# ASX Announcement

**10 June 2021**

## PHARMAXIS ANNOUNCES PROGRESSION OF CLINICAL TRIAL IN BONE MARROW CANCER

### PXS-5505 MULTINATIONAL TRIAL IN MYELOFIBROSIS CLEARED BY SAFETY COMMITTEE TO ADVANCE TO SECOND STAGE OF DOSE ESCALATION STUDY

Clinical stage drug development company Pharmaxis Ltd (ASX: PXS) today announced it has completed dosing the first of three stages in its phase 1c clinical trial (MF-101) studying a potential new treatment for the bone marrow cancer myelofibrosis. The study's safety monitoring committee that advises on the study progress, has given the green light to progress to the second dose level after reviewing factors including the safety and pharmacokinetic properties of PXS-5505 in myelofibrosis patients.

Participating Australian and South Korean hospitals will shortly commence dosing of the second dose level for a treatment period of 28 days. The dose escalation phase of the study will inform the selection of the optimum dose of PXS-5505 to be used in the six-month dose expansion phase (24 patients) to evaluate safety and efficacy. Sites in other countries including the USA are currently being approached in anticipation of the dose expansion phase commencing recruitment later this year.

Pharmaxis CEO Gary Phillips said, "PXS-5505 has demonstrated good tolerability and very consistent pharmacokinetic properties in the first dose escalation stage of MF-101. This is very much in line with the results from our phase 1 healthy volunteer study reported last year. Further data from this first dose will be available in coming weeks but based on the pharmacokinetic profile I anticipate that we will see significant inhibition of all lysyl oxidase enzymes even at this lowest level of dose. We look forward to seeing the data from the next two dose cohorts in 2H 2021 and delivering safety and efficacy data from the 6-month dose expansion study by the end of next year."

PXS-5505 is an orally taken drug that inhibits the lysyl oxidase family of enzymes. 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 for the treatment of myelofibrosis from the FDA.

The phase 1c/2a trial 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.

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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## About Pharmaxis

Pharmaxis Ltd is an Australian pharmaceutical research 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 is scheduled to begin 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 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.