



Quarterly Shareholder Update – September 2015

Pharmaxis – an emerging powerhouse in drug development



Dear Shareholder,

The last quarter has seen Pharmaxis move quickly to build on the transformational deal with Boehringer Ingelheim (Boehringer) for our drug discovery PXS-4728A. We have announced two significant milestones - the completion of the phase 1 clinical trial of PXS-4728A, and a new research collaboration with UK biotech company Synairgen plc.

Investment levels at the drug discovery stage are low compared to the cost of the clinical studies we conducted for Bronchitol and we have a clear strategy to manage risk and maximise our chances of success. I believe Pharmaxis is now well on track to become a powerhouse in drug development by leveraging its platform in amine oxidase chemistry on validated targets for inflammatory and fibrotic diseases with high unmet needs.

Successful completion of the phase 1 trial for PXS-4728A not only clears the way for Boehringer to proceed with its development program but also validates and adds credibility to our expertise in drug development. The next milestone payment is due to Pharmaxis at the commencement of phase 2 – which we expect to occur by the first quarter of 2017.

The collaboration with Synairgen aims to develop a Pharmaxis drug candidate for the treatment of idiopathic pulmonary fibrosis. By sharing the risk and cost of the preclinical and clinical development on a drug for this disease we are able to increase the overall number of amine oxidase chemistry based programs we are pursuing and increase our opportunities for success.

In late September the Company hosted investor briefings to provide additional insight into the Pharmaxis drug discovery program and in particular the disease targeted by several of our programs – Non-Alcoholic Steatohepatitis (NASH). We were very pleased that Professor Jacob George, (Westmead Millennium Institute and University of Sydney) participated in the briefing and shared his expertise on the prevalence and emerging treatment options for this disease.

With a cash balance of \$50.3 million at 30 September 2015 and net cash expenditure for the last quarter of \$4.3 million the Company is well positioned to benefit from upcoming milestones from our existing partnership deals with Boehringer and Chiesi while working to develop several new drugs.

This report outlines our recent progress and immediate plans in more detail.

Sincerely,

A handwritten signature in black ink that reads "Gary Phillips". The signature is fluid and cursive, with a long horizontal stroke extending to the right.

Chief Executive Officer

Drug discovery

Successful phase 1 of drug sold to Boehringer Ingelheim (PXS-4728A)

In September 2015 Pharmaxis announced positive results for all primary and secondary endpoints from the phase 1 clinical trial of PXS-4728A, the drug acquired by Boehringer Ingelheim in May 2015. Boehringer is developing PXS-4728A as a treatment for cardiometabolic diseases such as NASH. PXS-4728A is a highly selective inhibitor of an enzyme and adhesion protein which reduces inflammation and oxidative stress.

Pharmaxis had a commitment to complete the phase 1 study as a part of the deal with Boehringer and earlier in the year reported positive results from the initial phase 1a single ascending dose stage of this clinical trial. The subsequent phase 1b multiple ascending dose stage was conducted in 24 healthy subjects divided into three groups with each taking active or placebo once a day for 14 days. Three different dosages of PXS-4728A were trialled.

Once daily oral dosing of PXS-4728A for 14 days at doses between 3 and 10 mg was found to be safe and well tolerated. The data confirmed the high oral bioavailability of PXS-4728A and most importantly, showed these low doses are efficacious in inhibiting the enzyme and cause a long lasting inhibition. PXS-4728A is therefore ideally suited for potential use as a chronic treatment; a once a day tablet that causes 24 hour inhibition of the target enzyme at low doses.

Importantly, these positive phase 1 results enable Boehringer Ingelheim to proceed with further development of the program. Under our agreement, Boehringer is responsible for all development, regulatory, manufacturing and commercialisation activities, and Pharmaxis is entitled to total potential future milestones of €390 million (~A\$523 million) to approval for 2 indications plus sales milestones and earn out payments at a high single digit percentage of sales.

Boehringer is currently designing and preparing for the phase 2 clinical trial. While Pharmaxis is not involved in the program, based on usual drug development timeframes we would expect the phase 2 trial to commence by the first quarter of 2017.

Collaboration with Synairgen plc to develop a Pharmaxis drug for lung disease

In August 2015 Pharmaxis announced a research collaboration with UK biotechnology company Synairgen plc (LSE: SNG) to develop a selective inhibitor to the lysyl oxidase type 2 enzyme (LOXL2) to treat the fatal lung disease idiopathic pulmonary fibrosis (IPF).

IPF affects approximately 100,000 people in the US. Whilst current products are expected to produce global revenues in excess of \$1.1 billion by 2017 there remains a clear need for new treatments. This was evidenced recently when Bristol Myers Squibb announced an agreement to acquire a phase 2 drug for the treatment of IPF and myelofibrosis with total potential payments of US\$1.25 billion. We are targeting the LOXL2 enzyme because it is known to promote scar tissue which hardens and irreparably damages the lungs of IPF patients. It is hoped that the inhibition of LOXL2 will slow the build-up of scar tissue and improve survival rates that are worse than for many cancers.

Under the terms of the agreement Synairgen will fund further activity of the program, use its BioBank and in vitro lung model platform, and collaborate with the IPF research team at the University of Southampton in the UK to complete pre-clinical and early clinical development. The IPF program will be managed by a joint steering committee through to the end of phase 1 or phase 2a clinical trials, at which time the collaboration will seek a license partner. Pharmaxis and Synairgen will share any

licensing revenues in accordance with the ratio of total investment by the two companies at that time. The share of licensing revenues is expected to be approximately equal for a compound licensed for IPF after early clinical development.

The significant interest among leading clinicians and pharmaceutical companies in the role of LOXL2 in a number of different diseases highlighted the need for Pharmaxis to collaborate for selected indications in order to fully exploit the potential value of the Company's intellectual property. Synairgen has a demonstrated excellence in respiratory drug development, having successfully licensed its inhaled IFN-beta Phase 2 program to AstraZeneca. By collaborating with Synairgen Pharmaxis aims to accelerate the development of a highly competitive once a day oral treatment for patients with IPF while continuing to independently develop LOXL2 inhibitors for other potential indications.

The collaboration is progressing well and is currently evaluating drug candidates to enter preclinical development in 2016.

Drug development pipeline – other programs

The Pharmaxis drug discovery team is making solid progress with other programs based around the Company's amine oxidase chemistry platform:

1. LOXL2 inhibitor for NASH, liver and kidney fibrosis – *lead optimisation stage*
2. LOX/LOXL2 for fibrosis and metastatic cancer – *exploratory stage in conjunction with leading US and UK clinicians*
3. SSAO/MAO-B inhibitor for neuro inflammation – *lead candidate selected. Working through manufacturing scale up*
4. SSAO/MPO inhibitor for respiratory inflammation – *exploratory stage*

If successful, programs 1 and 3 above will move into preclinical development in 2016.

Pharmaxis and NASH

NASH is commonly found in people who are overweight or obese. Given the increasing rate of obesity around the world, the condition is likely to become a major cause of liver disease and shortened lifespans in coming years.

It's estimated that one in three people have fatty liver disease and that up to 10% of those will have NASH. One of the main ways to manage NASH is for the sufferer to reduce fat in the liver by losing weight through exercise and healthy eating. However, with changes in behaviour difficult to achieve, drug therapies are needed. There are currently no approved therapies available to treat NASH and Deutsche Bank estimates that the global market could be worth in excess of US\$35b by 2025.

It is not surprising that large Pharma companies are focusing their resources on developing and acquiring drug development programs in this area. In 2015, Gilead acquired Phenex a treatment for NASH in Phase 2 trials in a deal worth \$470 million while AstraZeneca bought the rights for a pre-clinical drug candidate to treat NASH from Regulus. Companies like Genefit and Intercept which have drugs that have completed phase 2 trials in NASH have market values in excess of US\$1b.

Pharmaxis has two programs targeting NASH. PXS-4728A, the drug sold to Boehringer, aims to address NASH by inhibiting an enzyme that promotes inflammation. By inhibiting inflammation, disease progression to scarring (fibrosis) and cancer of the liver is hoped to be reduced. The

Company's second program seeks to inhibit the LOXL2 enzyme that is key to scarring (fibrosis) in the liver.

A copy of the recent presentation by Professor Jacob George explaining NASH is available on the [Pharmaxis website](#).

Bronchitol for cystic fibrosis

Bronchitol®, an inhaled dry powder for the treatment of cystic fibrosis, has been the subject of two large scale global clinical trials conducted by Pharmaxis. The product is approved and marketed in Europe and Australia and a third large multicentre clinical trial is currently underway aiming to secure approval in the United States.

United States

In the US Pharmaxis has partnered with Chiesi Farmaceutici SpA which is funding (up to US\$22 million) the international phase 3 clinical trial designed to meet the remaining clinical requirements of the US Food and Drug Administration (FDA). Under the terms of the agreement and following a positive outcome of the trial, Chiesi will have responsibility for completing the new drug application with the FDA and the commercialisation of Bronchitol in the United States. We are already working closely with Chiesi on all aspects of securing US marketing approval for Bronchitol.

The clinical trial (CF303) commenced recruitment in October 2014 and is being conducted in over 120 sites across more than 20 countries. At 9 October 2015, 251 patients had been recruited into the trial, which has a targeted full recruitment of up to 440 patients. The trial has however been designed so that the total recruitment target can be altered based on an independent review of blinded data when 300 patients have been recruited – expected to occur in the current quarter. We are also waiting to see if recruitment rates return to the levels experienced before the northern hemisphere summer. If pre-summer recruitment rates do not return and the independent review of the blinded data indicates the full 440 patients should be targeted, the recruitment period will be extended into 2016. The Company has taken the precautionary step of extending the trial into additional countries to support recruitment efforts. Our current expectation is that CF303 will cost approximately US\$24 million to extend both the length and engaged sites of the trial, of which Chiesi is reimbursing the first US\$22 million.

Europe

In the EU Pharmaxis has appointed Chiesi as its exclusive distributor for the currently launched markets of the UK and Germany. Chiesi is an experienced and respected partner in key global markets and sells Bronchitol as part of its cystic fibrosis portfolio.

Chiesi assumed responsibility for the marketing, sales and distribution of Bronchitol from 1 June 2015. The sale of Bronchitol in Germany (launched in 2012) and in the United Kingdom (launched in 2013) account for more than ninety five percent of current European Bronchitol sales.

During the quarter the Company completed dosing in its phase 2 clinical trial in paediatric patients, the conduct of which is a required post marketing commitment of the European approval for adult patients granted in 2011. The trial results will be available before the end of the year.

Other territories

Approval & pricing reimbursements applications continue to progress in various countries outside of the EU in Eastern Europe, the Middle East and Brazil. Bronchitol has just received full reimbursement for all patients in Turkey. Russian and Brazilian approval is expected over the next two quarters.

Corporate

2015 Annual General Meeting

The 2015 AGM is to be held at the Christie Conference Centre, 3 Spring Street, Sydney, NSW, 2000 on 19 November at 2.30 pm (Sydney time). The notice of meeting and proxy form was sent to shareholders on 16 October 2015.

2015 Statutory Annual Report

The 2015 report is now available on the [Pharmaxis website](#).

Pharmaxis in the media

Following Pharmaxis' announcement of the successful phase 1 clinical trial of PXS4728A, the Company's chief executive officer Gary Phillips conducted a range of interviews with business journalists and analysts providing insight and comment on the Company's achievements and business strategy. Those interviews along with the company's current corporate summary can be accessed on the [Pharmaxis website](#).

Financials

The financial results for the September 2015 quarter better reflect the ongoing business of the Company. Key financial metrics are as follows:

	A\$'000	30-Sep-15	30-Sep-14
Sales revenue		2,084	1,443
Net loss		(6,169)	(8,334)
Segment results – adjusted EBITDA			
Bronchitol & Aridol		(1,151)	(3,462)
New drug development		(984)	(325)
Corporate		(423)	(1,016)
Total		(2,558)	(4,803)
Cash flow – cash used in			
Operations		(3,432)	(8,803)
Investing activities		(446)	(70)
Financing activities		(442)	(446)
Total		(4,320)	(9,319)
Cash at bank		50,324	24,912

An income statement for the quarter is attached. Highlights and commentary for the quarter:

1. The closing cash of \$50.3 million and the reduced net quarterly cash usage places the Company in a strong position.
2. Bronchitol sales for the quarter increased as Chiesi built inventory in its central warehouse and those of its local country wholesalers.

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3. Other revenue income for the September 2015 quarter includes \$2.4 million in relation to the reimbursement by our US partner Chiesi of the current phase 3 clinical trial of Bronchitol and amounts charged to Synairgen under our research collaboration agreement for drug discovery services. The comparative periods include an R&D tax credit of \$1.1 million. The company has not recorded an R&D tax credit in relation to the current period. The Company may not be eligible due to its revenue (including the reimbursement of clinical trial costs from Chiesi) potentially exceeding \$20 million in the 2016 financial year. This will be reviewed each quarter.
 4. Employee costs decreased by 30% when current period redundancy payments are excluded.
 5. Clinical trial costs for the quarter predominantly relate to the phase 3 clinical trial in cystic fibrosis, reimbursed by Chiesi up to US\$22 million. Costs in the prior period related to both the phase 3 clinical trial (A\$0.9 million) and the phase 2 paediatric trial conducted in Europe.
 6. Drug discovery costs have increased in line with the Company's increased focus on developing new drugs from its amine oxidase chemistry platform.
 7. Other includes a realised foreign exchange gain of \$0.6 million and an unrealised foreign exchange loss in relation to the financing agreement with NovaQuest.
 8. Finance expenses relates to the financing agreement with NovaQuest and the Company's finance lease for its Frenchs Forest facility. Subsequent to the Company entering into an Amended and Restated Financing Agreement with NovaQuest in December 2014, the finance expense recorded in relation to the agreement has decreased.
 9. Investing activities predominantly related to new analytical equipment for drug discovery.

Financial statements

(unaudited)

	Three months ended		Segment information - three months ended 30 September 2015			
	30-Sep-15	30-Sep-14	Bronchitol & Aridol	New Drug Development	Corporate	Total
Income statements						
Revenue						
Revenue from sale of goods						
Bronchitol	1,638	1,007	1,638	-	-	1,638
Aridol	446	423	446	-	-	446
Other products	-	13	-	-	-	-
	2,084	1,443	2,084	-	-	2,084
Other income - interest	319	237	-	-	-	-
Other revenue	2,418	1,400	2,168	167	83	2,418
	4,821	3,080	4,252	167	83	4,502
Expenses						
Employee costs	2,811	3,687	1,401	380	631	2,412
Administration & corporate	505	927	107	25	304	436
Rent, occupancy & utilities	296	357	134	19	143	296
Clinical trials	2,370	1,324	2,317	53	-	2,370
Drug development	639	146	-	639	-	639
Sales, marketing & distribution	324	301	324	-	-	324
Safety, medical and regulatory affairs	408	352	408	-	-	408
Manufacturing purchases	365	369	365	-	-	365
Other	2,339	570	347	34	(572)	(190)
Depreciation & amortisation	750	859	-	-	-	-
Finance expenses	175	2,497	-	-	-	-
	10,983	11,389	5,403	1,151	506	7,060
Net Loss before tax/Adjusted EBITDA	(6,162)	(8,309)	(1,151)	(984)	(423)	(2,558)
Income tax expense	(7)	(25)				
Net Loss after tax	(6,169)	(8,334)				
Interest revenue						319
Finance costs						(175)
Depreciation and amortisation expense						(750)
Redundancy costs						(194)
Non-recurring legal expenses						(68)
Unrealised FX loss on NovaQuest						(2,531)
Share-based payment expenses						(205)
Loss before income tax						(6,162)