Press release

Annual results 2021

- **Cash position of €7.9 million as of December 31, 2021**
- **Positive clinical results throughout the year**
- **Strategic partnership with IRIS Pharma to accelerate development in ophthalmology**

Toulouse, FRANCE, Lakeland, USA, April 28th, 2022, 6.00 pm CEST – **ABIONYX Pharma** (**FR0012616852 – ABNX – PEA PME eligible)**, a new generation biotech company dedicated to the discovery and development of innovative therapies for patients, today presents its annual results 2021 and provides an update on activity to date. Audit procedures on the consolidated financial statements have been carried out. The certification report will be issued after completion of the procedures required for the filing of the universal registration document.

Selected financial information

(*as of 31 December 2021/Consolidated financial statements under IFRS*)

Millions €	2021	2020
Revenue	0.7	0
Cost of goods and services sold	(0.4)	0
R&D expenditures	(3.8)	(1.7)
Administrative, sales and marketing expenses	(2.3)	(1.3)
Other income and expenses	(0.1)	
Operating income	**(5.9)**	**(3.0)**
Financial income	0.3	1.2
Financial expense	(0.2)	(0.1)
Net financial items	0.1	**1.1**
Net income	**(5.8)**	**(1.9)**
Net cash flows related to operating activities	(6.7)	(0.6)
Net cash flow from investing activities	1.3	(0.1)
Net cash flows related to financing activities	4.0	1.5
Cash position variation	**(1.4)**	**0.8**
Cash and cash equivalents at the end of the period	**7.9**	**9.2**

Fiscal year 2021 was marked by the acquisition, on December 3, 2021, of 100% of the shares of IRIS Pharma Holding, which itself holds 100% of the shares of IRIS Pharma, a company recognized as one of the world's experts in pharmacokinetics and preclinical research within the ophthalmology community.

Details of the main changes in the consolidated financial statements

ABIONYX Pharma's activities being dedicated to the discovery and development of innovative therapies to improve the lives of patients, generated a revenue of €27 K during the fiscal year 2021.

Following the acquisition of Iris Pharma, the Group mainly provides two types of services:

- Pre-clinical activities, for a revenue of €143 K since the acquisition
- Clinical activities representing a revenue of €505 K since the acquisition.

Costs of goods and services sold amounted to €416 K in 2021, corresponding to the costs associated with the pre-clinical and clinical studies carried out in December by Iris Pharma, which has been included in the scope of consolidation since the purchase of IRIS Pharma Holding shares.

Research and development expenditures amounted to €3,838 K over the period, compared to €1,698 K in fiscal year 2020, and correspond to the ramp-up of clinical studies in renal indications and ophthalmology and the increase in personnel costs, in particular due to the recruitment of employees for ophthalmology-related activities. However, these costs remain limited in view of all the clinical trials conducted and in progress in LCAT, sepsis and COVID-19 in 2021. As a reminder, the Phase 2a study was entirely funded by the Italian CBVF consortium.

Administrative, sales and marketing expenses amounted to €2,336 K in 2021 compared to €1,270 K the previous year. This increase is explained by the growth in activity linked to the integration of IRIS Pharma and by expenses linked to the free share grants on plans that have been definitively awarded and those that have been granted.

After taking all these factors into account, **operating income** fell from a loss of €2,968 K at 31 December 2020 to a loss of €5,952 K at 31 December 2021.

The **financial income** amounts to €130 K on December 31, 2021 against €1,082 K on December 31, 2020, which included the waiver of debt granted by Bpifrance for €900 K€ on the CER-209 program.

The **net income** thus shows a loss of €5,822 K on December 31, 2021 compared to a loss of €1,886 K on December 31, 2020.

Cash and cash equivalents amounted to 7,935 K€ on December 31, 2021 against 9,154 K€ on December 31, 2020.

Key highlights in 2021

Despite the continuation of the pandemic and for a managed cash burn in view of the tangible results delivered, the year 2021 has been very rich in transforming strategic events and positive clinical results.

Initiation of the randomized Phase 2a study named RACERS which concluded with positive interim clinical results

The first highlight of the year was the start of the randomized Phase 2a study called RACERS, a RAndomized trial comparing short-term infusions of CER-001 at different doses to prevent induced Acute Kidney Injury in high-risk septic patients. This clinical study is being conducted in partnership with the University of Bari and the Consorzio per Valutazioni Biologiche e Farmacologiche (CBVF) consortium, which is funding the entire study. The first patient could only be enrolled in June 2021 due to the pandemic. In April 2022, the Company reported positive interim results for this Phase 2a clinical trial in the treatment of patients with sepsis that demonstrate rapid reversal of the cytokine cascade in sepsis patients, rapid improvement in biomarkers of inflammation, including leukocytosis, compared to standard therapy. No treatment-related side effects were observed during the study.

Positive Clinical Results from CER-001 in an ultrarare kidney disease

In March 2021, ABIONYX announced positive clinical results from CER-001 in an ultra-rare kidney disease published in exclusively in the journal *Annals of Internal Medicine*. This publication revealed the efficacy of CER-001 in the renal and ophthalmic indication, which constitutes a breakthrough therapeutic innovation in renal diseases and ophthalmology, and the systemic mechanism of action of CER-001. As a reminder, the patient who was about to undergo dialysis due to the rapid decline in kidney 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.

Strategic partnership with GTP Biologics (Fareva Group) and V-Nano (VBI Therapeutics group)

At the end of March 2021, ABIONYX announced the signature of a strategic partnership with GTP Biologics (Fareva Group) and V-Nano (VBI Therapeutics Group) for the bioproduction of the bio-HDL in France. This strategic partnership allows the bioengineering redevelopment of the production of bio-HDL and ensures the relocation of the bioproduction to France.

A “raison d’être” stated in its articles "To develop innovative therapies in indications without effective or existing treatment, even the rarest ones, for the benefit of patients "

In June 2021, following these first scientific publications and the graceful availability of its bioproduct in rare diseases, ABIONYX Pharma communicated its “raison d'être”, which was stated in its articles: "To develop innovative therapies in indications with no effective or existing treatment, even the rarest ones, for the benefit of patients”. This determination has allowed the remobilization of all ABIONYX Pharma's collaborators, suppliers and shareholders around the common objective of developing innovative therapies and fundamental responsibility towards patients waiting for solutions in the rarest diseases.

Orphan Drug Designation from EMA for CER-001 in the kidney indication and ophthalmology

In July 2021, ABIONYX Pharma received a positive opinion from EMA within the framework of the the Orphan Drug Designation process for CER-001 for the rare disease LCAT deficiency. This Orphan Drug Designation was one of the first achievements of the new strategy in a rare renal disease and was a key step in the repositioning in renal and ophthalmological diseases. This designation also allowed the securing of the bioproduction of CER-001.

Strategic partnership with IRIS Pharma for the development of the first class of bioproducts in ophthalmology

In October 2021, ABIONYX Pharma announced positive preclinical results in a model of uveitis that allowed the Company to consider the strategic development of the first class of bioproducts in ophthalmology based on its bio-HDL. These preclinical results led to the initiation of strategic exchanges with IRIS Pharma, one of the world leaders in preclinical and clinical research in ophthalmology, which concluded in November 2021 with the signature of an agreement for the contribution of 100% of IRIS Pharma Holding's capital and the launch of a €4.2 million cash capital increase by private placement. ABIONYX has thus become a specialist in bioproduct candidates for ophthalmology in addition to renal diseases, with a potential portfolio of 3 new bioproducts candidates that could enter the clinical phase and 14 indications in ophthalmology. IRIS Pharma has become a subsidiary of ABIONYX and remains independent in its service activities for the largest pharmaceutical and biotech groups in ophthalmology.

New positive clinical results for CER-001 in ultra-rare kidney disease

In November 2021, ABIONYX Pharma announced new positive clinical results for CER-001 in renal diseases associated with LCAT deficiency published in the *Journal of Internal Medicine*. This publication highlighted the efficacy in the renal indication, demonstrated the ability to normalize lipoproteins and further clarified the mechanism of action of CER-001.

An equally tight and strategic beginning to 2022

The beginning of 2022 remains equally eventful as in January 2022, ABIONYX Pharma received a Compassionate Access Authorization from the ANSM for its bio-HDL (CER-001) in COVID-19, in which it was able to announce in March 2022 first clinical results published in the journal *Biomedecines*, demonstrating that CER-001 limits the effects of inflammation, improves reverse cholesterol transport and reduces inflammatory markers and cytokines.

At the end of March 2022, ABIONYX Pharma announced that the Food and Drug Administration (FDA) had granted orphan drug designation (ODD) to CER-001 for the treatment of LCAT deficiency, in renal dysfunction and/or ophthalmic disease. The orphan drug designation follows positive results in two compassionate use cases that demonstrated for the first time that bio-HDL treatment can reduce lipid deposition in the kidney, slow the decline in kidney function while eliminating the need for dialysis, beneficially remodel lipoproteins, and alleviate visual impairment due to corneal lipid deposits. The orphan drug designation opens a new strategy for the clinical development of bio-HDL in renal and ophthalmic diseases in the United States.

All of the preclinical and clinical results for 2021 and the beginning of this year point to an acceleration of the development of CER-001 in severe renal diseases, which have not seen a breakthrough innovation for a long time, and in ophthalmology with the first class of bioproducts. The Company continues to await further preclinical and clinical results.

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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