

Quarterly Shareholder Update – March 2020



Dear Shareholder,

COVID-19 has dominated the news during the last quarter and brought about changes in our environment that few of us could have predicted at the end of last year. Pharmaxis has been impacted in a number of ways and you can find a comprehensive analysis in this update. We have quickly adapted our operations to keep our clinical trial and production schedules on track but we will need to wait to see how the global economic downturn impacts capital markets and deal making in our sector. Your Board and Management team is keeping a watchful eye on how the environment is changing and examining contingencies to ensure we can still deliver on our business goals.

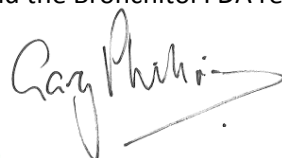
Our valuation has been impacted by both the Boehringer decision reported in the last quarter and the COVID-19 inspired market effect. However, I believe Pharmaxis remains a solid investment for the following reasons:

- **Boehringer AOC3 inhibitor program**
It's down but not out. A recent discussion with Boehringer emphasized that despite the decision to stop development in NASH, the clinical trial in diabetic retinopathy will complete on time (Q2 2020) and that a decision on its progression is expected in H2 2020. They already have a plan for the next study should the current 3-month phase 2a study show significant efficacy in this patient group.
- **LOXL2 partnering**
While slowed by the diversion of attention of potential partners in the last quarter there has certainly been no reduction in the number interested parties. The data generated by the small phase 1 study completed this quarter has been helpful in maintaining momentum and underlined the consistent quality of our science. We expect the further development of this program to be partner funded.
- **Anti-cancer Systemic pan-LOX program**
This is the third distinct program from our amine oxidase platform and having shown promise in several cancer models we now have our first phase 1 safety study in healthy volunteers successfully completed and sufficient data from long term toxicity studies to allow us to conduct 6-month phase 2 cancer studies. Following discussion with key opinion leading clinicians we believe that myelofibrosis is a firm opportunity and preliminary feedback from the FDA has encouraged us to submit an IND application mid-year with the aim of starting a 6-month safety and efficacy study in primary myelofibrosis starting in Q4 2020. [Read our press release](#) for detailed information.
- **Bronchitol US FDA review**
Our US license partner Chiesi completed the human factor study requested by the FDA in its complete response letter last year and remain on schedule to submit its response to the FDA this quarter. The US\$10m milestone due to Pharmaxis in the event of an approval would be payable on receipt of the launch stock which we forecast to be in Q4 2020.

In an environment reshaped by COVID -19, cash preservation is critical and I remain confident that our strategy of laying off development risk on projects that require significant investment via partnering, and fast tracking programs like the systemic pan-LOX inhibitor which can reach clinical proof of concept with smaller shorter clinical trials, are the right blend of risk mitigation and value driving events for shareholders. We have \$20m of cash on hand and other programs in our pipeline will receive minimal investment until the partnering of the LOXL2 program is completed and the Bronchitol FDA review is complete.

Sincerely,

Gary Phillips; Chief Executive Officer

A handwritten signature in black ink that reads "Gary Phillips". The signature is written in a cursive, flowing style with a long horizontal stroke at the end.

Pipeline at a glance

Disease/condition	Drug	Status
Cystic fibrosis	Bronchitol	Approved
Asthma	Aridol	Approved
AOC3 inhibitor	BI1467335	Partnered Phase 2
LOXL2 inhibitor	PXS-5382; PXS-5338	Phase 1 completed
Systemic pan-LOX inhibitor	PXS-5505	Phase 1 completed
Topical pan-LOX inhibitor	PXS-6302	Pre-clinical

Impact of COVID-19

Like all businesses Pharmaxis has reorganised the way it operates in response to the COVID-19 pandemic. In seeking to protect the community, our staff and our ability to continue to manufacture our pharmaceutical products needed by the cystic fibrosis community we quickly moved to home-based work broadly across the company. Our IT infrastructure already supported remote work. Employees in manufacturing, quality and drug discovery who need to come to the Frenchs Forest facility have been divided into two separate teams to increase our resilience to individual members becoming infected. Given the level of uncertainty around potential lock downs we also increased production of Bronchitol to ensure we could fulfill our customer orders for the next quarter and beyond.

The various clinical studies being conducted by Pharmaxis and our partners appear to have escaped any impact.

- The company completed both its phase 1b trial of its systemic pan-LOX inhibitor and a further phase 1 dosing study of its LOXL2 inhibitor in March
- Chiesi completed the Bronchitol human factor study in early March
- Boehringer has fully recruited its phase 2a study with the AOC3 inhibitor acquired from Pharmaxis in diabetic retinopathy and final dosing completed in February – with only follow up visits remaining.

We have to date identified two direct impacts of the pandemic on our business:

- In a number of countries, including those where we market Aridol, respiratory specialists have been advised to limit lung function testing to more severe cases due to health risks arising from patients exhaling multiple times with force as part of the test. We have seen a significant decrease in Aridol sales across all markets since mid-March. At this stage we do not know how long this advice will remain in place. Aridol sales averaged \$172,000 per month for the first nine months of the financial year.
- The significant reduction in international flights has made it more difficult to secure transport of our products to overseas markets in suitable temperature controlled aircraft. To date we have been able to secure freight routes although costs are higher. We have increased manufacturing output to allow earlier shipment of distributor orders while freight routes are available.

Other aspects of our business continue uninterrupted other than for minor delays in response times across business generally.

Drug discovery

Boehringer Ingelheim development of BI 1467335

BI 1467335 (formerly known as PXS-4728A) was acquired by Boehringer Ingelheim in 2015 with an upfront payment to Pharmaxis of \$41 million.

Boehringer initiated phase 2a proof of clinical principle trials for NASH in August 2017 and for the eye disease diabetic retinopathy (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, bringing the total received from Boehringer to A\$83 million.

Under the terms of the agreement Boehringer has total responsibility for the development program.

In December 2019 Boehringer advised it was discontinuing the development of BI 1467335 for the treatment of NASH, the reasons for which were discussed in the December 2019 Quarterly Shareholder Update.

The second study of the drug in DR is fully recruited and Boehringer has recently advised the study is expected to complete in the current quarter and report in H2 of 2020. Future development will be determined by BI in H2 2020 following completion of the phase 2a study.

Pharmaxis receives payments upon achievement of certain development milestones. Future potential payments by Boehringer to Pharmaxis for the DR program include:

- Commencement of phase 3 clinical trial milestone: €37m (approximately A\$62m)
- Filing, approval and pricing milestones: total of €140m (approximately A\$230m)
- Sales related payments increasing from high single digits
- Sales milestones

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.

LOXL2 inhibitor program

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 that have completed phase 1 clinical trials and 3-month toxicology studies (PXS-5382 and PXS-5338). Doses that resulted in 85% or greater inhibition over 24 hours of the target enzyme in the phase 1 studies were significantly below the Human Equivalent No Observed Adverse Effect Level doses in all toxicity studies and thus demonstrated an adequate safety margin to start phase 2 studies of at least 3 months in length.

Over the course of 2019 Pharmaxis scientists and collaborators demonstrated the link between LOXL2 inhibition in diseased organs, a reduction in collagen crosslinking in fibrotic tissue and clinical effect as measured by the area of fibrosis. Further progressing the program in the first quarter of 2020, Pharmaxis completed a small phase 1 study that demonstrated an improved pharmacokinetic profile in a number of different dosing regimens.

Pharmaxis is currently pursuing a number of different partnering options with international pharma companies to enable this drug to enter the clinic in phase 2 trials and will provide more information when the process concludes.

Systemic LOX inhibitor program

Pharmaxis is progressing two lysyl oxidase (LOX) programs from its amine oxidase chemistry platform, both of which are planned to be partnered after phase 2 clinical trials.

The most advanced LOX program has developed an oral drug (PXS-5505) that inhibits all lysyl oxidase family members (LOX, LOXL1, 2, 3 & 4) that has now completed phase 1 studies.

The compound has shown significant reductions in fibrosis in in-vivo models of kidney, liver and lung fibrosis, myelofibrosis and pancreatic cancer. It is suited to the treatment of severe fibrosis as well as cancer with prominent stroma (connective tissue) or fibrotic metastatic niches.

Following positive results from the clinical phase 1a single ascending dose study the drug progressed into the multiple ascending dose stage (phase 1b) in October 2019. This phase 1b study successfully completed during the quarter. The drug was well tolerated and no safety signals were identified during the study. Importantly for potential clinical benefit, the data confirmed the results of the phase 1a study showing a drug with good pharmacokinetics and a dose related inhibition of members of the LOX family of enzymes.

With the completed phase 1b study and longer term 6-month toxicity study results, Pharmaxis now has all the data required to support the commencement of clinical proof of concept studies in cancers where fibrosis plays a key role. Pre-clinical studies strongly support its potential use in myelofibrosis, a rare bone cancer with a poor prognosis and limited therapeutic options. Data from academic collaborators who are investigating other cancers where fibrosis plays a significant role is still being collected. These include pancreatic cancer, oral cancer, glioblastoma and mesothelioma where there is strong pre-clinical evidence that several members of LOX family play a critical role.

Following discussion with clinical opinion leaders in Australia, Europe and the US the Company has determined to run its first phase 2 study for PXS-5505 in myelofibrosis where a study duration of 6 months should allow meaningful endpoints of safety, changes in blood cell count and reduction in fibrosis measured in bone biopsies. The Company has taken advice from the US FDA and is currently preparing to file an Investigational New Drug (IND) application later in Q3.

The Company is well advanced in the design of a study protocol for a clinical trial in myelofibrosis and also in selection of a clinical research organisation to run the study.

Topical LOX inhibitor program

The Company's other LOX program has developed a drug for topical application with the potential for use in scar revision, keloid scarring and scarring from burn wounds.

A lead candidate has been selected (PXS-6302) and is currently in pre-clinical development including initial stability of the topical formulation, ongoing evaluation in various disease models of scarring and 13 week GLP toxicology studies that will report in H1 2020.

The Company has ongoing discussions with an Australian based hospital burns units that is interested in commencing a series of investigator initiated clinical studies to assess the safety and initial efficacy of this drug in burns related scars.

Mannitol business

Bronchitol and Aridol

Bronchitol® (mannitol) 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 Australia, Europe, Russia 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, Canada and South Korea.

United States

The Company's US partner Chiesi Group is responsible for the commercialisation of Bronchitol in the United States.

Following receipt of a complete response letter from the FDA in June 2019 and subsequent discussions with the FDA, Chiesi has revised its product packaging and user instructions for Bronchitol. During the March quarter Chiesi completed the human factor study (HFS) required to demonstrate that healthcare professionals can properly administer the mannitol tolerance test – an initial test to ensure patients hypersensitive to mannitol are not prescribed Bronchitol. Chiesi expects to file its updated response to the FDA in the current quarter and, based on the standard response timetable the FDA review of the Bronchitol NDA is expected to be completed in mid-year 2020.

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, Italy, Norway, Sweden, Finland, Denmark, Cyprus and Greece.

Pharmaxis also markets Bronchitol in Austria via its German based logistics provider and in Spain and Switzerland via exclusive distributors.

Other territories

Bronchitol is sold in Australia by Pharmaxis and in Turkey, the Czech Republic and Russia by exclusive distributors.

During the quarter Pharmaxis engaged For Benefit Medicines (FBM) to provide field support to cystic fibrosis treating teams in Australia.

FBM is an Australian company pioneering the concept that all field support profits generated from the sale of a registered product can be donated to fund medical research and patient support in that therapeutic area. FBM currently has products in various therapeutic areas including cystic fibrosis. Any profits made by FBM from its field support of these products in

Australia will be donated to Cystic Fibrosis Australia to fund local medical research.

Bronchitol sales

Bronchitol sales for the quarter and nine months ended 31 March 2020 and 31 March 2019 are as follows:

\$'000	Three months		Nine months	
	2020	2019	2020	2019
Australia	423	239	994	782
Western Europe	163	936	1,025	1,017
Russia & Eastern Europe	44	125	779	158
Total	\$630	\$1,300	\$2,798	\$1,957

Pharmaxis ex-factory sales for the current quarter reflect the buying patterns of its international distributors where only smaller orders were shipped. Subsequent to the end of the quarter the Company has invoiced more than \$1m in distributor orders. Unit in-market sales of Bronchitol by Chiesi in the UK, Germany and Italy for the nine months year increased 10% over the nine months to March 2019.

Pharmaxis Bronchitol distributors typically order on a six monthly basis.

Aridol

As noted earlier in this update, as a result of the current COVID-19 pandemic Aridol sales are expected to reduce in a number of major markets where lung function tests are restricted to more severe cases.

Aridol sales

Aridol sales for the quarter and nine months ended 31 March 2020 and 31 March 2019 are as below. Following two large orders in the previous financial year, there were no US Aridol orders during the half from the Company's North American distributor.

\$'000	Three months		Nine months	
	2020	2019	2020	2019
Australia	118	105	377	340
Europe	339	277	841	733
USA & Canada			73	659
South Korea		252	257	482
Total	\$457	\$634	\$1,548	\$2,214

Corporate

Subscribe to our emails

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

Financials

Key financial metrics

(unaudited)	Three months ended		Nine months ended	
	31-Mar-20	31-Mar-19	31-Mar-20	31-Mar-19
Income statements				
Sales of Bronchitol & Aridol	1,087	1,934	4,346	4,171
Total revenue	1,283	2,333	5,304	5,283
Total expenses	(10,822)	(8,662)	(25,162)	(24,199)
Net loss after tax	(9,539)	(6,329)	(19,858)	(18,916)
Segment results – adjusted EBITDA				
Mannitol business	(1,464)	(1,405)	(3,733)	(3,194)
New drug development	(3,095)	(3,303)	(7,124)	(9,226)
Corporate	(582)	(947)	(2,283)	(3,045)
Total	(5,141)	(5,655)	(13,140)	(15,465)
Statement of cash flows				
Cash inflow/ (outflow) from:				
Operations	(4,863)	(6,114)	(8,555)	(16,394)
Investing activities	(130)	(295)	(458)	(857)
Financing activities	(620)	(465)	(1,860)	21,307
Total cash generated/(used)	(5,613)	(6,874)	(10,873)	4,056
Cash at bank	20,251	35,129	20,251	35,129

Highlights

- Revenue
 - See above for detail and commentary on Bronchitol and Aridol sales.
 - The Company has not booked an estimated R&D tax incentive of \$0.9 million and \$2.3 million in relation to quarter and nine months respectively as it expects revenue for the financial year to be above the \$20 million cap after which the incentive is not payable.
- Expenses
 - When unrealised foreign exchange rate gains and losses in relation to the NovaQuest financing agreement are excluded, total expenses for the quarter were \$7.5 million (2019: \$8.8 million) and for the nine months \$21.7 million (2019: \$23.1 million). The reduction in total expenses compared to the prior quarter and nine months is primarily due to lower drug discovery expenditure and positive changes in inventory offset by increased clinical trial costs and depreciation and amortisation.
- Cash
 - The Company finished the quarter with \$20 million in cash.
 - Operational cash flows for the nine months include receipt of the 2019 R&D tax incentive of \$6.2 million.

Segment information

A\$'000								
Segment information - three months ended								
(unaudited)	31-Mar-20				31-Mar-19			
Income statements	Mannitol Business	New Drug Development	Corporate	Total	Mannitol Business	New Drug Development	Corporate	Total
Revenue								
Sale of Bronchitol	630			630	1,300			1,300
Sale of Aridol	457			457	634			634
	1,087			1,087	1,934			1,934
Tax credit								
Other revenue	5		104	109	6		128	134
	1,092		104	1,196	1,940		128	2,068
Expenses								
Employee costs	(1,516)	(943)	(392)	(2,851)	(1,566)	(752)	(465)	(2,783)
Clinical trials		(1,023)		(1,121)		(795)		(795)
Drug discovery		(1,056)		(1,056)		(1,591)		(1,591)
Changes in inventories	(80)			(80)	(1,052)			(1,052)
Other expenses	(960)	(73)	(294)	(1,229)	(727)	(165)	(610)	(1,502)
Total expenses	(2,556)	(3,095)	(686)	(6,337)	(3,345)	(3,303)	(1,075)	(7,723)
Adjusted EBITDA	(\$1,464)	(\$3,095)	(\$582)	(\$5,141)	(\$1,405)	(\$3,303)	(\$947)	(\$5,655)

Commentary for the quarter

- Mannitol Business:
 - Sales of Bronchitol and Aridol are detailed and discussed in the commentary above.
 - Employee expenses for the quarter were consistent with the prior period.
 - Changes in inventory include the net transfer of manufacturing labour and overhead to and from inventory and therefore reflects manufacturing activity and increases/decreases in inventory. The current period also includes several one-off costs to upgrade and prepare the manufacturing facility to supply the US market.
 - The increase in other expenses reflects higher distribution costs in the EU and one-off consultant costs.
- New Drug Development:
 - Clinical trial expenses include the phase 1 trial for the Systemic LOX program of \$598,000 (2019: \$436,000) and a small phase 1 dosing study in LOXL2 (\$426,000). In 2019, the clinical trial expenses included the main phase 1 trials conducted in the LOXL2 program (\$348,000).
 - Drug discovery expenses include work on the Topical LOX topical program (\$518,000 for the quarter; \$493,000 in 2019); and work on the Systemic LOX program (\$350,000 for the quarter; \$815,000 in 2019).
- Corporate
 - The decrease in employee expenses reflects one off adjustments to payroll on-cost accruals.
 - The decrease in other expenses includes foreign currency exchange rate gains in the current quarter and a reallocation of occupancy costs effective from the beginning of the current financial year.

A\$'000								
Segment information - nine months ended								
(unaudited)	31-Mar-20				31-Mar-19			
Income statements	Mannitol Business	New Drug Development	Corporate	Total	Mannitol Business	New Drug Development	Corporate	Total
Revenue								
Sale of Bronchitol	2,798			2,798	1,957			1,957
Sale of Aridol	1,548			1,548	2,214			2,214
	4,346			4,346	4,171			4,171
Tax credit		259		259				
Other revenue	15		367	382	20		378	398
	4,361	259	367	4,987	4,191		378	4,569
Expenses								
Employee costs	(4,553)	(2,472)	(1,293)	(8,318)	(4,447)	(2,166)	(1,499)	(8,112)
Clinical trials	98	(2,191)		(2,191)	621	(1,857)		(1,236)
Drug discovery		(2,367)		(2,367)		(4,792)		(4,792)
Changes in inventories	(826)			(826)	(1,110)			(1,110)
Other expenses	(2,813)	(353)	(1,357)	(4,425)	(2,449)	(411)	(1,924)	(4,784)
Total expenses	(8,094)	(7,383)	(2,650)	(18,127)	(7,385)	(9,226)	(3,423)	(20,034)
Adjusted EBITDA	(\$3,733)	(\$7,124)	(\$2,283)	(\$13,140)	(\$3,194)	(\$9,226)	(\$3,045)	(\$15,465)

Commentary for the nine months

- Mannitol Business:
 - Sales of Bronchitol and Aridol are detailed and discussed in the commentary above.
 - Employee expenses for the quarter were consistent with the prior period.
 - Clinical trial credits were received in both the December 2019 and December 2018 quarters from the contract research organisation that managed the clinical trial CF303.
 - Changes in inventory include the net transfer of manufacturing labour and overhead to and from inventory and therefore reflects manufacturing activity and increases/decreases in inventory.
 - The increase in other expenses reflects higher distribution costs in the EU and one-off consultant costs.
- New drug development:
 - Clinical trial expenses include the phase 1 trial for the Systemic LOX program that commenced in the March quarter of 2019 (\$987,000) and a limited phase 1 dosing study in LOXL2 (\$181,000). In 2018, the clinical trial expenses related to the main phase 1 trials conducted in the LOXL2 program.
 - Drug discovery expenses include work on the Topical LOX topical program (\$340,000 for the half; \$449,000 in 2018) and the Systemic LOX program (\$413,000 for the half; \$1.0 million in 2018). Prior period expenses included work on the LOXL2 program (\$731,000 in 2018).
- Corporate
 - The decrease in other expenses includes a reallocation of occupancy costs effective from the beginning of the current financial year.

Income statements

A\$'000 (unaudited)	Three months ended		Nine months ended	
	31-Mar-20	31-Mar-19	31-Mar-20	31-Mar-19
Revenue				
Revenue from sale of goods	1,087	1,934	4,346	4,171
Interest	86	265	316	714
R&D tax incentive			259	
Other	110	134	383	398
Total revenue	\$1,283	\$2,333	\$5,304	\$5,283
Expenses				
Employee costs	(3,040)	(3,085)	(9,045)	(9,074)
Administration & corporate	(456)	(489)	(1,609)	(1,687)
Rent, occupancy & utilities	(263)	(359)	(746)	(1,038)
Clinical trials	(1,024)	(794)	(2,093)	(1,235)
Drug development	(1,056)	(1,591)	(2,367)	(4,792)
Sales, marketing & distribution	(387)	(227)	(1,055)	(761)
Safety, medical and regulatory affairs	(188)	(138)	(675)	(616)
Changes in inventories	(80)	(1,052)	(826)	(1,110)
Other	(128)	(272)	(502)	(692)
Depreciation & amortisation	(808)	(662)	(2,424)	(1,954)
Foreign currency exchange gains & losses	(3,249)	130	(3,370)	(1,115)
Finance costs	(143)	(123)	(450)	(125)
Total expenses	(10,822)	(8,662)	(25,162)	(24,199)
Net profit (loss) before tax	(9,539)	(6,329)	(19,858)	(18,916)
Income tax expense				
Net profit (loss) after tax	(\$9,539)	(\$6,329)	(\$19,858)	(\$18,916)

Summary balance sheets

A\$'000 (unaudited)	31-Mar-20	30-Jun-19
Assets		
Cash	20,251	31,124
R&D tax incentive		5,962
Accounts receivable	780	1,171
Inventory	2,854	2,116
PP&E	9,605	9,123
Other	2,496	2,032
	\$35,986	\$51,528
Liabilities		
Accounts payable and accrued expenses	3,067	4,194
Lease liability (Frenchs Forest facility)	8,585	7,171
Financing agreement (not repayable other than as a % of US & EU Bronchitol revenue)	26,867	23,626
Other liabilities	1,784	1,723
	\$40,303	\$36,714