

Press release

InFlectis BioScience and Laboratoire de Génétique Médicale de Strasbourg enter into a strategic partnership in ophthalmology

- **Exclusive, worldwide license on intellectual property rights to develop and market a pharmacological combination to treat retinal ciliopathies**
- **3 year collaborative research program to advance and mature the retinal pharmacological therapy approach towards the clinical development**

Nantes & Strasbourg, France - January 31st, 2017. InFlectis BioScience SAS, a biopharmaceutical company specializing in the discovery and development of drugs for the treatment of protein misfolding diseases, and Laboratoire de Génétique Médicale de Strasbourg (UMRS 1112 – Inserm – Université de Strasbourg) today announced the signature of a 3-year research partnership and a license agreement for the development and marketing of a novel pharmacological therapy approach for retinal degeneration in ciliopathies.

The strategic collaboration will explore the underlying molecular mechanisms involved in the etiology of a group of selected rare retinal diseases, and will evaluate InFlectis BioScience's drug candidates in the pharmacological approach of Laboratoire de Génétique Médicale de Strasbourg, as well as different administration routes of pharmacological therapy.

The partnership will benefit on the one hand from the Laboratoire's translational approach, from laboratory's world-class expertise in fundamental research in retinal ciliopathies to point-of-care patient applications, and on the other hand from InFlectis BioScience expertise in pharmacological drug development, especially in the development of selective inhibitors of PPP1R15A (GADD34), a stress-induced PP1 phosphatase regulatory subunit involved in the unfolded protein response. A distinctive feature of this partnership lies in the continuous interaction between the parties.

"Eight years of fundamental research have been necessary to reach validation stage of our novel pharmacological approach in animal models of retinal ciliopathies. This has been possible thanks to the financial support of French state, patient associations like RETINA France and UNADEV together with SATT Conectus Alsace. We are glad that InFlectis brings resources and complementary expertise to advance our pharmacological approach toward clinical development, hopefully for the patient benefit" jointly declared Hélène Dollfus and Vincent Marion, respectively Laboratory Director and Associated Director of Laboratoire de Génétique Médicale de Strasbourg.

Philippe Guédat, CEO of InFlectis BioScience SAS, said: "*Here at InFlectis, we are convinced that by connecting excellence of academia and industry, and building partnered teams, we will promote and speed-up the development of innovative therapies*". Pierre Miniou, CBO added: "*This project is aligned perfectly with our research development strategy and extends our expertise in eye-diseases*".

Conectus Alsace, acting on behalf of Laboratoire de Génétique Médicale de Strasbourg, will receive financial support from InFlectis BioScience for the research phases, as well as upfront payment associated with the license agreement. InFlectis BioScience could make further potential payments to Laboratoire de Génétique Médicale de Strasbourg upon reaching development and commercial milestones, as well as royalty payments on annual net sales.

Notes for editors:

ABOUT RETINAL CILIOPATHIES

Primary cilium is a ubiquitously expressed organelle in eukaryotic cells where it plays crucial roles in the function of the cell. In the ciliopathies, a fast growing class of severe disorders related to defects of genetic origins, the modified primary cilium of the photoreceptor, also known as the connecting cilium, is frequently defective. Ciliary dysfunction induces disturbances in the trafficking and docking of specific proteins involved in its biogenesis or maintenance. The well-conserved ciliary process, intraflagellar transport (IFT), is a complex process carried out by multimeric ciliary particles and molecular motors of major importance in the photoreceptor cell. It is defective in a growing number of ciliopathies leading to retinal degeneration. Retinitis pigmentosa related to these ciliary dysfunctions can be an isolated feature or a part of a syndrome such as Bardet-Biedl syndrome (BBS). Research on key ciliopathies such as the Bardet Biedl syndrome (BBS) has led to the discovery of several major cellular processes carried out by the primary cilium structure.

ABOUT LABORATOIRE DE GENETIQUE MEDICALE DE STRASBOURG- INSERM UNIT U1112 (www.grand-est.inserm.fr)

The Laboratory of Medical Genetics (INSERM U1112) is focusing on research about rare and ultra-rare genetic diseases, in a translational state of mind: starting from the patient's clinical observation, the discovery of the causative gene (over 7 seven genes identified in the laboratory), the underlying mechanism of action, to therapeutic target identification. The laboratory is also studying the clinical and biological consequences of mutations in humans (genotype-phenotype correlations, biomarkers ...). Various fields of genetic diseases have been investigated such as syndromic retinitis pigmentosa with a specific interest for Bardet-Biedl syndrome and related ciliopathies, due to a defect of the primary cilia; this has opened new research avenues about the cellular mechanisms underlying the most disabling manifestations of ciliopathies namely: retinal degeneration, obesity and kidney failure. By studying these rare diseases, the laboratory is also working on common conditions such as obesity and diabetes. Pharmacological and gene therapies approaches are also developed and have reached preclinical status such as for the retinal pharmacological therapy project.

ABOUT SATT CONECTUS ALSACE (www.satt.conectus.fr)

Conectus Alsace was the first SATT (Accelerator of Technology Transfer) emerge in France within the Call for Projects of the Program "Investments for the future". Financed 100 % by the State through the National Valorization Fund operated by the French National Research Agency, it operated under the private law statute, whose shareholders include: the University of Strasbourg, the CNRS, the University of Haute-Alsace, Inserm, INSA de Strasbourg, the ENGEEES and the Caisse des Dépôts et Consignations that brings the participation of the State. Its activities are organised around the investment in intellectual property, the maturation of the technologies and licensing, as well as the sale of services in the domain of partnership research and technology transfer.

ABOUT INFLECTIS BIOSCIENCE (www.infectisbioscience.com)

InFlectis BioScience aims to discover and develop new molecules for the treatment of protein misfolding diseases. The company plans to demonstrate the clinical effectiveness of its drug candidate IFB-088 for the treatment of Charcot-Marie-Tooth diseases type 1A (CMT-1A), then partner with a pharmaceutical company for its subsequent development and commercialization. The company is also developing IFB-088 for the treatment of rare eye diseases. Meanwhile, InFlectis BioScience develops new chemical series for the treatment of non-orphan diseases whose etiology also lies in the accumulation of misfolded proteins. Based in Nantes in Western France, InFlectis BioScience is supported by Atlanpole (www.atlanpole.com), the science park of the economic area of Nantes Atlantique.

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