
**PHARMAXIS ANNOUNCES FIRST PATIENT ENROLLED IN
CLINICAL TRIAL FOR NEW CANCER TREATMENT**

PXS-5505 STUDY IN MYELOFIBROSIS OPENS AT AUSTRALIAN AND INTERNATIONAL SITES

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced it has enrolled the first patient in a clinical trial studying a potential new treatment for the bone marrow cancer myelofibrosis.

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.

Pharmaxis has completed site initiation at several Australian and South Korean hospitals and the first patient has been enrolled. The dose escalation phase of the study that aims to select the optimum dose of PXS-5505. This first phase, that will recruit up to 18 patients, is expected to conclude and report in 2H 2021 and will be followed by a six-month dose expansion phase (24 patients) to evaluate safety and efficacy. Sites in other countries including the USA will be added for the dose expansion phase.

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.

Pharmaxis CEO Gary Phillips said, "PXS-5505 has demonstrated good tolerability and highly effective inhibition of the enzyme in phase 1 studies. Its potential to modify the course of the disease by directly targeting bone marrow fibrosis will make PXS-5505 an ideal monotherapy or adjunct to approved therapies in this indication. There remains a high level of unmet need in myelofibrosis and many other drugs in development have challenging side effect profiles."

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.

Trial Design	
Name of trial	PXS5505-MF-101: A phase 1/2a study to evaluate safety, pharmacokinetic and pharmacodynamic dose escalation and expansion study of PXS-5505 in patients with primary, post-polycythaemia vera or post-essential thrombocythemia myelofibrosis
Trial number	NCT04676529
Primary endpoint	To determine the safety of PXS-5505 in patients with myelofibrosis
Secondary endpoints	<ul style="list-style-type: none"> • Determine appropriate therapeutic dose • Characterize pharmacokinetic and pharmacodynamic parameters • Determine reduction in bone marrow fibrosis • Determine response rates as defined by International Working Group (IWG)- Myeloproliferative Neoplasms Research and Treatment criteria • Evaluate efficacy of PXS-5505 in spleen size reduction measured by CT or MRI scan • Evaluate the efficacy of PXS-5505 on MF related symptoms based on MF-SAF scores (Myelofibrosis Symptom Assessment Form)
Blinding status	Open label
Placebo controlled	No
Trial design	Randomised, multicentre, 4 week duration phase 1 (dose escalation) followed by 6 month phase 2 (dose expansion)
Treatment route	Oral
Treatment frequency	Twice daily
Dose level	Dose escalation: three escalating doses Dose expansion: one dose
Number of subjects	Dose escalation: minimum of three patients to maximum of 18 patients Dose expansion: 24 patients
Subject selection criteria	Patients with primary or secondary myelofibrosis who are intolerant, unresponsive or ineligible for treatment with approved JAK inhibitor drugs
Trial locations	Dose escalation: Australia (2 sites) and South Korea (4 sites) Dose expansion: Australia, Korea, USA
Commercial partners involved	No commercial partner

#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 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 commenced 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.