

Quarterly Shareholder Update – June 2017



Dear Shareholder,

Pharmaxis completed a quarter with several notable achievements that position the Company for a new phase of growth.

The phase 2 NASH trial being conducted by Boehringer Ingelheim on the drug PXS-4728A acquired from Pharmaxis in 2015 commenced recruiting subjects in June. When the first patient is dosed it will trigger a milestone payment to Pharmaxis of approximately A\$26 million.

In addition, Boehringer has also advised that a phase 2 trial in a second indication is scheduled to commence in the second half of 2017, and this event will trigger a milestone payment to Pharmaxis of approximately A\$14 million. Boehringer's plans to pursue two indications in parallel is a signal of the confidence they have in the drug and doubles the potential development milestone payments Pharmaxis can receive.

Also during the quarter, our LOXL2 development program, which is being conducted in collaboration with UK biotech Synairgen plc, remained on track to progress into a phase 1 clinical study in the second half of 2017. Meetings held at the BIO international partnering meeting in June confirmed the high level of interest in this program from multinational Pharma companies.

In June, Pharmaxis reported on the international clinical trial of Bronchitol – a study which paves the way for our partner Chiesi to conclude the US application to market the product for adult cystic fibrosis patients. This step also completes the change in our business model as we reduce our exposure to late stage clinical development and commercialisation risk and generate increasing value from early stage clinical development and partnering.

Our Drug Discovery team have done outstandingly well to generate five lead candidate small molecule drugs to four different targets in the last two years since we sold the first phase 1 asset to Boehringer. To reflect the increasing opportunity to create shareholder value in this early stage pipeline we have recently strengthened our Board experience and expertise through the appointment of Dr Kathleen Metters. Dr Metters has international experience in new drug development from a distinguished career which included a period as head of Worldwide Basic Research at Merck & Co. Dr Metters will be a significant asset as we grow our drug development pipeline and her appointment builds on what is already a strong and committed leadership group at Board level.

The report below outlines our progress in more detail.

Sincerely,



Chief Executive Officer

Drug discovery

Boehringer Ingelheim phase 2 NASH trial recruiting, second indication phase 2 trial planned for 2017

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 the drug progresses towards approval as well as other sales related payments post approval.

Boehringer has advised that a phase 2 NASH trial has commenced recruitment and this is reported on the U.S National Institutes of Health website ClinicalTrials.gov. The dosing of the first patient will trigger a milestone payment to Pharmaxis of €18 million (approximately A\$26 million).

Following on from its report last quarter concerning plans for the clinical development for a second indication, Boehringer has now advised that a phase 2 trial in a second indication will commence in the second half of 2017, triggering the payment of a €10 million (approximately A\$14 million). Total milestones through to approval for a second indication are in aggregate the same as for the first indication (€195million for each), but weighted more towards the approval milestones.

LOXL2 inhibitor program preclinical candidates in toxicology studies

The Pharmaxis drug discovery group has developed a number of selective inhibitors to the lysyl oxidase type 2 enzyme (LOXL2) utilising the same amine oxidase platform that delivered the BI partnered drug PXS-4728A. LOXL2 is a key enzyme in the formation of excessive collagen depositions and important in the liver disease NASH, cardiac fibrosis, kidney fibrosis, the fatal lung disease idiopathic pulmonary fibrosis (IPF) and also plays a role in some solid cancers.

Pharmaxis is working with its collaborator, UK biotechnology company Synairgen plc (LSE: SNG) to commence a phase 1 study in the second half of 2017.

Large pharma company interest

The role of LOXL2 in fibrotic diseases such as NASH and pulmonary fibrosis is of significant interest to many large pharma companies as its inhibition is one of the very few truly anti-fibrotic mechanisms in clinical development. The annual US BIO conference in June 2017 provided an opportunity to share updates with them on our scientific progress and the timing of our partnering process which we plan to initiate in the second half of 2017 and conclude with a partnering deal in 2018.

Drug development pipeline – other programs include

- A LOX inhibitor program which has potential anti-fibrotic application in scarring. Pharmaxis is working together with the University of Western Australia, the Fiona Wood Foundation and the Royal Perth Hospital Burns Unit on this project. The research is currently focused on formulation and is scheduled to commence formal preclinical toxicology studies in the second half of 2017. Research work is also exploring other severe fibrotic indications where the Company's LOX inhibitor may also have application.
- The SSAO/MPO program which is developing a dual inhibitor with potential anti-inflammatory application in respiratory and cardiovascular disease. The research is currently focused on fully profiling the drugs under development and identifying the appropriate indications to pursue.
- The SSAO/MAOB inhibitor program has potential anti-inflammatory application in a number of indications. Further investment in this project has been postponed whilst awaiting the results from a number of academic collaborators focusing on identifying the appropriate indication.

Bronchitol for cystic fibrosis

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.

United States

A third international multicentre clinical trial (CF303) designed to meet the remaining clinical requirements of the US Food and Drug Administration (FDA) reported in June 2017. The trial was conducted in 126 sites across 21 countries and enrolled 423 adult CF patients.

- The phase 3 trial of Bronchitol (mannitol) in adults with cystic fibrosis (CF) met its primary endpoint by demonstrating superiority over the comparator on (FEV1 change from baseline over 26 week treatment period), with an effect of 54 ml (p=0.020).
- The study had recruited adult CF patients with all grades of disease that were already on the best standard of care. The improvement in lung function of 2.2% (p=0.025) was less than that seen in the adult CF population in previously reported Bronchitol phase 3 studies.
- No statistically significant differences between treatment groups in secondary endpoints were recorded, although a trend was observed in favour of Bronchitol for other lung function parameters (FVC).
- Bronchitol had a good safety profile with similar rates of adverse events seen compared to control.

Pharmaxis and its US partner Chiesi believe the results are sufficient to underpin a resubmission of the Bronchitol New Drug Application to the FDA which is expected to occur in 2018. Chiesi funded US\$22 million of the clinical trial, the total cost of which is approximately US\$26 million. Under the terms of the agreement Chiesi will be responsible for completing the New Drug Application with the FDA and the commercialisation of Bronchitol in the United States. The Company continues to work closely with Chiesi on all aspects of securing US marketing approval for Bronchitol.

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

Europe

In the EU, Pharmaxis has appointed Chiesi as its exclusive distributor for the markets of the UK, Ireland and Germany. In May 2017 Chiesi was also appointed exclusive distributor for the Italian market and it expects to launch Bronchitol in Italy in the second half of 2017. During the 2016 financial year Chiesi built up its initial inventory levels of Bronchitol and in the 2017 financial year allowed these levels to reduce, resulting in decreased sales by Pharmaxis in 2017.

During the March 2017 quarter regular six monthly supply recommenced. The next shipments are expected in the second half of 2017. Ongoing Bronchitol sales by Pharmaxis will continue to reflect the expected six monthly ordering by Chiesi rather than in-market use of the product.

Chiesi unit sales to pharmaceutical wholesalers in the UK and Germany for the June 2017 quarter were 5% below the level of sales in 2016 and for the financial year were 3% above 2016.

Pharmaxis also sells Bronchitol in Austria, Denmark and Norway via its German based logistics provider, and commenced selling into Spain during the quarter via its exclusive distributor. Sales to these other European markets totaling \$35,000 in the June 2017 quarter, \$158,000 for the year to date.

Other territories

Bronchitol is sold in Australia by Pharmaxis and in Turkey and Russia by exclusive distributors.

Pharmaxis' application to expand country wide reimbursement in Australia will be determined in the second half of 2017 by the Pharmaceutical Benefits Advisory Committee.

Sales for the quarter and year to date were as follows:

A\$'000	Three months		Twelve months	
	June 17	June 16	June 17	June 16
Australia	170	139	736	693
Russia	-	-	643	-
Turkey	71	48	277	48

Aridol

Aridol sales increased 14% for the quarter to \$589,000 and 11% year to date to \$2.0million. Pharmaxis is working towards reintroducing Aridol to the United States via a suitably experienced distributor.

Corporate



New board appointment – Dr Kathleen Metters

On 7 June Pharmaxis announced the appointment of Dr Kathleen Metters to the Board of Directors, following a global search for a candidate who could contribute to the Pharmaxis business model of creating value through excellence in drug discovery and development. Dr Metters has a long and distinguished career in drug discovery in big pharma. Dr Metters spent 9 years as a senior executive with Merck & Co. including a 4 year period as Senior Vice President and Head of

Worldwide Basic Research. That was followed by an appointment to design and establish External Discovery and Preclinical Sciences in order to expand Merck’s scientific network.

After leaving Merck in 2011 Dr Metters was appointed President and Chief Executive Officer for Lycera Corp., a biopharmaceutical company pioneering innovative approaches to novel oral medicines for treatment of autoimmune diseases and cancer. Under her leadership, Lycera developed a robust pipeline of proprietary and partnered immune modulator programs which formed the basis, in June 2015, of an exclusive global collaboration with Celgene Corporation.

Dr Metters is ideally qualified to make a significant contribution to the Pharmaxis business. As required by the Pharmaxis constitution, Dr Metters will offer herself for re-election at the company’s 2017 Annual General Meeting.

Further detail can be found on the [Pharmaxis website](#)

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Financials

Key financial metrics

(unaudited)	A\$'000		Three months ended		Twelve months ended	
			30-June-17	30-June-16	30-June-17	30-June-16
Income statements						
Sales			866	703	4,823	6,135
Total revenue			6,945	4,795	18,001	19,020
Total expenses			(11,067)	(6,919)	(36,347)	(35,476)
Net profit (loss) after tax			(4,122)	(2,124)	(18,346)	(16,463)
Segment results – adjusted EBITDA						
Bronchitol & Aridol			(2,421)	(2,141)	(7,100)	(8,228)
<i>Bronchitol & Aridol – excluding net clinical trial costs</i>			<i>(1,803)</i>	<i>(1,582)</i>	<i>(5,546)</i>	<i>(5,228)</i>
New drug development			199	92	(4,114)	(2,625)
Corporate			(1,005)	(1,132)	(4,017)	(3,988)
Total			(3,227)	(3,181)	(15,231)	(14,841)
Statement of cash flows						
Cash inflow/ (outflow) from:						
Operations			(4,228)	(1,559)	(15,181)	(11,989)
Investing activities			(328)	(146)	(725)	(1,381)
Financing activities			(434)	(425)	(1,721)	(1,714)
Total cash used			(4,990)	(2,130)	(17,627)	(15,084)
Foreign currency exchange rate changes impact on cash			(5)	(169)	(78)	155
Cash at bank			21,504	39,209	21,504	39,209

Highlights

- Sales revenue for the quarter increased over 2016 for both Bronchitol and Aridol. As discussed above the ordering patterns of our distributors in major overseas markets are not evenly spread throughout the year and as such there were no sales to the UK, German or Russian markets in the current quarter comparative quarter (June 2016).
- Total revenue for the quarter increased primarily because of an increased recognition of the clinical trial CF303 reimbursement by Chiesi, and the recognition of a \$3.1 million R&D tax incentive in relation to the 2017 financial year compared to a \$2.1 million incentive in 2016.
- Underlying core expenses for the quarter were mainly unchanged from the comparable period, however four specific items accounted for a net decrease in total expenses of \$661,000.
 - Clinical trial expenses in relation to clinical trial CF303 increased by \$0.8 million.
 - Drug development expenses increased by \$1.0 million reflecting increased levels of research activity in several projects.

- Foreign exchange losses of \$0.8 million in the June 2016 quarter increased to a gain of \$0.2 million in the current quarter, including an unrealised loss in relation to the financing agreement with NovaQuest of approximately \$0.9 million in the June 2016 quarter increasing to a gain of \$0.1 million in the current quarter.
- Other expenses include 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. Other expenses were a credit of \$552,000 in the June 2016 quarter and \$211,000 in the current quarter, reflecting a transfer of manufacturing and overhead into inventory.
- Segment information provided below provides a useful overview of the business.
- We have separately shown the Bronchitol & Aridol segment without clinical trial costs and related reimbursements to better identify the base segment.
- Note that the decrease in the Corporate Adjusted EBITDA reflects foreign exchange gains of \$48,000 in the June 2016 quarter increasing to \$114,000 in the June 2017 quarter.
- Closing cash for the quarter was \$21.5 million. Cash used during the quarter was \$5.0 million, \$17.7 million for the full year. Note that Pharmaxis has been funding clinical trial CF303 since Chiesi completed its contribution of US\$22 million in December 2016.
- As noted above the Company also expects to receive approximately \$26 million from Boehringer Ingelheim when it commences dosing in a phase 2 trial of PXS-4728A in the coming weeks.

Segment information

A\$'000								
Segment information - three months ended								
(unaudited)	30-June-17				30-June-16			
Income statements	Bronchitol & Aridol	New drug developmt	Corporate	Total	Bronchitol & Aridol	New drug developmt	Corporate	Total
Revenue								
Sale of Bronchitol	277			277	187			187
Sale of Aridol & other	589			589	516			516
	866			866	703			703
Clinical reimbursement	2,592			2,592	1,248			1,248
Tax credit	54	3,045		3,099	544	1,556		2,100
Other revenue	168	0	86	254	31	356	58	445
	3,680	3,045	86	6,811	2,526	1,912	58	4,496
Expenses								
Employee costs	(1,581)	(512)	(530)	(2,623)	(1,349)	(530)	(579)	(2,458)
Clinical trials	(3,210)			(3,210)	(2,454)			(2,454)
Drug development		(2,165)		(2,165)		(1,138)		(1,138)
Other expenses	(1,310)	(169)	(561)	(2,040)	(864)	(152)	(611)	(1,627)
Total expenses	(6,101)	(2,846)	(1,091)	(10,038)	(4,667)	(1,820)	(1,190)	(7,677)
Adjusted EBITDA	(\$2,421)	\$199	(\$1,005)	(\$3,227)	(\$2,141)	\$92	(\$1,132)	(\$3,181)

A\$'000								
Segment information - twelve months ended								
(unaudited)	30-June-17				30-June-16			
Income statements	Bronchitol & Aridol	New drug developmt	Corporate	Total	Bronchitol & Aridol	New drug developmt	Corporate	Total
Revenue								
Sale of Bronchitol	2,785			2,785	4,302			4,302
Sale of Aridol & other	2,038			2,038	1,833			1,833
	4,823	0	0	4,823	6,135	0	0	6,135
Clinical reimbursement	8,463			8,463	8,200			8,200
Tax credit	70	3,089		3,159	544	1,556		2,100
Other revenue	188	328	336	852	31	1,024	317	1,372
	13,544	3,417	336	17,297	14,910	2,580	317	17,807
Expenses								
Employee costs	(6,037)	(2,026)	(2,058)	(10,121)	(5,560)	(1,782)	(2,116)	(9,458)
Clinical trials	(10,017)			(10,017)	(11,846)	(109)		(11,955)
Drug development		(5,068)		(5,068)		(2,910)		(2,910)
Other expenses	(4,590)	(437)	(2,295)	(7,322)	(5,732)	(404)	(2,189)	(8,325)
Total expenses	(20,644)	(7,531)	(4,353)	(32,528)	(23,138)	(5,205)	(4,305)	(32,648)
Adjusted EBITDA	(\$7,100)	(\$4,114)	(\$4,017)	(15,231)	(\$8,228)	(\$2,625)	(\$3,988)	(\$14,841)

Income statements

(unaudited)	Three months ended		Twelve months ended	
	30-June-17	30-June-16	30-June-17	30-June-16
Revenue				
Revenue from sale of goods	866	703	4,823	6,135
Clinical trial cost reimbursements	2,592	1,248	8,463	8,200
Interest	135	298	703	1,213
Drug discovery service fee	(2)	257	328	925
R&D tax incentive	3,100	2,100	3,160	2,100
Other	254	189	524	447
Total revenue	\$6,945	\$4,795	\$18,001	\$19,020
Expenses				
Employee costs	(2,860)	(2,862)	(11,063)	(10,529)
Administration & corporate	(549)	(531)	(1,947)	(2,082)
Rent, occupancy & utilities	(303)	(321)	(1,148)	(1,296)
Clinical trials	(3,210)	(2,453)	(10,017)	(11,955)
Drug development	(2,165)	(1,138)	(5,068)	(2,910)
Sales, marketing & distribution	(346)	(246)	(1,061)	(1,101)
Safety, medical and regulatory affairs	(430)	(344)	(1,379)	(1,707)
Manufacturing purchases	(311)	(785)	(1,326)	(1,928)
Other	(211)	552	(437)	(382)
Depreciation & amortisation	(768)	(755)	(3,059)	(3,028)
Foreign currency exchange gains & losses	232	(837)	781	(843)
Finance expenses	(146)	2,975	(623)	2,459
		(174)		(174)
Total expenses	(11,067)	(6,919)	(36,347)	(35,476)
Net profit (loss) before tax	(4,122)	(2,124)	(18,346)	(16,456)
Income tax expense	-	-	-	(7)
Net profit (loss) after tax	\$(4,122)	\$(2,124)	\$(18,346)	\$(16,463)