Media Release

25 February 2019

BOEHRINGER INGELHEIM COMPLETES ENROLLMENT OF PHASE 2A CLINICAL TRIAL OF PHARMAXIS DEVELOPED DRUG IN NASH

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced completion of enrollment in Boehringer Ingelheim's Phase 2a clinical trial in patients with non-alcoholic steatohepatitis (NASH). The trial is assessing BI 1467335 (formerly known as PXS-4728A), an oral inhibitor of amine oxidase, copper containing 3 (AOC3), that works by blocking leucocyte adhesion and tissue infiltration in inflammatory processes underlying NASH. BI 1467335 was acquired from Pharmaxis in May 2015 and is also in an ongoing Boehringer Ingelheim Phase 2a clinical trial for diabetic retinopathy.

The Phase 2a NASH trial is a multi-centre, double-blind design in 114 patients with clinical evidence of NASH. The trial is being conducted in nine countries across North America and Europe. The primary objectives are to establish proof of clinical principle, investigate suitable dosing, and to evaluate the safety of BI 1467335. Patients have been randomized to either one of four dosages of BI 1467335 or to placebo for a 12-week treatment period followed by a 4-week observation period. The trial is expected to report in the second half of 2019. A subsequent Phase 2b study will seek to confirm and extend these findings.

Non-alcoholic fatty liver disease (NAFLD), the most common liver disorder in Western industrialized nations, and its more serious form NASH, is highly prevalent amongst patients with type 2 Diabetes. NASH is a major cause of liver fibrosis and cirrhosis and is an area of high unmet medical need with no treatments currently available. The high prevalence of type 2 diabetes and obesity is expected to make NASH one of the most common causes of advanced liver disorders in coming decades. 25% of the general adult population in the world has NAFLD and the prevalence of NASH has been found to range from 1.5% to 6.45% in current research, a number twice as high as 20 years ago.

Pharmaxis CEO Gary Phillips said, "We are delighted to see the progress made on this study and look forward to this trial reporting in the second half of this year. Whilst we do not receive any milestone payments under our contract with Boehringer Ingelheim for a positive trial result, this will still be the first proof of clinical principle of the Pharmaxis drug acquired in 2015. A positive result will increase the likelihood of receiving future milestones with the next one being triggered by the start of a phase 3 study."

#ends#

SOURCE: Pharmaxis Ltd, Sydney, Australia
CONTACT:
Media: Felicity Moffatt: T +61 418 677 701, E felicity.moffatt@pharmaxis.com.au
Investor relations: David McGarvey: T +61 438 880 106, E david.mcgarvey@pharmaxis.com.au

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 the United States, 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) (also known as amine oxidase, copper containing 3 (AOC3)), 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

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.