Press Release

# ABIONYX Pharma receives positive opinion from EMA within the framework of the Orphan Drug Designation process for CER-001 for the rare disease LCAT Deficiency

- **Culmination of new strategy in an ultra-rare kidney disease**
- **New step in repositioning the product for renal and ophthalmologic diseases**
- **Secured biomanufacturing of CER-001 for future trials**

**Toulouse, FRANCE, Lakeland MI, UNITED-STATES, July 28, 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, announced today that the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) has issued a positive opinion on the company's application for Orphan Drug Designation (ODD) for its drug candidate CER-001, as a potential treatment for lecithin-cholesterol acyltransferase (LCAT) deficiency clinically characterized by hemolytic anemia and renal failure, most often leading to renal transplantation, and corneal opacities. CER-001 is a first-in-class bio-HDL mimetic that directly targets a key underlying metabolic defect of LCAT deficiency.

## The culmination of a new strategy to use CER-001 in an ultra-rare renal disease

This positive opinion from the COMP of the EMA for an orphan drug designation for bio-HDL is of strategic importance for the development of our product. This designation offers an important advantage by providing access to the centralized marketing authorization procedure as well as 10 years of market exclusivity in the European Community from the date of obtaining a marketing authorization. This positive opinion is also a recognition of the need for innovative treatments for patients with LCAT deficiency, a serious and lifelong disease. Several nATU applications have been received necessitating the decision to secure biomanufacturing to produce clinical batches for patient use and a European regulatory filing. CER-001 is the only bio-HDL to receive this important designation in Europe and the first potential disease-modifying treatment for patients with LCAT deficiency.

Orphan drug designation in the European Union (EU) is granted by the European Commission on the basis of a positive opinion issued by the COMP of the EMA. To obtain this designation, a drug candidate must be intended to treat a severely debilitating or life-threatening condition that affects fewer than 5 in 10,000 people in the EU, and there must be sufficient clinical or non-clinical data to suggest that

the investigational drug can produce clinically relevant results. Orphan drug designation by the EMA provides companies with certain benefits and incentives, including assistance with clinical protocols, differentiated assessment procedures for health technology assessments in certain countries, access to a centralized marketing authorization procedure valid in all EU member states, reduced regulatory fees, and 10 years of market exclusivity from the time a marketing authorization is granted.

## New step in repositioning the product for renal and ophthalmologic diseases

The prevalence of LCAT deficiency is estimated to be less than 1 in 1,000,000. Several named compassionate use programs (nATUs) are evaluating the efficacy, safety, tolerability and compliance of CER-001 in patients with LCAT deficiency in Europe.

As LCAT deficiency also affects the cornea, this orphan designation paves the way for clinical development in ophthalmology, as announced in the context of expanding the potential of CER-001's innovation and its pleiotropic action as a natural recombinant HDL.

In addition to nATUs, ABIONYX Pharma is currently conducting a Phase 2a clinical trial evaluating the efficacy, safety, pharmacokinetics and pharmacodynamics of CER-001 treatment in adults with sepsis at high risk of Acute Kidney Injury, a disease affecting more than 2 million people worldwide.

## Securing the bioproduction of CER-001

As anticipated, the company had decided to launch purchases in view of the scarcity in the supply of raw materials and on the production deadlines of the various inputs for the manufacture of its bio-HDL due to COVID 19. Therefore, the company, after having signed its strategic production agreement, has secured the bioproduction of CER-001 vials for its various upcoming trials. CER-001 is now one of the most advanced biomedicines in France, and ABIONYX Pharma controls the entire value chain of its biomedicine, from patents in renal and ophthalmologic diseases to its bioproduction, through the various clinical stages. The company targets a marketing application for CER-001 in LCAT deficiency as soon as possible.

### 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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