Press release

ABIONYX Announces Positive Clinical Results From CER-001 in an ultrarare kidney disease Published in The Annals of Internal Medicine

- **Demonstrated efficacy in renal and ophthalmic indication**
- **Confirmation of the breakthrough therapy status of CER-001 in kidney and ophthalmic diseases**
- **Systemic mechanism of action of CER-001**

Toulouse, FRANCE, March 2, 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 positive clinical results from CER-001 in an ultrarare kidney disease published in the **Annals of Internal Medicine**, the highest cited and ranked internal medicine journal.

The patient, who was about to undergo dialysis due to the rapid decline in renal function, was able to avoid the need for dialysis during his treatment with CER-001. In addition, the patient who was suffering from lipid deposits in the corneas saw the visual blurring disappear. This clear improvement in visual function is still observed after 1 year of follow-up.

As a reminder, the French Drug Safety Agency (Agence Nationale de Sécurité du Médicament or ANSM), granted a named patient Temporary Authorization for Use ("ATU nominative") for CER-001 in an untreated, ultra-rare renal disease early 2020.

The patient with inherited mutations in the lecithin-cholesterol acyltransferase (LCAT) gene developed glomerulopathy and corneal lipid deposits and displayed very low circulating levels of high-density lipoprotein (HDL) and apoA-I. In this ATUn granted in the familial LCAT deficiency disease the patient was treated with CER-001, an apoA-I-containing HDL mimetic, to help in preventing rapidly progressive kidney failure.

CER-001 was completely safe and very well tolerated at doses of 10-30 mg/kg/week

CER-001 was given intravenously at a dose of 10 mg/kg thrice a week for 3 weeks, then twice a week for 3 weeks and once a week for 3 weeks. Thereafter, the dose was increased to 20 mg/kg/week for 6 weeks to reach the weekly dose that stabilized eGFR.

Whereas eGFR rapidly decreased from 41 to 19 mL/min/1.73 m^2 during the 9 months that preceded the start of CER-001, eGFR only decreased from 19 to 17 mL/min/1.73 m^2 during the 11 months following treatment introduction.

Urinary protein-to-creatinine ratio (uPCr) decreased from 7 to 0.25 g/g at day 10, with undetectable albuminuria at that time, but returned to initial values thereafter. However

nephrotic syndrome disappeared with serum albumin increased from 29 to 37 g/L during the treatment period and then the follow-up.

The publication states that CER-001 prevented further kidney function decline. The patient who was about to be dialysed due to rapidly declining kidney function, was able to avoid the need for dialysis during their treatment with CER-001.

No other treatment was introduced strongly suggesting that stabilization of the renal function relied on the administration of CER-001.

The publication confirms the breakthrough therapy status of CER-001 in kidney diseases

The publication mentions that CER-001 effects on renal progression should be assessed in more common proteinuric diseases, including diabetic nephropathy or extra-membranous glomerulonephritis (two diseases characterized by a reduction in local LCAT activity, gloemrular lipid deposits, and inflammatory processes) , or other nephropathies related to lipid deposits.

The publication reveals the systemic mechanism of action of CER-001 and broadens the field of breakthrough innovations to ophthalmology

The publication mentions positive extra-renal clinical results and the disappearance of visual blurring secondary to corneal deposits. This clear improvement in visual function is still observed after 1 year of follow-up.

The clear improvement of the blurred vision at the end of the follow-up suggests that the anti-inflammatory properties and/or the increase in the Reverse Cholesterol Transport (RCT) of CER-001 can improve vision in FLD patients. This finding and previous data showing the role of apoA-I in the development of corneal clouding and blurring vision will pave the way to interventional studies testing CER-001 in patients developing lipid corneal deposits from other pathologies such as secondary lipid keratopathy or inherited corneal dystrophy.

Prof. Stanislas Faguer, nephrologist at Department of Nephrology and Organ Transplantation Reference Center for Rare Kidney Diseases, Hospital Rangueil, CHU Toulouse, states :" *These positive clinical data demonstrate that CER-001 prevented significantly kidney function decline. ApoA-I containing HDL mimetic CER-001 is the first treatment that has proven its ability to slow the progression of renal failure and reduce visual discomfort secondary to corneal lipid deposits in a patient with FLD. We look forward to conducting new clinical studies of CER-001 to assess its ability to improve the prognosis of other orphan or common kidney diseases for which no effective treatment is currently available."*

Cyrille Tupin, Managing Director of ABIONYX Pharma concludes :" *These positive clinical data published in one of the most cited medical journals prove the efficacy of our bioproduct and reveal the relevant repositioning of CER-001 in a severe renal indication where there has been no breakthrough therapeutic innovation in the last 10 years. This represents an important step forward in the clinical development of CER-001. I would like to warmly thank Professor Faguer for his major contribution and all the teams at the Toulouse University Hospital for their continued collaboration. We are very grateful to the French National Agency for Drug Safety for accepting Prof. Faguer's request for ATUn, as well as to the patient who fully supported the therapeutic project that led to this discovery. The ABIONYX team continues to explore other potential medical benefits associated with the CER-001, an HDL mimetic. We are committed to advancing the clinical development of the mimetic HDL as quickly and safely as possible while investigating new indications in ophtalmology so that we can help address new rare or orphan diseases with no existing treatment* ».

Data tables can be found in the article https://www.acpjournals.org/doi/10.7326/L20-1300

About Annals of Internal Medecine

Annals of Internal Medicine is an academic medical journal published by the American College of Physicians (ACP). It is one of the most widely cited and influential specialty medical journals in the world. The most recent (2019) Impact Factor for Annals of Internal Medicine is **21.317** (Clarivate Analytics). According to the new 2019 Journal Citations Reports from Clarivate Analytics, Annals is the highest cited and ranked internal medicine journal in the category of Medicine, General and Internal.

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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