



Press Release

ABIONYX Pharma announces positive preclinical results in a model of uveitis and launches the strategic development of the first class of biomedicines in ophthalmology based on its bio-HDL

- **Positive preclinical results in a model of uveitis**
- **Development of the first class of biomedicines in ophthalmology with bio-HDL**

Toulouse, FRANCE, Lakeland MI, UNITED-STATES, October 7, 2021, 7.30 am CEST – ABIONYX Pharma (FR0012616852 - ABNX - PEA PME eligible), a next-generation biotech company dedicated to the discovery and development of innovative therapies, announces today, after having already obtained an Orphan Drug designation for CER-001 in the treatment of LCAT deficiency, positive preclinical results in ophthalmology and the launch of a strategic development of the first class of biomedicines from its bio-HDL for the treatment of ocular pathologies.

Positive preclinical results in a model of uveitis

Following the positive clinical results which included the disappearance of visual blur related to corneal lipid deposits in a patient suffering from LCAT deficiency, treated within the framework of a Temporary Use Authorization, and the clear improvement of the patient's visual functions which was still observed after 1 year of follow-up (initial results published exclusively in the scientific journal "Annals of Internal Medicine; follow-up information on file), ABIONYX Pharma conducted preclinical studies in ophthalmology to determine the ocular tolerance of its bio-HDL and the potential spectrum of efficacy in new indications.

Bio-HDL was found to be completely safe and very well tolerated on the ocular surface and inside the eye, regardless of the route of administration in a preclinical study, either by intravenous injection, by application of drops to the surface of the eye or by injection inside the eye itself (into the vitreous).

In addition, bio-HDL was tested in a proof of concept efficacy study using a recognized and validated preclinical model of uveitis. In this Endotoxin-Induced Uveitis (EIU) model, uveitis was triggered by a dose of lipopolysaccharide (LPS) injected in the vitreous. Subsequent treatment with CER-001 injected into the vitreous showed a statistical reduction in the signs of uveitis, as measured by protein concentration and cellular infiltration in the aqueous humor.

These initial preclinical results indicate the major therapeutic potential of bio-HDL in ophthalmology, and more broadly the role of lipids in ocular pathologies.

Dr. Christophe Baudouin, Professor of Ophthalmology in Paris, Head of the Ophthalmology Department at the Centre Hospitalier National d'Ophtalmologie de l'Hôpital des Quinze-Vingts (Paris), Director of the "S12" research team at the Institut de la Vision, and member of the prestigious international societies, American Society of Ophthalmology and Academia Ophthalmologica Internationalis, states: *"The latest scientific work in the field shows that lipids and their metabolism are involved in many ocular pathologies, for example dysfunction of the meibomian glands and macular degeneration. By testing CER-001, a biomimetic HDL produced in France, in ocular pathology models, we will be able to help choose the best ocular indication for this product, with the aim of providing patients with a new and effective treatment."*

Development of the first class of biomedicines in ophthalmology with bio-HDL

Following the granting of Orphan Designation by the European Medicines Agency (EMA) for its drug candidate CER-001 as a potential treatment for LCAT deficiency, ABIONYX Pharma's bio-HDL is one of the most advanced biomedicines in France that could soon be marketed in ophthalmology.

As a reminder, there are two forms of LCAT deficiency:

- familial LCAT deficiency (FLD), which results from a complete deficiency and is clinically characterized by hemolytic anemia, renal failure (frequently leading to renal transplantation), and corneal opacities; and
- "Fish Eye Disease" resulting from a partial deficiency and clinically characterized by more severe corneal opacities without renal involvement.

As the orphan drug designation obtained covers both a renal and an ophthalmological indication, the clinical development of bio-HDL in ophthalmology may be very rapid.

Lipids are of major interest for the eye in several respects: its structural role, its functional role, its pathophysiological links and its therapeutic potential.

Dr. Catherine Creuzot-Garcher, Professor of Ophthalmology in Dijon, Head of the Ophthalmology Department at the University Hospital of Dijon, University Professor, co-leader of the Eye, Nutrition and Cellular Signaling team at the Taste and Food Science Center of Dijon, and **Dr. Niyazi Acar (PhD)**, leader of the Eye, Nutrition and Cellular Signaling team at the Taste and Food Science Center of Dijon state: *"The study and development of the therapeutic potential of CER-001, a biomimetic HDL, in the treatment of eye diseases will allow us to better understand the role of lipids in the physiology and dysfunctions of the eye, particularly in the retina, and to provide our patients with an innovative solution for the future."*

The anti-inflammatory and/or reverse lipid transport enhancing properties of CER-001, which may improve vision in patients with deficient LCAT activity, combined with the new preclinical results in uveitis, pave the way for clinical studies testing bio-HDL in patients developing corneal lipid deposits from other pathologies, and allow ABIONYX Pharma to launch a strategic development of the first class of biomedicines in ophthalmology based on its bio-HDL.

About ABIONYX Pharma

ABIONYX Pharma is a new generation biotech company that aims to contribute to health through innovative therapies in indications where there is no effective or existing treatment, even the rarest ones. Thanks to its partners in research, medicine, biopharmaceuticals and shareholding, the company innovates on a daily basis to propose drugs for the treatment of renal and ophthalmological diseases, or new HDL vectors used for targeted drug delivery.

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