The apoA-I companY

Press Release

ABIONYX Pharma announces new positive results in a uveitis model for the strategic development of the first class of biomedicines in ophthalmology based on its recombinant apoA-I

- **Significant efficacy of CER-001 after a single intraocular administration in a uveitis model with severe inflammation compared with standard and best-in-class drugs**

- **Appointment of Mr Jérôme Martinez as Senior Advisor for Ophthalmology**

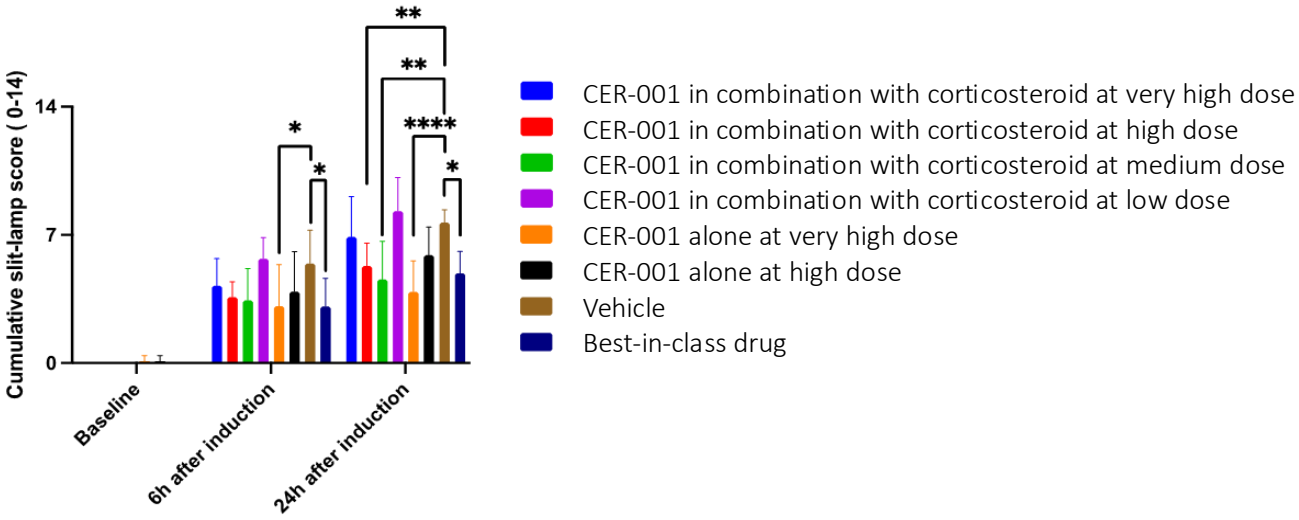
Toulouse, FRANCE, Lakeland MI, USA, November 21, 2023, 7.00 a.m. CET - **ABIONYX Pharma**, (**FR0012616852 - ABNX - PEA PME eligible**), a new generation biotech company dedicated to the discovery and development of innovative therapies based on the world's only recombinant human apoA-1, announces new positive results in ophthalmology for the first class of CER-001-based biomedicines for the treatment of ocular pathologies, and the appointment of Mr. Jérôme Martinez as ABIONYX Pharma's Senior Advisor in ophthalmology.

New positive long-term preclinical results evaluating the efficacy of CER-001 after a single intraocular administration in a uveitis model with severe inflammation

Following the beneficial clinical findings related to the disappearance of visual blurring linked to corneal deposits in a patient suffering from LCAT deficiency treated under a Temporary Authorization for Use, and a marked improvement in the patient's visual function (results published exclusively in the scientific journal "Annals of Internal Medicine" in 2021), and still observed after more than a year of follow-up, ABIONYX Pharma conducted new preclinical studies in ophthalmology to qualify the efficacy spectrum of recombinant apoA-I alone and in combination with a corticosteroid, and to broaden its potential in new indications.

After demonstrating the safety of CER-001, the recombinant apoA-I was tested again to assess its action in reducing inflammatory reactions and its tolerability after a single intraocular administration (IVT) in a model of LPS-induced uveitis.

Slit lamp examinations (cumulative score)



74 animals participated in this study, divided into 8 groups. Six hours after LPS injection, statistically significant reductions in inflammation, measured by slit-lamp examination, were observed for the groups treated with CER-001 alone or in combination with a corticosteroid compared with the vehicle-treated group. CER-001 (cumulative score 3.1 ± 2.3, p= 0.0254) and best-in-class drug (cumulative score 3.1 ± 1.5, p= 0.0228) were superior to vehicle. No statistically significant differences were observed for the other groups treated with standard therapies.

Twenty-four hours after induction, the significance observed at six hours for the CER-001 and best-in-class drug-treated groups versus the vehicle-treated group continued, demonstrating a reduction in inflammation (cumulative score 3.9 ± 1.7 and 4.9 ± 1.2, respectively and $p < 0.0001$ and = 0.0018, respectively). The downward trend observed at six hours compared with the vehicle-treated group was confirmed by statistical significance for CER-001 alone and CER-001 in combination (cumulative score 5.3 ± 1.3 and 4.6 ± 2.1, respectively and $p = 0.0081$ and = 0.0018, respectively). No statistically significant differences were observed for all other groups. Results for the CER-001 and CER-001 combination groups were comparable or better than those for the best-in-class alone group.

Cell infiltration in aqueous humor

Twenty-four hours after LPS injection, the highest level of induced inflammation was reached in the vehicle-treated group, with median values of 5920 cells/µL. A statistically significant decrease in leukocyte infiltration was observed in the groups treated with CER-001 alone and CER-001 in combination with a corticosteroid, compared with the vehicle-treated group. For all other groups, no significance was observed.

CER-001 alone or in combination with a corticosteroid, as tested in this preclinical study, proved safe and well tolerated on the ocular surface and inside the eye, when given by injection inside the eye.

These new preclinical results reconfirm the major therapeutic potential of the only recombinant apoA-I in ophthalmology. The anti-inflammatory and/or reverse lipid transport-enhancing properties of CER-001, and these new preclinical results in uveitis, pave the way for clinical trials testing apoA-I in patients with other severe inflammatory ocular diseases.

Appointment of Mr Jérôme Martinez as ABIONYX Pharma's Senior Advisor in ophthalmology

Jérôme Martinez has been appointed Senior Advisor to ABIONYX Pharma for the development of CER-001 in ophthalmology. Jérôme has over 30 years' experience in the management of pharmaceutical and biotech companies in France and abroad. Before joining ABIONYX Pharma, Jérôme Martinez was COO in France for the Japanese pharmaceutical company SANTEN, specializing in ophthalmology and rheumatology. From 2004 to 2011, he was Chairman of the Board of Novagali Pharma, a laboratory specializing in ophthalmology, where he oversaw Novagali Pharma's IPO and its sale to SANTEN in 2012. With a background in pharmacology, Jérôme Martinez holds a Master of Law in Health Administration from the Paris XI University, and an MBA from HEC Paris / Keio University in Japan. He is also a graduate of the JL Kellogg Graduate School of Management at Northwestern University in Chicago and is certified in the International Director Program at INSEAD.

Mr. Jérôme Martinez, ABIONYX Pharma's Senior Advisor in ophthalmology, concludes: "*After being profiled as a bioproduct in ophthalmology, and tested in the reduction of inflammatory reactions, recombinant apoA-I alone also proved to be effective in a uveitis model with severe inflammation. More importantly, this efficacy was compared with other market standards, notably the best-in-class, and proved to be more than significant not only as a stand-alone bioproduct, but also in combination with a corticosteroid, after a single intraocular injection. This is very promising for the development of the first class of biomedicines in ophthalmology in the very broad field of severe indications involving major vascular or lipid disorders such as AMD or DME*."

About uveitis

Acute anterior uveitis is a recurrent inflammatory disease of the eye that occurs frequently and can have potentially blinding sequelae. The pathogenesis of this disease is poorly understood. Various circumstantial observations suggest that the innate immune system plays a very important role in the development of uveitis.

Patients suffering from acute anterior uveitis complain of photophobia (sensitivity to light), often severe. Other symptoms may include redness of the eye, tearing and decreased vision. Characteristic examination findings include vessel congestion, the presence of cells and proteins in the aqueous humor, and miosis. In severe cases, hypopyon and/or fibrin may form.

Clinically, chronic progressive or recurrent forms of non-infectious uveitis are treated with topical and/or systemic corticosteroids.

However, long-term use of these drugs can lead to deleterious ocular and systemic side effects such as glaucoma, cataracts, osteoporosis, hypertension and diabetes. The use of alternative steroid-sparing immunosuppressive agents has also shown clinical benefits, but itself carries undesirable risks. Given these limitations, there is a clear demand for the development of new therapeutic strategies. Recent advances in our understanding of the resolving mechanisms of inflammation, and the discovery of several inflammatory mediators, have led to a whole new range of potential therapeutic possibilities.

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 apoA-I vectors used for targeted drug delivery.

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