Press release

ABIONYX Pharma Receives FDA Orphan Drug Designation (ODD) for CER-001 for the Treatment of LCAT Deficiency Presenting as Kidney Dysfunction and/or Ophthalmologic Disease

- **ODD follows positive results in two compassionate use cases**
- **Published data from compassionate use cases demonstrated for the first time that bio-HDL therapy can reduce lipid deposits in the kidney, slow the decline of kidney function while eliminating the need for dialysis, beneficially remodel lipoproteins, and improve visual impairment due to corneal lipid deposits**
- **Orphan designation provides a new strategy for clinical development of bio-HDL in kidney diseases and ophthalmologic diseases in the US**

Toulouse, FRANCE, March 29, 2022, 5.45pm CEST – **ABIONYX Pharma** (**FR0012616852 – ABNX – PEA PME eligible)**, a new generation biotech company dedicated to the discovery and development of innovative therapies for patients, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan-drug designation (ODD) to the Bio-HDL CER-001 for the treatment of lecithin-cholesterol acyltransferase (LCAT) deficiency. The designation covers both partial LCAT deficiency, presenting as Fisheye Disease, and complete LCAT deficiency presenting with renal symptoms and corneal opacities. Progression of LCAT deficiency, for which there is no approved treatment, can ultimately lead to renal failure requiring dialysis or kidney transplant, and/or to complete corneal opacification requiring transplant.

The European Medicines Administration (EMA) granted ODD status to CER-001 for the treatment of LCATdeficiency in July 2021. Positive clinical results from CER-001 in LCAT disease have previously been published. In the Annals of Internal Medicine in March 2021, a case study of a patient who was about to undergo dialysis due to the rapid decline in renal function was described. The patient was able to avoid the need for dialysis during her treatment with CER-001 and in addition, lipid deposits in her corneas which had caused signficant visual blurring, improved with treatment. The improvement in visual function was still observed after 1 year of follow-up. A second case was described in the Journal of Internal Medicine in November 2021 and showed that CER-001 reduced glomerular lipid deposits and slowed the patient's decline in renal function. Furthermore, CER-001 remodeled his plasma lipoproteins by reducing the level of LpX, large abnormal lipid complexes known to be renally toxic.

"*We are pleased to have received ODD for CER-001 just eight months after the ODD in Europe and one week after the first positive clinical results in COVID-19. These OD designations from the FDA underscore the importance of bringing this important therapeutic option to patients with LCAT Deficiency both as a kidney disease and as an ophthalmic disease*," commented Cyrille TUPIN, CEO of ABIONYX Pharma. "*We look forward to presenting new clinical results in the coming months and providing additional insight into the potential of our Bio-HDL therapy platform in both kidney diseases and ophthalmologic diseases. The ODD paves the way for ABIONYX Pharma to launch a new strategic clinical development of the bio-HDL in kidney diseases and ophthalmologic diseases in the US."*

Orphan-drug designation is granted by the FDA to a drug or biologic intended to treat a rare disease or condition, which generally includes a disease or condition that affects fewer than 200,000 individuals in the U.S. Supporting the development and evaluation of new treatments for rare diseases is a key priority for the FDA. The designation is granted based on the mechanism of action of the drug or biologic taken into consideration with the pathogenesis of the disease or condition, its course and prognosis as well as the availability of treatments and/or resistance to available treatments.

Orphan drug designation qualifies sponsors for incentives including: tax credits for qualified clinical trials, exemption from user fees, and a potential for seven years of market exclusivity after approval.

Next financial press release : **Annual Results, April 28th 2022**

About the Bio-HDL CER-001

CER-001 is the first-in-class bio-HDL mimetic that directly targets a key underlying metabolic defect of LCAT deficiency. The bio-HDL is one of the most advanced biomedicines and is a potential novel treatment for kidney diseases, sepsis or COVID-19, but also for ophthalmologic diseases involving lipid abnormalities. These abnormalities could be modified by pharmacological agents that increase plasma ApoA-I and HDL levels, but more importantly increase the number of functional HDL. The antiinflammatory properties and/or the increase in the Reverse Cholesterol Transport (RCT) of CER-001 can prevent decline in kidney function and improve vision in LCAT patients. Bio-HDL as a biomedicine was found to be completely safe and very well tolerated by more than 600 patients involved in all previous clinical studies.

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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