Press release

ABIONYX Pharma Reports Positive Results from Phase 2a Pilot Clinical Trial Evaluating CER-001 in the Treatment of Septic Patients at High Risk of Developing Acute Kidney Injury

- **Positive results on primary and secondary endpoints, identifying dose for future development**
- **Direct and significant effect of CER-001 on endotoxin removal and consequent reduction in the inflammatory cascade or "cytokine storm"**
- **Significant protective effect of CER-001 on endothelial functionality**
- **Trends towards fewer ICU days for patients treated, lower requirement for organ support and improved 30-day survival were also seen**
- **Trial reinforces the well-established safety profile of CER-001**
- **Efficacy results consistent with those observed in COVID-19**

Toulouse, FRANCE, Lakeland, USA, January 16th, 2023, 12.00 pm 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 reported that the pilot Phase 2a clinical trial evaluating CER-001, the only natural recombinant apoA-I, as a treatment for septic patients at high risk of developing Acute Kidney Injury (AKI) met its primary objective. There are no approved treatments for septic patients in the world.

Loreto Gesualdo, full Professor, Head of the Nephrology, Dialysis and Transplantation Unit, University of Bari Aldo Moro, Italy, and lead investigator of the RACERS study stated: "*We are incredibly excited to share results from this pilot Phase 2a trial evaluating CER-001 in septic patients at high risk of developing acute kidney injury. There is a complete lack of treatment options for septic patients at high risk of developing acute kidney injury, a disease that in 2019 had an estimated 13.7 million related deaths globally (Lancet 2022; 400: 2221–48). The trial shows promising positive results across a variety of primary and secondary endpoints. CER-001 was significantly able to scavenge endotoxins, modulate the cytokine storm, and provide endothelial protection. The trend observed in reducing renal damage, the need for organ support and ICU-day stay underscores the potential clinical significance of these results.*"

The RACERS study included 20 patients with gram-negative sepsis who were at high risk for acute kidney injury due to high levels of endotoxin activity and decline in function of one or more organ systems. Patients received either standard of care treatment alone, or in combination with one of three dosage regimens of CER-001 (five patients per group).

The main objective of this pilot study was to investigate whether the use of CER-001 at different doses, in combination with standard of care (SOC) treatment, is safe and effective, providing a potential new strategy to treat septic patients, reducing the inflammatory response to endotoxin and preventing the progression to AKI according to KDIGO (Kidney Disease: Improving Global Outcomes) criteria, as well as safety and tolerability of the dosage regimens in order to select the optimal dose of CER-001.

One of the metabolic characteristics of bacterial (like sepsis) or virus infections (like sars-Cov-2) is the strong decrease of circulating lipoprotein and particularly the High-Density Lipoprotein (HDL) with its main containing protein apolipoprotein A-I (apoA-I). As an example, apoA-I level was recently described as the biomarker predictive of long-term mortality after surgical sepsis[1]. The rational of Abionyx was to restore, using CER-001, the apoA-I levels to reestablish all the functionality of this individualized biomarker leading to potential benefit in sepsis pathology.

RACERS pilot study has shown for the first time in a human pilot trial that the recovery of a normal apoA-I level in patient stop the cytokine storm and improve the clinical outcomes. CER-001 demonstrated rapid and sustained reduction in endotoxin levels and consequent reduction in the inflammatory cascade or "cytokine storm" relative to SOC alone. Endothelial biomarkers demonstrated a significant protective effect of CER-001. Trends towards fewer ICU days, lower requirement for organ support and improved 30-day survival.Evaluation of safety data, taken together with the pharmacokinetic and pharmacodynamic data, has identified the dose that will be used in subsequent studies.

Connie Peyrottes, Senior VP clinical development at ABIONYX Pharma, added: *"In this pilot study, CER-001 was shown to directly decrease endotoxin and inhibit inflammation, limiting the associated downward spiral that septic patients often experience. The broad pleiotropic effect of our apoA-I bioproduct can target multiple facets of septic disease, rather than focusing on a single step in the inflammatory process. Primary and secondary endpoints showed benefits of CER-001 therapy when added to standard treatments. The positive results from this Phase 2a trial show CER-001 has the potential to be a gamechanger for critical illnesses marked by inflammation and organ failure across different high mortality clinical indications which continue to have high unmet medical needs."*

Michael Davidson, M.D., Chairman of the Scientific Advisory Board of ABIONYX concluded: *"The impressive results of this Phase 2a study confirm that CER-001 may potentially treat sepsis and other severe, acute inflammatory diseases. These results are consistent with the previously published results in COVID-19 and opens a new chapter in the development of CER-001 in the field of short-term therapy for acute conditions."*

The observed safety and efficacy in RACERS were generally consistent with historical data including clinical results for CER-001 in COVID-19 that were published recently in the scientific journal "Frontiers in Medicine", a specialty medicine journal, in September 2022.

The potential use of CER-001 in septic patients is currently under clinical development. These data will be discussed with regulatory authorities, starting with Europe but also the U.S. later this year in order to design an appropriate clinical and regulatory development strategy for this disease state that currently has no available treatment options.

[1] Guirgis, Faheem W.,et al. *Annals of Intensive Care* 11, nº 1 (décembre 2021): 82. https://doi.org/10.1186/s13613-021-00865-x.

About RACERS

RACERS is a clinical trial named RACERS (a RAndomized 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 assessed the role of CER-001, a novel, in preventing Acute Kidney Injury (AKI) in septic patients. The core component of the program is 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 activity. These important effects were also demonstrated with CER-001 in a rigorous preclinical model of sepsis-induced AKI developed in collaboration with an Italian Veterinarian Hospital (Surgical Section, Chief: Prof. Antonio Crovace). 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), was a randomized, open labelled, placebo-controlled, parallel-group study evaluating the safety and efficacy of intravenously administered CER-001 in patients with sepsis at high risk for AKI based on their endotoxin levels and Sequential Organ Failure Assessment (SOFA score). A total of 20 patients were randomized to receive 8 doses of CER-001 over 6 days on top of standard of care, or standard of care alone. The primary endpoint of the study was 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 clinical study was partnered with the University of Bari.

About CER-001 in COVID

The complete data are available in the article: “Apolipoprotein-A-I for severe COVID-19-induced hyperinflammatory states: A prospective case study”

https://www.frontiersin.org/articles/10.3389/fphar.2022.936659/full

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.

Contacts:

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

NewCap
Media relations
Arthur Rouillé
abionyx@newcap.eu
+33 (0)1 44 71 00 15