pharmaxis

An Emerging Powerhouse In Drug Development

Who we are

Pharmaxis is an Australian -based pharmaceutical research company focused on developing innovative treatments for inflammatory and fibrotic diseases with high unmet needs. The company is on track to become a powerhouse in drug development and has already evidenced its scientific approach with two partnerships.

Pharmaxis is a leader in amine oxidase chemistry and mechanism-based inhibitors. It has a proven ability to deliver Phase 2 trial ready compounds and an exciting pipeline of drug candidates for valuable targets. Its management team has strong industry relationships to deliver global transactions with major pharma partners, as well as the preclinical, early and late phase clinical experience to secure successful drug development.

The company had a cash balance of $54 million at 30 June 30 2015, and together with income from already launched drugs has sufficient funding for it to reach significant value milestones from existing partner deals.

Drug discovery strategy

Pharmaxis aims to improve success rates, keep drug discovery costs down and speed up development timelines by focusing on drug targets that have been independently validated in diseases with limited treatment options and where its amine oxidase platform can be utilised.

The first products from the company's amine oxidase platform have been inhibitors of the enzymes semicarbazide-sensitive amine oxidase (SSAO) and lysyl oxidase type 2 (LOXL2). Both of these enzymes are involved in the inflammatory and fibrotic progression of numerous serious chronic diseases.

In May 2015 Pharmaxis sold its Phase 1 SSAO inhibitor PXS-4728A in a significant deal with big pharma. The company's strategy is to take all of its drugs through to at least Phase 1 clinical studies.

Partnerships and Collaborations

In May 2015 pharmaceutical giant Boehringer Ingelheim acquired Pharmaxis' PXS-4728A, primarily for development to treat non-alcoholic steatohepatitis (NASH), a severe, progressive form of fatty liver disease that can lead to scarring and cirrhosis. The deal is potentially worth more than A$750 million in milestone payments to Pharmaxis if the drug is developed and commercialised with high single digit royalties also payable on sales in a market which Deutsche Bank recently estimated could be worth more than US$35 billion by 2025. The transaction was transformational for Pharmaxis and significant globally for a Phase 1 drug. It also provided validation for Pharmaxis' drug platform which is now attracting the interest of other major pharmaceutical companies.

In August 2015 UK-based drug discoverer Synairgen and Pharmaxis commenced a collaboration to develop a LOXL2 therapy to treat pulmonary fibrosis. The collaboration is another demonstration of the international interest in Pharmaxis' drug discovery capability and provides the company with another potential drug from the amine oxidase platform. Under the partnership, Synairgen will fund further development until Phase 1 trials are conducted, when a license partner will be sought.

Pharmaxis has also partnered with pharmaceutical company Chiesi for the commercialisation of cystic fibrosis treatment Bronchitol in the US. The US is the largest market in the world for cystic fibrosis. Chiesi is funding a Phase 3 trial for Bronchitol to meet Food and Drug Administration requirements and will pay Pharmaxis a milestone on commercial launch and then royalties and milestones on sales. Bronchitol is currently on the market in Europe where it is sold via distributors and in Australia.

The diseases we are focused on:

Pharmaxis is targeting the following inflammatory and fibrotic diseases using its amine oxidase platform:

- Non-alcoholic steatohepatitis (NASH)
- Liver fibrosis
- Pulmonary fibrosis
- Cystic fibrosis

Other diseases involving inflammation and fibrosis that are of interest:

- Alzheimer's disease
- Parkinson's disease
- Stroke
- Kidney fibrosis

Pharmaxis' PXS-4728A 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.

NASH is commonly found in people who are overweight or obese. Given the increasing rate of obesity around the world, made worse by sedentary lifestyles and poor food choices, the condition is likely to become a major cause of liver disease and transplantations 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 notoriously difficult to achieve, drug therapies are needed. There are currently no approved therapies available to treat NASH.

Pharmaxis' LOX inhibitor program aims to treat fibrotic diseases by specifically targeting the LOXL2 enzyme which is up regulated in diseased patients and has been found to be responsible for excessive fibrosis by facilitating the cross linking of collagen.

There is considerable interest from large pharmaceutical companies for therapies that treat inflammatory and fibrotic diseases, especially NASH given its prevalence and potential market size. In 2015, Gilead acquired Phenex Pharmaceutical's treatment for NASH that was 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. Also in 2015, Bristol-Myers Squibb entered an agreement for the right to acquire Promedior and its therapy in Phase 2 trials to treat idiopathic pulmonary fibrosis and myelofibrosis for up to $1.25 billion.

Upcoming milestones

2016

- Potential milestone payment on Boehringer Ingelheim beginning Phase 2 trial for PXS-4728A to treat NASH (late 2016/early 2017)
- Complete pre-clinical program on LOXL2 candidate to treat a more severe form of NASH/liver fibrosis
- Complete pre-clinical program with Synairgen on LOXL2 therapy to treat pulmonary fibrosis
- Final patient to complete Chiesi funded cystic fibrosis trial in US and reporting of trial results

2017

- FDA decision on approval of Bronchitol for the US
- Commence Phase 1 trial with Synairgen of LOXL2 therapy for pulmonary fibrosis and partner drug with another pharmaceutical company
- Milestone payment on launch of Bronchitol in US
- Commence Phase 1 trial of LOXL2 candidate to treat more severe form of NASH/liver fibrosis and partner with another pharmaceutical company

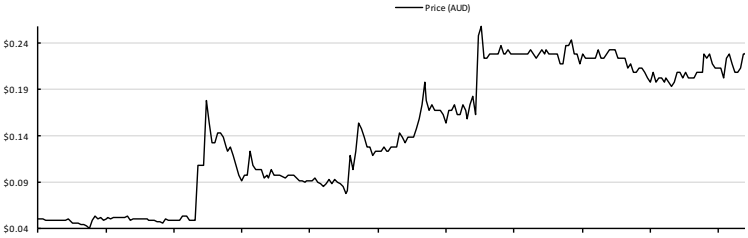
Investor details

Share price at (1 October 2015): A$0.23

Number of shares on issue: 317,126,457

Market cap: A$72.9M

PXS Share Price (2 Oc 2014 - 2 Oct 2015)



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