pharmaxis

Quarterly Report to Shareholders

Issue 34 | Jan – Mar 2012

Pharmaxis Ltd ABN 75 082 811 630

Producing human healthcare products to treat and manage respiratory diseases

Overview of Pharmaxis

The Business
Pharmaxis is a specialty pharmaceutical company with activities spanning product research & development through to manufacture, sales and marketing. The company's therapeutic interests include lung diseases such as asthma, bronchiectasis and chronic obstructive pulmonary disease (COPD) and the genetic disorder, cystic fibrosis.

Based in Sydney, Pharmaxis manufactures its two lead products for commercial sale, clinical trials and for compassionate use and has offices in Exton, Pennsylvania and Slough in the UK.

Aridol
Aridol®, is a mannitol bronchial challenge test and is registered for sale and marketing in Australia, Europe, South Korea and the United States. Aridol is designed to assist in the detection of hyper-responsive, or twitchy, airways, which is one of the hallmarks of asthma. The product is fully reimbursed in the USA and in Korea.

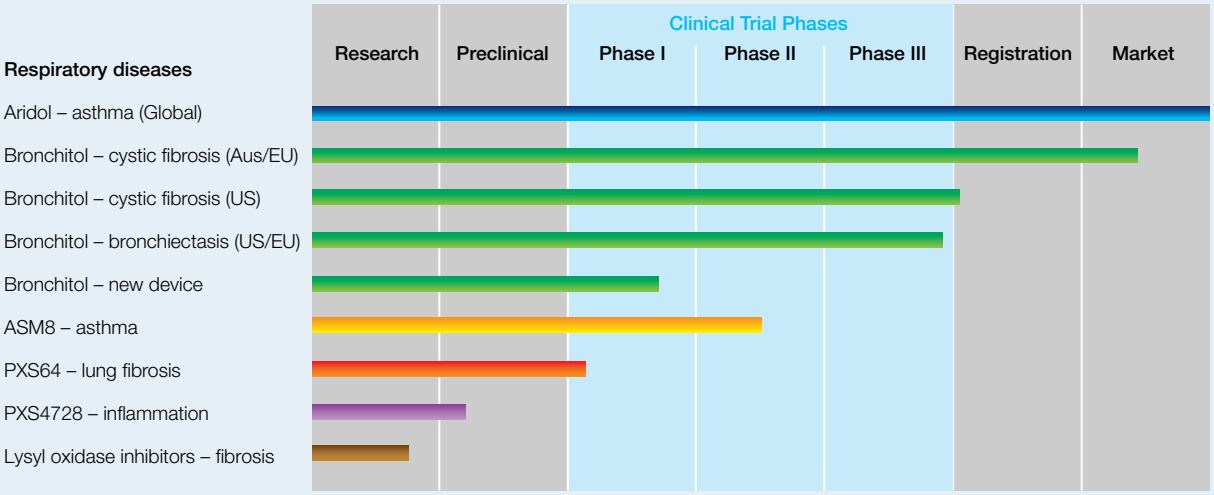
Bronchitol
Bronchitol® has been designed to assist with lung clearance and lung defence for people with cystic fibrosis. Two regulatory Phase III trials for cystic fibrosis have been completed involving over 90 hospitals around the world. Bronchitol is approved for marketing in Australia and approval is pending in Europe.
A marketing application for the US is being assembled.

ASM8
This potential new drug for the treatment of asthma has completed a number of clinical trials in people affected by allergic asthma and it is currently in Phase II clinical trials.

PXS64
This prodrug of PXS25 has been developed for the treatment of lung fibrosis and a Phase I trial with PXS25 has been completed.

PXS4728
This potential new anti-inflammatory drug has now entered preclinical evaluation and is first in a new class of exquisitely selective SSAO inhibitors.

Pharmaxis Product Development at March 2012



CEO Report

This report to shareholders covers the first three months of 2012.

Pharmaxis is now transitioning from a business that was primarily concerned with various forms of product development to a business that is primarily concerned with sales and marketing of its products.

Full reimbursement for Aridol in the USA has been in force since the beginning of the year and we are seeing the benefits in an increased awareness of the product and an increased willingness for pulmonary function laboratories to engage with the product. The sales and marketing investment is modest and, consequently, has to be highly targeted. The U.S. Aridol business is managed by Stephen Beckman from the Pharmaxis office outside Philadelphia.

Bronchitol, however, remains the product that, for now, shapes the future of the company. While we wait to receive the marketing authorisation certificate from the European Medicines Agency, sales and marketing personnel have been recruited and trained in both Germany and the UK. Both these countries are moving to a value based pricing model and we have been working with the UK's National Institute for Health and Clinical Excellence (NICE) to help provide an assessment of Bronchitol. Of course, there remains a degree of uncertainty for a product like Bronchitol that is not yet available in the marketplace and for which there is limited experience outside the confines of a well controlled clinical trial. By necessity, therefore, a number of assumptions have to be made in building a cost effectiveness model and these will be refined as experience of the product becomes more widespread. Accordingly, it would not be a surprise if NICE took an initial conservative position on Bronchitol until additional experience is available. NICE provide an advisory service to the National Health Service in the UK and there is a similar system in place in Germany. Bronchitol has an advantage in this system because of the extensive clinical trial programme that underpins it. In addition, it represents a new class of treatment, fulfils an unmet clinical need and provides a demonstrable benefit to society. In Germany Bronchitol's designation as an orphan product exempts it from review by their national pricing and reimbursement body.

UK cost effectiveness of Bronchitol

In Australia, a Bronchitol reimbursement application to the Pharmaceutical Benefits Advisory Committee concluded last quarter and we were pleased to receive a positive recommendation which will see the product become available to Australian patients with cystic fibrosis in a cost effective manner.

Bronchitol to be launched in Australia

The US marketing application, or NDA, represents another important crystallisation of Bronchitol's value and opens up additional markets such as South America and Canada. During its prosecution with the FDA, Pharmaxis will be exploring all available options to ensure this value is captured.

This report documents progress in building the business over the last few months.

Alan D Robertson, Chief Executive Officer

Forthcoming Events

- The commercial launch of Bronchitol in Germany and the UK
- Submitting the U.S. New Drug Application (NDA) to the FDA
- Reimbursement and commercial launch of Bronchitol in Australia

Bronchitol for Cystic Fibrosis in Europe

Throughout Europe, Bronchitol is indicated for the treatment of cystic fibrosis in people aged 18 years and over.

Before introducing Bronchitol to the European cystic fibrosis community, the European Medicines Agency (EMA) has to grant to the company an appropriate Marketing Authorisation Certificate. The company has been notified by the EMA that it has adopted a positive opinion on the marketing authorisation application. Ahead of receiving the marketing certificate, the company is preparing to introduce Bronchitol in Europe and this will be during the April to June quarter of 2012. The European introduction is being managed from our office in the UK and involves the German company Arvato that supply logistics services and the Quintiles organisation that supply sales and marketing personnel. During this period, the 35th European Cystic Fibrosis Conference is being held in Dublin and, as part of this scientific meeting, there will be a Pharmaxis symposium to introduce and to debate Bronchitol and its role in cystic fibrosis.

Bronchitol is to be launched first in Germany and the UK-countries for which a formal drug pricing and reimbursement application is not required. In other countries, notably France, an application for reimbursement will be lodged following receipt of the European marketing authorisation certificate. Patients suffering with severe, chronic diseases in France are typically reimbursed 100% for all services and medicines associated with the management of their condition, however, a reimbursement application can take as long as six to nine months to complete.

CF clinical studies published

Germany has 7,500 patients with CF treated through 110 CF centres, whereas the UK has 9,000 CF patients treated through 55 centres. The first task of the sales and marketing team will be to ensure that Bronchitol is listed on the hospital formularies and included in the appropriate health care budgets. The awareness of Bronchitol is already high through publication of the clinical trial data and through presentations at international scientific congresses.

Publication of scientific results are an important validation of the quality and integrity of clinical trials and represent an important communication to the clinicians responsible for patient care and welfare. Both Phase 3 Bronchitol clinical trials have now been published in well respected, peer reviewed journals. During the March 2012 quarter, the second Phase 3 (CF302) clinical trial results were published in the American Journal of Respiratory and Critical Care Medicine (Am J Respir Crit Care Med Vol 185, Iss. 6, pp 645–652, Mar 15, 2012) with Professor Aitken from the University of Washington Medical Center, Seattle, Washington as the leading author. This scientific paper follows a 2011 publication in the European Respiratory Journal of the first Phase 3 (CF301) clinical trial results.

Bronchitol for cystic fibrosis in USA

The United States represents the largest single market opportunity for Bronchitol as there are approximately 30,000 people with CF managed through 150 hospitals.

U.S. marketing application to be submitted

Before a drug can be marketed in the USA it requires approval from the Food and Drug Administration, which will conduct a thorough review of all the available information on the new product including its clinical effectiveness, its safety and the method of manufacture. Bronchitol has been the subject of two rigorous reviews in Australia and in Europe and the US application will seek consent from the FDA to market Bronchitol for people with cystic fibrosis 6 years of age and over.

The FDA review may also involve discussion of Bronchitol by an advisory committee that is charged with the responsibility of providing expert advice to the FDA. The Prescription

Drug User Fee Act in the USA allows the FDA to collect fees from sponsoring pharmaceutical companies and to provide guidance on the likely timeline during which a decision will be reached on any new drug evaluation. Currently, this is 10 months from when the application is submitted.

Bronchitol priced in Australia

Bronchitol for Cystic Fibrosis in Australia

Bronchitol has been approved in Australia for the treatment of cystic fibrosis in patients aged 6 years and over. The approval was finalised in early 2011, however, before the drug can be supplied to patients in a cost effective manner, it has to be included on the Pharmaceutical Benefits Scheme (PBS). The Australian Government's PBS provides reliable, timely and affordable access to a wide range of prescription medicines for all Australians. The inclusion of a new drug on the PBS is a rigorous process that, in the first instance, requires a positive opinion to be adopted by the Pharmaceutical Benefits Advisory Committee (PBAC). This government appointed committee considers the merits of all new drugs from a cost effectiveness perspective. Following an extensive twelve month review, Bronchitol was recommended by the PBAC in March of this year for inclusion on the PBS. Final listing on the PBS can sometimes take a few months after receipt of a positive PBAC recommendation.

In the meantime, Bronchitol is supplied to individual named patients as part of the Pharmaxis Physician Familiarisation Programme, whereby Pharmaxis subsidises the cost of the drug to the patient. There are nearly 3,000 people in Australia with cystic fibrosis, treated through 20 hospitals and Bronchitol is now included on all the relevant hospital formularies.

Bronchitol presentation at TSANZ

The Thoracic Society of Australia and New Zealand held their annual scientific conference in Canberra in March. This meeting is largely concerned with asthma and COPD, however, there are also presentations and discussion on other diseases such as cystic fibrosis and bronchiectasis. The clinical experience gained with Bronchitol so far was presented by Professor Wark from the University of Newcastle to a group of clinicians interested in cystic fibrosis.

Mucus clearance is a key goal for people living with cystic fibrosis and Bronchitol is expected to become a major part of cystic fibrosis management.

Bronchitol for bronchiectasis

Mucus clearance is also a key goal for people living with bronchiectasis and Bronchitol is in an extensive clinical trial to help broaden the utility of the product and assist people with this debilitating condition.

There is no particular product available for clearing mucus from the airways of patients with bronchiectasis and, a drug that is often used to help clear mucus in CF (dornase alfa), has been shown to be detrimental in treating people with bronchiectasis.

Bronchiectasis Phase 3 trial on track

The purpose of the ongoing trial with Bronchitol is to examine the efficacy and safety of 52 weeks of treatment. Previous studies with Bronchitol demonstrated improvement in mucociliary clearance; mucus rehydration; improvement in quality of life and respiratory symptoms. The results of this study in combination with a previously completed 3 month study seek to extend the evidence to support Bronchitol's use as a therapy in people with bronchiectasis.

Bronchitol should improve the overall health and hygiene of the lung through regular and effective clearing of the mucus load. As a consequence of the reduction in mucus load, the frequency of bronchiectasis related pulmonary exacerbations and the need for exacerbation related antibiotic treatment should fall.

485 subjects have been recruited to the trial and the last patient had their first visit in December of last year. The trial is double blinded and the last patient is expected to have their last visit in December 2012. It is expected the data will be available sometime early next year. In the meantime, a great deal of work is in progress to ensure the trial data is captured and stored effectively and participating hospitals are kept informed of progress.

ASM8 for asthma

Potential new drug for asthma

ASM8 is a potential new drug to treat the inflammation associated with severe allergic asthma. It is a combination of two oligonucleotides delivered to the lung as a nebulised solution. Oligonucleotides are a specific class of drug that interfere with protein synthesis in a controlled and highly targeted fashion. ASM8 reduces the production of important pro-inflammatory proteins and has been studied in a number of clinical trials involving both healthy volunteers and people with allergic asthma. The current clinical trial involves administering ASM8 for 14 days and then challenging the volunteers with the very specific allergen that usually triggers their asthmatic response. The trial has been conducted in a small number of patients in four hospitals in Canada. The trial is exploratory in nature and seeks to define the appropriate dosing schedule for future longer term trials.

ASM8 inhibits protein synthesis

Asthma is a difficult condition to treat and it is the number one reason for children attending the emergency rooms of hospitals. In Australia, asthma affects more than 10% of the population and for many of those patients there are no effective remedies to help control their disease. ASM8 is designed to provide an alternative solution for those severe asthmatics who are not controlled by existing drugs.

PXS 25/64

Potential new drug for IPF

PXS25 is a small molecule that appears to be expertly suited to controlling fibrosis. Over many years of research, it has been well demonstrated that the protein TGFβ is required to drive fibrosis. One of the challenges in tackling this protein as a treatment of fibrosis is not to interfere with its critically important function in other parts of the body. PXS25 does just that and, in an important paper published late last year, it was shown that PXS25 could block the function of TGFβ in promoting kidney fibrosis. Moreover, it is able to do this without causing toxicity. In considering how to produce an improved version of PXS25, a prodrug has been synthesised that has improved cellular permeability and, in cell culture, is even more effective at blocking the function of TGFβ than PXS25. The active ingredient inside the cell remains PXS25. This potential pharmaceutical is PXS64 and some of its attributes in tackling fibrosis were presented at a major US scientific meeting in March.

PXS64 shows promise as a unique small molecule that is well suited for treating and, perhaps, reversing idiopathic pulmonary fibrosis (IPF) – a condition of the lung that leads to the early death of the patient. IPF affects as many as 500,000 people in the major pharmaceutical markets of the world and, so far, only one drug has been approved for marketing in certain countries.

PXS 4728

Potential new inflammation drug

PXS4728 is a small molecule inhibitor of a protein known as SSAO. This enzyme is a key regulator of inflammation and a small molecule inhibitor should, therefore, be an effective anti-inflammatory agent. PXS4728 has been chosen from many hundred potential inhibitors as a molecule that has the required selectivity for its target protein and has the right pharmaceutical properties. PXS4728 performs well in cellular assays and in models

of inflammation and is currently in scale up synthesis with a view to entering formal preclinical safety testing later this year.

PXS4728 is effective when delivered orally and has properties indicative of once a day dosing in patients. The first clinical trials will be in healthy volunteers to measure the pharmacokinetic properties of the drug and also to determine its safety. These studies will be scheduled for the beginning of 2013 following completion of the preclinical safety testing.

Aridol

Aridol is a lung test approved for use in identifying bronchial hyperresponsiveness to assist in the diagnosis of asthma in Europe, Australia, parts of Asia and the USA. The USA represents the market of greatest potential and, as of the 1st January 2012, Aridol became fully reimbursable for the lung function testing labs and the clinician conducting the Aridol test.

Aridol sales grow in the USA

Aridol has been well received in the U.S and the improved reimbursement environment will help sales. It is a product that provides new information to the clinicians that is quick and convenient. The investment in selling and marketing Aridol is modest and the effort is driven from the Pharmaxis office outside of Philadelphia. The U.S. sales for the quarter were a substantial increase on the previous quarter and represent a relatively even mix between new customers and reorders from existing customers. Approvals by the hospital formulary committees remains at 100% and, on average, the approval rate is over 10 per month.

During the quarter the American Academy of Allergy, Asthma & Immunology held their 2012 annual meeting in Orlando, Florida. This is one of the most important US scientific meetings for people involved with asthma research and there were twelve presentations involving Aridol and its place in assisting with detecting bronchial hyper-responsiveness.

Financial Overview of the Quarter

The cash position at the end of the quarter was $92 million.

For the March 2012 quarter, sales of $298,000 compared to $318,000 in the March 2011 quarter and $341,000 in the December 2011 quarter. Sales of Aridol in the US were $107,000 compared to $70,000 in the December 2011 quarter.

Research and development dominate expenses

Research and development expenses of $6.5 million for the March 2012 quarter compares to $7.8 million in the March 2011 quarter and $8.1 million in the December 2011 quarter. Clinical trials and manufacturing development account for 34% and 27% respectively of expenditure in the current quarter. The decreased expenditure in the current quarter primarily reflects reduced clinical trial expenditure.

Commercial expenses of $3.0 million compares to $2.7 million in the March 2011 and December 2011 quarters. Preparation for the launch of Bronchitol in Europe increased costs for the March quarter, primarily driven by recruitment in Germany.

Administration expenditure of $1.4 million compares to $1.2 million in the March 2011 quarter and $1.7 million in the December 2011 quarter.

Operating activities used cash of $9.1 million compared to $10.2 million in March 2011 and $8.4 million in the December 2011 quarter. Investing activities used cash of $130,000 compared to $297,000 in March 2011 and $38,000 in the December 2011 quarter.

Financial Statement Data – Unaudited
(International Financial Reporting Standards)

('000 except per share data)

Income Statement Data

	Three months ended		Nine months ended	
	31-Mar-12	31-Mar-11	31-Mar-12	31-Mar-11
	A$	A$	A$	A$
Revenue from sale of goods	298	318	958	677
Cost of sales	(111)	(148)	(365)	(266)
Gross profit	187	170	593	411
Interest	1,049	697	2,081	2,468
Other income	761	82	2,433	332
Expenses				
Research & development	(6,461)	(7,832)	(21,821)	(25,552)
Commercial	(2,972)	(2,668)	(7,358)	(6,329)
Administration	(1,383)	(1,206)	(3,998)	(3,999)
Finance expenses	(209)	(215)	(567)	(648)
Total expenses	(11,025)	(11,921)	(33,744)	(36,528)
Loss before income tax	(9,028)	(10,972)	(28,637)	(33,317)
Income tax expense	29	(58)	123	(65)
Loss for the period	(8,999)	(11,030)	(28,514)	(33,382)
Basic and diluted earnings (loss) per share – $	(0.029)	(0.048)	(0.109)	(0.147)
Depreciation & amortisation	1,164	1,167	3,511	3,573
Fair value of securities issued under employee plans	211	352	756	1,182

Balance Sheet Data

	As at	
	31-Mar-12	30-Jun-11
	A$	A$
Cash and cash equivalents	91,550	44,343
Property, plant & equipment	28,368	30,570
Intangible assets	14,585	15,954
Total assets	141,576	94,572
Total liabilities	(22,155)	(23,742)
Net assets	119,421	70,830

Cash Flow Data

	Three months ended		Nine months ended	
	31-Mar-12	31-Mar-11	31-Mar-12	31-Mar-11
	A$	A$	A$	A$
Cash flows from operating activities	(9,086)	(10,174)	(28,164)	(27,395)
Cash flows from investing activities	(130)	(297)	(84)	(1,140)
Cash flows from financing activities	(423)	(304)	75,445	(563)
Impact of foreign exchange rate movements on cash	(13)	62	10	(405)
Net increase (decrease) in cash held	(9,652)	(10,713)	47,207	(29,503)

Share Data

	Ordinary Shares as at	
	31-Mar-12	30-Jun-11
Ordinary shares on issue	305,891	228,290
Options over ordinary shares outstanding	11,430	13,297

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Contact Details

Further information on Pharmaxis can be obtained from www.pharmaxis.com.au or by contacting David McGarvey, Chief Financial Officer:

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