



Press Release

## **ABIONYX announces the publication of pre-clinical data in the journal Metabolism demonstrating that CER-001 improves lipid profile and kidney function for an ultra-rare kidney disease**

- **Beneficial effects on lipid profile and renal function**
- **Reducing urinary albumin to creatinine ratio and restoring nestin and nephrin content in the glomerulus**
- **A treatment option for patients with ultra-rare kidney disease**

**Toulouse, FRANCE, December 15, 2020, 7:00pm CET – 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 the publication of a set of pre-clinical data in the journal Metabolism demonstrating that CER-001 improves lipid profile and kidney function in an emphasis kidney disease model representative of LCAT (lecithin-cholesterol acyltransferase) deficiency.

CER-001 is an HDL mimetic previously tested in different pathological conditions, but never in a kidney disease prior to ABIONYX Pharma's ATUn.

In a genetically modified mouse model of LCAT deficiency, CER-001 had beneficial effects not only on lipid profile but also on renal function, reducing urinary albumin to creatinine ratio and restoring nestin and nephrin content in the glomerulus.

These new data give us a new opportunity for the use of CER-001 for the treatment of renal diseases. The company will continue to work on strengthening the understanding the mechanism of action of CER-001 in kidney disease.

*“This publication in a reference journal in the field of metabolism encourages us to continue exploring the effect of CER-001. The removal of lipids from the kidney in an experimental model of LCAT deficiency is a major breakthrough. Results are consistent and really support a beneficial effect of CER-001 on the kidney, the most affected organ in human LCAT deficiency.”* said Dr. Laura Calabresi, PhD., Professor of Pharmacology, University of Milano, Department of Pharmacological and Biomolecular Sciences.

This study was designed to investigate the catabolic fate of CER-001, and to evaluate the effects of CER-001 on kidney disease. Wild-type mice received CER-001 for 2 weeks. Plasma lipid/lipoprotein profile and HDL subclasses were analyzed. In a second set of experiments, mice were induced to develop the ultra-rare renal LCAT disease and treated with CER-001. Lipid profile, renal function, and kidney histology were evaluated.

As a conclusion, three major outcomes have emerged from this study:

- Treatment with CER-001 ameliorates the dyslipidemia, reducing plasma triglycerides and increasing HDL levels.
- More importantly, treatment with CER-001 ameliorates renal function in a mouse model with ultra-rare renal disease, reducing urinary albuminuria and restoring podocyte functionality.
- The data set the basis for the potential use of CER-001 in other renal diseases.

This scientific publication, entitled “**CER-001 ameliorates lipid profile and kidney disease in a mouse model of Familial LCAT Deficiency**», is available online in the journal Metabolism: [https://www.metabolismjournal.com/article/S0026-0495\(20\)30328-0/fulltext](https://www.metabolismjournal.com/article/S0026-0495(20)30328-0/fulltext)

ABIONYX is awaiting data from other ongoing preclinical studies.

#### About ABIONYX Pharma

ABIONYX Pharma is a new generation biotech company dedicated to the discovery and development of innovative therapies for patients. The biotech assets inherited from CERENIS Therapeutics constitute a rich portfolio of valuable programs for the treatment of metabolic diseases as well as with a HDL targeted drug delivery platform.

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