

Press Release

Boehringer Ingelheim discontinues development of BI 1467335 for NASH

- BI 1467335 was part of Boehringer Ingelheim's growing NASH R&D pipeline portfolio
- Study of BI 1467335 in diabetic retinopathy will be continued

Ingelheim, Germany and Sydney, Australia, 18 December 2019 – Boehringer Ingelheim and Pharmaxis Ltd today announced the discontinuation of the development of BI 1467335 for the treatment of NASH (non-alcoholic steatohepatitis). BI 1467335 was acquired from Pharmaxis in 2015.

In a 12 week Phase IIa trial investigating BI 1467335 in patients with clinical evidence of NASH, treatment was well tolerated and no related serious adverse events occurred. The study met the pre specified targets for inhibition of plasma amine oxidase copper-containing 3 (AOC3)¹ activity by BI 1467335 compared to placebo as well as clinically relevant changes in NASH biomarkers. However, following assessment of another recently completed Phase I study, Boehringer Ingelheim decided not to further develop BI 1467335 in this indication based on the risk of drug interactions of the compound in NASH patients. Further studies with BI 1467335, including a Phase IIa study in diabetic retinopathy which has completed recruitment and is due to report 2H 2020, remain unaffected by the decision.

Boehringer Ingelheim continues to be fully committed to building a comprehensive next generation portfolio of first-in-class treatments with breakthrough potential to enable resolution of NASH across all stages of the disease.

Pharmaxis CEO, Gary Phillips said, "We are disappointed that BI 1467335 is not advancing in NASH. We look forward to further scientific discussion when the full data and analysis from this Phase IIa clinical trial and Boehringer Ingelheim's recently reported Phase I study are available for review."

About the Phase IIa trial with BI 1467335 in NASH

The Phase IIa trial (ClinicalTrials.gov Identifier: NCT03166735) was a multi-centre, double-blind design in 114 patients with clinical evidence of NASH. Objectives were to establish the proof of clinical principle, investigate suitable dosing, and to evaluate the safety of BI 1467335. Patients were randomized to either one of four dosages of BI 1467335 or to placebo for a 12-week treatment period.

About NASH

Non-alcoholic fatty liver disease (NAFLD), the most common liver disease in Western industrialized nations, and its more serious form non-alcoholic steatohepatitis (NASH) are especially highly prevalent in patients with metabolic disorders such as type 2 diabetes and obesity. NASH is a major cause of liver fibrosis and cirrhosis and is an area of high unmet medical need with no approved 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 diseases in coming decades.

1 out of 4 adults is assumed to have NAFLD, and the prevalence of NASH has been estimated to range from 1.5 per cent to 6.45 per cent in current research, a number twice as high as 20 years ago.

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

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About Boehringer Ingelheim

Improving the health of humans and animals is the goal of the research-driven pharmaceutical company Boehringer Ingelheim. The focus in doing so is on diseases for which no satisfactory treatment option exists to date. The company therefore concentrates on developing innovative therapies that can extend patients' lives. In animal health, Boehringer Ingelheim stands for advanced prevention.

Family-owned since it was established in 1885, Boehringer Ingelheim is one of the pharmaceutical industry's top 20 companies. Some 50,000 employees create value through innovation daily for the three business areas human pharmaceuticals, animal health and biopharmaceuticals. In 2018, Boehringer Ingelheim achieved net sales of around 17.5 billion euros. R&D expenditure of almost 3.2 billion euros, corresponded to 18.1 per cent of net sales.

As a family-owned company, Boehringer Ingelheim plans in generations and focuses on long-term success. The company therefore aims at organic growth from its own resources with simultaneous openness to partnerships and strategic alliances in research. In everything it does, Boehringer Ingelheim naturally adopts responsibility towards mankind and the environment.

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 Ltd 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/>