

Quarterly Shareholder Update – September 2018



Dear Shareholder,

The September Quarter saw the Company raise capital for the first time since 2011. The fact that we raised \$24m at a premium to the market and further strengthened our register with some well regarded specialist healthcare investors was very pleasing and I believe a strong signal of the underlying value in Pharmaxis. The timing of the raise was driven by a number of factors which go to the core of our strategy to maximise the value of our pipeline whilst managing risk by partnering assets at the optimum stage of development. It gives us the financial strength and

resources to progress one of the best early stage pipelines for a biotech company of our size and increases our value potential for the years ahead.

The first of our LOXL2 inhibitors successfully completed its phase 1 study and confirmed its best in class profile with 24-hour inhibition of the target enzyme from a single daily dose. The other inhibitor will report later this quarter and together with the remaining 3-month toxicity studies that are also due to report this quarter, will complete the data package that several companies are now reviewing. Providing these results are favourable, I anticipate that we will move quickly to commercial partnering discussions. Our healthy cash balance puts us in a strong position to take our time and negotiate the best deal for our shareholders.

In other good news, it became apparent during the June Quarter that one of our pre-clinical assets from the stable of amine oxidase compounds was starting to show real promise. This oral LOX inhibitor has since cleared 28 day tox studies and subject to some confirmatory animal model studies will be ready to go into phase 1 clinical studies in early 2019. This compound blocks the whole family of lysyl oxidase enzymes (LOX, LOXL1, 2, 3 and 4). As such it is a very potent anti-fibrotic and would seem suited to diseases where there is a very high unmet need and where fibrosis plays a major role. Pancreatic cancer and myelofibrosis are the diseases which are the most well validated in the literature and our pre-clinical studies suggest that we could have a very effective drug on our hands. The Company will release more information on our plans in this quarter as the data becomes available. I am very pleased that the capital raise gives us the financial strength to accelerate the development path of this drug irrespective of the timing of any LOXL2 partnering deal.

In other news, Boehringer Ingelheim has confirmed that the phase 2 NASH study that they are undertaking on the drug acquired from Pharmaxis will report in 1H 2019. Boehringer expects a one-month delay but in the context of a crowded market where many NASH studies are struggling to recruit this is a commendable performance. The second phase 2 study in diabetic nephropathy is subject to a longer 8-month delay but we remain pleased with the effort BI is investing to realise a timely outcome in both diseases.

Finally, its very pleasing to report a number of achievements this quarter to move the mannitol franchise towards profitability. The FDA audited and cleared the Pharmaxis Sydney production facility to supply the US market with Aridol. The first sales to our Aridol North American distributor will happen this quarter. We also expect Chiesi to file a Bronchitol submission to the FDA before year end. Meanwhile in Russia we cleared a vital step on the way to national reimbursement for Bronchitol when the Essential Drugs Committee voted to include Bronchitol in their 2019 list.

I hope you enjoy the rest of this comprehensive update.

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 – 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.

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

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.

Under the deal signed in 2015, Boehringer has total responsibility for the development program 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.

Boehringer provides regular reports on the development programs for BI 1467335, and updates on certain development matters as they arise. During the September quarter Boehringer reviewed the recruitment of both trials and provided Pharmaxis with revised trial completion dates. The NASH trial completion date was pushed back by one month and is still within the first half of 2019, as previously advised by Pharmaxis. The DR trial completion date was pushed back by 8 months and is now early in the first half of 2020. Boehringer explained that the trial delay has been caused mainly by slower than expected site initiation and recruitment. As part of the significant efforts being undertaken to complete recruitment, Boehringer advise that they are adding additional sites in countries where recruitment rates are best.

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

LOXL2 inhibitors in the clinic

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 were well tolerated in 28 day regulatory toxicity studies with a good safety profile.

During the quarter Pharmaxis announced positive results from the Phase 1 clinical trial for the first of LOXL2 inhibitor compounds. The double-blind placebo controlled study consisted of two stages. The first single ascending dose stage was conducted in 48 healthy subjects divided into six groups with each taking a single dose ranging from 10mg to 400mg or placebo. The second multiple ascending dose stage was conducted in 24 healthy subjects divided into three groups which each received a single daily dose ranging from 100mg to 400mg or placebo for 14 days.

The excellent drug like properties demonstrated in earlier pre-clinical testing were confirmed. There were no adverse safety findings in either the first or second stages of the study and the pharmacokinetic profile showed the expected dose related increases in exposure.

In addition to studying the safety and pharmacokinetic profile, the clinical trial also investigated the degree to which the drug can inhibit the target enzyme LOXL2 which is implicated in several different fibrotic diseases. Importantly, Pharmaxis has been able to demonstrate a large and highly significant inhibition of this enzyme in blood serum for a full 24 hours from a single dose and that daily dosing over a 14-day period now meets our targeted effect of greater than 80% inhibition at the 400mg dose.

The Phase 1 trial for the second Pharmaxis LOXL2 compound being studied has recently completed dosing and will report in the December 2018 quarter.

Pharmaxis has also initiated 3-month tox studies in both compounds to further de-risk the program and add more value for prospective pharmaceutical company partners. Both compounds have successfully completed the first of their two species studies. The remaining studies will report in the December quarter of 2018.

Pharmaxis has conducted an extensive pre-clinical program on the compounds and the role of LOXL2 in the predictive in vivo animal models for anti-fibrotic diseases such as NASH and Idiopathic Pulmonary Fibrosis (IPF). The compounds show excellent efficacy in several different in vivo fibrosis models including fibrosis of the liver, lung, kidney and heart.

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. The excellent pharmacokinetic parameters and the significant and long lasting inhibition of the target LOXL2 enzyme demonstrated by the program compounds has led to increased interest from major pharmaceutical companies looking for good anti fibrotic programs to acquire. This quarter the Company expects to complete the data package which a number of potential partners are currently reviewing. Commercial discussions are expected to quickly follow and conclude in 2019.

Drug development pipeline – other programs

In addition to the SSAO inhibitor (BI 1467335) and the LOXL2 program, Pharmaxis has other programs developed from its amine oxidase chemistry platform. Lead candidates have been identified in two of these programs and both have commenced preclinical studies with the objective of having one target phase 1 ready within the next six months.

- An oral drug inhibiting all lysyl oxidase family members with potential anti-fibrotic application in severe fibrotic indications. This candidate has positive results in in vivo models of myelofibrosis and pancreatic cancer and additional confirmatory studies are being run at the present time. The drug has recently satisfactorily completed the 28 day preclinical

tox studies required for the commencement of phase 1 studies.

- a dual acting drug inhibiting SSAO and myeloperoxidase (MPO) for the treatment of inflammation.

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 is responsible for the commercialisation of Bronchitol in the United States and is currently preparing to resubmit a New Drug Application with the FDA. Chiesi expect to complete and file the NDA in the December quarter of 2018.

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 been navigating the process to have Bronchitol reimbursed nationally. Importantly, during the September quarter the Russian Ministry of Health's Essential Drugs Committee decided to include Bronchitol on the Essential Drugs List of Russia from 2019. Pharmaxis is working through the final aspects of the reimbursement approval process. In the meantime, the Company's Russian distributor has received approval to supply Bronchitol to a number of patients via an individual reimbursement scheme.

Bronchitol was launched in the Czech Republic in the June quarter of 2018.

Bronchitol sales for the quarter

Bronchitol sales for the quarter were \$424,000 compared to \$605,000 in the September 2017 quarter.

\$'000	30 Sept 18	30 Sept 17
Australia	249	148
Western Europe	37	457
Eastern Europe	139	-
Total	\$424	\$605

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

There were no sales to Chiesi for the UK and Germany in the quarter, compared to sales of \$458,000 in the September 2017 quarter.

Sales to Eastern Europe included sales to Turkey of \$125,000 compared to nil in the September 2017 quarter.

There were no sales by Pharmaxis to its Russian distributor during the quarter.

Pharmaxis distributors typically order Bronchitol on a six monthly basis and the only distributor shipment in the September 2018 quarter was to Turkey.

Aridol

In August 2018 the US Food and Drug Administration approved the Pharmaxis manufacturing facility in Sydney, Australia to produce Aridol® for the US market. Aridol was first approved by the FDA in 2011 as an aid to diagnosing asthma, and commercialised by

Pharmaxis in the US until its withdrawal from the market in 2013 as part of a corporate restructuring. Aridol will commence US commercial sales in the fourth quarter of 2018 via Pharmaxis' exclusive distributor 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 take approximately 12 months.

Aridol sales for the quarter

Aridol sales for the quarter were A\$475,000 compared to A\$444,000 in the September 2017 quarter.

\$'000	30 Sept 18	30 Sept 17
Australia	118	103
Europe	204	186
South Korea	153	155
Total	\$475	\$444

Corporate

Mr Edward Rayner appointed to Pharmaxis Board of Directors.

The extraordinary general meeting of shareholders held on 17 September elected biotech investment specialist Edward Rayner as a non-executive director of the Company.

Mr Rayner is an investment director at Arix Bioscience plc (LSE: ARIX), a global healthcare and life science company, and led the Arix investment in the recent Pharmaxis share placement. Arix now holds 11 percent of the Company.

Before joining Arix Bioscience at its inception, Mr Rayner spent 18 years as an equity analyst and portfolio manager in Europe and Australia. From 2004 to 2014, he was based in Sydney Australia, initially as head of research at Alliance Bernstein and then a senior portfolio manager at AMP Capital where he managed the growth equity portfolios and launched a small companies fund. As part of his responsibilities he focused on the healthcare sector.

Mr Rayner brings global experience as a specialist biotech investor to the Pharmaxis Board.

Pharmaxis completes A\$24 million placement

During the quarter the Company completed a \$24 million placement to institutional and sophisticated investors. The placement issue price of \$0.325 per share was a 3.1% premium to the last closing price prior to the announcement of the placement. The funds raised will be used to strengthen the Company's balance sheet as it conducts partnering negotiations for its LOXL2 program.

The placement received strong support from specialist healthcare investors including existing shareholder BVF Partners LP who have increased their shareholding to 22.9% and new shareholder Arix Bioscience plc who now hold an 11.1% shareholding in the Company. It is the first Australian investment by Arix which is a global healthcare and life science company based in the United Kingdom and listed on the main market of the London Stock Exchange.

Pharmaxis director Dr Simon Buckingham to retire at 2018 AGM

On 4 October the Company announced that non-executive director Dr Simon Buckingham will retire at the 2018 AGM. Dr Buckingham joined the

Pharmaxis Board in July 2012 and has served as a non-executive director for two terms of three years each, a critical time which has seen the Company repositioned as a drug developer focused on inflammatory and fibrotic diseases.

Dr Buckingham will continue to consult for the Company on the LOXL2 inhibitor partnering process.

2018 Annual General Meeting

The 2018 Annual General Meeting will be held on 22 November 2018 at the offices of Computershare, Level 4, 60 Carrington Street, Sydney NSW 2000 on 22 November 2018 at 2.30 pm (Sydney time). The Notice of Meeting and Proxy Voting Form were distributed to shareholders on 19 October. The formal part of the Meeting will cover consideration of the Company's financial statements and remuneration report, the re-election of a non-executive director, the grant of securities to the Chief Executive Officer and renewal of the proportional takeover provisions in the constitution of the Company.

Subscribe to our emails

If you would like to be advised directly by email each time Pharmaxis issues a media release, please [subscribe](#) at our website.

Financials

Key financial metrics

A\$'000	Three months ended	
(unaudited)	30-Sept-18	30-Sept-17
Income statements		
Sales of Bronchitol & Aridol	900	1,049
Milestones from sale of drug	-	26,891
Total revenue	1,232	28,911
Total expenses	(8,765)	(7,572)
Net profit (loss) after tax	\$(7,533)	\$21,347
Segment results – adjusted EBITDA		
Bronchitol & Aridol	(1,849)	(1,460)
New drug development	(3,209)	24,498
Corporate	(1,098)	(981)
Total	(\$6,156)	\$22,057
Statement of cash flows		
Cash inflow/ (outflow) from:		
Operations	(6,209)	17,662
Investing activities	(333)	(110)
Financing activities	22,227	(427)
Total cash generated/(used)	\$15,685	\$17,125
Cash at bank	\$46,758	\$38,629

Highlights for the quarter

- In comparing the revenue for the quarter with the prior comparable period please note:
 - There were no major shipments to Bronchitol distributors in the quarter, as discussed above.
 - The milestone from sale of drug in the September 2017 quarter related to a milestone received from Boehringer Ingelheim on the commencement of a phase 2a clinical trial in NASH.
 - Total revenue in the comparable 2017 quarter also included a reimbursement payment of \$681,000. The clinical trial to which the payment related was substantially completed in 2017.
- Expenses
 - Total expenses increased for the quarter, driven predominantly by unrealised foreign exchange gains and losses on the NovaQuest financing agreement changing from a gain of \$420,000 in the September 2017 quarter to a loss of \$720,000 in the September 2018 quarter.
 - Clinical trials expenses in the quarter related to the New Drug Development LOXL2 phase 1 trial while in the September 2017 quarter the expenses relate to both the LOXL1 phase 1 (\$199,000) and the Bronchitol phase 3 trial (CF303).

- o Finance costs for the quarter were a net credit of \$125,000 reflecting a small adjustment to the carrying value of the financing agreement.
 - o Other expenses for the quarter include \$145,000 (a credit of \$113,000 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.
- Cash
 - o During the quarter the Company completed a \$24 million placement to institutional and sophisticated investors.
 - o During the quarter the company invested \$278,000 in patent applications in relation to the LOXL2 program.
 - o The closing cash position at 30 September 2018 was \$46.8 million.

Segment information

A\$'000	Three months ended							
(unaudited)	30-Sept-18				30-Sept-17			
Income statements	Bronchitol & Aridol	New drug developm't	Corporate	Total	Bronchitol & Aridol	New drug developm't	Corporate	Total
Revenue								
Sale of Bronchitol	425	-	-	425	605	-	-	605
Sale of Aridol	475	-	-	475	444	-	-	444
	900	-	-	900	1,049	-	-	1,049
Milestones from sale of drug	-	-	-	-	-	26,891	-	26,891
Clinical reimbursement	-	-	-	-	681	-	-	681
Other revenue	9	-	124	133	88	-	109	197
	909	-	124	1,033	1,818	26,891	109	28,818
Expenses								
Employee costs	(1,397)	(774)	(559)	(2,730)	(1,428)	(577)	(508)	(2,513)
Clinical trials	-	(636)	-	(636)	(776)	(199)	-	(975)
Drug discovery	-	(1,675)	-	(1,675)	-	(1,544)	-	(1,544)
Other expenses	(1,361)	(124)	(663)	(2,148)	(1,074)	(73)	(582)	(1,729)
Total expenses	(2,758)	(3,209)	(1,222)	(7,189)	(3,278)	(2,393)	(1,090)	(6,761)
Adjusted EBITDA	(\$1,849)	(\$3,209)	(\$1,098)	(\$6,156)	(\$1,460)	\$24,498	(\$981)	\$22,057

Commentary

- Bronchitol & Aridol:
 - o Sales revenue decreased as detailed in commentary above.
 - o Clinical trial reimbursements and clinical trial costs ceased following completion of study CF303 in the 2018 financial year.
 - o Other expense increases reflect the net transfer of overhead as discussed above (increase of \$258,000).
- New drug development:
 - o Revenue in the September quarter of 2017 includes the milestone received from Boehringer Ingelheim of \$27 million as discussed above.
 - o Clinical trial expenses relate to the 2 phase 1 trials being conducted in the LOXL2 program.
 - o Drug discovery expenses include work on the LOXL2 program (\$159,000 for the quarter; \$675,000 in 2017), and the two programs currently in preclinical studies - the LOX program

(\$897,000 for the quarter; \$580,000 in 2017) and the SSAO/MPO program (\$551,000 for the quarter; \$177,000 in 2017).

Income statements

A\$'000	Three months ended	
(unaudited)	30-Sept-18	30-Sept-17
Revenue		
Revenue from sale of goods	900	1,049
Milestones from sale of drug	-	26,891
Clinical trial cost reimbursements	-	681
Interest	199	93
Drug discovery service fee	-	-
R&D tax incentive	-	-
Other	133	197
Total revenue	\$1,232	\$28,911
Expenses		
Employee costs	(3,071)	(2,817)
Administration & corporate	(574)	(592)
Rent, occupancy & utilities	(330)	(277)
Clinical trials	(636)	(975)
Drug development	(1,675)	(1,544)
Sales, marketing & distribution	(253)	(246)
Safety, medical and regulatory affairs	(311)	(184)
Manufacturing purchases	(340)	(465)
Other	(361)	(25)
Depreciation & amortisation	(641)	(781)
Foreign currency exchange gains & losses	(698)	482
Finance costs	125	(148)
Total expenses	(8,765)	(7,572)
Net profit (loss) before tax	(7,533)	21,339
Income tax credit/(expense)	-	8
Net profit (loss) after tax	(\$7,533)	\$21,347

Summary balance sheets

A\$'000		
(unaudited)	30-Sep-18	30-June-18
Assets		
Cash	46,758	31,073
R&D tax credit receivable	-	-
Accounts receivable	700	1,787
Inventory	2,251	2,398
PP&E	11,825	12,451
Intangible assets	704	446
Other	1,529	1,942
	\$63,767	\$50,097
Liabilities		
Accounts payable and accrued expenses	3,077	4,926
Lease liability (Frenchs Forest facility)	8,004	8,268
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	23,164	22,754
Other liabilities	2,920	3,031
	\$37,165	\$38,979
Net Assets	\$26,602	\$11,118