ASX Announcement

2 November 2020

PHARMAXIS PRIORITISES BREAKTHROUGH CLINICAL PROGRAM ON MYELOFIBROSIS

PXS-5505 STUDY TO RECRUIT 42 PATIENTS AT AUSTRALIAN AND INTERNATIONAL SITES

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced details of the clinical development plan for its lead drug pipeline asset being studied for the bone marrow cancer myelofibrosis.

PXS-5505 has already received IND approval and Orphan Drug Designation from the FDA and will proceed to an open label study recruiting up to 42 patients with myelofibrosis in Australia and international sites. PXS-5505 is a pan-LOX inhibitor that seeks to reverse the bone marrow fibrosis that drives morbidity and mortality in the disease.

Pharmaxis will recruit myelofibrosis patients who are intolerant, unresponsive or ineligible for treatment with approved JAK inhibitor drugs in two stages; a dose escalation phase to select the optimum dose followed by a six-month dose expansion phase (24 patients) to evaluate safety and efficacy. Preparation for the dose escalation phase is well advanced and will include sites in Australia and South Korea. The first patient is expected to be recruited in Q1 2021. Follow up studies are planned to look at safety and efficacy in combination with JAK inhibitors which are the current standard of care.

Pharmaxis CEO Gary Phillips said, "Phase 1 studies have already evidenced that PXS-5505 has a good safety profile. Good tolerability allied to the potential to modify the course of the disease by directly targeting bone fibrosis will make PXS-5505 an ideal monotherapy or adjunct to approved therapies in this indication where there remains a high level of unmet need and where many other drugs in development have challenging side effect profiles."

Dr Gabriela Hobbs, Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital said, "JAK inhibition alone is insufficient in the treatment of patients with myelofibrosis; it is not associated with changes in underlying disease biology and it can worsen blood counts, leading to high drug discontinuation rates over time. The trial utilizing PX-5505 is supported by a sound scientific rationale, based on pre-clinical work demonstrating the importance of lysyl oxidase in the development of myelofibrosis. PXS-5505 has a unique mechanism of action that has the potential for disease modification. I am looking forward to seeing the effect of this drug in clinical trials."

Separately, Pharmaxis announced today that its US licensee Chiesi Farmaceutici SpA (Chiesi) had received approval from the Food and Drug Administration (FDA) for Pharmaxis developed drug Bronchitol® (mannitol) in cystic fibrosis ("FDA Approves Bronchitol for US Market"). US$10 million in approval and launch milestones give Pharmaxis cash of A$34 million proforma[1] as of June 2020 which, together with the contribution from the mannitol business, provides a cash runway that covers development of PXS-5505 to conclusion of its phase 2 trial for the treatment of myelofibrosis.

Pharmaxis CEO Gary Phillips said, "The US approval of Bronchitol, associated milestone payments and positive cash flow from our mannitol business allow us to move confidently ahead with the

development of PXS-5505 in myelofibrosis which has been fast tracked from patent filing through long term tox studies and phase 1 trials to a phase 1c/2 trial start within two years."

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 cancers where it aims to breakdown the fibrotic tissue in the tumour and enhance the effect of existing chemotherapy.

Mr Phillips commented, "We have been very encouraged by the enthusiastic response of scientists and clinicians globally to our pan-LOX inhibitor program. The role of LOX enzymes in cancer is the subject of an increasing number of academic publications that that can now be tested in the clinic with PXS-5505. We are engaged in numerous collaborations that aim to widen the therapeutic uses of PXS-5505 through grant supported independent investigator-initiated studies."

Pharmaxis also provided an update on the other candidates in its amine oxidase pipeline. Partnering activities on its LOXL2 inhibitor (PXS-5382) are continuing with ongoing discussions of commercial terms and scientific diligence with several parties. In addition, other assets are being progressed with the aid of independent investigator studies and grants.

The Company will host an investor conference call at 11.00am this morning (Sydney time) in relation to today's announcements. A separate announcement will provide access details. A recording of the call will be available later today on the Pharmaxis website.

(1) Proforma cash at June 2020 is calculated as follows: $15m cash at 30 June 2020 plus A$5m R&D tax credit received 14 October 2020 plus A$14m Chiesi milestone payments.

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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.