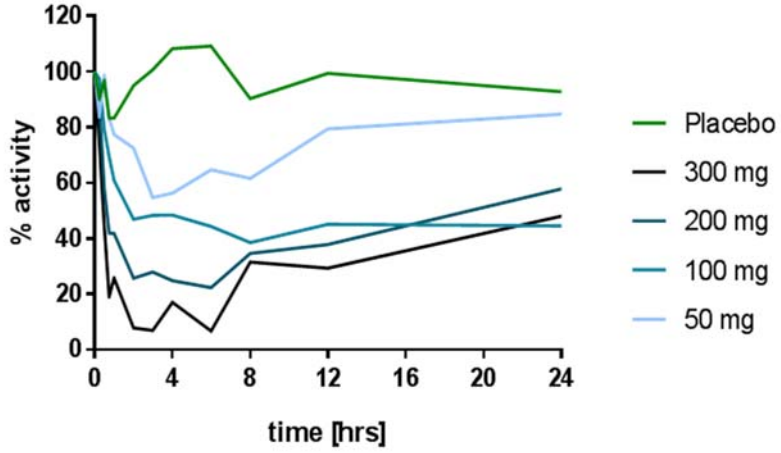
Media Release

24 October 2019

PHARMAXIS CANCER DRUG PROGRESSING IN CLINIC
ORAL PAN-LOX INHIBITOR NOW IN
PHASE 1B FOLLOWING POSITIVE PHASE 1A DATA

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced that following positive clinical Phase 1A study results, it has begun dosing healthy subjects in the second stage of its study of an oral anti-fibrotic Lysyl Oxidase (LOX) inhibitor. This drug is being targeted to treat cancers including myelofibrosis and pancreatic cancer.

The double-blind placebo- controlled study commenced in February 2019 and consists of two stages. The first single ascending dose stage (phase 1A) was conducted in 40 healthy subjects divided into five groups with each taking a different single oral dose or placebo. The drug was well tolerated and no safety signals were identified during the study. Importantly for potential clinical benefit, the data showed a drug with good pharmacokinetics and a dose related inhibition of the LOX family of enzymes with the upper doses causing significant inhibition for a full 24 hours after a single application.



Dose dependent reduction in LOX activity in plasma

The second multiple ascending dose stage (phase 1B) commenced on 15 October in 16 healthy subjects divided into two groups with each group receiving a different dose or placebo daily for 14 days. The clinical trial is due to report in Q1 2020.

Pharmaxis CEO Gary Phillips said, “The second part of this study was originally due to report mid-year but we delayed it while we completed 3-month toxicity testing to further de-risk the drug. This means that with successful completion of this phase 1 study we will have all the data required to support the commencement of clinical proof of concept studies in either myelofibrosis or pancreatic cancer. Pharmaxis plans to discuss this program with the FDA in coming months prior to filing an IND that supports entering phase 2 studies in 2H 2020.”

“Our expertise in the biology and chemistry of lysyl oxidase inhibitors, including the LOX and LOXL2 programs has been featured in a number of international conferences and publications in recent months. We have developed proprietary technology to measure LOX and its related family of enzymes in both animal models and human plasma that enables us to clearly understand the

role these enzymes have in fibrotic disease. This will significantly aid patient selection and proof-of-mechanism in the upcoming phase 2 trials."

The program compound is an oral once-a-day drug that inhibits all lysyl oxidase family members (LOX, LOXL1, 2, 3 & 4). The compound successfully cleared pre-clinical safety and 3-month toxicity studies and has shown significant reductions in fibrosis in *in-vivo* models of kidney, lung and liver fibrosis as well as myelofibrosis and pancreatic cancer. It is suited to the treatment of severe fibrosis as well as cancer with prominent stroma (connective tissue) or fibrotic metastatic niches. Pharmaxis is evaluating plans to develop the compound for myelofibrosis and pancreatic cancer where there is a clear unmet need and the possibility of an anti-fibrotic adding to the limited efficacy seen with current standard of care.

#ENDS#

SOURCE: Pharmaxis Ltd, Sydney, Australia

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About Pharmaxis

Pharmaxis Limited is an Australian pharmaceutical research company and a global leader in drug development for inflammation and fibrotic diseases. The company has a highly productive drug discovery engine, drug candidates in clinical trials and significant future cash flows from partnering deals.

Leveraging its small-molecule expertise and proprietary amine oxidase chemistry platform, Pharmaxis has taken four in-house compounds to Phase 1 trials in just five years. Global pharmaceutical company Boehringer Ingelheim acquired the Pharmaxis anti-inflammatory AOC3 inhibitor in 2015 and is developing it (BI 1467335) for two diseases: the liver condition Non-alcoholic Steatohepatitis (NASH) and diabetic retinopathy (DR). Total potential milestone payments to Pharmaxis from these programs is €419 million ($625 million).

The company's successor amine oxidase program has developed an oral anti-fibrotic LOXL2 inhibitor, aimed at NASH, pulmonary fibrosis (IPF) and other high-value fibrotic heart and kidney diseases, with a commercial partnering process underway. Two further new drugs from the same program are expected to begin proof-of-efficacy trials in 2020. Pharmaxis' Mannitol platform has yielded the products Bronchitol® for cystic fibrosis, which is marketed in Europe, Russia and Australia, with United States FDA approval pending; and Aridol® for the assessment of asthma, which is sold 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. http://www.pharmaxis.com.au/

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 partnering our LOXL2 program or any of the other 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.