

Boehringer Ingelheim discontinues development of BI 1467335 for Diabetic Retinopathy

- BI 1467335 was acquired from Pharmaxis in 2015 as part of Boehringer Ingelheim's growing Retinal Health R&D pipeline portfolio.
- Boehringer Ingelheim has terminated the agreement with Pharmaxis with 90 days' notice during which time Pharmaxis can request return of all existing data and intellectual property.
- Pharmaxis will continue its development of small molecule amine oxidase inhibitors with a phase 2 study in myelofibrosis due to commence later this year.
- Boehringer Ingelheim will continue to advance its comprehensive portfolio of next generation retinal therapy approaches.

Ingelheim, Germany and Sydney, Australia, 7 September 2020 – Boehringer Ingelheim and Pharmaxis Ltd today announced the discontinuation of the development of anti-inflammatory AOC₃ inhibitor BI 1467335 for the treatment of patients with moderate-severe non-proliferative diabetic retinopathy (NPDR). BI 1467335 was acquired from Pharmaxis in 2015 and Boehringer Ingelheim will now terminate the agreement.

In a Phase IIa trial in patients with moderate-severe NPDR, BI 1467335 met its primary endpoint in ocular safety with the treatment being well tolerated. Boehringer Ingelheim decided not to further develop BI 1467335 in this indication based on the lack of a clear efficacy signal and risk of dose dependent drug interactions of the compound in NPDR patients identified in another Phase I study.

Pharmaxis CEO, Gary Phillips said, "We understand Boehringer Ingelheim's decision to stop development of BI 1467335 in NPDR based on the risk of dose dependent drug interactions at the dose level that was tested in the Phase IIa trial. Based on recent publications AOC₃ remains an important clinical target. We will review the data collected in more detail to evaluate potential opportunities in other indications that already have supportive pre-clinical data and where the risk of drug interactions are of less concern. In the meantime, we look forward to the upcoming FDA decision on granting a marketing authorisation for Bronchitol in the US cystic fibrosis market and our focus remains on advancing our anti-cancer pan-LOX inhibitor program into a phase 2 myelofibrosis study following the recent IND approval."

Boehringer Ingelheim takes a holistic approach to the development of novel retinal disease therapies, targeting key mechanisms in the pathogenesis of retinal diseases. By leveraging existing expertise in oncology, inflammation, neurodegeneration, fibrosis and cardiometabolic diseases, the company is building a growing portfolio of next generation retinal therapy approaches in various stages of development up to Phase 2 in macular degeneration and diabetic retinal diseases.

About the Phase IIa trial with BI 1467335 in NPDR (ROBIN study)

The Phase IIa trial (ClinicalTrials.gov Identifier: NCT03238963) was a Phase IIa multi-centre, double-masked design in 79 patients with moderately severe to severe non-proliferative diabetic retinopathy (NPDR) without center-involved diabetic macular edema (CIDME). The primary objective was to evaluate the safety and tolerability of BI 1467335. Secondary objective was the proportion of patients with a two step improvement in diabetic retinopathy severity score (DRSS). Patients were randomized to either BI 1467335 or to placebo for a 12-week treatment period with an additional 12-week follow-up period afterwards.

About Diabetic Retinopathy

Diabetic retinopathy (DR) is the leading cause of vision-loss in adults aged 20-74. It progresses from mild nonproliferative diabetic retinopathy to moderate and severe nonproliferative diabetic retinopathy (NPDR), characterized by retinal hemorrhages and vascular changes in the retina, to proliferative diabetic retinopathy (PDR), characterized by the growth of new blood vessels on the retina. Diabetic Macular Edema (DME), characterized by retinal thickening from leaky blood vessels, can develop at all stages of retinopathy. Of an estimated 285 million people with diabetes mellitus worldwide, approximately one third have signs of DR and of these, a further one third of DR is vision-threatening DR (severe NPDR, PDR and DME).

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About Boehringer Ingelheim

Making new and better medicines for humans and animals is at the heart of what we do. Our mission is to create breakthrough therapies that change lives. Since its founding in 1885, Boehringer Ingelheim is independent and family-owned. We have the freedom to pursue our long-term vision, looking ahead to identify the health challenges of the future and targeting those areas of need where we can do the most good. As a world-leading, research-driven pharmaceutical company, more than 51,000 employees create value through innovation daily for our three business areas: Human Pharma, Animal Health, and Biopharmaceutical Contract Manufacturing. In 2019, Boehringer Ingelheim achieved net sales of 19 billion euros. Our significant investment of almost 3.5 billion euros in R&D drives innovation, enabling the next generation of medicines that save lives and improve quality of life.

We realize more scientific opportunities by embracing the power of partnership and diversity of experts across the life-science community. By working together, we accelerate the delivery of the next medical breakthrough that will transform the lives of patients now, and in generations to come.

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

About Pharmaxis

Pharmaxis Limited 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 with drug candidates in clinical trials.

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. The Company's first compound is an anti-inflammatory AOC₃ inhibitor developed in 2015. The company's amine oxidase program has since 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; a systemic pan-LOX inhibitor for acute fibrosis and cancer that will enter a phase 2 study in 2020; and a topical pan-LOX inhibitor for scarring that is expected to commence phase 1 studies in 2H 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/>