

Quarterly Shareholder Update – December 2017



Dear Shareholder,

Pharmaxis closed out 2017 in a good position having made significant progress in drug development with value inflection points in a number of programs now in sight. This goes to the very core of what it means to be a research-based biotech company. We have assembled a very talented team, identified novel ways to cure diseases which are poorly treated and generated new drugs which we then test rigorously until they pass or fail. This process of innovation carries risk but our commitment is driven by the potential benefits for patients, shareholders and the wider community if we succeed.

I rarely make comment in relation to the company's share price but given the number of commentators and now broker analysts that say they are perplexed by the lack of movement in our stock price despite a stream of positive announcements, it seems an opportune time as we head into 2018 to point out what those valuation inflection points are.

In August 2017 Pharmaxis received \$27m from Boehringer in a milestone payment for starting a phase 2a study in the liver disease NASH with the drug acquired from us in 2015. We received another \$15m earlier this month when Boehringer started a second phase 2a trial in diabetic retinopathy with the same drug. Whilst we don't earn anymore milestone payments until commencement of phase 3 studies, the risk adjusted valuation of the Boehringer deal will be fundamentally impacted by the results of these two concurrent studies. The NASH study is due to read out in the September quarter and the diabetic retinopathy study in the December quarter this year. Both these studies will provide the first clinical proof of concept in humans and, if positive, would represent a huge step forward in their progress towards the clinic and the delivery of more financial rewards for Pharmaxis.

The second key event will be the mid-year read out from the phase 1 studies that are underway on the two lead candidates from our anti fibrotic LOXL2 program. Our decision late last year to take full scientific and commercial control of the collaboration with UK biotech Synairgen was driven by our increasing confidence in this program and the very high level of interest we are receiving from the large Pharmaceutical companies that are active in the liver and lung fibrosis markets. We have used some of the cash that the BI deal has provided to substantially increase our interest in the commercial returns from the program in exchange for a one-off payment to Synairgen.

The end of phase 1 is not a classic major value inflection point for a new drug given that you have shown safety in healthy volunteers but not yet any efficacy in patients with the disease being targeted. Despite this I believe that this point in our LOXL2 program is significant from a valuation perspective. There are very few anti fibrotic drugs under development and the number of companies chasing a good asset is high. This means that companies are looking earlier and earlier in the development pipeline of biotechs to identify promising drugs to add to their development portfolio. I met with many of these companies at the JP Morgan conference in San Francisco earlier this month and several are already investing considerable resources in doing due diligence on our science whilst the phase 1 studies are ongoing. I anticipate that a major deal in the second half of this year will be our reward if the phase 1 studies are successful.

Sincerely,

A handwritten signature in black ink, appearing to read 'Ray Ph...'. The signature is written in a cursive style with a long horizontal stroke extending to the right.

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 – NASH and diabetic retinopathy (DR). Boehringer initiated phase 2a proof of clinical principle trials for both development programs in 2017. Pharmaxis received €18 million (A\$27 million) when the NASH trial dosed its first patient in August 2017 and €10 million (A\$15 million) when the DR trial dosed its first patient in January 2018.

The NASH study is estimated to complete in July 2018 and the DR trial in November 2018. We eagerly await the results of these first trials of efficacy in humans.

NASH is an area of significant interest to large pharma companies at the present time and in addition to BI 1467334, Pharmaxis has a LOXL2 inhibitor under development for NASH, as discussed below.

DR 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 of DR is vision-threatening.

Under the deal signed in 2015, Boehringer has total responsibility for the development program and Pharmaxis receives payments for multiple indications. The total development milestones in the deal (€419m /A\$625m), would be payable to Pharmaxis should both indications be approved.

Two LOXL2 inhibitors commence phase 1 clinical trials

Two small molecule inhibitors to the LOXL2 enzyme commenced phase 1 clinical trials in the second half of 2017 and will complete single ascending dose and multiple ascending dose stages by the middle of 2018. Importantly, the phase 1 trials will also be able to measure the extent to which the compounds inhibit the LOXL2 enzyme due to the successful development of a test in the December quarter by the drug

discovery team working with an international organisation.

To increase the potential value of the program, Pharmaxis has also initiated 3 month tox studies and the scale up of manufacturing so that the successful partner will be able to commence phase 2 studies without delay.

The Pharmaxis drug discovery group developed the inhibitors to the LOXL2 enzyme utilising the same amine oxidase platform that delivered BI-1467335. The 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 extensive pre-clinical program performed on the program compounds confirmed they have all the characteristics of a successful once a day, oral drug, showing excellent efficacy in several different in vivo fibrosis models including fibrosis of the liver, lung, kidney and heart. In regulatory toxicity studies, the compounds were well tolerated with a good safety profile.

Partnering plan for LOXL2 program

The recent JP Morgan biotech conference held at the start of each year in San Francisco provided an opportunity for Pharmaxis to meet again with eight of the dozen large pharma companies interested in the Company's LOXL2 program.

Pharma is interested in the Pharmaxis program as it is one of the very few truly anti-fibrotic mechanisms in clinical development. A number of the pharma companies are well advanced in due diligence of the LOXL2 program.

Pharmaxis plans to partner the LOXL2 program in the second half of 2018 after the phase 1 trials report.

Pharmaxis acquires full control and increases its stake in LOXL2 program

In December Pharmaxis and UK biotechnology company Synairgen plc, the Company's collaborator on the LOXL2 program, announced a number of significant changes to the collaboration. Pharmaxis has taken full control of the scientific and commercial control of the collaboration and also substantially increased its

interest in the program in return for a one-off payment to Synairgen.

Changes in the collaboration financial arrangements include:

- Pharmaxis has significantly increased its share of any partnering deal for the LOXL2 program in fibrotic diseases to 83%.
- Pharmaxis has assumed full funding responsibility for the ongoing collaboration program.
- Pharmaxis made a cash payment to Synairgen of £5 million (approximately A\$9m).

While the collaboration with Synairgen in the program's pre-clinical stage served its purpose very well, the increased interest by large pharma companies in NASH, together with Pharmaxis' enhanced financial position, meant that Pharmaxis wished to develop the program under its control with its own resources in order to maximise the potential partnering opportunity. Having assumed control Pharmaxis immediately doubled the program scope to encompass two lead candidates with unique properties that it intends to take to the end of phase 1 trials with additional toxicology data to be delivered in parallel to enable the lead candidates to be phase 2 ready by mid-2018.

Drug development pipeline – other programs

In addition to the SSAO inhibitor (BI 1467335) and the LOXL2 program, Pharmaxis has two other programs that have come out of its amine oxidase chemistry platform. In the December quarter both of these programs commenced the toxicity studies that are prerequisite to being phase 1 ready later in 2018.

- a drug inhibiting both myeloperoxidase (MPO) and SSAO with potential anti-inflammatory application in respiratory and cardiovascular disease.
- a drug inhibiting all the LOX family of enzymes with potential anti-fibrotic application in scarring and severe fibrotic indications including some cancers. A poster presentation featuring a successful animal study using one of our LOX inhibitors to treat a model of myelofibrosis was included at the prestigious American Society of Haematology meeting (ASH) in December.

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 clinical team has now finalised the complete study report in relation to the international multicentre clinical trial of Bronchitol (CF303) and delivered it to the Company's US partner Chiesi who are responsible for completing and filing the New Drug Application with the FDA and the commercialisation of Bronchitol in the United States.

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 royalties and will be the exclusive supplier of Bronchitol for the US market.

Western Europe

In the EU, Pharmaxis has appointed Chiesi as its exclusive Bronchitol distributor for the markets of the UK, Ireland, Germany and Italy. As expected Chiesi purchased Bronchitol for the German and Italian markets in the December quarter with orders for all three markets scheduled to ship in the March quarter.

Pharmaxis also markets Bronchitol in Austria, Denmark and Sweden via its German based logistics provider, and Spain via an exclusive distributor. Sales for Western Europe in the quarter were \$587,000 compared to \$26,000 in the December 2016 quarter, the increase reflecting increased sales to Chiesi for Germany.

Other territories

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

Russia represents a potential significant opportunity for Bronchitol with approximately 4,000 CF patients on the Russian Cystic Fibrosis Registry and an annual market (2015) for CF drugs to deal with mucus clearance of approximately US\$29 million. Following the receipt of approval in 2016 for both adult and paediatric CF patients, Pharmaxis has been navigating the process to have Bronchitol reimbursed nationally. Whilst the national reimbursement process is progressing, the Company has been successful in obtaining approval for a few hundred patients to receive Bronchitol on an individual reimbursement basis and the first tender of Bronchitol in relation to these patients is scheduled for the first quarter of 2018.

Sales in Australia were \$220,000 for the quarter compared to \$201,000 in the December 2016 quarter. Following the August 2017 positive recommendation from the Australian Pharmaceutical Benefits Advisory Committee, the Australian government announced extended reimbursement of Bronchitol. Effective from 1 January 2018, eligible people with cystic fibrosis who are taking Pulmozyme® (dornase alfa), another CF medication, will be able to add reimbursed Bronchitol to their treatment regime.

Aridol

Aridol sales for the quarter were A\$536,000 compared to A\$475,000 in the December 2016 quarter, with the increase primarily due to a higher level of sales to Korea in the current quarter.

Pharmaxis has expanded the territory of its US distributor, specialty pharmaceutical company Methapharm Inc, to include Canada. Pharmaxis and Methapharm are scheduled to file regulatory submissions in the current quarter for both the United States and Canada.

Corporate

Bell Potter Initiates Coverage

In December 2017 Bell Potter Securities Limited initiated research coverage of Pharmaxis with an initial valuation of \$0.54. An updated report issued early January increased the valuation to \$0.56.

For a copy of their reports contact the analyst Tanushree Jain (tnjain@bellpotter.com.au)

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Financials

Key financial metrics

(unaudited)	A\$'000		Three months ended		Six months ended	
			31-Dec-17	31-Dec-16	31-Dec-17	31-Dec-16
Income statements						
Sales of Bronchitol & Aridol			1,402	793	2,451	1,690
Sale of drug candidate			-	-	26,891	-
Total revenue			2,433	1,900	31,344	6,910
Total expenses			(17,859)	(8,850)	(25,432)	(17,945)
Net profit (loss) after tax			(15,419)	(6,950)	5,920	(11,035)
Segment results – adjusted EBITDA						
Bronchitol & Aridol			13	(2,489)	(1,447)	(3,941)
New drug development			(3,626)	(1,303)	20,872	(2,421)
Corporate			(10,654)	(850)	(11,635)	(2,078)
Total			(14,267)	(4,642)	7,790	(8,440)
Statement of cash flows						
Cash inflow/ (outflow) from:						
Operations			(13,023)	(2,721)	4,639	(8,894)
Investing activities			(125)	(64)	(235)	(214)
Financing activities			(436)	(430)	(863)	(856)
Total cash generated/(used)			(13,584)	(3,215)	3,541	(9,964)
Cash at bank			25,045	29,245	25,045	29,245

Highlights for the quarter

- The financial results for the quarter were dominated by the costs associated with changing the research collaboration agreement with Synairgen. Under the amended agreement the Company paid £5.0 million (A\$8.8 million) to Synairgen and also incurred associated legal and professional fees of \$806,000. These payments are included in the Corporate segment.
- The closing cash position at 31 December of \$25 million was increased early in the New Year by receipt of the income tax credit of \$3.3 million in respect of the 2017 financial year.
- The commencement of dosing in Boehringer's phase 2a trial in diabetic retinopathy in early January triggered a milestone payment of A\$15 million which will be received in the February 2018.
- Sales revenue for the quarter increased 77% over the comparable quarter in 2017.
- Net loss after tax for the quarter was \$15.4 million compared to a loss of \$7.0 million in the December quarter of 2016. Excluding the Synairgen related expenses of \$9.6 million the loss for the quarter was \$5.8 million.
- Total expenses for the quarter before Synairgen related expenses decreased by \$570,000 (7%) compared to the December 2016 quarter. The following items account for the net decrease in total expenses.
 - Clinical trial expenses in relation to clinical trial CF303 changed from \$1.6 million in the December 2016 quarter to a credit of \$610,000 in the December 2017 quarter as a result of the

contract research organization that conducted the study reconciling its pass through trial site costs.

- Clinical trial expenses in relation to new drug development increased by \$1.0 million as the LOXL2 program entered phase 1 studies.
- Drug development expenses increased by \$1.0 million reflecting increased levels of research activity in several projects.
- Unrealised foreign exchange losses in relation to the financing agreement reduced by 1.3 million
- Other expenses were a credit of \$200,000 in the December 2016 quarter and \$354,000 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 as well as other inventory adjustments.

Segment information

A\$'000								
Segment information - three months ended								
(unaudited)	31-Dec-17				31-Dec-16			
Income statements	Bronchitol & Aridol	New drug developm't	Corporate	Total	Bronchitol & Aridol	New drug developm't	Corporate	Total
Revenue								
Sale of Bronchitol	866			866	318	-	-	318
Sale of Aridol	536			536	475	-	-	475
	1,402			1,402	793	-	-	793
Sale of drug candidate	-	-	-	-	-	-	-	-
Clinical reimbursement	506	-		506	725	-	-	725
Tax credit	-	160	-	160	-	-	-	-
Other revenue	90	5	119	214	6	106	85	197
	1,998	165	119	2,282	1,524	106	85	1,715
Expenses								
Employee costs	(1,278)	(758)	(487)	(2,524)	(1,445)	(466)	(426)	(2,337)
Clinical trials	610	(1,015)		(405)	(1,628)	-	-	(1,628)
Drug discovery	-	(1,894)		(1,895)	-	(860)	-	(860)
Other expenses	(1,317)	(124)	(704)	(2,145)	(940)	(83)	(509)	(1,532)
Change in collaboration	-	-	(9,580)	(9,580)	-	-	-	-
Total expenses	(1,985)	(3,791)	(10,771)	(16,549)	(4,013)	(1,409)	(935)	(6,357)
Adjusted EBITDA	\$13	(\$3,626)	(\$10,652)	(\$14,267)	(\$2,489)	(\$1,303)	(\$850)	(\$4,642)

Commentary for the quarter

- Sales of Bronchitol increased primarily due to increased sales to Chiesi for Germany and Italy.
- Sales of Aridol increased primarily because of a higher level of sales to Korea in the current quarter, although sales in all markets increased.
- Clinical trial reimbursements and clinical trial costs reduced following reporting of study CF303 in June 2017. As the study has now fully reported no further reimbursement revenue or clinical trial costs will be booked. Reconciliation in the December quarter of final pass through site costs by the contract research organisation responsible for the study identified a credit due to Pharmaxis.
- Completion of the 2017 research & development income tax credit resulted in a claim \$160,000 higher than the amount booked at 30 June 2017. The full credit of \$3.3 million was received in January 2018.
- Increased new drug development expenses for the quarter reflects
 - Recent staff increases

- Two phase 1 clinical trials in relation to the LOXL2 program which commenced dosing during the quarter.
- Drug discovery expenditure for the quarter including in relation to the LOXL2 program (\$892,000) as well as on the SSAO/MPO program and the LOX programs (\$822,000) both of which have commenced preclinical tox studies.
- Bronchitol & Aridol Other Expenses increased mainly due to the change in net transfer of labour and overhead into inventory as discussed above.
- As noted above, the Company incurred expenses totaling \$9.6 million in relation to the amended research collaboration agreement with Synairgen concluded in December 2017.

A\$'000								
Segment information - six months ended								
(unaudited)	31-Dec-17				31-Dec-16			
Income statements	Bronchitol & Aridol	New drug developm't	Corporate	Total	Bronchitol & Aridol	New drug developm't	Corporate	Total
Revenue								
Sale of Bronchitol	1,471	-	-	1,471	710	-	-	710
Sale of Aridol	980	-	-	980	980	-	-	980
	2,451			2,451	1,690			1,690
Sale of drug candidate	-	26,891	-	26,891	-	-	-	-
Clinical reimbursement	1,187	-	-	1,187	4,301	-	-	4,301
Tax credit	-	160	-	160	-	-	-	-
Other revenue	177	5	229	411	14	330	165	509
	3,815	27,056	229	31,100	6,005	330	165	6,500
Expenses								
Employee costs	(2,706)	(1,335)	(996)	(5,037)	(2,930)	(974)	(1,073)	(4,977)
Clinical trials	(166)	(1,214)	-	(1,380)	(5,398)	-	-	(5,398)
Drug discovery	-	(3,439)	-	(3,439)	-	(1,592)	-	(1,592)
Other expenses	(2,390)	(196)	(1,288)	(3,874)	(1,618)	(185)	(1,170)	(2,973)
Change in collaboration	-	-	(9,580)	(9,580)	-	-	-	-
Total expenses	(5,262)	(6,184)	(11,864)	(23,310)	(9,946)	(2,751)	(2,243)	(14,940)
Adjusted EBITDA	(\$1,447)	\$20,872	(\$11,635)	\$7,790	(\$3,941)	(\$2,421)	(\$2,078)	(\$8,440)

Commentary for the half year

- Sale of Bronchitol increased primarily due to increased sales to Chiesi for Germany, Italy and the UK.
- Clinical trial reimbursements and clinical trial costs reduced following reporting of study CF303 in June 2017.
- Increased new drug development expenses for the half reflects
 - Recent staff increases
 - The commencement of 2 phase 1 clinical trials in relation to the LOXL2 program
 - Drug discovery expenditure for the half including in relation to the LOXL2 program (\$1.6 million) as well as on the SSAO/MPO program and the LOX programs (\$1.6 million).
- Bronchitol & Aridol Other Expenses increased mainly due to the change in net transfer of labour and overhead into inventory as discussed above.

Income statements

A\$'000 (unaudited)	Three months ended		Six months ended	
	31-Dec-17	31-Dec-16	31-Dec-17	31-Dec-16
Revenue				
Revenue from sale of goods	1,402	793	2,451	1,690
Sale of drug candidate	-	-	26,891	-
Clinical trial cost reimbursements	506	724	1,187	4,301
Interest	151	185	244	409
Drug discovery service fee	-	106	-	330
Other	373	92	571	180
Total revenue	\$2,432	\$ 1,900	\$ 31,344	\$ 6,910
Expenses				
Employee costs	(2,832)	(2,563)	(5,649)	(5,455)
Administration & corporate	(736)	(529)	(1,328)	(1,065)
Rent, occupancy & utilities	(324)	(300)	(601)	(544)
Clinical trials	(405)	(1,628)	(1,380)	(5,398)
Drug development	(1,895)	(860)	(3,439)	(1,592)
Sales, marketing & distribution	(303)	(250)	(549)	(457)
Safety, medical and regulatory affairs	(189)	(318)	(373)	(693)
Manufacturing purchases	(288)	(419)	(753)	(733)
Other	(353)	215	(379)	526
Depreciation & amortisation	(784)	(765)	(1,565)	(1,523)
Foreign currency exchange gains & losses	(27)	(1,273)	455	(689)
Finance costs	(143)	(160)	(291)	(322)
Costs in relation to change in collaboration agreement	(9,580)	-	(9,580)	-
Total expenses	(17,859)	(8,850)	(25,432)	(17,945)
Net profit (loss) before tax	(15,427)	(6,950)	5,912	(11,035)
Income tax credit/(expense)	8	-	8	-
Net profit (loss) after tax	\$(15,419)	\$ (6,950)	\$ 5,920	\$ (11,035)

Summary balance sheets

A\$'000		
(unaudited)	31-Dec-17	30-June-17
Assets		
Cash	25,045	21,504
R&D tax credit receivable	3,260	3,100
Accounts receivable	1,238	1,262
PP&E	13,679	14,860
Other	4,913	4,708
	\$49,105	\$45,434
Liabilities		
Accounts payable and accrued expenses	6,196	6,134
Lease liability (Frenchs Forest facility)	8,774	9,251
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	21,701	22,141
Other liabilities	2,412	4,387
	\$39,083	\$41,913