

Media Release

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PHARMAXIS BUSINESS PLAN RESOURCED TO COMPLETE PARTNERING AND CLINICAL DEVELOPMENT MILESTONES

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today provided an update on its business plan following a decision by Boehringer Ingelheim (BI) not to progress studies of the AOC3 inhibitor acquired from Pharmaxis in 2015 for the treatment of NASH. The reason provided by BI for the discontinuation after a successful phase 2a study that met its safety and efficacy endpoints was the potential for drug interactions in NASH patients. A second study of the drug in diabetic retinopathy with associated milestone payments will continue with future development to be decided by BI following completion of the current phase 2a study due to report 2H 2020.

Boehringer Ingelheim's decision has no impact on short term cash as the next milestone payment for NASH was not scheduled until the commencement of a phase 3 study in several years' time.

Pharmaxis closed the September quarter with \$23 million cash, and in October received its 2019 R&D tax incentive of \$6.2 million. In addition, Pharmaxis anticipates receipt of a US\$10 million milestone payment for Bronchitol® from its US licensee Chiesi in Q3 2020 subject to FDA approval midyear.

The company's drug development pipeline includes two drug projects that can be brought to significant valuation points with the current cash position:

- **Partnering of best in class anti fibrotic LOXL2 inhibitor program**
The company confirms that it is engaged in discussions with potential partners and that those discussions will continue into next year. A variety of deal structures are being considered including global pharma and regional pharma deals, all of which fund the future LOXL2 inhibitor development program.
- **Commencement of clinical proof of concept studies in myelofibrosis**
The company's systemic pan-LOX inhibitor program has recently cleared 3-month tox studies and is anticipated to successfully conclude the final stage of a phase 1 study in healthy volunteers in Q1 2020. The company will shortly seek approval from the FDA on orphan status for the treatment of the bone marrow cancer myelofibrosis and seek feedback via the IND process on progressing to phase 2 trials in this disease where there is a high level of unmet need and an existing market value in excess of US\$1 billion per annum.

Pharmaxis CEO, Gary Phillips said, "We are obviously disappointed by BI's decision not to progress the compound acquired from Pharmaxis in NASH. This was unexpected. We understand the significant hurdles to overcome in the early stages of drug development. That is why the company has pursued a strategy of generating multiple research pipeline opportunities underpinned by our cash position and a partnering strategy which offlays development risk where appropriate."

The company also has a topical pan LOX inhibitor program that has potential in scar revision. This program is currently completing late stage pre-clinical testing and is anticipated to enter a phase 1 clinical trial in healthy volunteers in 2020.

Mr Phillips commented, "The topical LOX inhibitor has significant market potential in both post-surgical and cosmetic indications. The company will carefully consider the optimal development plan based on the financial resources required and the level of interest from pharma companies with an interest in dermatology."

#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. Boehringer Ingelheim acquired the Pharmaxis anti-inflammatory AOC3 inhibitor in 2015 to develop it (BI 1467335) for two diseases: the liver condition Non-alcoholic Steatohepatitis (NASH) and diabetic retinopathy (DR).

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.