Press Release

ABIONYX Pharma announces its 2021 half year financial results and provides an update on its development activities

- **Orphan Drug Designation status granted for CER-001 for Lecithin Cholesterol Acyltransferase (LCAT) Deficiency as both a renal disease and an ophthalmic disease**
- **Progress in the fully-funded Phase 2a clinical trial, RACERS, evaluating CER-001 in sepsis at high risk of developing acute kidney injury**

Toulouse, FRANCE, Lakeland MI, UNITED-STATES, September 9, 2021, 6.45 pm CEST – **ABIONYX Pharma (FR0012616852 - ABNX - PEA PME eligible),** a next-generation biotech company dedicated to the discovery and development of innovative therapies, announces its 2021 half year financial results and provides an update on its development activities.

Orphan Drug Designation status granted for CER-001 for LCAT Deficiency as both a renal disease and an ophthalmic disease

Following the receipt of a positive opinion from the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) regarding the Orphan Drug Designation application for its bio-HDL, the company has obtained the Orphan Drug Designation (ODD) from the European Commission for its drug candidate CER-001, as a potential treatment for LCAT deficiency. Two forms of LCAT deficiency exist: Familial LCAT Deficiency (FLD) resulting from a complete deficiency and clinically characterized by hemolytic anemia, renal failure, most often leading to renal transplantation, and by corneal opacities; and Fish Eye Disease (FED), resulting from a partial deficiency and clinically characterized by corneal opacities without renal involvement. The Orphan Drug Designation obtained therefore covers both a renal and an ophthalmologic indication.

Obtaining the ODD allows for assistance from the EMA with clinical protocol design, access to a centralized marketing authorization procedure valid in all EU member states, the possibility for conditional marketing authorization, reduced regulatory fees, and 10 years of market exclusivity from the time a marketing authorization is granted. In addition to the development advantages of obtaining the ODD, this designation marks a strong and strategic regulatory recognition as the only bio-HDL to receive this status in Europe.

The efficacy of CER-001 was announced last March when positive clinical results were published in the scientific journal “Annals of Internal Medicine” in this rare kidney disease. As a reminder, the patient who was on the verge of dialysis due to the rapid decline of his kidney function was able to avoid the need for dialysis during treatment with CER-001. In addition, the patient who suffered from lipid

deposits in the corneas experienced the disappearance of visual blur, a clear improvement in visual function that continued to be observed after 1 year of treatment-free follow-up.

Progress in the Phase 2a clinical trial, RACERS, evaluating CER-001 in sepsis at high risk of developing acute kidney injury

Following the announcement last June of the enrollment of the first patient in the Phase 2a clinical trial evaluating CER-001, the Bio-HDL, as a potential treatment for patients with sepsis at high risk of developing acute kidney injury, RACERS, the study, evaluating the safety and efficacy of CER-001 in a total of 20 patients, continues to advance. The primary endpoints of the study will be the onset and severity of acute kidney injury according to KDIGO criteria, as well as the safety and tolerability of dosing regimens, in order to select the optimal dose of CER-001. The clinical study is being conducted in partnership with the University of Bari and the Consorzio per Valutazioni Biologiche e Farmacologiche (CBVF) and is already fully funded.

Selected Financial Information *(at June 30 / IFRS Consolidated accounts)*

Millions €	H1 2021	H1 2020
Sales	0.03	0
R&D expenses	(2.03)	(0.41)
Administrative and commercial expenses	(0.58)	(0.63)
Operating income	**(2.59)**	**(1.04)**
Financial products	0.08	0.19
Financial expenses	(0.04)	(0.07)
Financial Result	**0.04**	**0.12**
Net income	**(2.55)**	**(0.92)**
Net income per share (€)	**(0.10)**	**(0.04)**
Net cash flow from operating activities	(4.27)	0.57
Net cash flow from investing activities	(0.01)	(0.02)
Net cash flow from financing activities	(0.05)	(0.07)
(Decrease) / Increase in cash position	**(4.32)**	**0.48**
Cash and cash equivalents at end of period	**4.83**	**8.81**

Details of the main changes in the consolidated financial statements

ABIONYX Pharma recorded its first sales of €27 K in Q1, corresponding to the supply of CER-001 for the treatment of a patient for one month in the context of a new ATUn, following positive results obtained in March 2021 in the treatment of LCAT deficiency in a patient in France.

The increase in research and development expenses, which amounted to €2,029 K for the period, compared to €412 K in the first half of 2020, corresponds mainly to the evolution of subcontracting and consulting expenses which amounted to €1,584 K. This increase is mainly related to the launch of a new production campaign for the CER-001 bio product, initiated at the end of 2020, and the restart of new R&D activities.

General and administrative expenses amounted to €584 K as of June 30, 2021, compared to €629 K as of June 30, 2020. After taking these expenses into account, the operating profit went from a loss of €1,041 K as of June 30, 2020 to a loss of €2,586 K as of June 30, 2021. The financial income shows a surplus €41 K in the first half of 2021, compared to the €121 K income recorded for the first half of 2020. The net result shows a loss of €2,546 K as of June 30, 2021, compared to a loss of €920 K as of June 30, 2020. Cash and cash equivalents amounted to €4.8 million on June 30, 2021.

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.

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