Media Release

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PHARMAXIS CANCER DRUG PXS-5505 PROFILED IN TWO POSTERS AT AMERICAN SOCIETY OF HEMATOLOGY CONFERENCE

- **Further analysis of interim data from 6 patients in phase 2 myelofibrosis study; PXS-5505 continues to exhibit an excellent safety profile with encouraging signs of clinical activity in patients ineligible for a JAK inhibitor.**
- **Pharmaxis to discuss next steps in clinical development of PXS-5505 for myelofibrosis with FDA in Q1 2023.**
- **Potential application for PXS-5505 in myelodysplastic syndrome highlighted by promising data released from pre-clinical studies of bone marrow samples from patients.**

Clinical stage drug development company Pharmaxis Ltd (ASX: PXS) reports that two scientific posters have been presented at the American Society of Hematology conference (ASH) in New Orleans providing clinical evidence on the disease modifying effect of its pan LOX inhibitor PXS-5505 in bone marrow cancer, and pre-clinical evidence in other myeloid neoplasms.

Poster 1: Interim data from the ongoing phase 2 myelofibrosis study (MF-101)

The poster reinforced the conclusions reported in October for the first 6 patients to have completed 6 months' treatment.

- PXS-5505 is well tolerated and the clinical responses support continued investigation in myelofibrosis with no dose limiting toxicity seen and preliminary indication of disease stabilisation.
- PXS-5505 demonstrates potent and sustained inhibition of lysyl oxidases in patients with 5 out of 6 patients either stable or showing improved bone marrow fibrosis scores of ≥1 grade.
- Results are encouraging given the poor prognosis seen after ruxolitinib discontinuation with a median overall survival of only 11-14 months typical of this study population.

The poster further detailed the hematological response to PXS-5505 for these 6 patients:

- 5 had stable/improved hemoglobin counts including one patient that had an anemia response (Hgb increase >20 g/L) at week 18 with no red blood cell transfusions (Figure 1)
- 5 had stable/improved platelet counts over 24 weeks' therapy (Figure 2)

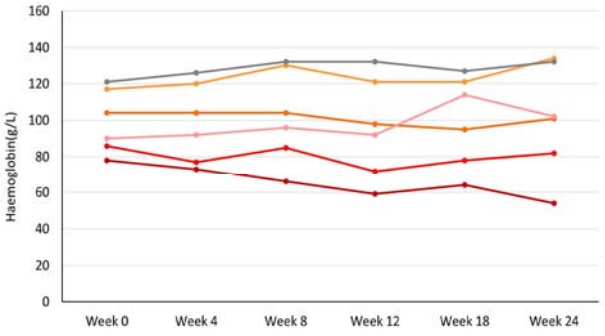

Fig 1: Hemoglobin (g/L) concentration in patients by study visit

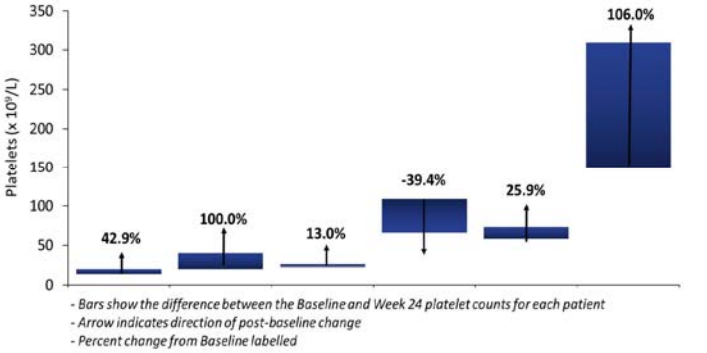

Fig 2: Platelet counts change from baseline at week 24 (by patient)

Poster 2: Inhibition of Lysyl Oxidases Synergizes with 5-Azacytidine to Restore Erythropoiesis in Myeloid Neoplasms

Given the limited response rates and frequent relapses during currently approved drug treatments in myeloid neoplasms, an urgent unmet need exists in diseases such as myelodysplastic syndrome. The altered bone marrow niche in myeloid neoplasms is increasingly being recognized as a therapeutic target where upregulated lysyl oxidase activity contributes to the excessive deposition of extracellular matrix proteins.

This poster reports on ground breaking work done in collaboration with Professor Wolf-Karsten Hofmann and Professor Daniel Nowak at Heidelberg University, Germany. The full results will be the subject of a future publication.

The American Society of Hematology conference in New Orleans is one of the premier gatherings of scientists and clinicians in this field of research and this year was attended by more than 20,000 people.

Gary Phillips, Pharmaxis CEO said, "It is very encouraging to see the many positive responses at ASH to the pioneering work Pharmaxis has been conducting in the role of lysyl oxidase in a variety of diseases. For our pan-LOX inhibitor, PXS-5505, there is increasing data that we have a safe and well tolerated drug achieving high target engagement and with the potential to make a real difference to patients, not only in myelofibrosis but also other myeloid neoplasms.

"Based on the ongoing data from MF-101 we will schedule discussions with the FDA in Q1 2023 on the next steps of clinical development for PXS-5505 in myelofibrosis."

While Pharmaxis' primary focus is the development of PXS-5505 for myeloid neoplasms such as myelofibrosis and myelodysplastic syndrome, the drug also has potential in several other cancers including liver and pancreatic cancer where it aims to break down the fibrotic tissue in tumours and enhance chemotherapy treatment. An investigator led phase 1c study in newly diagnosed hepatocellular cancer patients, where PXS-5505 will be used in addition to immunotherapy standard of care, is open for recruitment at Rochester University New York.

#ENDS#

See MF-101 poster here.

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.