Press release

ABIONYX Announces New Positive Clinical Results for CER-001 in kidney disease associated with LCAT deficiency Published in The Journal of Internal Medicine

- **Newly demonstrated efficacy in renal indication**
- **Demonstration of the lipoprotein remodeling capacity of CER-001**
- **Clarification of the mechanism of action of CER-001**

Toulouse, FRANCE, November 15, 2021, 7.30am 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 announces new positive clinical results for CER-001 in kidney disease associated with familial LCAT deficiency (FLD) published in the **Journal of Internal Medicine**, another ranked internal medicine journal.

The worsening of kidney function in the patient was slowed by CER-001 infusions. Kidney biopsy showed a reduction of lipid deposits and a stabilization of the disease. The results demonstrate that CER-001 has the potential to be beneficial in other renal diseases characterized by renal lipid deposits.

As a reminder, compassionate treatment with CER-001 of a named Italian patient with LCAT deficiency was authorized by the Friuli Venezia Giulia Regional Ethical Committee, (Opinion CEUR-2020-EAP-012-ASUFC) in February 2020.

The patient had a homozygous mutation in the lecithin-cholesterol acyltransferase (LCAT) gene and developed an aggressive glomerulopathy requiring three separate kidney transplantations over 20 years. Nine months after the third renal graft, the kidney function had already dropped by half. In this compassionate treatment of renal disease associated with LCAT deficiency, the patient was treated with CER-001, an apoA-I-containing HDL mimetic, to help ameliorate progressive kidney failure.

CER-001 reduces kidney lipid deposits

After 12 weeks of treatment, histological analysis showed a reduction in glomerular lipid deposits, despite the presence of fibrosis and atrophy. The worsening of kidney function was slowed by the treatment. Both albumin-to-creatinine and protein-to-creatinine ratios increased in the first three weeks of treatment, then dropped in the following weeks. Treatment was well-tolerated.

CER-001 remodels plasma lipoproteins reducing LpX

The patient presented with dramatically low HDL-cholesterol, and abnormal prominent large lipoprotein complexes, named LpX, which are known to be toxic to the kidneys. Treatment with CER-001 led to a normalization of the lipoprotein profile, with a decrease of LpX in favour of normal-sized lipoproteins.

Beneficial effect of CER-001 is mediated by lipoprotein remodelling and direct cholesterol removal from kidney cells

To clarify the mechanism(s) of action for the beneficial effect observed with CER-001, in vitro experiments were carried out using podocytes, the kidney cells involved in FLD-induced kidney damage. Incubation of podocyte cells with the patient's plasma collected at different time points before and during CER-001 treatment progressively led to less lipid accumulation in kidney cells, confirming that the drug-induced remodelling of plasma lipoproteins is responsible for the reduced cholesterol deposit in cells.

The present report confirms the beneficial effects on the kidney of the HDL mimetic CER-001 previously observed in the case of LCAT deficiency in France and provides novel insights into the mechanisms of kidney function stabilization exerted by the drug.

The beneficial effect is mediated by at least a dual action of CER-001, which directly effluxes cholesterol from podocytes, but also induces normalization of plasma lipoproteins by a still unknown mechanism, thus reducing toxic lipid deposits in the kidney.

Dr. Laura Calbresi states: "*These positive clinical data demonstrate again that CER-001 prevented significant decline in kidney function. FLD is a rare dyslipidaemia with severe kidney complications, presently with no cure. CER-001 may represent a therapeutic option, with the objective of delaying end-stage renal disease. CER-001 also has the potential to be tested in more common kidney diseases characterized by kidney lipid deposits.*"

Data presentations, including images of kidney biopsies before and after CER-001 treatment, can be found in the article at onlinelibrary.wiley.com

About Journal of Internal Medecine

The Journal of Internal Medicine (Impact Factor: 8.989) is an international peer-reviewed scientific journal in continuous publication since 1863. JIM publishes original work in clinical science from bench to bedside covering a broad field of internal medicine and its subspecialties. JIM supports and organises scientific meetings in the form of symposia within the scope of the journal.

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.

Contacts:

NewCap
Investor relations
Louis-Victor Delouvrier
abionyx@newcap.eu
+33 (0)1 44 71 98 53

NewCap
Media relations
Nicolas Merigeau
abionyx@newcap.eu
+33 (0)1 44 71 94 98