Research Note

Pharmaxis

Rising from the ashes

Chief Research Analyst

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Name:	**Pharmaxis Ltd**
Country:	**Australia**
Price:	**AUD 0.23**
ISIN Code:	**AU000000PXS5**
Reuters Code:	**PXS.AX**
Market Cap (AUD m):	**72.0**
EV (AUD m):	**10.0**
Cash & cash eq. (AUD m):	**62.0*)**
Shares outstanding (m):	**312.85**
Volume:	**2,704,540**
Free float:	**100%**
52-week Range:	**0.04-0.27**

) Including payment Boehringer Ingelheim

	2013A	2014A	2015E
Total Income	3.237	5.036	60.000
Net Loss/Profit	(43.535)	(51.818)	28.000
Net loss per share (cents)	(14.1)	(16.8)	8.9
R&D costs	23.862	17.702	22.000
Cash increase/(decrease)	(17.544)	(29.802)	26.000
Cash and marketable sec.	63.943	34.182	60.000

Executive Summary

- Pharmaxis is an Australian based pharmaceutical company with a portfolio of products at various stages of development and approval. The company's development pipeline is centred on its expertise in amine oxidase chemistry and includes Semicarbazide-Sensitive Amine Oxidase Inhibitors (SSAO) for Non-alcoholic Steatohepatitis (NASH) and inflammatory diseases including Chronic Obstructive Pulmonary Disease (COPD), and Lysyl Oxidase Inhibitors (LOX) targeting fibrotic diseases including pulmonary fibrosis and some cancers.

- Its product Bronchitol is a spray-dried form of mannitol, delivered to the lungs by a specially designed, portable inhaler. The product is approved for marketing for the treatment of cystic fibrosis for patients aged over six years in Australia and for patients aged 18 years and over throughout the European Union and in Israel. A phase III trial to enable completion of an NDA for the US market is underway.

- Recently the company announced its new strategy that is focused on innovation and partnering. The new direction follows the completion of two years of restructuring that included partnering Bronchitol in major markets worldwide. Key elements of this strategy are to build a regional biotech powerhouse in fibrosis and inflammation, and creating value by partnering earlier stage development (Phase I or II) projects such as the SSAO and LOXL2 inhibitor programs.

- A very successful example of implementing this strategy was already announced last week when the company published that Boehringer Ingelheim has exercised its option to acquire the investigational drug PXS4728A, to develop it for the treatment of the liver-related condition NASH and to prevent its consequences. NASH is the progressive form of non-alcoholic fatty liver disease (NAFLD), the most common liver disorder in

Western industrialized nations. With a total potential value of more than AUD 750 million, this deal is truly groundbreaking for the Australian biotech sector. Pharmaxis will receive an upfront payment of EUR 27.5 million and, subject to the continuing successful development and commercialization of the PXS4728A program, follow on payments.

- There are a number of key milestones to focus on in the next 6-12 months which include: Phase I results on the PXS4728A program, identification of a lead candidate in LOXL2 (Lysyl Oxidase Inhibitor targeting fibrotic diseases including pulmonary fibrosis and some cancers) as well as bringing it into preclinical and Phase I. And last but not least updates on CF303 on enrolment and completion of the trial.

- The company's current cash position is AUD 62 million, which includes the milestone payment of Boehringer of AUD 39 million. The finalization of the restructured Bronchitol business has led to a reduced investment of more than 50% and will shorten the time to profitability. Also the distribution and supply agreement with Chiesi Farmaceutici has provided funding of the phase III with Bronchitol in the US.

- Based on NPV based valuation, we believe that Pharmaxis is still substantially undervalued at the current share price of AUD 0.23. Using our valuation model and taking into account the deal with Boehringer Ingelheim, the Company's current total value is AUD 300 million, or AUD 0.96 per share. This represents a substantial upside from the current share price.

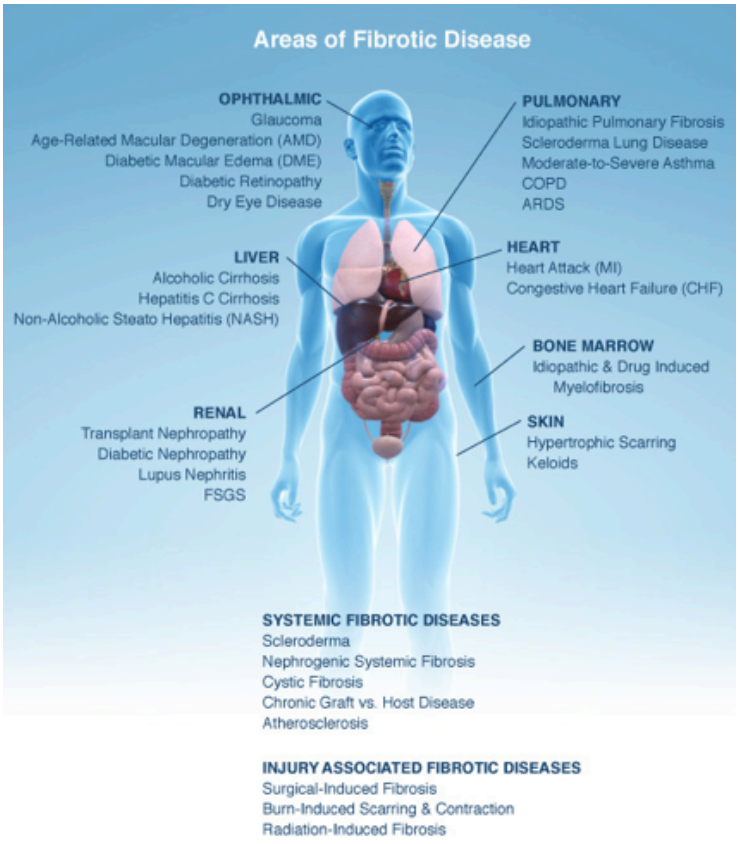
Company Profile & Technology

Pharmaxis is an Australia based specialty pharmaceutical company with a portfolio of products at various stages of development and approval. Its product Bronchitol for cystic fibrosis is marketed in Europe and Australia and a phase III trial to enable completion of an NDA for the US market is underway. Its product Aridol for the assessment of asthma is sold in Europe, Australia and Asia. The company's development pipeline is centred on its expertise in amine oxidase chemistry and includes Semicarbazide-Sensitive Amine Oxidase Inhibitors (SSAO) for Non-alcoholic Steatohepatitis (NASH) and inflammatory diseases including Chronic Obstructive Pulmonary Disease (COPD), and Lysyl Oxidase Inhibitors (LOX) targeting fibrotic diseases including pulmonary fibrosis and some cancers. In the past two years the company was busy transforming the company with a focus on innovation and partnering including its main product Bronchitol in major markets worldwide. Important part of the strategy is to create value via partnering. Collaborations with big pharma reduce the risk profile and accelerates the development program. The recent sale of the early stage drug PXS4728A to Boehringer Ingelheim is a perfect example of the successful transition. The deal also accentuated management experience in capital markets, business development and partnering. Pharmaxis aims to build a regional powerhouse in fibrosis and inflammation.

Technology Platform: Amino Oxidase based enzymes

The inhibition of amino oxidase base enzymes has a broad array of potential applications. Scientific research showed that there is a strong positive correlation between amino oxidase activity and lung related diseases like COPD, Asthma, Cystic Fibrosis (CF), Pulmonary Fibrosis and liver related diseases like NASH (Nonalcoholic Steatohepatitis). LOX and LOXL-2 play a clear role in the pathogenesis of lung and liver fibrosis and cancer as they are involved in the creation and maintenance of the pathologic microenvironment. Given the house keeping function of LOX and the excellent target validation from a LOXL-2 selective antibody, Pharmaxis has chosen to focus its development efforts on small molecule LOXL-2 selective inhibitors for the treatment of

fibrosis. For over 50 years lysyl oxidase activity has been linked to fibrosis. Arresto Biosciences developed a functional LOXL-2 antibody that Gilead Sciences acquired in 2010 as a Phase 1 ready asset for USD 225 million. The antibody is currently in a broad Phase IIb clinical trial program.



Fibrosis is a leading cause of morbidity and mortality worldwide. Fibrosis is defined by the excessive accumulation of extracellular matrix components in organs or tissues, altering their architecture, and disrupting their normal function. It can affect almost any organ or tissue and has a broad association with various diseases and injuries. Despite this, there are minimal therapies available on the market to treat fibrosis and it therefore presents as a high unmet medical need.

Agreement with Boehringer Ingelheim

In March 2015, Pharmaxis signed an agreement with Boehringer Ingelheim for its inhibitor PXS4728A. Boehringer's primary interest in PXS4728A is directed at the liver related disease Non-Alcoholic Steatohepatitis (NASH). NASH is the progressive form of non-alcoholic fatty liver disease (NAFLD), which is the most common liver disorder in Western industrialized nations with an estimated 30% prevalence in the United States for NAFLD and 3-5% for NASH. NASH is regarded as a major cause of cirrhosis of the liver and is an area of high unmet clinical need. The high prevalence of type 2 diabetes and obesity, which can lead to NASH and other non-alcoholic fatty liver diseases, is expected to make NASH potentially the most common cause of advanced liver disease in coming decades and the market has been estimated to exceed USD 3.5 billion by 2025.

On May 25th, Boehringer Ingelheim announced that it has exercised its option and acquired the PXS4728A, to develop it for the treatment of NASH and to prevent its consequences. Pharmaxis has developed it through to phase I clinical studies, demonstrating oral bioavailability, long-lasting target inhibition and good tolerability and safety. Pharmaxis will receive an upfront payment of EUR 27.5 million (approximately AUD 39 million) and, subject to the continuing successful development and commercialisation of the PXS4728A program, the following payments:

- up to a total of EUR 55 million in development milestone payments tied to the commencement of phase II and III clinical trials

- up to a total of EUR 140 million in regulatory milestone payments upon filing of applications for marketing approval and receipt of regulatory and pricing approvals for a PXS4728A program product in the major pharmaceutical markets (i.e., USA, EU, and China or Japan) for the first indication

- additional milestone payments similar in total to those set forth above upon achievement of the same development and regulatory milestone events by a PXS4728A program product for a second indication

- earn-out payments on annual net sales of PXS4728A program products at tiered percentages starting in the high single digits

- commercialisation milestone payments upon achievement of specified levels of annual net sales of PXS4728A program products

The total deal has therefore a potential value of more than AUD 750 million. It goes without saying that this deal is very positive for Pharmaxis and will bring the company back on the forefront of the Australian biotech sector, which we think is a clear sign of strength and resilience of the company.

Pipeline and Product Portfolio

Pharmaxis has a broad pipeline of products in development, primarily focused on treating respiratory diseases. The target disease states are under-treated and many have broad patient bases. Next to that, the company conducts its own clinical trials as part of the regulatory approvals process for Aridol and Bronchitol. Pharmaxis also supports independent trials of its products to provide further evidence of the efficacy and safety.

Bronchitol: Pharmaxis' lead product in Cystic Fibrosis

Pharmaxis' product is Bronchitol, a drug designed to reduce the amount of mucus build-up in the lungs of patients suffering from chronic respiratory conditions. Pharmaxis has developed Bronchitol primarily for the treatment of cystic fibrosis. Bronchitol is a proprietary formulation of mannitol administered as a dry powder in a convenient hand-held inhaler. Bronchitol hydrates the lungs, helps restore normal lung clearance, and allows patients to clear mucus more effectively. Clinical studies have shown Bronchitol to be effective, and well tolerated in treating patients with cystic fibrosis. Bronchitol is an Australian discovered and developed product, which was approved for marketing in Australia in February 2011 and listed for reimbursement in August 2012. In April 2012 Bronchitol was approved for marketing in the European Union by the European Medicines Agency. In October 2012 Bronchitol was approved by NICE in the UK, opening the door for reimbursement by the National Health Service.

There have been limited therapeutic advances in the past decade to help clear congested lungs for patients with cystic fibrosis, a condition which affects 75,000 people worldwide. Bronchitol has been awarded fast-track status in the U.S., and orphan drug designation in both the US and EU.

End of 2014, the company entered into an exclusive distribution and supply agreement with pharmaceutical company Chiesi Farmaceutici SpA for the commercialisation of Bronchitol in the United States. Under the terms of the agreement, Chiesi is responsible for funding up to USD 22 million of the cost of the phase III clinical trial of Bronchitol.

Aridol: Innovative Lung Function Test

Pharmaxis' product Aridol is an innovative lung function test designed to help doctors diagnose and manage asthma by detecting active airway inflammation through measuring airway hyper-responsiveness. By having patients inhale Aridol via a simple hand-held device, doctors can determine the severity of a patient's inflammation and prescribe the right amount of medication to bring it under control. The Aridol challenge test potentially prevents inappropriate treatment and may help patients better comply with their therapies.

Aridol is approved for sale in Australia, major European countries and South Korea. It is the first and only approved Europe-wide lung function test and the world's first approved indirect challenge test for asthma, a condition which affects 52 millions people worldwide.
Aridol is involved in investigator-sponsored clinical trials worldwide.

Upcoming Milestones

There are a number of key milestones to focus on in the next 6-12 months. A major milestone for 2015H1 has already been achieved:

2015

- Exercise option to acquire PXS4728A by Boehringer Ingelheim ☑
- PXS4728A Phase I results
- Full enrolment CF303
- LOXL2 – lead candidate identified

2016

- LOXL2 – lead candidate into preclinical
- LOXL2 – initiation Phase I
- CF303 – completion trial Phase III Cystic Fibrosis
- CF303 – publication and FDA filing
- At least one Drug discovery program reaches pre clinical valuation point

Analyst: Marcel Wijma MSc

Marcel Wijma, Chief Research Officer and managing partner, has a longstanding history in financial biotech research. After selling Van Leeuwenhoeck Research (VLR) to SNS Securities in 2006, he established an award winning analyst team in biotech/life sciences at SNS Securities. In 2009, Marcel was awarded by Financial Times/Starmine as being one of the Top-3 biotech analysts in Europe. Later that year, Marcel purchased VLR from SNS Securities after which the company was reconstituted. At VLR, he leads the professional VLR research organisation, which is augmented by selected external financial researchers with a specialisation in Life Sciences. Mr. Wijma has a Masters degree in Financial Economics from Erasmus University in Rotterdam.