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Media Release

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PHARMAXIS LAUNCHES NEW INTERNATIONAL CLINICAL TRIAL SITES IN PHASE 2 BONE MARROW CANCER TRIAL

PXS-5505 MAINTAINING GOOD TOLERABILITY PROFILE IN PATIENTS ALREADY TREATED

Clinical stage drug development company Pharmaxis Ltd (ASX: PXS) today announced that it has initiated the first of 5 new trial sites in Taiwan as it progresses the phase 2 clinical trial of its drug PXS-5505 in patients with the bone marrow cancer myelofibrosis. These latest centres join 11 active sites across Australia and South Korea with a further 4 centres in the US expected to come on stream in coming months.

Pharmaxis CEO Gary Phillips said, "This planned initiation of new trial sites in Taiwan will bring the total number of active sites to 16. The study is making pleasing progress as it builds to a recruitment target of 24 patients. This latest expansion of sites is part of a focused effort by the company to bring patients into the trial and deliver results by the year end."

Mr Phillips added, "We know from the earlier dose escalation study that PXS-5505 will fully block the LOX enzymes that play a fundamental part in the bone marrow fibrosis that characterises myelofibrosis, giving hope that the drug can modify the course of the disease. The good tolerability profile, which is a key differentiator from current standard of care, was first demonstrated in healthy volunteers and then backed up by myelofibrosis patients on 1 month of therapy in the dose escalation study. I am pleased to report that we now have experience with patients who have had 3 or more months of therapy on PXS-5505 and that the good tolerability profile is being maintained."

The phase 2 trial known as MF-101 was cleared by the FDA under the Investigational New Drug (IND) scheme and 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

AUTHORISED FOR RELEASE TO ASX BY:

Pharmaxis Ltd Disclosure Committee. Contact: David McGarvey, Chief Financial Officer and Company Secretary: T +61 2 9454 7203, E david.mcgarvey@pharmaxis.com.au

CONTACT:
Media: Felicity Moffatt: T +61 418 677 701, E felicity.moffatt@pharmaxis.com.au
Investor relations: Rudi Michelson (Monsoon Communications) T +61 411 402 737, E rudim@monsoon.com.au

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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.

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.