Quarterly Shareholder Update – September 2016

Pharmaxis priorities for the quarter

Dear Shareholder,

Welcome to the update for the September quarter 2016 which will detail the significant progress made across the business in recent months. In talking to potential new investors I often find myself explaining the difference between Pharmaxis and other companies in the sector. Pharmaxis does stand out as having a relatively broad portfolio of drugs and drug candidates at various stages of development and commercialisation. The benefit of this model is that downside risk in the stock is mitigated by the number of opportunities we have in our pipeline. The challenge of this model is to stay focused on the opportunities which are going to deliver the greatest value for shareholders both in the short and long term. This quarterly update contains information on all aspects of the business but before you read on I just wanted to highlight our two key priority areas:

1. **Getting a breakthrough treatment in fibrosis to a significant value step**
   The Pharmaxis drug PXS-4728A acquired by Boehringer Ingelheim (BI) will deliver short term value (cash milestones) in 2017 when BI commence phase 2 studies in either one or two indications; the first of those being in the liver disease NASH. Whilst this is obviously important, BI is responsible for the development steps and this doesn't absorb too much of our time. On the other hand our LOXL2 inhibitor program is very much a work in progress and this next quarter promises to be exciting as we move from the exploratory phase of drug discovery to formal development, starting with toxicity studies and leading to phase 1 studies in the second half of 2017. This update reports on recent deal values in this therapeutic area for compounds against which we truly believe we will be competitive. LOXL2 is an excellent target in fibrosis and we have some promising drug candidates that will be significantly de-risked when we complete the toxicity trials in first half of 2017.
2. **Bringing the Bronchitol franchise to profitability**
   You will read about a plethora of actions and developments in a wide number of Bronchitol markets but the overriding corporate objective here is to continue to build volume, reduce our cost of goods and reach the point where the Bronchitol franchise delivers cash back to Pharmaxis. The announcement of Russian approval and the expectation of our first sales order before the end of the year is a significant step forward. The timeline for reporting of the CF303 clinical trial and subsequent US approval now that the study is fully recruited is clear and provides the largest single opportunity in the medium term for this part of the business.

I hope the above context helps you interpret this report which outlines our recent progress in more detail.

Sincerely,

Chief Executive Officer

Drug discovery

Boehringer Ingelheim advancing PXS-4728A towards a phase 2 clinical trial

Boehringer acquired PXS-4728A in May 2015 to develop initially as a treatment for non-alcoholic steatohepatitis (NASH). Under the terms of our agreement Boehringer has total responsibility for the development program and is required to make milestone payments to Pharmaxis as PXS-4728A progresses towards approval as well as other sales related payments post approval.

Boehringer is currently completing the prerequisite toxicology studies and drug formulation to enable the commencement of a phase 2 NASH trial in 2017, which will trigger a milestone payment to Pharmaxis of approximately A$25 million. Pharmaxis will receive its next periodic progress report from Boehringer in late 2016 at which time we expect to receive updated timing for the commencement of the trial - last advised as the first quarter of 2017.

Pharmaxis is also entitled to a milestone payment should Boehringer commence a phase 2 trial in a second indication.

Boehringer is a global leader in pharmaceutical products for cardiometabolic diseases and the NASH indication is a very large unmet need in this area being pursued by many pharmaceutical companies. Boehringer is therefore a strong and strategically aligned partner to develop PXS-4728A and we continue to be very pleased with their commitment to the development program.

LOXL2 inhibitor program evaluating preclinical candidates

Pharmaxis is developing selective inhibitors to the lysyl oxidase type 2 enzyme (LOXL2) utilising the amine oxidase platform that delivered PXS-4728A. The Company is focusing its efforts on NASH and kidney fibrosis and is collaborating with UK biotechnology company Synairgen plc (LSE: SNG) to develop a LOXL2 inhibitor to treat the fatal lung disease idiopathic pulmonary fibrosis (IPF). The LOXL2 enzyme also plays a role in some solid cancers.

The Pharmaxis drug discovery group has developed a small number of LOXL2 inhibitors that are suitable for further development. The lead optimisation work of the chemistry team is complete. We continue to work with Synairgen on selecting which drug candidates to take forward into the clinic for IPF and/or NASH.

Pharmaceutical company interest continues in the search for new drugs to treat NASH

The level of interest and corporate activity related to the search for drugs to address the “silent epidemic” of NASH continues to increase. The Washington Post reported on 21 September that “Non-alcoholic steatohepatitis, or NASH, is now in the sights of more than a dozen drugmakers. Allergan Plc became the latest when it recently announced two deals in less than 12 hours to acquire companies developing NASH treatments, including Tobira Therapeutics Inc. for as much as $1.7 billion in what Chief Executive Officer Brent Saunders described as a “very competitive situation.”

The cover story in Chemical & Engineering News (3 October, 2016) entitled “A silent liver disease epidemic” notes that “As nonalcoholic steatohepatitis, or NASH, stealthily becomes a leading cause of liver transplants, drug companies are racing to develop treatments.”

Pharmaxis continues to interact with more than a dozen larger companies as our NASH development program progresses and we expect that our LOXL2 small molecule inhibitor program will generate a competitive partnering process after completing phase 1 clinical trials, currently scheduled to commence in the second half of 2017.

Drug development pipeline – other programs

Other research initiatives at an earlier stage of development and involving lesser investment include

- SSAO/MAOB inhibitor program with potential anti-inflammatory application in a number of indications. We have a lead candidate for this program and are focusing on selecting the appropriate indication before proceeding to formal preclinical development and phase 1 studies.
- LOX inhibitor program with potential anti-fibrotic application in scaring. See "Stopping scars before they form" later in this report.
- SSAO/MPO inhibitor program with potential anti-inflammatory application in cardio vascular diseases.

We have research collaborations with a number of leading universities and academics assessing the above programs as well as the utility of our LOXL2 inhibitors in oral cancer, bone marrow myelofibrosis, NASH and wound scarring.

Pharmaxis expands Scientific Advisory Board

Pharmaxis has expanded its recently established scientific advisory board with the appointment of Professor Carol Pollock.

Prof Pollock is a distinguished academic nephrologist with more than 280 publications in basic research and clinical medicine. She is Chair of the NSW Cardiovascular Research Network and Chairs the Research Advisory Committee of the Australian and New Zealand Society of Nephrology. Professor Pollock has been the Chair of the Northern Sydney Local Health District Board since its inception in 2011 and since 2016 was Director and then Chair of the NSW Bureau of Health Information. She is a current Director of Kidney Health Australia and Chairs the International Society of Nephrology Meetings committee, responsible for delivering both research and educational meetings and policy forums across the globe.

Professor Pollock joins our existing scientific advisory board members Professor Jacob George and Dr Alan Robertson in overviewing the Company's drug discovery and development programs.

Further information concerning the scientific advisory board is available on the Pharmaxis website.

Bronchitol for cystic fibrosis

Bronchitol® is an inhaled dry powder for the treatment of cystic fibrosis (CF) and has been the subject of two large scale global clinical trials conducted by Pharmaxis. The product is approved and marketed in Europe and Australia and a third large multicentre clinical trial is currently underway aiming to secure approval in the United States.

Two important milestones were achieved in the September quarter – approval of Bronchitol in Russia and the completion of recruitment in our large multicentre phase 3 clinical trial.

Russian approval for Bronchitol

On 27 September we were pleased to announce the receipt of marketing approval of Bronchitol in Russia for the treatment of both paediatric and adult CF patients. Russia is the largest market accessed to date for Bronchitol.

This announcement carried extra significance because Bronchitol is the first medicine to be processed under new Russian laws to provide patients access to innovative medicines. The new orphan drug legislation was announced by the Russian Ministry of Health in January 2016, and Bronchitol was designated as an orphan drug the following month.

There are approximately 7,400 CF patients on the Russian Cystic Fibrosis Registry, but it is estimated there are between 3,000 and 6,000 CF patients living in rural regions not currently included on the registry. Last year the Russian market for CF drugs to deal with mucus clearance was approximately US$29 million. The first Russian

sales of Bronchitol are expected before the end of 2016.

Russia's Ministry of Health Orphan Committee will now consider Bronchitol's application for reimbursement under the country's program for guaranteed funding of seven orphan diseases known as the 7 Nosologies Program. CF drugs are purchased by the Russian Ministry of Health by way of a tender process.

There are 40 cystic fibrosis centres for children and 3 for adult patients in Russia. Additionally, some small centers are located within the pulmonology departments of paediatric hospitals.

President of the Russian Association of patients with cystic fibrosis Professor Nikolay Kapranov welcomed the approval saying, "The staff of the Russian and Moscow centres of cystic fibrosis are very pleased that Bronchitol has state registration in Russia and that our patients will have access to a new innovative product . We believe the vast majority of our CF patients will be able to have free access to this modern product once it is included in the 7 Nosologies Program."

Elena Amelina, Head of CF laboratory Scientific Research Center of Pulmonology MOH Russia said, "State registration of Bronchitol in Russia is a very important and timely step in the treatment of cystic fibrosis in this country. Bronchitol is an effective product in the treatment of cystic fibrosis giving improved drainage of the bronchus-pulmonary system. Given the process of state registration and inclusion in the list of orphan drugs is now complete, we urgently wait for Bronchitol's inclusion in the 7 Nosologies Program and the supply of the product in Russia so it can be included in the basic treatment of our patients."

Pharmaxis has been supported in its Russian application by its Russian distributor who will provide in-country logistical support for Bronchitol. Pharmaxis will engage the services of four CF medical specialists to support the use of Bronchitol in the clinic.

United States

On 15 July we announced the completion of recruitment for the clinical trial which commenced recruitment in October 2014. It is being conducted in 126 sites across 21 countries and, and enrolled 423 adult CF patients. Approximately 65% of patients have now completed the study, the results of which are expected to be reported in the second quarter of 2017.

In the US Pharmaxis has partnered with Chiesi Farmaceutici SpA (Chiesi) to conduct the international phase 3 clinical trial (CF303) designed to meet the remaining clinical requirements of the US Food and Drug Administration (FDA). Under the terms of the agreement and following a positive outcome of the trial, Chiesi will have responsibility for completing the New Drug Application with the FDA and the commercialisation of Bronchitol in the United States. We continue to work closely with Chiesi on all aspects of securing US marketing approval for Bronchitol.

Subject to a positive trial outcome, a decision on approval can be expected in 2018.

Chiesi is responsible for funding up to US$22 million of the cost of the trial, the total cost of which is expected to be approximately US$26 million. Mid to high teen percentage royalties and milestones totaling up to US$25 million are payable to Pharmaxis including US$10 million on the launch of Bronchitol.

Europe

In the EU, Chiesi has been Pharmaxis' exclusive distributor for the UK and Germany since 1 June 2015. Chiesi is an experienced and respected partner in key global markets and sells Bronchitol as part of its cystic fibrosis portfolio.

Having built local European inventory levels in the prior year, Chiesi only placed a smaller order of $128,000 which was delivered in the September quarter. In-market sales data for the quarter ended 30 September indicates total Chiesi sales in the UK and Germany increasing 11% over the immediately prior quarter (30 June 2016), subsequent to Chiesi marketing initiatives that commenced in 2016. Ongoing Bronchitol sales by Pharmaxis will continue to reflect the timing of Chiesi orders to replenish inventory rather than in market use of the product.

Pharmaxis is also currently selling Bronchitol in Austria, Denmark and Norway via its German

based logistics provider, with sales totaling $52,000 in the current September quarter.

Other territories

Bronchitol is sold in Australia by Pharmaxis (sales of $194,000 in the September quarter) as well as in Turkey by an exclusive specialist distributor.

Distributor appointment, approval and/or reimbursement applications continue to progress in various countries including Spain, Israel, Italy, several Eastern European countries and Brazil where Bronchitol will be marketed by distributors.

Corporate

2016 Annual General Meeting

The 2016 Annual General Meeting will be held on 29 November 2016 at the Christie Conference Centre, 3 Spring Street, Sydney, at 2.30 pm (Sydney time). The Notice of Meeting and Proxy Voting Form were distributed to shareholders on 26 October. The formal part of the Meeting will cover the consideration of the Company's financial statements and remuneration report, the re-election of a non-executive director and the grant of securities to our Chief Executive Officer.

Pharmaxis in the media

"Stopping scars before they form"

Pharmaxis is collaborating on a potential new scar treatment for burns, keloids and Dupuytren contracture. A news release by the American Chemical Society has highlighted early stage research from a group including The University of Western Australia, Fiona Wood Foundation, Royal Perth Hospital Burns Unit and Pharmaxis presented at the prestigious American Chemical Society meeting in Philadelphia.

The research team tested their molecules using a "Scar-in-a-jar" model, which mimics scar formation. In short, this technique involved culturing human fibroblasts from scar tissues in a petri dish. The cells overproduce and secrete collagen, as they would in a real injury. In the study, the researchers added Pharmaxis LOX inhibitors to cultures from patients with Dupuytren's, keloids and other scar tissue, and detected changes using two-photon microscopy combined with biochemical and immunohistochemical analyses.

The preliminary data strongly suggested that lysyl oxidase inhibition alters the collagen architecture and restores it to the normal architecture found in the skin.

Read the news release

"Pharmaxis breaks down Russia's drug barrier"

The Australian newspaper reported prominently on the approval of Bronchitol in Russia with a story on September 28:

"Small Australian drug developer Pharmaxis has become the first company in the world to get an orphan drug approved in Russia under new laws it campaigned for to allow the sale of its cystic fibrosis treatment."

Read the full article.

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Financials

Key financial metrics

A$'000	Three months ended	
(unaudited)	**30-Sep-16**	**30-Sep-15**
Income statements		
Sales	897	2,084
Total revenue	5,010	4,821
Total expenses	(9,095)	(10,983)
Net profit (loss) after tax	**(4,085)**	**(6,169)**
Segment results – adjusted EBITDA		
Bronchitol & Aridol	(1,452)	(1,151)
New drug development	(1,118)	(984)
Corporate	(1,228)	(423)
Total	**(3,798)**	**(2,558)**
Statement of cash flows		
Cash inflow/ (outflow) from:		
Operations	(6,173)	(3,432)
Investing activities	(150)	(446)
Financing activities	(426)	(442)
Total cash used	**(6,749)**	**(4,320)**
Foreign currency exchange rate changes impact on cash	-	507
Cash at bank	**32,460**	**50,325**

Highlights

- Sales revenue for the quarter was lower than the comparable period primarily due to Chiesi reducing its inventory of Bronchitol, as discussed above. This was partially offset by increased sales of Aridol, primarily attributable to increased sales to our Korean distributor.
- Total revenue for the quarter increased because of the higher expenditure in relation to clinical trial CF303 which is reimbursable by Chiesi.
- Underlying core expenses were in the main unchanged from the comparable period, however three specific items accounted for a net decrease in total expenses of $1.9 million.
    - Clinical trial expenses in relation to clinical trial CF303 increased by $1.4 million to $3.8 million
    - Foreign exchange losses of $1.9 million in the September 2015 quarter reversed to a gain in the current quarter of $584,000, including an unrealised loss for the September 2015 quarter of $2.6 million and an unrealised gain for the September 2016 quarter in relation to the financing agreement with NovaQuest.
    - Other expenses of $431,000 in the September 2015 quarter reversed to be a gain of $311,000 in the current quarter, this being the result of a larger recovery of overhead into the cost of inventory.

- Segment information provided below provides a useful overview of the business. Note that the increase in the Corporate adjusted EBITDA is the result of foreign exchange gains of $644,000 in the September 2015 quarter.
- Closing cash for the quarter was $32.5 million. Cash used during the quarter of $6.7 million includes significant movements in working capital:
    - Increase in inventory ($0.5 million) including the manufacture of Russian launch stock
    - Increase in accounts receivable ($0.7million) resulting mainly from an increase in reimbursable clinical trial costs paid in the quarter. At 30 September the Company had $2.4 million reimbursable clinical trial costs in accounts receivable.
    - Decrease in accounts payable and accrued liabilities - $1.7 million

The company has completed its R&D tax credit claim for the 2016 and expects to lodge its tax return and receive its credit of $2.1 million in the December quarter.

As noted above the Company also expects to receive approximately $25 million from Boehringer Ingelheim when it commences a phase 2 trial of PXS-4728A in 2017.

Segment information

A$'000	Segment information - three months ended							
(unaudited)	**30-Sep-16**				**30-Sep-15**			
Income statements	**Bronchitol & Aridol**	**New drug developmt**	**Corporate**	**Total**	**Bronchitol & Aridol**	**New drug developmt**	**Corporate**	**Total**
Revenue								
Sale of Bronchitol	392	-	-	392	1,638	-	-	1,638
Sale of Aridol & other	505	-	-	505	446	-	-	446
	897	**-**	**-**	**897**	**2,084**	**-**	**-**	**2,084**
Clinical reimbursement	3,576	-	-	3,576	2,168	-	-	2,168
Tax credit	-	-	-	-	-	-	-	-
Other revenue	8	224	80	312	-	167	83	250
	4,481	**224**	**80**	**4,785**	**4,252**	**167**	**83**	**4,502**
Expenses								
Employee costs	(1,485)	(508)	(647)	(2,640)	(1,401)	(380)	(631)	(2,412)
Clinical trials	(3,770)	-	-	(3,770)	(2,317)	(53)	-	(2,370)
Drug development	-	(732)	-	(732)		(639)	-	(639)
Other expenses	(678)	(102)	(661)	(1,441)	(1,685)	(79)	125	(1,639)
Total expenses	(5,933)	(1,342)	(1,308)	(8,583)	(5,403)	(1,151)	(506)	(7,060)
Adjusted EBITDA	**(1,452)**	**(1,118)**	**(1,228)**	**(3,798)**	**(1,151)**	**(984)**	**(423)**	**(2,558)**

Income statements

A$'000	Three months ended	
(unaudited)	**30-Sep-16**	**30-Sep-15**
Revenue		
Revenue from sale of goods	897	2,084
Clinical trial cost reimbursements	3,577	2,168
Interest	224	319
Drug discovery service fee	224	167
Other	88	83
Total revenue	**5,010**	**4,821**
Expenses		
Employee costs	(2,892)	(2,811)
Administration & corporate	(536)	(505)
Rent, occupancy & utilities	(244)	(296)
Clinical trials	(3,770)	(2,370)
Drug development	(732)	(639)
Sales, marketing & distribution	(207)	(324)
Safety, medical and regulatory affairs	(375)	(408)
Manufacturing purchases	(314)	(365)
Other	311	(431)
Foreign currency exchange gains & losses	584	(1,909)
Depreciation & amortisation	(758)	(750)
Finance expenses	(162)	(175)
Total expenses	**(9,095)**	**(10,983)**
Net profit (loss) before tax	**(4,085)**	**(6,162)**
Income tax expense	-	(7)
Net profit (loss) after tax	**(4,085)**	**(6,169)**