Quarterly Shareholder Update – March 2018

Dear Shareholder,

The first Quarter of 2018 was a significant one for the Australian biotech industry with a number of acquisitions in the sector highlighting the hidden value that many of us can see waiting to be appreciated. After the announcement of the Viralytics deal with Merck &Co I was interviewed on Sky Business News and asked about its implications for other Australian biotech companies and their shareholders. It's a question that is a very relevant one for Pharmaxis shareholders and has been a common theme of discussions we have held both with multinational Pharma companies at the recent International Liver Congress in Paris and at subsequent meetings with specialist healthcare investors in Australia, US and Europe. In short, the Viralytics deal exposed the large gulf in valuation between the market capitalisation of the company and the value identified by a large Pharma company and I found myself expanding frequently on the foundations of long term value generation in Pharmaxis. I believe there are three main drivers here which affect both short and long-term value:

1. A strong cash balance
   The receipt of a further $15m milestone payment from our deal with Boehringer Ingelheim boosted cash at the close of this quarter to $35m. This gives us a comparatively long operational runway with the certainty of enough funds to exploit the value in our pipeline and simultaneously build for the future.
2. An experienced and talented team
   Our business is to identify unmet needs in human health, discover and develop new solutions and then sell those assets on to large Pharma companies at the moment when we have maximised their value with the resources at our disposal. We have demonstrated our capability in this area with the program sold to Boehringer in 2015. Our appointment last year of a new Head of Chemistry and a new Board member, both of whom had distinguished careers in large Pharma, speak to our resolve to continue strengthening our team and building capabilities to both innovate and commercialise into the future.
3. A broad portfolio with multiple shots on goal
   Our mannitol business had a strong quarter underpinned by Bronchitol growth in Europe and Australia with further potential upsides from Russia and the US markets to come. Boehringer's trials of the Pharmaxis drug discovery are progressing in two indications where positive outcomes can unlock significant future value, and our anti fibrotic LOXL2 inhibitor program is reaching its conclusion and attracting strong interest from multiple potential large pharma partners.

A recent research analyst report from Bell Potter focused closely on assessing our Bronchitol business and the Boehringer deal in coming to a target valuation, noting the LOXL2 program is difficult to value until we see the shape of the partnering deal we hope to conclude in the second half of this year. In the meantime, I am very encouraged by the recent discussions we have had with companies that are looking for effective treatments in diseases such as NASH and Idiopathic Pulmonary Fibrosis. There is clearly value here if our drug candidates continue to perform well in the ongoing phase 1 and toxicity studies.

Sincerely,

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.

Boehringer has recently updated its estimated completion time for both trials to 1H 2019. While this is a delay in the results of the first trials of efficacy in humans, we remain extremely satisfied with the level of commitment demonstrated by Boehringer to this drug. This commitment is evidenced in part by the parallel development of two indications and that these two indications form part of Boehringer's holistic strategy to treat the complications of diabetes.

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.

We eagerly await the results of these first trials of efficacy in humans.

LOXL2 inhibitors in the clinic

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 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.

The Pharmaxis drug discovery group developed two small molecule inhibitors to the LOXL2 enzyme which were well tolerated in regulatory toxicity studies with a good safety profile. The two compounds commenced phase 1 clinical trials in the second half of 2017 and have both now completed the single ascending dose stage and are proceeding into the multiple ascending dose stages this quarter.

Importantly, Pharmaxis has successfully developed and validated a target engagement assay sensitive enough to measure LOXL2 enzyme activity in the phase 1 study subjects. Results from the single ascending dose stage for both compounds demonstrate that they produce a significant and long-lasting inhibition of LOXL2 that is dose dependent.

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.

Partnering plan for LOXL2 program

Large pharma is interested in the Pharmaxis LOXL2 program as it is one of the very few truly anti-fibrotic mechanisms in clinical development and a number of pharma companies have been following progress of the Pharmaxis LOXL2 program for more than two years. With the Phase 1 results expected mid 2018 Pharmaxis has initiated a formal due diligence process and a number of pharma companies are well advanced in assessing the LOXL2 program.

Pharmaxis plans to partner the LOXL2 program in the second half of 2018 after the phase 1 trials and longer-term toxicity studies report.

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. Lead candidates have been identified in both of these programs and both have 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 application in inflammatory bowel diseases, 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 pancreatic cancer and myelofibrosis.

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 who are responsible for the commercialisation of Bronchitol in the United States are currently preparing to resubmit the New Drug Application with the FDA. Following an extensive review by Chiesi of the data from all three phase 3 studies and the latest FDA guidelines of requirements for inhaled respiratory drugs, we expect Chiesi to complete and resubmit the NDA in the December quarter of 2018.

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, 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. Sales for Western Europe in the quarter were $963,000 compared to $887,000 in the March 2017 quarter and $587,000 in the December 2017 quarter.

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 with a recent submission expected to be reviewed in the second half of calendar 2018, the Company has been successful in obtaining approval for a few hundred patients to receive Bronchitol via an individual reimbursement scheme. During the quarter the Company's Russian distributor commenced supply of Bronchitol to a number of these patients and has orders from clinics for further supply.

Sales in Australia were significantly increased at $287,000 for the quarter compared to $187,000 in the March 2017 quarter and $220,000 in the December 2017 quarter. This improvement comes as a result of increased usage in new patients now covered under the widened government reimbursement for Bronchitol granted on Jan 1st 2018.

Aridol

Aridol sales for the quarter were A$421,000 compared to A$469,000 in the March 2017 quarter. Slight increases in the Australian and European markets were offset by reduced sales to the Company's Korean distributor, which fluctuate quarter by quarter.

Together with its US and Canadian Aridol distributor Methapharm Inc, Pharmaxis has filed a regulatory submission with the US FDA to enable Aridol to be sold again in the USA. The US FDA is expected to respond later this year.

A submission to Canadian authorities will be filed this quarter and the approval process is expected to take approximately 12 months.

Corporate

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Financials

Key financial metrics

A$'000	Three months ended		Nine months ended	
(unaudited)	**31-Mar-18**	**31-Mar-17**	**31-Mar-18**	**31-Mar-17**
Income statements				
Sales of Bronchitol & Aridol	1,743	2,267	4,194	3,957
Sale of drug candidate	15,239	-	42,130	-
Total revenue	17,276	4,144	48,620	11,056
Total expenses	(9,122)	(7,345)	(34,554)	(25,290)
Net profit (loss) after tax	**8,154**	**(3,199)**	**14,074**	**(14,234)**
Segment results – adjusted EBITDA				
Bronchitol & Aridol	(1,141)	(738)	(2,588)	(4,679)
New drug development	11,581	(1,892)	32,453	(4,313)
Corporate	(884)	(934)	(12,519)	(3,012)
Total	**9,556**	**(3,564)**	**17,346**	**(12,004)**
Statement of cash flows				
Cash inflow/ (outflow) from:				
Operations	10,279	(2,132)	14,918	(11,026)
Investing activities	(369)	(183)	(604)	(397)
Financing activities	(447)	(431)	(1,310)	(1,287)
Total cash generated/(used)	**9,463**	**(2,746)**	**13,004**	**(12,710)**
Cash at bank	**34,508**	**26,499**	**34,508**	**26,499**

Highlights for the quarter

- The financial results for the quarter were dominated by the A$15.2 million milestone payment received from Boehringer Ingelheim on the commencement of a phase 2a clinical trial in diabetic retinopathy, bringing the total milestones received this financial year from Boehringer to A$42.1 million.
- The closing cash position at 31 March was $34.5 million.
- An income tax credit of $3.3 million in relation to the 2017 financial year was received during the quarter.
- While sales revenue for the quarter was less than the comparable quarter in 2017, the comparable quarter included the initial sale of $643,000 to the Company's Russian distributor for Bronchitol. The next sale to Russia is expected later in the 2018 calendar year.
- Net profit for the quarter was $8.2 million compared to a loss of $3.2 million in the March quarter of 2017.
- Expenses
    - Total expenses increased for the quarter, driven by new drug development of which the LOXL2 inhibitor program is the major component.
    - Note that year to date expenses include $9.6 million of costs incurred in the December quarter associated with changes to the collaboration agreement with Synairgen.

- Clinical trial expenses in relation to clinical trial CF303 changed from $1.4 million in the March 2017 quarter to nil in the March 2018 quarter. Clinical trial expenses in the current quarter relate to the two phase 1 trials being run as part of the LOXL2 program.
- Drug development expenses increased by $0.5 million reflecting increased levels of research activity in several projects.
- Foreign exchange gains and losses include an unrealised gain in the comparative quarter of $1.4 million and an unrealised loss in the current quarter of $350,000.
- Other expenses for the quarter include a credit of $9,000 ($551,000 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, as well as other inventory adjustments.

Segment information

A$'000	Segment information - three months ended							
(unaudited)	31-Mar-18				31-Mar-17			
Income statements	Bronchitol & Aridol	New drug developm't	Corporate	Total	Bronchitol & Aridol	New drug developm't	Corporate	Total
Revenue								
Sale of Bronchitol	1,322	-	-	1,322	1,798	-	-	1,798
Sale of Aridol	421	-	-	421	469	-	-	469
	1,743	**-**	**-**	**1,743**	**2,267**	**-**	**-**	**2,267**
Sale of drug candidate	-	15,239	-	15,239	-	-	-	-
Clinical reimbursement	-	-	-	-	1,570	-	-	1,570
Tax credit	-	-	-	-	16	44	-	60
Other revenue	5	-	120	125	6	(2)	85	89
	1,748	**15,239**	**120**	**17,107**	**3,859**	**42**	**85**	**3,986**
Expenses					-	-	-	-
Employee costs	(1,499)	(696)	(445)	(2,640)	(1,526)	(540)	(455)	(2,521)
Clinical trials	(24)	(1,076)	-	(1,100)	(1,409)	-	-	(1,409)
Drug discovery	-	(1,788)	-	(1,788)	-	(1,311)	-	(1,311)
Other expenses	(1,366)	(98)	(559)	(2,023)	(1,662)	(83)	(564)	(2,309)
Total expenses	(2,889)	(3,658)	(1,004)	(7,551)	(4,597)	(1,934)	(1,019)	(7,550)
Adjusted EBITDA	**($1,141)**	**$11,581**	**($884)**	**$9,556**	**($738)**	**($1,892)**	**($934)**	**($3,564)**

Commentary for the quarter

- Bronchitol & Aridol:
  - The decrease in sales of Bronchitol arises from the comparable quarter including a sale to the Company's Russian distributor. The next sale to Russia is expected later in the 2018 calendar year.
  - Total sales of Aridol decreased primarily because of a lower level of sales to Korea in the current quarter. Sales in other markets increased.
  - Clinical trial reimbursements and clinical trial costs significantly reduced following reporting of study CF303 in June 2017.
  - Other expenses increased mainly due to the change in net transfer of labour and overhead into inventory as discussed above.
- New drug development:
  - The Company booked an A$15 million milestone payment from Boehringer Ingelheim on the commencement of a phase 2a clinical trial in diabetic retinopathy

- Clinical trial expenses relate to the 2 phase 1 trials being conducted in the LOXL2 program.
- Drug discovery expenses include work on the LOXL2 program ($509,000 for the quarter) and increased mainly as a result of the progression of the LOX ($536,000 for the quarter) and SSAO/MPO ($569,000 for the quarter) programs into preclinical – the final step before proceeding into phase 1.

A$'000	Segment information - nine months ended							
(unaudited)	31-Mar-18				31-Mar-17			
Income statements	Bronchitol & Aridol	New drug developm't	Corporate	Total	Bronchitol & Aridol	New drug developm't	Corporate	Total
Revenue								
Sale of Bronchitol	2,793	-	-	2,793	2,508	-	-	2,508
Sale of Aridol	1,401	-	-	1,401	1,449	-	-	1,449
	4,194	**-**	**-**	**4,194**	**3,957**	**-**	**-**	**3,957**
Sale of drug candidate	-	42,130	-	42,130	-	-	-	-
Clinical reimbursement	1,187	-	-	1,187	5,871	-	-	5,871
Tax credit	-	160	-	160	16	44	-	60
Other revenue	182	5	349	536	20	328	250	598
	5,563	**42,295**	**349**	**48,207**	**9,864**	**372**	**250**	**10,486**
Expenses								
Employee costs	(4,205)	(2,031)	(1,441)	(7,677)	(4,456)	(1,514)	(1,528)	(7,498)
Clinical trials	(190)	(2,290)	-	(2,480)	(6,807)	-	-	(6,807)
Drug discovery	-	(5,227)	-	(5,227)	-	(2,903)	-	(2,903)
Other expenses	(3,756)	(294)	(1,847)	(5,897)	(3,280)	(268)	(1,734)	(5,282)
Change in collaboration	-	-	(9,580)	(9,580)	-	-	-	-
Total expenses	(8,151)	(9,842)	(12,868)	(30,861)	(14,543)	(4,685)	(3,262)	(22,490)
Adjusted EBITDA	**($2,588)**	**$32,453**	**($12,519)**	**$17,346**	**($4,679)**	**($4,313)**	**($3,012)**	**($12,004)**

Commentary for the nine months

- Bronchitol & Aridol:
  - Sale of Bronchitol increased for Australia (16%), Western Europe including sales to Chiesi for Germany, Italy and the UK (84%), while the comparative period includes a sale of $643,000 for Russia where the next sale is expected later in the 2018 calendar year.
  - Clinical trial reimbursements and clinical trial costs reduced following reporting of study CF303 in June 2017.
  - The increase in Other expenses is primarily due to net transfer of labour and overhead into inventory as discussed above.
- New drug development:
  - Increased new drug development expenses for the three quarters reflects
    - Staff increases earlier in the financial year
    - 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 ($2.1 million for nine months) as well as on the SSAO/MPO program ($1.2 million for nine months) and the LOX programs ($1.5 million for nine months).
- Corporate:
  - Note the $9.6 million of costs incurred in the December quarter associated with changes to the collaboration agreement with Synairgen.

Income statements

A$'000	Three months ended		Six months ended	
(unaudited)	**31-Mar-18**	**31-Mar-17**	**31-Mar-18**	**31-Mar-17**
Revenue				
Revenue from sale of goods	1,743	2,267	4,194	3,957
Sale of drug candidate	15,239	-	42,130	-
Clinical trial cost reimbursements	-	1,570	1,187	5,871
Interest	169	159	413	568
Drug discovery service fee	-	-	-	330
Other	125	150	696	330
Total revenue	**$17,276**	**$ 4,144**	**$ 48,620**	**$ 11,056**
Expenses				
Employee costs	(2,933)	(2,758)	(8,582)	(8,203)
Administration & corporate	(494)	(333)	(1,822)	(1,398)
Rent, occupancy & utilities	(329)	(301)	(930)	(845)
Clinical trials	(1,100)	(1,409)	(2,480)	(6,807)
Drug development	(1,788)	(1,311)	(5,227)	(2,903)
Sales, marketing & distribution	(348)	(258)	(897)	(715)
Safety, medical and regulatory affairs	(200)	(256)	(573)	(949)
Manufacturing purchases	(488)	(282)	(1,241)	(1,015)
Other	(236)	(752)	(615)	(236)
Depreciation & amortisation	(787)	(768)	(2,352)	(2,291)
Foreign currency exchange gains & losses	(279)	1,238	176	549
Finance costs	(140)	(155)	(431)	(477)
Costs in relation to change in collaboration agreement	-	-	(9,580)	-
Total expenses	**(9,122)**	**(7,345)**	**(34,554)**	**(25,290)**
Net profit (loss) before tax	**8,154**	**(3,199)**	**14,066**	**(14,234)**
Income tax credit/(expense)	-	-	8	-
Net profit (loss) after tax	**$8,154**	**($3,199)**	**$14,074**	**($14,234)**

Summary balance sheets

A$'000		
(unaudited)	**31-Mar-18**	**30-June-17**
Assets		
Cash	34,508	21,504
R&D tax credit receivable	-	3,100
Accounts receivable	1,592	1,262
PP&E	12,965	14,860
Other	6,043	4,708
	$55,108	**$45,434**
Liabilities		
Accounts payable and accrued expenses	2,869	6,134
Lease liability (Frenchs Forest facility)	8,530	9,251
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	21,988	22,141
Other liabilities	3,252	4,387
	$36,639	**$41,913**
Net Assets	**$18,469**	**$3,521**