pharmaxis

Quarterly Report to Shareholders

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Pharmaxis Ltd ABN 75 082 811 630

pharmaxis

Photo courtesy of David Poole, The Daily Mail, London.

Living with Cystic Fibrosis

What does a person with cystic fibrosis look like? The glowing photograph on the left belies the struggles Kate Smith endures daily, often hourly, in living with cystic fibrosis:

'You'd hardly know I was ill if you were to look at me,' Kate wrote in the Daily Mail in London. 'But I am constantly exhausted. The rigorous exercise and physiotherapy regime required to help loosen the mucus on the lungs would tire anyone. The effect of absorbing a cocktail of drugs – up to 50 pills a day – plus inhalers, also saps the energy. The onset of infection often brings appalling pains in my chest and either side of my spine and I'm often hooked up to intravenous antibiotics.

'The reduced lung capacity makes it harder still to cope with everyday life, let alone a full-time job. In my early 20s I worked, but a severe infection brought on by my illness forced me to quit. Abandoning my career was the hardest decision I've made, but there was little choice.

'And always at the back of my mind there is a number – the average life expectancy for someone with my disease. That number is 31. I've just turned 30. Cystic fibrosis is with me day and night but the right treatment may hold it at bay. Techniques have developed rapidly in the past two decades. With smarter treatment, sufferers are living longer.'

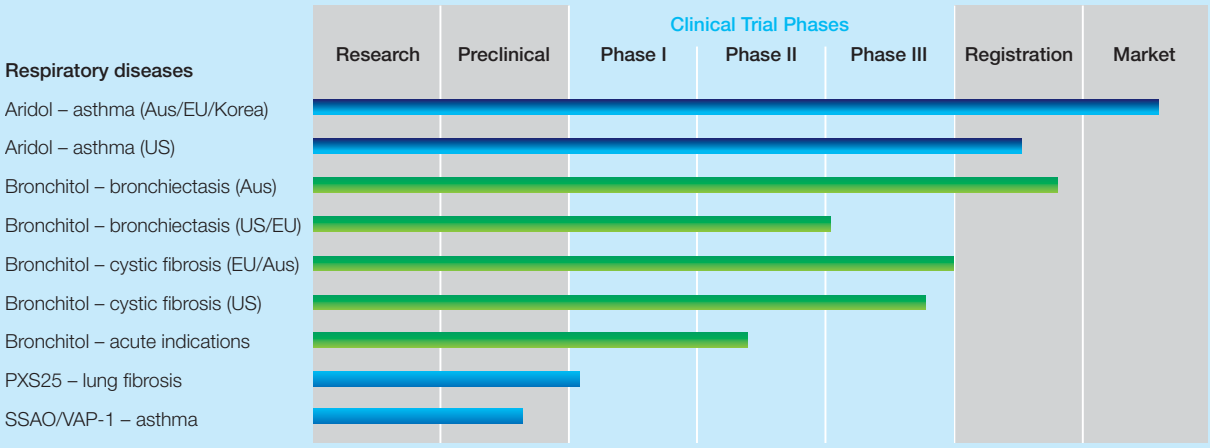
At Pharmaxis, we draw our motivation from a drive to help patients like Kate, who are in need of better treatments.

Overview of Pharmaxis

Pharmaxis is a specialty pharmaceutical company with activities spanning product research & development through to manufacture, sales and marketing. Our therapeutic interests include lung diseases such as cystic fibrosis, asthma, bronchiectasis and chronic obstructive pulmonary disease.

Our first product, Aridol, is now registered for sale in Australia, Europe and South East Asia and a request for marketing has been filed in the United States. Aridol is designed to assist in the management of both asthma and chronic obstructive pulmonary disease. Our second product, Bronchitol, has completed the first regulatory Phase 3 trials in both cystic fibrosis and bronchiectasis.

Our research group is developing two new potential therapies for chronic and debilitating lung conditions such as asthma and pulmonary fibrosis.



CEO Report

Welcome to our 24th quarterly report. During the quarter, we have been heavily involved in the final stages of putting a new drug on the international market. I am very pleased to have reached this stage after seven years of development. It is worth noting that it is a rare event to get to this position in drug development, where the attrition rate is very high indeed. The company is now entering new ground as far as Bronchitol is concerned and we have been working hard on raising the international awareness of the product and its role in the treatment of cystic fibrosis. Since the first data was revealed in May, we have presented to international audiences at the European CF meeting, the European Respiratory Society Congress, and the Australian CF meeting. A health economic dossier has been prepared and market research has been conducted amongst patients, payors and clinicians and a scoping meeting has been held with the National Institute of Clinical Excellence in the UK. The company is now well advanced in its preparation for the launch of Bronchitol, which we hope will be during the second half of next year.

Bronchitol market launch preparation

In addition to the positive results from the first pivotal CF study, a survey conducted in a small sample of clinical trial participants who went on to receive Bronchitol in the open label phase of the study reported positive responses. The reasons people believed Bronchitol was working included the following: better lung function test; being able to perform better at sport; not being as sick; less hospital visits; having easier and better physiotherapy sessions; achieving better quality and more restful sleep. This feedback from the field provides immense encouragement and we are very much looking forward to bringing Bronchitol to the international cystic fibrosis community.

Alan D Robertson, Chief Executive Officer

Third Quarter Highlights

Two new Phase 3 trials complete recruitment

- The second pivotal Phase 3 cystic fibrosis trial closed to recruitment having enrolled 319 subjects
- The first pivotal Phase 3 trial results of Bronchitol for cystic fibrosis were presented at three international scientific meetings
- Ethics and regulatory approval was received to commence a Phase 1 trial with PXS25
- Reimbursement received for Aridol in South Korea

Coming Events

Major milestones in coming months

- EU marketing application submission for Bronchitol as a treatment for cystic fibrosis
- FDA advisory committee meeting to discuss the Aridol New Drug Application
- FDA response to the Aridol New Drug Application
- Second Phase 3 trial of Bronchitol for bronchiectasis to commence dosing
- PXS25 starts Phase 1A clinical trial

Corporate News

Pharmaxis De-Lists from Nasdaq

During the quarter Pharmaxis voluntarily de-listed from the Nasdaq. This followed a review of current market requirements which found the benefits of the Nasdaq listing could no longer justify the related ongoing costs. The Company's primary listing on the Australian Securities Exchange will continue without change.

Annual Review, Report and AGM

Pharmaxis' 2009 Annual Review of its operations and Statutory Annual Report were released in September. The Annual Review is a concise report which includes highlights of the year's progress, an update of our products in development, an overview of operations and summary financial data. The Statutory Annual Report is a comprehensive document (144 pages this year) detailing all of Pharmaxis' statutory annual reporting and disclosure requirements. Both documents can be found online at www.pharmaxis.com.au/annual-reports

2009 AGM to be held on 21 October

Meanwhile, Pharmaxis' 2009 Annual General Meeting will be held at the Intercontinental Hotel Sydney, on the corner of Bridge and Phillip Streets in Sydney on Wednesday, 21 October, at 2.30pm. A notice of the meeting was issued to all shareholders in September. As well as reviewing the year's activities, shareholders will be invited to elect a new Board member, Richard van den Broek. If elected, Richard will replace Peter Farrell, who is retiring from the Board after three and a half years of valued service.

Marketing experts join company

Pre-marketing resources intensified

Two new Bronchitol product managers have been appointed in Australia and the UK to spearhead our pre-marketing efforts in those countries. Both have extensive experience in launching products for specialist orphan diseases and we are delighted to have them on board.

Pharmaxis presents at global pharma conference

Pharmaxis was one of two Australian companies invited to present at the UBS Global Life Sciences Conference in New York in September. The conference was attended by an international audience of institutional investors; and Pharmaxis shared a stage with major pharmaceutical firms at the four-day event.

Facilities

New factory commissioning on schedule

The commissioning of Pharmaxis' new manufacturing plant in readiness for the European launch of Bronchitol for cystic fibrosis is progressing to plan, with the three-storey spray dryer now operational. A specialised air handling plant, WFI water treatment plant, powder blender and blister packer have also been installed in recent months. The last major piece of equipment to be fitted is the automated capsule filler and the Factory Acceptance Tests at the manufacturer have been completed satisfactorily. The facility is due to be fully operational by the second quarter of 2010.

Publications

Four new peer reviewed scientific papers were published in major scientific journals during the quarter. The clinical results attesting to the performance of Aridol came from hospitals in Denmark, The Netherlands and the USA. These publications are an important part of growing the international awareness of Aridol and will help drive sales.

Bronchitol for Cystic Fibrosis

Second Phase 3 CF trial completes recruitment

Second trial closes recruitment

Enrolment has surpassed expectations for the second Phase 3 clinical trial of Bronchitol for the treatment of cystic fibrosis. The trial has now completed recruitment after passing its target of 300 subjects and enrolling 319 subjects with cystic fibrosis. The study results are expected to be available during the first half of 2010. This is the second of two trials in cystic fibrosis required by the U.S. FDA before a marketing application can be submitted in the United States. More than 600 cystic fibrosis patients have now been recruited into the two Bronchitol Phase 3 studies.

The latest trial is being conducted at 65 sites in seven countries, comparing 400 mg of Bronchitol twice a day to control. The primary objective is improvement in lung function. Pharmaxis has high expectations that like the previous study, this trial will show that Bronchitol significantly improves cystic fibrosis. After the trial results are released the marketing application for the U.S. will be submitted. More than 75,000 people in the major pharmaceutical markets are affected by cystic fibrosis and no products have been approved to improve lung hydration.

Marketing dossier set to be submitted

EU marketing application this month

In October, Pharmaxis will file an application with the European Medicines Agency (EMeA) to market Bronchitol in Europe for the treatment of cystic fibrosis. The marketing application is based on the positive results of the first Phase 3 trial reported in May this year. Since then, intensive resources have been committed to carefully compiling the extensive dossier for the EMeA. Throughout preparation of the application, Pharmaxis has been engaged in frequent and cooperative dialogue with the regulator to ensure the document meets its stringent requirements. The EMeA will announce its decision during the second half of 2010.

In preparation for Bronchitol being launched in Europe, the first phase of health economic modeling of Bronchitol in cystic fibrosis has been completed. This modeling will support the pricing applications that Pharmaxis will make for Bronchitol in the EU.

Strong profile at international congress

Bronchitol results generate great interest at major medical meeting

The results from the Phase III trial of Bronchitol in cystic fibrosis were presented in a late-breaking oral presentation at the 2009 European Respiratory Society congress in Vienna in September. Attended by more than 18,000 respiratory physicians from around the world, the Congress is a high profile annual event. Data from the pivotal study was presented by Dr. Diana Bilton, Consultant Physician and Honorary Senior Lecturer at the Department of Respiratory Medicine, The Royal Brompton Hospital, London, UK.

At the same meeting, a session entitled Bronchiectasis Comes of Age was overflowing, with 1,500 doctors filling the room and another 300 watching on screens outside, as the picture (left) shows. The session included a discussion of Bronchitol patient data collected thus far and the additional data expected from the second Phase 3 bronchiectasis trial. During the course of the Congress, Pharmaxis management met with the Bronchitol Advisory Board to discuss how we can better develop the product, both clinically and commercially. On many fronts the Congress was a valuable event for informing and hearing from opinion leaders on the company's products and targeted disease states.

The next major medical presentation by Pharmaxis will be at the North American Cystic Fibrosis meeting in Minneapolis in October. This will be attended by thousands of respiratory physicians, patients and institutional investors.

Bronchitol for Cystic Fibrosis

Bronchitol for Bronchiectasis

A second Phase 3 trial of Bronchitol in patients with bronchiectasis is ready to commence dosing and will recruit 474 patients throughout 57 hospitals across seven countries.

The study aims to show that Bronchitol reduces acute sickness and hospitalisation. If positive, the results will form the basis of marketing applications to both the U.S. and Europe.

Bronchitol for bronchiectasis is the subject of a marketing application being considered by the Australian Therapeutic Goods Administration; the conclusion of the process is expected by the end of the year.

Aridol

Aridol featured at global scientific congress

Aridol is attracting growing interest from independent research scientists worldwide, with a total of 14 poster presentations at the recent 2009 European Respiratory Society congress in Vienna. In addition, a major respiratory meeting in North America discussed the role of Aridol in asthma detection, the proceedings of which are due to be published in a major scientific journal.

Independent studies show positive results

Of particular interest was a clinical trial that demonstrated that in a random population sample, Aridol is as specific and sensitive at detecting people with asthma as the market leader methacholine. The study by C Porsbjerg et al also found Aridol better reflected ongoing airway inflammation, which is crucial when treating asthma. The data presented were strongly positive, reinforcing Aridol as a clinically relevant test.

US marketing application progress

The U.S. FDA is on track to advise before the end of the year whether our New Drug Application seeking approval to market Aridol in the States is successful.

NDA meeting in November

Late next month the FDA will hold an advisory committee meeting in Washington, where Pharmaxis and two clinical experts will present in support of the application. The meeting is open to the public and is often attended and addressed by influential patient groups and medical experts. Pharmaxis has been actively undertaking clinical awareness activities, engaging advisory boards and patient groups in both the US and Europe.

The advisory committee will then make a non-binding recommendation to the FDA, which is due to announce its decision on 27 December.

Pharmaxis is seeking approval for Aridol for ‘the assessment of bronchial hyper responsiveness to aid in the diagnosis of patients with symptoms of or suggestive of asthma.’

Bronchial challenge tests are designed to help in the correct diagnosis and assessment of asthma. If approved, Aridol will be the first dry powder bronchial challenge test available in the U.S.

Korea’s premium price

Korea price agreed

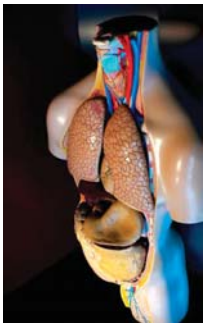
Full reimbursement has been granted to Aridol in Korea at a premium to other tests in the market. The price approval follows a long reimbursement process review by Korean authorities. A total of 13 countries have now launched Aridol, with Spain and Italy also recently approving the therapy for reimbursement.

Aridol sales gain traction in Europe

Aridol in Europe

Bronchial challenge tests are not a high priority medical item (relative to medicines for diseases such as cancer and cystic fibrosis) and the sales of Aridol have been delayed in all countries as Pharmaxis, or its distributors, have negotiated hospital purchasing procedures. Whilst this still affects countries like Italy and Spain who have recently launched Aridol, it is encouraging to see consistent increases in sales throughout the year from markets like Scandinavia and the UK where the product has been on the market for longer. In the UK, for example, we are adding new customers every month and there are still 65 pricing submissions in progress.

Early Stage Research Activities



Phase 1A trial underway for lung disease

Consistent with our mission of bringing new medicines for respiratory diseases through development to market, Pharmaxis has begun human testing of a potential new medicine for pulmonary fibrosis.

The Phase 1A trial of the molecule PXS25 began in early October, to assess its safety and tolerability in healthy volunteers. The trial in 64 subjects is underway to determine PXS25's suitability to progress to the second efficacy stage of testing. Pulmonary fibrosis is a degenerative disease of the lungs, affecting more than 500,000 people in the major pharmaceutical markets. The average life expectancy on diagnosis is four years, with no approved therapies available outside Japan.

Results from the first trial are expected during the 4th quarter of 2009.

A patent for PXS25 has been granted in Europe and is at the allowance stage in the USA. More information on our patents can be found at www.pharmaxis.com.au/patents

Financial Overview of the Quarter

At 30 September 2009 Pharmaxis had A$113 million in cash.

For the September 2009 quarter, Aridol sales of A$183,000 compared to A$106,000 in 2008 and A$141,000 in the June 2009 quarter. Interest income of $952,000 earned primarily on commercial bills, compares to $2.1 million in 2008, reflecting significantly lower interest rates and a lower average balance of funds invested.

Research and development expenses of A$8.1 million for the June 2009 quarter compares to A$6.0 million in the September 2008 quarter, and A$8.5 million in the June 2009 quarter. Expenditure on clinical and manufacturing development accounted for the changes.

Commercial expenses of A$1.3 million compares to A$1.4 million in the September 2008 quarter and A$1.9 million in the June 2009 quarter. Commercial expenditure includes the costs of preparing for the launch of Bronchitol in Europe and the US, the costs of preparing for sale of Aridol in the US and the costs of selling Aridol in Europe and Asia Pacific.

R&D dominates expenditure

Administration expenditure of A$1.7 million compares to A$1.3 million in the September 2008 quarter and A$1.5 million in the June 2009 quarter. Costs associated with the new facility and larger infrastructure are the major components of the changes. Finance costs represent the ongoing finance charge component of the capitalized finance lease for our new facility at Frenchs Forest.

Operating activities used A$10.0 million compared to A$4.9 million in 2008 and A$10.1 million in the June quarter. Investing activities of A$1.3 million compared to A$1.4 million in 2008 and A$1.8 million in the June quarter. Financing activities include exercise of employee options and reduction of the capitalized lease on our headquarters.

Financial Statement Data – Unaudited
(International Financial Reporting Standards)

('000 except per share data)

Income Statement Data

	Three months ended	
	30-Sep-09	30-Sep-08
	A$	A$
Revenue from sale of goods	183	106
Cost of sales	(47)	(29)
Gross profit	136	77
Interest	952	2,076
Other income	88	3
Expenses		
Research & development	(8,111)	(5,960)
Commercial	(1,251)	(1,371)
Administration	(1,721)	(1,283)
Finance expenses	(286)	–
Total expenses	(11,369)	(8,614)
Loss before income tax	(10,193)	(6,458)
Income tax expense	(11)	(6)
Loss for the period	(10,204)	(6,464)
Basic and diluted earnings (loss) per share – $	(0.047)	(0.033)
Depreciation & amortisation	505	252
Fair value of options issued under employee plan	604	611

Balance Sheet Data

	As at	
	30-Sep-09	30-Jun-09
	A$	A$
Cash and cash equivalents	113,438	124,993
Property, plant & equipment	32,559	32,698
Intangible assets	1,189	1,193
Total assets	151,822	163,997
Total liabilities	(23,508)	(26,306)
Net assets	128,314	137,691

Cash Flow Data

	Three months ended	
	30-Sep-09	30-Sep-08
	A$	A$
Cash flows from operating activities	(10,024)	(4,877)
Cash flows from investing activities	(1,324)	(1,430)
Cash flows from financing activities	(189)	11
Net increase (decrease) in cash held	(11,537)	(6,296)

Share Data

	Ordinary Shares as at	
	30-Sep-09	30-Jun-09
Ordinary shares on issue	217,900	217,659
Options over ordinary shares outstanding	14,683	15,075

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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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