Media Release

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PHARMAXIS SCIENCE UPDATE OUTLINES SCOPE FOR ANTI-FIBROTIC PIPELINE UNDERPINNED BY UNIQUE ASSAY TECHNOLOGY

STRONG COMPETITIVE PROFILE

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today provided an update on the company's anti-fibrotic drug pipeline highlighting both the therapeutic areas of interest and competitive positioning of its LOXL2 and pan LOX inhibitors.

Speaking at the 2019 AusBiotech conference in Melbourne, Australia, Pharmaxis CEO Gary Phillips outlined the steps Pharmaxis has taken to develop a best in class status for its Lysyl oxidase like 2 (LOXL2) inhibitors and strong pre-clinical data in myelofibrosis for its pan-LOX inhibitor PXS-5505.

Pharmaxis has recently presented data at scientific conferences[1] and in scientific publications[2a] detailing its unique proprietary assays that, for the first time, allow all of the LOX enzyme family to be measured in serum and tissue, even at very low levels. These data demonstrate that LOXL2 in particular has a relatively fast turnover in human serum and suggests that an effective drug will need to both penetrate fibrotic tissue and be present in concentrations high enough to inhibit LOXL2 at all times. Pharmaxis has used this unique assay technology in the development and assessment of all its current LOX and LOXL2 inhibitors.

Mr Phillips said, "Whilst there are several excellent publications demonstrating the increase in LOXL2 enzyme levels in patients with fibrotic diseases of the lung, liver, kidney, heart, and some cancers, the importance of LOXL2 has been put in doubt by the failure of the one competitive LOXL2 inhibitor drug to have been trialled in patients; Gilead's LOXL2 antibody simtuzumab. Gilead commented in the publication of its failed IPF study that their lack of an assay for LOXL2 inhibition limited their ability to confirm adequate target inhibition.

"The risk sensitivities of potential commercial partners for our LOXL2 inhibitor program were understandably heightened by the historic simtuzumab clinical failures but importantly, the work Pharmaxis has undertaken in assay sensitivity has provided very reassuring data. Over the course of this year, we have been able to reanalyse samples from existing studies and conduct new pre-clinical studies that have clearly shown the link between LOXL2 inhibition in diseased organs, a reduction in collagen crosslinking which is a hallmark of fibrosis and clinical effect as measured by the area of fibrosis. This data has energised the partnering process by underlining the relevance of LOXL2 and the superiority of our compounds. We are currently pursuing a number of different partnering options to enable this drug to commence phase 2 efficacy studies in patients with IPF or NASH."

Pharmaxis also has two separate programs for inhibitors that block all enzymes in the LOX family (LOX, LOXL1-4). Pharmaxis has supported a number of academic centres of excellence with supplies of drugs from these programs to use in pre-clinical studies. A recent publication[2b] has reported that two Pharmaxis compounds have significantly decreased the bone marrow fibrotic burden in two different models of primary myelofibrosis (PMF) which is a chronic myeloproliferative cancer with a poor prognosis and limited therapeutic options. Currently, only allogeneic stem cell transplantation is curative in those who are candidates, while administration of the JAK1/2 inhibitor ruxolitinib carries a risk of worsening cytopenia.

Mr Phillips commented, "We have been very encouraged by the feedback we have received from both academic and clinical thought leaders about the use of the Pharmaxis LOX inhibitor PXS-5505 in patients with PMF as an adjunct to existing standard of care and as a monotherapy. We are pressing ahead with plans to complete the phase 1b study that started earlier this month, engage with the FDA and commence patient studies in H2 2020."

The authors[2b] noted that LOX is an enzyme vital for collagen cross-linking and extracellular matrix stiffening and found to be upregulated in PMF. They concluded based on their multiple studies that the Pharmaxis compounds appear to be promising new candidates for the treatment of fibrosis in PMF. In addition, research efforts are ongoing in other centres of excellence to validate Pharmaxis' pan-LOX inhibitor for the treatment of pancreatic cancer.

REFERENCES:

1. Conferences:
   a. 3rd Annual IPF Summit; San Diego, August 27-29, 2019
   b. 14th World Congress on Inflammation; Sydney, September 15-19, 2019
   c. AusBiotech Annual Conference; Melbourne October 29-31, 2019 (available here)
2. Publications:
   a. "Identification and Optimization of Mechanism-Based Fluoroallylamine Inhibitors of Lysyl Oxidase-like 2/3" https://pubs.acs.org/doi/pdf/10.1021/acs.jmedchem.9b01283
   b. "Novel lysyl oxidase inhibitors attenuate hallmarks of primary myelofibrosis in mice" https://link.springer.com/article/10.1007%2Fs12185-019-02751-6

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About Pharmaxis

Pharmaxis Ltd 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.