - Clinical trial expenses relate to the phase 1 trials being conducted in the LOXL2 program which completed in the December 2018 quarter ($348,000; $1.1 million in 2018)) and the phase 1 trial for the LOX oral program that commenced in the current quarter ($436,000).
  - Drug discovery expenses include work on the LOXL2 program ($74,000 for the quarter; $509,000 in 2017), the LOX oral program ($815,000 for the quarter; $536,000 in 2018) and the LOX topical program ($493,000 for the quarter; nil in 2018).

A$'000	Segment information - nine months ended							
(unaudited)	31-Mar-19				31-Mar-18			
Income statements	Bronchitol & Aridol	New drug development	Corporate	Total	Bronchitol & Aridol	New drug development	Corporate	Total
Revenue								
Sale of Bronchitol	1,957	-	-	1,957	2,793	-	-	2,793
Sale of Aridol	2,214	-	-	2214	1,401	-	-	1,401
	4,171	**-**	**-**	**4,171**	**4,194**	**-**	**-**	**4,194**
Milestones from sale of drug	-	-	-	-		42,130	-	42,130
Clinical reimbursement	-	-	-	-	1,187	-	-	1,187
Tax credit	-	-	-	-		160	-	160
Other revenue	20		378	398	182	5	349	536
	4,191	**-**	**378**	**4,569**	**5,563**	**42,295**	**349**	**48,207**
Expenses								
Employee costs	(4,447)	(2,166)	(1,499)	(8,112)	(4,205)	(2,031)	(1,441)	(7,677)
Clinical trials	621	(1,857)	-	(1,236)	(190)	(2,290)	-	(2,480)
Drug discovery	-	(4,792)	-	(4,792)	-	(5,227)	-	(5,227)
Other expenses	(3,559)	(411)	(1,924)	(5,894)	(3,756)	(294)	(1,847)	(5,897)
Change in collaboration	-	-	-	-	-	-	(9,580)	(9,580)
Total expenses	(7,385)	(9,226)	(3,423)	(20,034)	(8,151)	(9,842)	(12,868)	(30,861)
Adjusted EBITDA	**($3,194)**	**($9,226)**	**($3,045)**	**($15,465)**	**($2,588)**	**$32,453**	**($12,519)**	**$17,346**

Commentary for the nine months

- Bronchitol & Aridol:
  - Sales of Bronchitol and Aridol are discussed in commentary above.
  - Clinical trial reimbursements and clinical trial costs ceased following completion of study CF303 in 2017.
  - Positive clinical trials expense consisted of a $621,000 unexpected refund from the clinical research organization that managed the CF303 clinical trial.
- New drug development:
  - The milestones from sale of drug in the prior period relate to two milestones received from Boehringer Ingelheim on the commencement of phase 2a clinical trial in NASH and diabetic retinopathy.
  - The Company does not expect to qualify for an R&D tax credit in 2019 due to total revenue for the year expected to exceed the $20 million cap.
  - Clinical trial expenses relate to the phase 1 trials being conducted in the LOXL2 program which completed in the December 2018 quarter ($1.4 million; $2.4 million in 2018)) and the phase 1 trial for the LOX oral program that commenced in the current quarter ($484,000).
  - Drug discovery expenditure for the period includes the LOXL2 program ($804,000 compared to $2.1 million in 2018), the LOX oral program ($1.8 million compared to $1.5 million in 2018) and the LOX topical program ($943,000 compared to nil in 2018).
- Corporate:
  - Note the $9.6 million of costs incurred in 2018 associated with changes to the collaboration agreement with Synairgen.

Income statements

A$'000	Three months ended		Nine months ended	
(unaudited)	**31-March-19**	**31-Dec-18**	**31-Dec-19**	**31-Dec-18**
Revenue				
Revenue from sale of goods	1,934	1,743	4,171	4,194
Milestones from sale of drug	-	15,239		42,130
Clinical trial cost reimbursements	-	1	-	1,188
Interest	265	169	714	413
Drug discovery service fee	-	-	-	
R&D tax incentive	-	-	-	160
Other	134	124	398	535
Total revenue	**$2,333**	**$17,276**	**$5,283**	**$48,620**
Expenses				
Employee costs	(3,085)	(2,933)	(9,074)	(8,582)
Administration & corporate	(489)	(494)	(1,687)	(1,822)
Rent, occupancy & utilities	(359)	(329)	(1,038)	(930)
Clinical trials	(794)	(1,100)	(1,235)	(2,480)
Drug development	(1,591)	(1,788)	(4,792)	(5,227)
Sales, marketing & distribution	(227)	(348)	(761)	(897)
Safety, medical and regulatory affairs	(138)	(200)	(616)	(573)
Manufacturing purchases	(299)	(488)	(931)	(1,241)
Other	(1,025)	(237)	(871)	(616)
Depreciation & amortisation	(662)	(787)	(1,954)	(2,352)
Foreign currency exchange gains & losses	130	(278)	(1,115)	177
Finance costs	(123)	(140)	(125)	(431)
Costs in relation to change in collaboration agreement	-	-	-	(9,580)
Total expenses	**(8,662)**	**(9,122)**	**(24,199)**	**(34,554)**
Net profit (loss) before tax	**(6,329)**	**8,154**	**(18,916)**	**14,066**
Income tax credit/(expense)	-	-	-	8
Net profit (loss) after tax	**($6,329)**	**$8,154**	**($18,916)**	**$14,074**

Summary balance sheets

A$'000		
(unaudited)	**31-Mar-19**	**30-June-18**
Assets		
Cash	35,129	31,073
R&D tax credit receivable	-	-
Accounts receivable	2,125	1,787
Inventory	2,251	2,398
PP&E	10,835	12,451
Other	2,484	2,388
	$52,824	**$50,097**
Liabilities		
Accounts payable and accrued expenses	3,198	4,926
Lease liability (Frenchs Forest facility)	7,462	8,268
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	23,442	22,754
Other liabilities	2,882	3,031
	$36,984	**$38,979**
Net Assets	**$15,840**	**$11,118**