Press release

# ABIONYX Pharma announces a new Compassionate Access Authorization (CAA) for CER-001 in the rare disease LCAT deficiency or Norum disease in Europe

- **4th CAA patient in Europe in the rare disease LCAT deficiency also known as Norum disease**
- **Scientific consistency and coherence of clinical data from first 3 patients**
- **Treatment safety and tolerability data as positive as ever**
- **Constructive discussions with European regulatory authorities to define a fast-track clinical development pathway for CER-001 for patients with this rare disease**
- **Treatment of LCAT deficiency or Norum disease presents a major medical challenge, given the disease's constant progression and rapid renal decline even following kidney grafting**

**Toulouse, FRANCE, Lakeland MI, USA, July 10th, 2023, 6.00 pm CEST** - **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 natural recombinant ApoA-I, announces that the company has been granted a new Compassionate Access Authorization (CAA) in Europe for CER-001 in the rare, untreated kidney disease LCAT Deficiency.

This is the fourth patient suffering from LCAT deficiency to be granted Compassionate Access to CER-001, in development by ABIONYX Pharma, in Europe. The four patients suffering from this rare disease, which has a major impact on quality of life and on lifespan, represent very different stages in the evolution of the disease, for which CER-001 may prove effective, whether or not the patients are transplanted, and whatever their age.

CER-001 would represent a unique therapeutic breakthrough for patients who need to be diagnosed as early as possible to avoid dialysis, followed by one or more transplants during their lifetime. The early administration of CER-001 in young patients with Norum disease could have a real beneficial effect on these patients, transforming their lives and, above all, delaying the end of their lives, while very significantly reducing the management of their disease, whether with dialysis or by one or more transplants.

The safety and tolerability data for this treatment are as positive as ever, with no adverse events reported for any of the patients.

Constructive discussions have been initiated with the European regulatory authorities to define a pathway for the rapid advancement of CER-001's clinical development in the patient population affected by this rare disease.

The treatment of LCAT deficiency or Norum disease is a major medical challenge, given the constant progression of the disease, leading to rapid renal decline and often premature death, and the total absence of approved treatments to slow or modify the progression of the disease.

ABIONYX Pharma continues to receive and fulfill Compassionate Access requests from hospitals around the world. The biomanufacturing of the latest and subsequent batches of CER-001 to the highest GMP quality standards will enable these requests to be met, and the clinical results of the first patients will be communicated exclusively via scientific publication.

### About LCAT deficiency or Norum disease

LCAT deficiency is a rare degenerative disease affecting around 1 in 1,000,000 newborns. It is characterized by renal failure and hemolytic anemia, sometimes by corneal opacities, and biochemically by severely reduced HDL-cholesterol levels. It is caused by mutations in the LCAT gene, whose enzymatic activity catalyzes the formation of cholesterol esters in lipoproteins. Accumulation of unesterified cholesterol in the body, notably in the cornea, red blood cells and kidneys, leads to severe degeneration and premature death. There are currently no treatment options to alter the course of LCAT deficiency, known as Norum disease after the Norwegian physician Kaare Norum who identified and studied it in the 1960s. To date there are no approved treatments for this disease.

### 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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