Press Release

ABIONYX announces the observation of positive therapeutic signals in Temporary Authorizations for Use (ATUn) in France and Italy for an ultra-rare kidney disease

- **Excellent tolerability of CER-001**
- **A potential modifying effect on the progression of a kidney disease without existing treatment**
- **A treatment option for patients with the ultra-rare kidney disease**

Toulouse, FRANCE, November 25, 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 announces the observation of positive therapeutic signals in named patient Temporary Authorizations for Use (ATUn), using CER-001 in patients with an ultra-rare kidney disease.

Based on the positive therapeutic signals detected in in-depth post-hoc analyses, ABIONYX confirms that the data set of the two ATUn granted in France and Italy supports a promising therapeutic activity of CER-001 in the progression of an ultra-rare renal disease. Due to the severity of their renal disease, patients who were about to be dialysed due to rapidly declining kidney function, were able to avoid the need for dialysis during their treatment with CER-001. These therapeutic signals on CER-001 confirm the potential for CER-001 to be used in severe indications, mainly renal at the moment.

In studies currently being considered for publication by peer-reviewed journals, researchers have shown that HDLs have a major anti-inflammatory role and impact on kidney function. These important effects were demonstrated in a genetically modified mouse model of kidney failure. Several studies of other models of renal pathology have previously shown that HDLs stimulate renal remodeling, a critical factor contributing to disease progression.

As a reminder, last January and February, ABIONYX received requests to provide CER-001 in France and Italy under named patient Temporary Authorizations for Use (ATUn) in an ultra-rare renal disease without existing treatment. These ATUn were granted at the request and under the sole responsibility of prescribing physicians, and are usually requested when the drug is likely to be of benefit to the patient.

Under the two named patient Temporary Authorizations for Use (ATUn) in France and Italy for the ultra-rare renal disease without existing treatment, CER-001 was administered chronically but with a frequency that decreased during the course of treatment in accordance with the protocol chosen by the prescribing physicians.

ABIONYX has conducted an in-depth analysis of all data that will be included in the publications and will share these results with regulatory authorities in order to redefine the development plan for CER-001 in the treatment of renal diseases without existing treatment. ABIONYX will work closely with patients, investigators, ethics committees and regulatory authorities to identify the next steps to be taken, in the best interest of patients, while ensuring the consent of all stakeholders.

These ATUn data do not allow a definitive conclusion on the efficacy endpoint at this time, but are likely to be of benefit to patients with severe kidney disease without existing treatment.

Cyrille Tupin, CEO of ABIONYX, concludes: *"We are extremely grateful to the patients, their families and the nephrology professors and physicians and all healthcare professionals involved in these named patient Temporary Authorizations for Use (ATUn) for their support and trust in the company. We are more than ever committed to the continued development of our biotech assets and the development of drugs for kidney diseases without effective treatment, where medical needs remain very high".*

Finally, 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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