



Press release

ABIONYX initiates a Phase 2a clinical trial with CER-001 in septic patients at high risk of developing Acute Kidney Injury

- This partnered clinical study with the University of Bari and the CBVF foundation is already fully funded
- Temporary Authorization for Named Use (ATUn) showed promising efficacy in renal diseases
- Evaluation of the clinical activity by dosage level of CER-001 in the prevention of Acute Kidney Injury in ICU patients with septicemia
 - A potentially modifying effect on the progression of the inflammatory cascade in sepsis
- Pending positive data, the company aims to move into Phase 2b by end of 2021

Toulouse, FRANCE, December 23, 2020, 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 that it has received authorization from the Italian authorities to launch a clinical trial named **RACERS** (a **RA**ndomized study comparing short-term **CER-001** infusions at different doses to prevent **Sepsis-induced acute kidney injury**) with CER-001 in septic patients at high risk of developing acute kidney injury.

Following the positive signals observed in the Temporary Authorization for Named Use (ATUn) in an ultra-rare kidney disease, the study will assess the role of CER-001, a HDL-mimetic, in preventing Acute Kidney Injury (AKI) in septic patients.

The core component of the program will be the launch of a 30-day Phase 2a clinical dose-finding trial with the Company's lead product candidate, CER-001, in the prevention of AKI in septic patients.

Researchers have demonstrated that in humans, reconstituted HDLs have a scavenger role in reducing circulating endotoxin, as well as major anti-inflammatory and endothelial activity¹.

¹Pajkrt, D. *et al.* Anti inflammatory effects of reconstituted high-density lipoprotein during human endotoxemia. *J. Exp. Med.* **184**, 1601–1608 (1996) and Birjmohun, R. S. *et al.* High-density lipoprotein attenuates inflammation and coagulation response on endotoxin challenge in humans. *Arterioscler. Thromb. Vasc. Biol.* **27**, 1153–1158 (2007).

These important effects were also demonstrated with CER-001 in a rigorous animal model of sepsis-induced AKI. Several other AKI/sepsis models showed that HDL is a critical factor in modifying the disease.

This clinical study, designed in concert with expert Italian nephrologists (nephrology Dialysis and Transplantation Unit, Chief: Prof. Loreto Gesualdo) and intensivists (Anesthesiology and Resuscitation Unit, Chief: Prof. Salvatore Grasso), will be a randomized, open labelled, placebo-controlled, parallel-group study evaluating the safety and efficacy of intravenously administered CER-001 in ICU patients with sepsis at high risk for AKI based on their Sequential Organ Failure Assessment (SOFA score). A total of 20 patients will be randomized to receive 8 doses of CER-001 or placebo over 6 days. The primary endpoint of the study will be the onset and severity of AKI according to KDIGO criteria as well as safety and tolerability of the dosage regimens in order to select the optimal dose of CER-001.

The secondary endpoints will include changes in endotoxin and IL-6 levels from baseline to Day 3, Day 6 and Day 9, changes in the SOFA score from baseline to Day 3, Day 6 and Day 9, changes in other key inflammatory markers (e.g., CRP, IL-8, MCP 1 and TNF- α), and changes in AKI biomarkers (TIMP-2 and IGFBP7).

Enrollment of patients in the study is expected to begin in the first half of 2021. The clinical study is partnered with the University of Bari and the Consorzio per Valutazioni Biologiche e Farmacologiche foundation (CBVF) and is already fully funded.

"We are pleased to expand our work on CER-001 into the clinical stage," said Professor Loreto Gesualdo, full Professor, Head of the Nephrology, Dialysis and Transplantation unit, University of Bari Aldo Moro, Italy. "It is important to note that the action of CER-001 could have a significant disease-modifying effect on the progression of the disease, by addressing endotoxins, renal remodeling and anti-inflammatory activities. Based on the preclinical HDL data to date, we believe that CER-001 could be an effective treatment for sepsis-induced AKI. Significant CER-001 activity has already been demonstrated in a validated swine model and we look forward to further evaluating this promising candidate in RACERS clinical study."

"We look forward to initiating this Phase 2a trial which re-launches and extends the clinical evaluation of CER-001 to a new metabolic pathology," said Connie Peyrottes, Senior VP clinical development at ABIONYX Pharma. "There is a tremendous medical need for effective therapies to prevent AKI in patients with sepsis. Given its excellent tolerability in clinical use to date, we believe that CER-001 could become an important therapeutic option for these patients. We would like to thank all of our collaborating disease experts, the University of Bari and the CBVF foundation for their commitment and support in conducting this strategic research program. "

About "Consorzio per Valutazioni Biologiche e Farmacologiche" (CVBF): cbvf.net

The "Consortium for Biological and Pharmacological Evaluations" (CVBF) provides scientific, methodological and regulatory support to European entities and companies willing to innovate in the pharmaceutical and biotechnological fields. The life sciences are the main area of interest, with activities including planning the development of innovative drugs for particular populations (rare diseases and pediatrics), research management, conducting clinical trials and providing ethical and regulatory advice.

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