

---

### PHARMAXIS RELEASES SUCCESSFUL RESULTS OF PHASE 1 CLINICAL TRIAL FOR BOEHRINGER INGELHEIM PARTNERED DRUG PXS-4728A

---

Pharmaceutical company Pharmaxis (ASX: PXS) today announced positive results for all primary and secondary endpoints from the phase 1 clinical trial of PXS-4728A, a drug that was recently acquired by Boehringer Ingelheim to develop as a treatment for cardiometabolic diseases such as Non-Alcoholic Steatohepatitis (NASH). PXS-4728A is a highly selective inhibitor of an enzyme and adhesion protein (SSAO/VAP-1 encoded by the AOC3 gene) which reduces inflammation and oxidative stress.

Further to the phase 1a single ascending dose stage that reported positive results in April 2015, this phase 1b multiple ascending dose stage was conducted in 24 healthy subjects divided into three groups with each taking active or placebo once a day for 14 days. Three different dosages of PXS-4728A were trialled.

Once daily oral dosing of PXS-4728A for 14 days at doses between 3 and 10 mg was found to be safe and well tolerated. The data confirmed the high oral bioavailability of PXS-4728A and most importantly, showed these low doses are efficacious in inhibiting the enzyme and cause a long lasting inhibition suggesting PXS-4728A can be dosed once a day.

These positive phase 1 results enable Boehringer Ingelheim to proceed with further development of the program.

Pharmaxis CEO Mr Gary Phillips said, "NASH is a disease that is becoming more prevalent and there is a clear need for more effective therapies. PXS-4728A inhibits an enzyme which has been highlighted in independent peer reviewed publications as an excellent target to treat NASH and this first human trial of the drug confirms its strong pre-clinical profile translates into human studies. It is rare to be able to demonstrate effective target engagement in a phase 1 study so the fact that the long lasting enzyme inhibition seen in the phase 1a study was reinforced when given once a day for 14 days adds to our confidence in PXS-4728A. We now look forward to the next value appreciating steps as the clinical development program proceeds with Boehringer."

Boehringer Ingelheim, a leader in cardiometabolic and respiratory research and development, acquired PXS-4728A on 15 May 2015. The results from the phase 1 study will be presented at an upcoming international congress focusing on liver diseases.

**#ends#**

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

**CONTACT:** Felicity Moffatt, phone +61 418 677 701 or email [felicity.moffatt@pharmaxis.com.au](mailto:felicity.moffatt@pharmaxis.com.au)

#### About Pharmaxis

Pharmaxis (ACN 082 811 630) is a specialist pharmaceutical company with a portfolio of products at various stages of development and approval. Its product Bronchitol® for cystic fibrosis is marketed in Europe and Australia and a phase 3 trial to enable completion of an NDA for the US market is underway. 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 Semicarbazide-Sensitive Amine Oxidase Inhibitors (SSAO) for Non-alcoholic Steatohepatitis (NASH) and inflammatory diseases including Chronic

---

Obstructive Pulmonary Disease (COPD), and Lysyl Oxidase Inhibitors (LOX) targeting fibrotic diseases including pulmonary fibrosis and some cancers. In May 2015, Boehringer Ingelheim acquired the Pharmaxis investigational drug PXS4728A, to develop it for the treatment of the liver-related condition NASH. 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).

### **About Boehringer Ingelheim**

The Boehringer Ingelheim group is one of the world's 20 leading pharmaceutical companies. Headquartered in Ingelheim, Germany, Boehringer Ingelheim operates globally with 146 affiliates and a total of more than 47,700 employees. The focus of the family-owned company, founded in 1885, is researching, developing, manufacturing and marketing new medications of high therapeutic value for human and veterinary medicine.

Social responsibility is an important element of the corporate culture at Boehringer Ingelheim. This includes worldwide involvement in social projects, such as the initiative "Making more Health" and caring for the employees. Respect, equal opportunities and reconciling career and family form the foundation of the mutual cooperation. In everything it does, the company focuses on environmental protection and sustainability.

In 2014, Boehringer Ingelheim achieved net sales of about 13.3 billion euros. R&D expenditure corresponds to 19.9 per cent of its net sales.

### **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 drug candidates. All forward-looking statements included in this media release are based upon information available to us as of the date hereof, and we assume no obligation to update any such forward-looking statement as a result of new information, future events or otherwise. We cannot guarantee that any product candidate will receive regulatory approval or that we will seek any such approval.

---