



## Media Release

15 November 2018

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

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Pharmaceutical company Pharmaxis (ASX: PXS) today announced positive results from the Phase 1 clinical trial for the second 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 5mg to 200mg 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 of either 50mg, 100mg, 200mg or placebo for 14 days.

Repeating the positive results seen in the Phase 1 trial of the first inhibitor compound announced on 10 October 2018, 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.

Significant target engagement of the LOXL2 enzyme by both compounds has now been demonstrated in blood serum for a full 24 hours from a single dose over a 14-day period, with the second compound achieving more than 85% inhibition over 24 hours from a 100mg daily dose, achieving the target for this program.

Pharmaxis CEO Gary Phillips said, "Several large pharma companies are interested in the Pharmaxis program where both of our LOXL2 inhibitors have now successfully completed phase 1 studies and demonstrated a best in class profile with 24-hour inhibition of the target enzyme from a single daily dose. In a further significant scientific advancement we have also managed to underline the relevance of the program to potential partners by using our proprietary research tools to confirm that our compounds directly inhibit the activity of the raised levels of LOXL2 seen in diseased tissue from NASH and IPF animal models."

Phillips continued, "The only remaining elements necessary to finalise the data package that companies are now conducting diligence on, are the 3-month toxicity studies on both compounds which are due to report later this quarter. This is slightly delayed versus initial expectations due to the availability of time slots at the contract organisations we use but successful 3-month studies will significantly enhance the LOXL2 program. Following the completion of the data package, Pharmaxis intends to conduct a final series of scientific briefings to potential partners before moving to commercial partnering discussions to secure a comprehensive licensing agreement in 2019."

The Company's LOXL2 program compounds are highly selective small molecule inhibitors of LOXL2 that can be administered orally and the soon to be completed pre-clinical development program supports the potential of both compounds to treat fibrotic disease in one or more organs.

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**SOURCE:** Pharmaxis Ltd, Sydney, Australia

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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.