The apoA-I companY

Press release

World Rare Disease Day: ABIONYX renews its commitment to treat LCAT Deficiency (Norum's disease) and provides an update on its advances

- **Commitment to the LCAT patient community for the creation of a patient association and assistance with early diagnosis**
- **Preparation for the submission of the Marketing Authorization Application to the EMA following the clinical phase 3 exemption and the agreement for the validation of only two bioproduction batches**
- **Benefit of Orphan Disease Designation for both renal and ophthalmological forms in Europe and the United States**

Toulouse, FRANCE, Lakeland, USA, February 28, 2025, 7:30 p.m. CET – **ABIONYX Pharma, (FR0012616852 - ABNX - eligible for PEA PME)**, a next generation biopharma company dedicated to the development of innovative biomedicines based on a recombinant apolipoprotein apoA-1 for the treatment of the most severe inflammatory diseases, today provides an update on its progress and confirms its commitment to the rare disease known as LCAT Deficiency or 'Norum's disease'.

Commitment to the LCAT patient community for the creation of a patient association and assistance with early diagnosis

In accordance with its *Raison d'Etre* as set out in its articles of association, *'To develop innovative therapies for indications with no existing or effective treatment, even the rarest, for the benefit of patients'*, ABIONYX Pharma is committed to the most severe inflammatory diseases where medical needs are the highest and for which there is no existing treatment.

As a result, in addition to targeting diseases with very severe inflammatory components such as septicemia, the company is involved in the development of a biomedicine for LCAT Deficiency or Norum's disease. This rare disease, which affects less than 1 in 1,000,000 people worldwide, is characterized by a rare lipoprotein metabolism disorder that causes very severe kidney and/or eye damage that can lead to dialysis, organ transplantation and/or vision loss. The mission to finally offer a treatment option to these LCAT patients is based on R&D expertise in metabolic diseases and marks a strong commitment to the community of these patients.

On the occasion of International Rare Diseases Day, which takes place every year on the last day of February, ABIONYX Pharma is expressing its commitment to improving the care pathway and patient

management, and to combating diagnostic failure, a real obstacle for patients with rare diseases. For example, the company has encouraged the identification of LCAT patients in ophthalmology in several leading French medical centers, including Cochin Hospital in Paris and Rangueil Hospital in Toulouse, based on the more frequent identification of the ocular form of the disease, also known as 'Fish Eye Disease'.

ABIONYX Pharma is determined to work tirelessly to break down the barriers faced by the LCAT community worldwide. The company is helping to raise awareness of this very rare disease and has been able to offer its biological drug free of charge to patients in Europe on a compassionate use basis, despite still having limited financial resources. These actions have been carried out for the benefit of people who have difficulty accessing treatment. ABIONYX thus hopes that each LCAT patient can remain in better health thanks to the earliest reliable diagnosis and specific treatment.

In this respect, ABIONYX Pharma is already committed to supporting the creation of a European association of LCAT patients to help recognize this very rare disease and its two particular renal and ophthalmological forms. Finally, the company is working closely with the Filière de Santé Maladies Rares (Rare Diseases Health Network) and nephrology and ophthalmology learned societies to promote the early (biochemical and genetic) diagnosis of this genetic disease with dramatic consequences.

Preparing to submit the Marketing Authorisation application to the EMA following the clinical phase 3 exemption and the agreement for the validation of two bioproduction batches

Following a positive opinion from the EMA for CER-001 in LCAT deficiency to submit data from only two prospective validation batches instead of the three batches usually required, ABIONYX Pharma will present by the end of the year the clinical data of CER-001 related to its compassionate use in treated LCAT patients from four European countries, with a view to applying for a marketing authorization (MA).

It should be noted that the first positive clinical results of CER-001 in LCAT deficiency were published exclusively in the scientific journal 'Annals of Internal Medicine' in March 2021. They revealed that the patient, who was about to be put on dialysis due to the rapid decline of her renal function, was able to avoid the need for dialysis thanks to her treatment with CER-001. The patient, who suffered from lipid deposits in the corneas, also noticed that her blurred vision had disappeared.

Benefit of Orphan Drug Designation for both renal and ophthalmological conditions in Europe and the United States

In early 2021, the European Medicines Agency (EMA) issued a positive opinion on the company's application for orphan drug designation for the candidate biomedical drug CER-001 as a potential treatment for LCAT deficiency. In early 2022, the Food and Drug Administration (FDA) in turn granted orphan drug designation (ODD) to CER-001 for the treatment of LCAT deficiency in renal dysfunction and/or ophthalmological disease

This orphan disease designation offers strong recognition of LCAT deficiency, which is still too little known in Europe and the United States, and highlights the urgent need for innovative treatment for patients suffering from this serious, disabling and permanent disease. This designation will enable the secure acceleration of future commercial development, thanks to access to the centralized marketing authorization (MA) procedure in Europe and in the US, as well as commercial exclusivity for 10 to 12 years from the granting of the MA.

The designation of orphan drug by the EMA and the FDA offers the company significant incentives and benefits, including assistance with clinical protocols, differentiated evaluation procedures for health technology assessments, and above all reduced regulatory costs.

About ABIONYX Pharma

ABIONYX Pharma is a next-generation biopharma company dedicated to the development of innovative biomedicines for the most severe inflammatory diseases for which there is no effective or existing treatment, even in the rarest indications. The company accelerates the development of breakthrough therapies thanks to in-depth expertise in lipid science and a technological platform based on recombinant apoA-I. ABIONYX Pharma is committed to radically improving the results of sepsis treatments and intensive care.

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