Pharmaxis Ltd

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Pharmaxis submits Investigational New Drug application for treatment of myelofibrosis

Pharmaxis Ltd (ASX:PXS) (OTCMKTS:PXSLY) (FRA:UUD) has submitted an Investigational New Drug (IND) application to the US Food and Drug Administration (FDA) for a planned phase 1/2 study of PXS-5505 for the treatment of myelofibrosis.

The protocol incorporates a one-month dose-escalation phase followed by six months' treatment in an open label study of patients who are not on a JAK inhibitor.

"Next major step forward"

Chief executive officer Gary Phillips said: "This IND submission incorporates the pre-IND feedback Pharmaxis received from the FDA earlier this year and is the next major step forward in the clinical development program of PXS-5505 for the treatment of myelofibrosis."

The study is planned to commence in the December quarter of 2020 and is expected to conclude in 2022.

Phillips said: "We are leveraging our leadership in lysyl oxidase science to bring new treatment options for these severely underserved patients and strongly believe that our novel approach of inhibiting all of the lysyl oxidase family members could reduce bone marrow fibrosis and have beneficial effects on blood cell production and consequently other aspects of the disease."

IND application inclusions

The IND application includes reports on the phase 1 studies in healthy volunteers completed in the June quarter of 2020 as well as numerous individual studies that characterise the pharmacology, pharmacokinetics, and toxicology of PXS-5505 in a number of animal species, including the effects in various animal models of disease.

Additionally, the application describes the GMP manufacture of the drug substance and drug product to be used in human clinical trials.

FDA feedback on the IND is expected within 30 days and Pharmaxis will outline the final study design and timing at that point.

The CEO added, "After evaluating the safety and efficacy as a monotherapy in this first phase 1/2 study we plan further studies to include myelofibrosis patients being treated with JAK inhibitors which are the existing standard of care for many patients.

"We are also actively exploring how PXS-5505 can be progressed in a number of other fibrotic diseases and cancers, including pancreatic cancer, where we have compelling pre-clinical data."

Price: 0.094

Market Cap: $37.3 m

1 Year Share Price Graph

Share Information

Code:		PXS
Listing:		ASX
52 week	**High**	**Low**
	0.285	**0.053**
Sector:		**Pharma & Biotech**
Website:		**www.pharmaxis.com.au**

Company Synopsis:

Pharmaxis Ltd (ASX:PXS) is a specialty pharmaceutical company focused on the development of new products for the diagnosis and treatment of chronic respiratory and immune disorders.

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What is myelofibrosis?

Myelofibrosis is a disorder in which normal bone marrow tissue is gradually replaced with a fibrous scar-like material. Over time, this leads to progressive bone marrow failure.

Under normal conditions, the bone marrow provides a fine network of fibres on which the stem cells can divide and grow. Specialised cells in the bone marrow known as fibroblasts make these fibres.

In myelofibrosis, chemicals released by high numbers of platelets and abnormal megakaryocytes (platelet forming cells) over-stimulate the fibroblasts. This results in the overgrowth of thick coarse fibres in the bone marrow, which gradually replace normal bone marrow tissue.

Over time this destroys the normal bone marrow environment, preventing the production of adequate numbers of red cells, white cells and platelets.

This results in anaemia, low platelet counts and the production of blood cells in areas outside the bone marrow for example in the spleen and liver, which become enlarged as a result.

The cause of myelofibrosis remains largely unknown and it can occur at any age but is usually diagnosed later in life, between the ages of 60 and 70.

It can be classified as either JAK2 mutation positive (having the JAK2 mutation) or negative (not having the JAK2 mutation).

Pharmaceutical research

Pharmaxis is an Australian pharmaceutical research company and the leading developer of therapeutics targeting lysyl oxidase enzymes to treat fibrosis and cancer.

The company has a highly productive drug discovery engine, drug candidates in clinical trials and significant future cash flows from partnering deals.

Leveraging its small-molecule expertise and proprietary amine oxidase chemistry platform, Pharmaxis has taken four in-house compounds to Phase 1 trials in recent years.

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