

## Media Release

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### PHARMAXIS EARNS A\$15 MILLION MILESTONE PAYMENT AS BOEHRINGER INGELHEIM CLINICAL TRIAL IN DIABETIC RETINOPATHY DOSES 1<sup>ST</sup> PATIENT

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- Boehringer Ingelheim expands clinical work with drug acquired from Pharmaxis into 2<sup>nd</sup> indication
- Phase 2a Studies into NASH and Diabetic Retinopathy both due to report in 2H 2018
- Drug potential being maximised under Boehringer Ingelheim's holistic cardio metabolism R&D strategy
- A\$15m milestone payment as 2<sup>nd</sup> indication unlocks full deal potential of A\$625m

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced dosing of the first patient in Boehringer Ingelheim's Phase 2a clinical trial in patients with diabetic retinopathy (DR), triggering a €10 million (~A\$15 million) milestone payment to Pharmaxis. DR is the second disease to be targeted with BI 1467335 originally discovered by Pharmaxis. BI 1467335 is also in an ongoing Phase 2a clinical trial for non-alcoholic steatohepatitis (NASH).

The [Phase 2a clinical trial](#) of BI 1467335 in diabetic retinopathy will randomise 100 patients to either drug or placebo for a 12-week treatment period with an additional 12-week follow-up period. The study is expected to report in the second half of 2018. A subsequent Phase 2b study will seek to confirm and extend these findings.

Diabetic retinopathy is the leading cause of vision-loss in adults aged 20-74. Of an estimated 285 million people with diabetes mellitus worldwide, approximately one third have signs of the disease.

Boehringer Ingelheim's two-pronged clinical program with BI 1467335, an oral inhibitor of amine oxidase, copper containing 3 (AOC3)<sup>1</sup> discovered by Pharmaxis, is focused on tackling the common diseases related to the complications of diabetes which affect millions of people worldwide. The drug was acquired by Boehringer Ingelheim in 2015 with an upfront payment of A\$41m to initially study the chronic liver condition NASH.

Gary Phillips, Pharmaxis CEO said, "This milestone payment marks the expansion of the Boehringer Ingelheim development plan for BI 1467335 into two diseases, where all the potential development milestones in the deal (~A\$625m), would be payable to Pharmaxis should both indications be approved. Both development plans attract the same total development payments through to approval, with the 2nd indication milestone payments weighted more towards approval. The next milestones from BI are payable if they choose to commence phase 3 trials, at which time the two indications would attract a total of €62 million in milestones."

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*If you would like to be advised directly by email each time Pharmaxis issues a media release, please register on our website at <http://www.pharmaxis.com.au/investor-centre/subscribe/>.*

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<sup>1</sup> Also known as vascular adhesion protein-1 (VAP-1) or semicarbazide-sensitive amine oxidase (SSAO)

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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 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), and is developing it for the treatment of the liver-related condition Non-alcoholic Steatohepatitis (NASH) and diabetic retinopathy. 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.

#### **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](http://www.boehringer-ingelheim.com) or in its annual report: <http://annualreport.boehringer-ingelheim.com>.