

## Media Release

11 October 2018

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### PHARMAXIS RELEASES POSITIVE RESULTS OF PHASE 1 CLINICAL TRIAL FOR LOXL2 INHIBITOR COMPOUND

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Pharmaceutical company Pharmaxis (ASX: PXS) today announced positive results from the Phase 1 clinical trial for the first of its Lysyl Oxidase Like 2 (LOXL2) inhibitor compounds being developed to treat fibrotic diseases such as Non-Alcoholic Steatohepatitis (NASH) and Idiopathic Pulmonary Fibrosis (IPF).

The double-blind placebo controlled study consisted of two stages. The first single ascending dose stage was conducted in 48 healthy subjects divided into six groups with each taking a single dose ranging from 10mg to 400mg or placebo. The second multiple ascending dose stage was conducted in 24 healthy subjects divided into three groups which each received a single daily dose ranging from 100mg to 400mg or placebo for 14 days.

The excellent drug like properties demonstrated in earlier pre-clinical testing were confirmed. There were no adverse safety findings in either the first or second stages of the study and the pharmacokinetic profile showed the expected dose related increases in exposure.

In addition to studying the safety and pharmacokinetic profile, the clinical trial also investigated the degree to which the drug can inhibit the target enzyme LOXL2 which is implicated in several different fibrotic diseases. Importantly, Pharmaxis has been able to demonstrate a large and highly significant inhibition of this enzyme in blood serum for a full 24 hours from a single dose and that daily dosing over a 14-day period now meets our targeted effect of greater than 80% inhibition at the 400mg dose.

Pharmaxis CEO Gary Phillips said, "I'm delighted that the excellent pharmacokinetic parameters and the significant and long lasting inhibition of the target LOXL2 enzyme demonstrated in the single dose stage of the study earlier this year completely translated into the profile we have seen in the multiple dosing study. This drug profile has led to increased interest from major pharmaceutical companies looking for good anti fibrotic programs to acquire. Today's announcement that enzyme inhibition is further enhanced after daily dosing over 14 days goes a long way to completing the data package on which we will base continuing scientific and commercial discussions with potential partners during the current quarter."

The Phase 1 trial for a second Pharmaxis LOXL2 compound being studied has recently completed dosing and will report in the current quarter.

The company's LOXL2 program compounds are highly selective small molecule inhibitors of LOXL2 that can be administered orally. The ongoing pre-clinical development program supports the potential of both compounds to treat fibrotic disease in several organs. This support has been enhanced by recent breakthroughs in Pharmaxis proprietary assay technology that have demonstrated target engagement in animal tissue from the pre-clinical studies as well as serum. 28-day animal toxicity studies have been completed in two species for both compounds and the remaining 3 month studies are due to be completed later this quarter.

Pharmaxis has previously announced its intention to partner the LOXL2 program after phase 1 studies are complete. Mr Phillips said, “We believe that the best in class LOXL2 inhibition and the availability of two compounds with differentiated pharmacokinetic profiles make this program very attractive and we look forward to concluding a licensing deal with a partner committed to develop the compounds in indications where there remain a lack of treatment options and significant commercial opportunities.

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#### **About Pharmaxis**

Pharmaxis (ACN 082 811 630) is an Australian pharmaceutical research company focused on inflammation and fibrosis with a portfolio of products at various stages of development and approval. Its product Bronchitol® for cystic fibrosis is marketed in Europe, Russia and Australia. Its product Aridol® for the assessment of asthma is sold in Europe, Australia and Asia. The company’s development pipeline is centred on its expertise in amine oxidase chemistry and includes a series of Lysyl Oxidase Inhibitors under clinical development targeting fibrotic diseases of the heart, kidney, liver and lung. In May 2015, Boehringer Ingelheim acquired the Pharmaxis investigational drug PXS-4728A, a potent inhibitor of Semicarbazide-Sensitive Amine Oxidase (SSAO), to develop it for the treatment of the liver-related condition Non-alcoholic Steatohepatitis (NASH) and other inflammatory diseases. Pharmaxis is listed on the Australian Securities Exchange (symbol PXS). The company’s head office, manufacturing and research facilities are located in Sydney, Australia. For more information about Pharmaxis, please see [www.pharmaxis.com.au](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. 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.