Media Release

9 November 2021

FDA CLEARS PHARMAXIS CANCER DRUG TO PROGRESS TO PHASE 2 STUDY IN LIVER CANCER

IND APPROVED TO TRIAL PXS-5505 ADDED TO CURRENT STANDARD OF CARE IN NEWLY DIAGNOSED UNRESECTABLE HEPATOCELLULAR CARCINOMA PATIENTS

UNIVERSITY OF ROCHESTER MEDICAL CENTER AND PHARMAXIS IN DISCUSSIONS TO COMMENCE INVESTIGATOR-LED STUDY IN 2022

Clinical stage drug development company Pharmaxis Ltd (ASX: PXS) today announced that an Investigational New Drug application (IND) for a trial of PXS-5505 in hepatocellular carcinoma (HCC) patients has been cleared by the United States Food and Drug Administration (FDA). The IND was submitted by the University of Rochester Medical Center, New York State, following the positive pre-clinical results reported in August 2021 at the Americas Hepato-Pancreato-Biliary Association conference in Miami, USA. The trial design approved by the FDA calls for PXS-5505 to be added to current chemotherapy standard of care; combination of a PD-L1 inhibitor and an anti-VEGF drug as first line therapy in newly diagnosed patients with unresectable HCC carcinoma.

Primary liver malignancies have doubled in incidence over the last two decades. These malignancies are now the 4th leading cause of cancer-related mortality worldwide with a 19.6% 5-year relative survival rates. Currently, just 20-30% HCC are resectable at presentation with many patients relying on chemotherapy. A prominent feature of HCC is the presence of highly fibrotic tissue that increases tumour stiffness, and decreases access of drugs into the tumour. Under the guidance of Dr. Roberto Hernandez-Alejandro, MD (Chief Division of Transplantation / Hepatobiliary Surgery), the research team at the University of Rochester Medical Center, New York State, have been investigating the role of lysyl oxidase enzymes in liver cancer and whether Pharmaxis' cancer drug PXS-5505 can improve the efficacy of current chemotherapy drugs by inhibiting these enzymes.

Dr. Roberto Hernandez-Alejandro said, "At the University of Rochester Wilmot Cancer Center, we are excited about the prospect of combining PXS-5505 with standard first line therapy for our unresectable hepatocellular carcinoma patients. The incidence of hepatocellular carcinoma is rising in part due to increasing incidence of cirrhosis and non-alcoholic steatohepatitis. Beyond resection, effective systemic therapies for this disease are lacking, thus new treatment regimens are of significant clinical need."

Dr. Nabeel Badri, (Wilmot Cancer Institute, University of Rochester) added, "PXS-5505 is a potent inhibitor of lysyl oxidase, a key enzyme in collagen crosslinking. By inhibiting the formation of fibrotic tissue in the tumor we hope to improve delivery and effectiveness of immunotherapy drugs which have so far had a limited impact of the survival of our patients. Through preclinical testing and translational research, we have developed a promising clinical trial design that has the potential to benefit these patients and improve our understanding of hepatocellular carcinoma."

The IND submitted by Rochester referenced the previous successful IND lodged by Pharmaxis for the ongoing phase 2 trial of PXS-5505 in myelofibrosis. The approved trial design envisages a dose escalation stage where the safety of PXS-5505 in combination with a PD-L1 inhibitor and an anti-VEGF drug will be assessed at several different doses as well as measures designed to explore the impact of PXS-5505 on fibrosis and drug perfusion. This will be followed by a 6-month trial of the selected dose with both safety and efficacy endpoints.

Pharmaxis CEO Gary Phillips said, "We highly value our collaboration with the research team at University of Rochester. The rapid progression from the compelling pre-clinical work presented for the first time in August to a successful IND submission is very encouraging and we look forward to

concluding arrangements for the commencement of the dose escalation study in 2022. PXS-5505 has recently progressed to a phase 2 clinical trial looking for evidence of disease modifying effects in the bone cancer myelofibrosis as a monotherapy so exploring its additional potential to address cancers where fibrosis is limiting the clinical benefit of current chemotherapy- such as liver and pancreatic cancer- would be significantly value adding."

#ENDS#

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.

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 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.

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.