Media Release

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FOLLOWING FDA REVIEW, PHARMAXIS TO ACCELERATE PLANS FOR PXS-5505 COMBINATION STUDY WITH JAK INHIBITOR IN MYELOFIBROSIS PATIENTS

- FDA PROVIDES FEEDBACK AFTER REVIEW OF INTERIM SAFETY AND EFFICACY DATA FROM ONGOING MONOTHERAPY TRIAL MF-101
- PHARMAXIS PLANS TO COMMENCE TRIAL OF COMBINATION THERAPY WITH MYELOFIBROSIS STANDARD OF CARE TREATMENT IN CY 2023

Clinical stage drug development company Pharmaxis Ltd (ASX: PXS) today announced it will add a combination treatment arm to the current Phase 2 clinical trial of PXS-5505 in myelofibrosis (MF). Following helpful feedback from the U.S. Food and Drug Administration (FDA), the trial will be widened to include myelofibrosis patients already receiving a JAK inhibitor as standard of care in combination with PXS-5505.

Pharmaxis has previously reported interim data from MF-101 in a monotherapy setting demonstrating a well-tolerated drug that leads to stable or improved symptoms, haematological cell counts and fibrosis grades. In a Type C Meeting review, the FDA examined a package of safety and efficacy information from the monotherapy trial and provided guidance on the number of patients, treatment dosage, study duration and endpoints for a study in combination with a JAK inhibitor.

Recruitment for the current study has reached 21 out of a targeted 24 patients on monotherapy with 20 sites active worldwide. Pharmaxis now plans to submit a protocol amendment to global regulators, including the FDA, that will add an arm to the existing MF study MF-101 and utilise its existing trial sites. Based on the FDA feedback, it is anticipated that the trial design can be streamlined to initiate the combination arm at the same dose currently used in the monotherapy arm and commence later this year.

Pharmaxis CEO, Gary Phillips said, “The agreement with the FDA to expand the patient population in the ongoing phase 2 study to include those patients currently on a JAK inhibitor is an important step forward in realising the benefits of lysyl oxidase inhibition for all myelofibrosis patients and in maximising the commercial opportunity for PXS-5505. We are already in discussion with the existing trial site investigators who have welcomed the opportunity to extend the patient population for the study and anticipate significantly accelerated recruitment.”

In recent months Pharmaxis has reported interim data from MF-101, two poster presentations at the American Society of Hematology and the publication of ground breaking pre-clinical data in myelodysplastic syndrome (MDS) in Nature Communications for the Company’s lead asset, PXS-5505. Following a review of its development strategy Pharmaxis has decided to focus its resources on these haematological malignancies and will not at this point be progressing the previously planned study in hepatocellular carcinoma (HCC) patients in an investigator initiated clinical trial by the University of Rochester.

Pharmaxis CEO Gary Phillips said, “Our collaboration with the research team at University of Rochester remains highly valued and their work is continuing with further pre-clinical evaluation of our pipeline assets but for now we have decided not to pursue HCC given the timelines for recruitment and the need to focus our resources.”

Pharmaxis will provide further details of study design, timelines and costs for the PXS-5505 / JAK Inhibitor combination arm of the MF-101 study after it receives feedback from regulators on the amended protocol, expected in Q2 2023.

#ENDS#

SOURCE: Pharmaxis Ltd, Sydney, Australia

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About Pharmaxis

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. The FDA has granted an IND for a phase 1c/2a clinical trial in liver 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 other inflammatory diseases. PXS-4728 is being studied in collaboration with Parkinson's UK as a best in class SSAO/MAOB inhibitor to treat sleep disorders and slow progression of neurodegenerative diseases like Parkinson's by reducing neuroinflammation.

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

Forward-looking statements in this media release include statements regarding our expectations, beliefs, hopes, goals, intentions, initiatives or strategies, including statements regarding the potential of products and drug candidates. All forward-looking statements included in this media release are based upon information available to us as of the date hereof. Actual results, performance or achievements could be significantly different from those expressed in, or implied by, these forward-looking statements. These forward-looking statements are not guarantees or predictions of future results, levels of performance, and involve known and unknown risks, uncertainties and other factors, many of which are beyond our control, and which may cause actual results to differ materially from those expressed in the statements contained in this document. For example, despite our efforts there is no certainty that we will be successful in developing or partnering any of the products in our pipeline on commercially acceptable terms, in a timely fashion or at all. Except as required by law we undertake no obligation to update these forward-looking statements as a result of new information, future events or otherwise.