Press Release

ABIONYX Pharma received positive feedback from the EMA for CER-001 in LCAT Deficiency

- **Acceptability of submitting data from 2 prospective process validation batches for drug substance and drug product manufacturing at the time of Marketing Authorization Application (MAA)**

Toulouse, FRANCE, Lakeland MI, USA, October 21st, 2024, 8:00 p.m. 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, has submitted on July 11, 2024, a formal Application for scientific advice to the European Medicines Agency (EMA) for recombinant human apolipoprotein A-I, CER-001, for treatment of lecithin-cholesterol acyltransferase (LCAT) deficiency. This submission completes the rolling review process, with clinical data and partial Chemistry, Manufacturing, and Controls (CMC) data submitted by ABIONYX Pharma.

ABIONYX Pharma requests whether the Committee for Medicinal Products for Human Use (CHMP) which is the European Medicines Agency's (EMA) committee responsible for human medicines agrees with the proposal to submit, at the time of filing of the CER-001 dossier for granting EU conditional approval for the LCAT deficiency indication, data from 2 prospective process validation batches for Drug Substance and Drug Product manufacturing.

EMA concludes that the proposal to submit data from 2 prospective process validation batches for drug substance and drug product at the time of MAA manufacturing could be acceptable.

As recommended, ABIONYX Pharma will pursue its development plan regarding viral safety, method description and validation specificity till MAA submission.

ABIONYX Pharma has provided CER-001 under named compassionate use to eight patients with LCAT Deficiency in 4 European countries. All patients have now completed six months of treatment. As agreed in previous advice received from the CHMP, these cases will form the clinical basis for the MAA submission.

Based on the scientific advice from the EMA, the Company has now clarified the requirements to initiate the process to submit a MAA.

About CER-001

CER-001 is an engineered HDL particle which contains recombinant human apolipoprotein A-I (apoA-I), complexed with phospholipids. HDL particles have been shown to be highly effective scavengers of bacterial endotoxins, such as lipopolysaccharide (LPS), with the ability to inactivate LPS and target it for removal by the liver. In addition, its significant capacity to uptake cholesterol and lipids accumulated in tissues (particularly kidneys) makes CER-001 a important tool in the treatment of Norum 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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