Press release

ABIONYX Pharma announces its strategy in ophthalmology and new positive preclinical results for the deployment of two innovative technology platforms: apotherapy and biovectorization

- **Very promising preclinical results of the apoA-I biovector in an acute inflammation model**
- **Development of safe corticoids to limit traditional corticoid-related side effects when used in AMD or DME**
- **Project to separate the ophthalmology activities and change the name of IRIS Pharma Holding to APOGEYE Pharma**

Toulouse, FRANCE, Lakeland MI, USA, March 31, 2022, 6:45 p.m. 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 announced today announced its strategy in ophthalmology and new positive preclinical results in two innovative technological platforms: apotherapy and biovectorisation.

Cyrille TUPIN, Chief Executive Officer of ABIONYX Pharma, said: "*The acquisition of IRIS Pharma, one of the world leaders in Contract Research in ophthalmology, a little over a year ago, has borne fruit and has enabled us to structure a value-creating strategy based on our flagship asset, the only natural recombinant apoA-I lipoprotein in the world and one of the most advanced biomedicines. Indeed, studies with IRIS Pharma have determined the deployment of a strategy based on two technological platforms: apotherapy that means an innovative therapy based on our natural recombinant apoA-I alone, and biovectorization meaning incorporation of various active ingredients into our proprietary apoA-I complex used as a vector. The preclinical results of our biovector in ophthalmology are very promising, especially for corticoids, in order to optimise their efficacy while limiting their side effects, which are one of the major concerns in ophthalmology today worldwide.*"

Two innovative technological platforms: apotherapy and biovectorization

ABIONYX Pharma is developing two innovative technological platforms: apotherapy only based on the apoA-I and biovectorization using apoA-I complexes as a vector to deliver several active ingredients. These two platforms have multiple possible applications in ophthalmology. ABIONYX Pharma has chosen to focus its apotherapy approach initially on the ultra-rare LCAT disease, known as Norum's disease, and on uveitis. ABIONYX Pharma's drug candidates for LCAT and uveitis, CER-001 and ABNX-100, respectively, are intended to be administered as systemic intravenous injections to target patients with corneal opacity or ocular inflammation in the setting of uveitis to achieve significant functional

visual improvement. ABIONYX Pharma's most advanced drug candidate in ophthalmology, CER-001, targeting Fish-Eye Diseas in LCAT Deficiency, is currently being used under compassionate approval in Europe. As a reminder, CER-001 has been granted Orphan Drug status in Europe by the EMA and in the United States by the FDA. ABNX-100 in uveitis will enter the clinical phase as soon as the company has received regulatory approval for the advancement of sepsis, as its systemic treatment is very similar to the targeted treatment in uveitis in apotherapy.

Uveitis is a disease characterized by inflammation of the tunica vascularis of the eye called the uvea, which is relatively common in industrialized countries with an estimated incidence of more than 100 cases per 100,000 population in Europe and the United States. Uveitis can cause reduced vision and, if not diagnosed early and managed appropriately, ultimately lead to blindness. Since ABIONYX's recombinant natural apoA-I complexes have been shown to be effective in resolving systemic inflammation as demonstrated in the Phase 2 RACERS study, in addition to offering a reparative action on epithelial cells, it is a drug candidate of choice for uveitis for which current treatments have limited effects.

As far as biovectorization is concerned, uveitis can also be treated, but two other major ophthalmology indications are targeted, namely Age-related Macular Degeneration (AMD) and Diabetic Macular Edema (DME) through two other drug candidates, ABNX-1010 and ABNX-3010.

Very promising results of biovectorization

ABIONYX Pharma has been pursuing the development of new *apoA-I lipoprotein* vectors for targeted drug delivery since 2018. Several preclinical studies have already validated the concept, showing that apoA-I complexes can be used as a specific drug delivery platform targeting tumor cells or immune cells. Results from a TARGET study validating this delivery method had been reported in December 2018.

For ophthalmology, an apoA-I complex encapsulating a corticoid was developed by ABIONYX Pharma and tested at IRIS Pharma. Many active ingredients used in ophthalmology are hydrophobic, and the main quality of the apoA-I complex, which contains lipids, is its ability, thanks to its structure and size, to solubilize and transport this type of active ingredient. Given the use of corticoids in ophthalmology and their side effects (cortico-induced cataract, glaucoma, etc.), the development of an apoA-I complex carrying a corticoid appears to be of great value to increase the benefits of treatment for patients, by enabling intraocular injection. ABNX-3010 was therefore tested to validate a proof of concept in a recognized and validated preclinical model of uveitis. Groups treated with ABNX-3010 via the vitreous route showed statistically a near-total reduction in the signs of uveitis, as measured by protein concentration and cell infiltration in the aqueous humor.

The safety profile of apoA-I complex encapsulating a corticoid, as well as ABIONYX apoA-I complexes alone, allow for preclinical and clinical development at the ocular surface and inside the eye, targeting all corneal or retinal indications. These preclinical results for the biovectorization of apoA-I validate a new therapeutic approach based on lipid mechanisms in ocular pathologies, and foreshadow a major therapeutic potential in ophthalmology.

Phase 1/2 clinical trials for ABNX-1010 and ABNX-3010 could begin during 2024, subject to regulatory approval. The biovector technology platform for ophthalmology was developed with IRIS Pharma's teams. This biovector platform, like the ophthalmology apotherapy platform, is protected by new patents for 20 years.

The anti-inflammatory properties of apoA-I, observed in ophthalmologic disease in different IRIS Pharma models, may contribute to improved vision in patients suffering from uveitis, and more generally in other indications with an inflammatory component. *apoA-I* as structured phospholipid complexes allowing the encapsulation of active ingredients, notably the most hydrophobic ones such as corticoids, opens the field to new types of treatments in ophthalmology and a new generation of biomedicines of which the "safe corticoid" is the first candidate in ophthalmology.

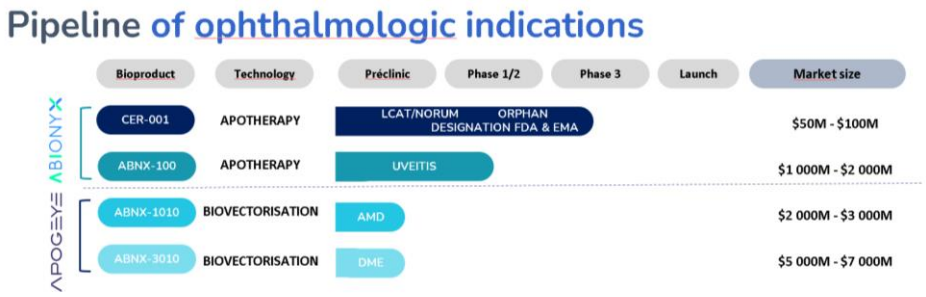
Project to separate the ophthalmology activities and change the name of IRIS Pharma Holding to APOGEYE Pharma

Given the strong development dynamics of the ophthalmology activities, ABIONYX Pharma has decided to separate these specific biotech activities, and plans to transfer its ophthalmology biotech activities to IRIS Pharma Holding (IPH), with in particular the implementation of a license agreement on biovectorization dedicated to ophthalmology.

In order to identify the activities specific to this subsidiary, IRIS Pharma Holding will be renamed APOGEYE Pharma, and thus more clearly valorize apotherapy and biovectorization activities in ophthalmology. The separation of the ophthalmic activities also provides ABIONYX Pharma with strategic flexibility for future partnerships.

New pipeline of ophthalmologic indications

Based on this new strategy, ABIONYX Pharma communicates a new and more precise pipeline of indications.



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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