Press Release

Boehringer Ingelheim initiates Phase IIa study of compound acquired from Pharmaxis in debilitating liver disease NASH

- Boehringer Ingelheim commences Phase II program of investigational drug candidate BI 1467335 acquired from Pharmaxis with a 12 week Phase IIa proof of clinical principle study in non-alcoholic steatohepatitis (NASH)
- New study underscores Boehringer Ingelheim's aspiration to deliver more first in class medicines with breakthrough potential for patients with cardio metabolic diseases
- Pharmaxis to receive €18 million milestone payment in a significant further endorsement of the company's ability to generate value from its early stage pipeline.

INGELHEIM, Germany and SYDNEY, Australia – August 24, 2017 – Boehringer Ingelheim and pharmaceutical company Pharmaxis (ASX: PXS) announce that Boehringer Ingelheim has initiated a European and North American Phase IIa trial in NASH with BI 1467335 (formerly known as PXS-4728A), acquired from Pharmaxis in May 2015. The compound is an oral inhibitor of amine oxidase, copper containing 3 (AOC3)[1], and works by blocking leucocyte adhesion and tissue infiltration in inflammatory processes underlying NASH.

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.

In 2016 Boehringer Ingelheim obtained Fast Track Designation from the US Food and Drug Administration (FDA) for the development of BI 1467335 in NASH. Fast track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions which fill an unmet medical need. The designation provides opportunities for Boehringer Ingelheim to accelerate the development of this investigational drug candidate in NASH.

This Phase IIa trial is a multi-centre, double-blind design in 150 patients with clinical evidence of NASH. The primary objectives are to establish proof of clinical principle, investigate suitable dosing, and to evaluate the safety of BI 1467335. Patients will be randomized to either one of four dosages of BI 1467335 or to placebo for a 12-week treatment period.[2] A subsequent Phase IIb study will seek to confirm and extend these findings.

Dr. Christopher Corsico, Chief Medical Officer Boehringer Ingelheim Boehringer Ingelheim commented, "Advancing BI 1467335 into Phase II clinical research is important news for patients with NASH. Boehringer Ingelheim is committed to developing novel therapeutics designed to address unmet medical need and improve public health. Boehringer Ingelheim looks forward to further studying this novel compound in NASH patients".

[1] Also known as vascular adhesion protein-1 (VAP-1) or semicarbazide-sensitive amine oxidase (SSAO)
[2] https://clinicaltrials.gov/show/NCT03166735

Boehringer Ingelheim has a long history of excellence in the discovery and development of medicines for cardiometabolic disease patients. It has established a broad portfolio of marketed products for thromboembolic diseases, type 2 diabetes, acute myocardial infarction, hypertension and cardio-renal risk reduction. The cardiometabolic diseases pipeline extends beyond type 2 diabetes and anticoagulation with a focus on innovative drugs for the treatment of the devastating consequences of diabetes.

Pharmaxis CEO Mr. Gary Phillips said, "Pharmaxis selected Boehringer Ingelheim as our partner for PXS-4728A both because of the company's reputation as a leader in cardio metabolic research and development, and its outstanding track record in advancing external research. Today's announcement of the start of this Phase IIa clinical trial for NASH is excellent news and is very significant for Pharmaxis. It triggers the payment of an €18 million (A$27m) milestone to Pharmaxis and opens the path to a total of €195 million in milestone payments as the drug progresses through development and approval for this indication. The initiation of Phase II trials in a second indication later this year by Boehringer Ingelheim can bring the total potential value of the partnership with Boehringer Ingelheim to €418.5m (A$627m) plus sales milestones and high single digit earn-out payments on annual net sales."

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About Boehringer Ingelheim
Innovative medicines for people and animals have for more than 130 years been what the research-driven pharmaceutical company Boehringer Ingelheim stands for. Boehringer Ingelheim is one of the pharmaceutical industry's top 20 companies and to this day remains family-owned. Day by day, some 50,000 employees create value through innovation for the three business areas human pharmaceuticals, animal health and biopharmaceutical contract manufacturing. In 2016, Boehringer Ingelheim achieved net sales of around 15.9 billion euros. With more than three billion euros, R&D expenditure corresponds to 19.6 per cent of net sales.

Social responsibility comes naturally to Boehringer Ingelheim. That is why the company is involved in social projects such as the "Making More Health" initiative. Boehringer Ingelheim also actively promotes workforce diversity and benefits from its employees' different experiences and skills. Furthermore, the focus is on environmental protection and sustainability in everything the company does.

More information about Boehringer Ingelheim can be found on www.boehringer-ingelheim.com or in our annual report: http://annualreport.boehringer-ingelheim.com.

About Pharmaxis
Pharmaxis (ACN 082 811 630) is an Australian research pharmaceutical 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 that will enter clinical development in 2017 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.