
PHARMAXIS ANNOUNCES FIRST RESULTS IN BONE MARROW CANCER TRIAL**FIRST DOSE LEVEL IN PXS-5505 MYELOFIBROSIS STUDY SHOWING STRONG INHIBITION OF TARGET ENZYMES LOX AND LOXL2 IN PATIENTS****DOSING COMMENCED IN SECOND COHORT FOLLOWING SUCCESSFUL RECRUITMENT**

Clinical stage drug development company Pharmaxis Ltd (ASX: PXS) today announced results of data analysis from the first of three stages in its phase 1c clinical trial (MF-101) studying a potential new treatment for the bone marrow cancer myelofibrosis. In addition to the good tolerability and consistent pharmacokinetic properties previously announced, the inhibition of two target enzymes, LOX and LOXL2, were assessed with Pharmaxis proprietary assays and found to be highly statistically significant.

Pharmaxis CEO Gary Phillips said, “We are delighted to see that along with excellent tolerability we are achieving levels of LOX and LOXL2 inhibition in myelofibrosis patients that are already exceeding those levels seen in the preclinical models of myelofibrosis. In these models PXS-5505 caused disease modifying effects with improvements in blood cell count, diminished spleen size and reduced bone marrow fibrosis. Comparing these results with those achieved in the phase 1 study with healthy volunteers, we are now very confident that we will achieve a level of inhibition of the lysyl oxidase enzyme family that allows us to fully test the clinical relevance of these targets in myelofibrosis patients in the upcoming 6-month dose expansion study.”

The second dose cohort of the clinical trial is already fully recruited and dosing of all patients has commenced at participating sites in Australian and South Korean hospitals. Following 28 days on this second dose the safety and pharmacokinetics will be assessed before starting myelofibrosis patients on the final and highest dose. The dose escalation phase of the study will inform the selection of the optimal dose of PXS-5505 to be used in the six-month dose expansion phase 2a (24 patients) to evaluate safety and efficacy. Sites in other countries including the USA are currently being engaged in anticipation of the dose expansion phase commencing recruitment later this year.

The phase 1c/2a trial MF-101 cleared by the FDA under the Investigational New Drug (IND) scheme aims to demonstrate that PXS-5505, the lead asset in Pharmaxis’ drug discovery pipeline, is safe and effective as a monotherapy in myelofibrosis patients who are intolerant, unresponsive or ineligible for treatment with approved JAK inhibitor drugs. An effective pan-LOX inhibitor for myelofibrosis would open a market that is conservatively estimated at US\$1 billion per annum.

While Pharmaxis’ primary focus is the development of PXS-5505 for myelofibrosis, the drug also has potential in several other cancers including liver and pancreatic cancer where it aims to breakdown the fibrotic tissue in the tumour and enhance the effect of chemotherapy treatment.

#ENDS#

SOURCE: Pharmaxis Ltd, Sydney, Australia

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Pharmaxis Ltd is an Australian clinical stage drug development company developing drugs for inflammatory and fibrotic diseases, with a focus on myelofibrosis. The company has a highly productive drug discovery engine built on its expertise in the chemistry of amine oxidase inhibitors, with drug candidates in clinical trials. Pharmaxis has also developed two respiratory products which are approved and supplied in global markets, generating ongoing revenue.

Pharmaxis is developing its drug PXS-5505 for the bone marrow cancer myelofibrosis which causes a build up of scar tissue that leads to loss of production of red and white blood cells and platelets. The US Food and Drug Administration has granted Orphan Drug Designation to PXS-5055 for the treatment of myelofibrosis and permission under an Investigational Drug Application (IND) to progress a phase 1c/2 clinical trial that began recruitment in Q1 2021. PXS-5505 is also being investigated as a potential treatment for other cancers such as liver and pancreatic cancer.

Other drug candidates being developed from Pharmaxis' amine oxidase chemistry platform are targeting fibrotic diseases such as kidney fibrosis, NASH, pulmonary fibrosis and cardiac fibrosis; fibrotic scarring from burns and other trauma; and inflammatory diseases such as Duchenne Muscular Dystrophy.

Pharmaxis has developed two products from its proprietary spray drying technology that are manufactured and exported from its Sydney facility; Bronchitol® for cystic fibrosis, which is approved and marketed in the United States, Europe, Russia and Australia; and Aridol® for the assessment of asthma, which is approved and marketed in the United States, Europe, Australia and Asia.

Pharmaxis is listed on the Australian Securities Exchange (PXS). Its head office, manufacturing and research facilities are in Sydney, Australia. www.pharmaxis.com.au

About PXS-5505

PXS-5505 is an orally taken drug that inhibits the lysyl oxidase family of enzymes, two members LOX and LOXL2 are strongly upregulated in human myelofibrosis. In pre-clinical models of myelofibrosis PXS-5505 reversed the bone marrow fibrosis that drives morbidity and mortality in myelofibrosis and reduced many of the abnormalities associated with this disease. It has already received IND approval and Orphan Drug Designation from the FDA.

About 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.

Myelofibrosis can occur at any age but is usually diagnosed later in life, between the ages of 60 and 70 years. The cause of myelofibrosis remains largely unknown. It can be classified as either JAK2 mutation positive (having the JAK2 mutation) or negative (not having the JAK2 mutation).

Source: Australian Leukemia Foundation: <https://www.leukaemia.org.au/disease-information/myeloproliferative-disorders/types-of-mpn/primary-myelofibrosis/>

Forward-looking statements

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