Press Release
April 24th, 2018

Lysogene signs partnership agreement with Dr. Hervé Moine, researcher at the IGBMC[1] and the SATT[2] Conectus Alsace, in the Fragile X syndrome

- Development of a gene therapy product for the treatment of patients with Fragile X syndrome
- Implementation of the project through a unique partnership
- First results anticipated in 2019

CAMBRIDGE, MA, U.S and Paris, France – April 24, 2018, at 08:00am CEST – Lysogene (FR0013233475 – LYS), a leading biopharmaceutical company pioneering gene therapy technologies to treat central nervous system (CNS) diseases, announces the signing of a partnership agreement with Dr. Hervé Moine, researcher in Prof. Jamel Chelly's team at the IGBMC Illkirch (CNRS, Inserm and Strasbourg University) and SATT Conectus. This partnership has been facilitated by the *Fondation Maladies Rares*, through its POC Club (Proof Of Concept) promoting research and knowledge management with academic and industrial actors in the field.

The agreement concerns the development of a gene therapy product for the treatment of patients with Fragile X syndrome. Fragile X syndrome affects approximately 1 birth in 4,000 to 5,000 boys and 1 birth in 8,000 girls, or about 110,000 patients in Europe. It is the most common inherited cause of intellectual disability as well as the most common cause of autism spectrum disorder. No treatment is currently available that has been shown to correct the neurological manifestations of the disease.

The partnership aims to develop a new therapeutic approach based on the pioneering work of Dr. Hervé Moine. The Strasbourg team is using gene therapy to compensate for reduced DgkK, an innovative therapeutic target and potentially a key function in neuronal gene regulation, whose synthesis is regulated by Fragile X Mental Retardation Protein (FMRP), the missing protein responsible for Fragile X syndrome.

This partnership is expected to be implemented through an original co-design format. Driven by the SATT Conectus, this project brings together, in early-stage development, an innovative project driven by a researcher with the financial support of Conectus and an industrial partner with gene therapy development expertise. If the project is successful, Lysogene has a license option, guaranteeing priority access to the technology.

[1] IGBMC *: Institute of Genetics, Molecular and Cell Biology* - www.igbmc.fr
[2] SATT : *Organisation for Accelerating Tech Transfer*

The first results of this project are anticipated in 2019.

"We are pleased by this agreement with prestigious partners in the field of Fragile X. This association, which should lead to an exclusive licensing agreement, is a recognition of Lysogene's know-how in the development of therapies for rare CNS diseases, "said **Philippe Mendels-Flandre, Chief Operating Officer at Lysogene**.

"*SATT Conectus is very motivated by this promising development. Our investment of over €390 k into this project (GETEX project), and the associated intellectual property, will allow us to validate the proposed gene therapy concept and ensure it addresses the unmet market needs. We are also happy to partner with Dr. Hervé Moine and Lysogene, a key partner chosen, after a rigorous selection process, for their expertise in gene therapy development for central nervous system diseases*", commented **Nicolas Carboni, President of SATT Conectus.**

"*I am delighted by the Foundation's role, through the POC Club, in associating Lysogene, Dr. Hervé Moine and Satt Conectus*," says **Christine Fetro, Head of Support for therapeutic proof of concept at the *Fondation Maladies Rares***. "*Accelerating the development of drug candidates for rare diseases is one of the Foundation's core missions*."

"*The involvement of the Fondation Maladies Rares, the expertise of Lysogene and the support of Conectus, will enable us to test a new therapeutic approach for Fragile X, based on the discovery of a fundamental disease mechanism. Our team is very proud of this partnership, which is a unique opportunity to see our academic work developed into a treatment, awaited by families*" adds **Dr. Hervé Moine**.

Karen Aiach, CEO and Founder of Lysogene, says she is "*I am delighted to see Lysogene develop a new program to serve patients with no current therapeutic alternatives*."

Next financial milestones:

- Shareholders' meeting in June, 2018
- Q2 2018 revenue and cash position expected on July 16, 2018 (after market close)

About Lysogene

Lysogene is a gene therapy company focused on the treatment of rare diseases of the central nervous system (CNS). The company has built a unique capability to enable a safe and effective delivery of gene therapies to the CNS to treat lysosomal diseases and other genetic disorders of the CNS. A pivotal clinical trial in MPS IIIA is expected to start in 2018, a phase 1-2 clinical trial in GM1 Gangliosidosis in 2019, while we are currently collaborating to define the clinical development path for the treatment of Fragile X syndrome. www.lysogene.com

Lysogene is listed on the Euronext regulated market in Paris (ISIN code: FR0013233475)

For more information, please visit www.lysogene.com.

About SATT* Conectus Alsace

In close connection with the major players in public research, SATT Conectus Alsace offers companies innovations from academic laboratories of excellence located in Alsace. It finances the most promising inventions up to 500 k €, to enable them to reach a proof of concept, indicator of success. Large groups, SMEs and start-ups gain access to pioneering technologies, de-risked, and ready to be industrialized. Thanks to Conectus, manufacturers can also co-develop, with public researchers, these high potential innovative projects. Conectus also enables companies to identify cutting-edge academic skills to accelerate their own R & D projects. Conectus manages and coordinates exchange and transaction between the company and the researcher, guaranteeing simplicity and speed. Since 2012, thanks to Alsacian academic excellence of international caliber, SATT Conectus has significantly accelerated the transfer of technologies which have had a direct economic benefit.

Since 2012: 141 active intellectual property titles ○ 738 signed collaboration agreements ○ 79 innovative projects funded ○ 76 transfers completed ○ 15 startups created ○ € 27M raised from investors (2017 figures).

Shareholders: CNRS, INSERM, ENGEES, INSA, UNIVERSITE DE STRASBOURG, UNIVERSITE DE HAUTE ALSACE, CAISSE DES DEPOTS
www.conectus.fr ○ http://conectlabs.conectus.fr/ ○ @ConectusAlsace

** Organisation for Accelerating Tech Transfer*

About the Fondation Maladies Rares

The *Fondation Maladies Rares* is a scientific cooperation foundation, created in 2012 as part of the 2nd National Rare Diseases Plan, through the collabortation of 5 founding members: the AFM-Telethon, Alliance Maladies Rares, Inserm, Conference of the General Directors of University Hospitals and Conference of University Presidents. Its public interest mission is to accelerate rare diseases research with three strategic objectives: 1) to identify the cause of rare diseases and to aid diagnosis, 2) to help the development of new treatments and 3) to break the isolation of patients and their families.

To meet this mission, the foundation supports and finances research projects, provides advice and accompanies research teams in the field.

Results since 2012:
→ 301 research projects financed in France
→ 15 technology platforms partnering with call for projects
→ over 170 researchers accompanied
→ 88 therapeutic proof of concept closely followed

http://fondation-maladiesrares.org/

About the IGBMC

Founded in 1994 by Pierre Chambon and Dino Moras, the Institute of Genetics, Molecular and Cell Biology (IGBMC) is one of the leading biomedical research centers in Europe and the largest French research unit associating Inserm, the CNRS and the University of Strasbourg. IGBMC was recognized as a Laboratory of Excellence in 2011.

It brings together a scientific community of about 750 people from 42 different nationalities. The research activity of the IGBMC consists of 4 scientific departments divided into about 50 teams. The institute hosts a hundred doctoral students and masters each year, thanks to high-level training programs such as the PhD program and the IMCBio University School of Research. In addition, the IGBMC has 4 platforms and 9 advanced scientific services for internal use, which are also open to the outside scientific community, and benefits from the technological potential of the infrastructures: FRISBI, PHENOMIN, France Génomique, and INGESTEM.

The goal of the institute is to develop transdisciplinary research at the interface of biology, biochemistry, medicine and physics, but also to attract students from around the world with its offer of training at the highest level in the biomedical sciences field. Located at the Illkirch Innovation Park and on the university campus in the suburbs of

Strasbourg, the IGBMC is positioned in an exceptional academic and industrial scientific environment that greatly favors collaborations and technology transfer.

The IGBMC is a pioneering institute for the study of Fragile X syndrome and contributed in the early 1990s, simultaneously with other institutes, to identify the gene responsible for this genetic defect.

http://www.igbmc.fr/

About the Genetics and pathophysiology of neurodevelopmental and epileptogenic diseases team at the IGBMC
The research team, led by Dr. Jamel Chelly, is based at the Institute of Genetics, Molecular and Cell Biology (IGBMC, CNRS UMR 7104 - Inserm U 1258) in Illkirch. The team is studying the genetic bases and molecular mechanisms of several neurodevelopmental diseases. Recent work, supervised by Hervé Moine, on Fragile X syndrome, has shown that the FMRP protein plays a role in neuronal communication and production of the enzyme diacylglycerol kinase (Dgkk). The restoration of a normal level of Dgkk by gene therapy offers new therapeutic perspectives that will be tested in this program.

About Fragile X syndrome
Fragile X syndrome is the most common inherited cause of intellectual disability and autism spectrum disorders. Patients may experience severe behavioral changes, including hyperactivity, impulsivity, anxiety, low language acquisition, and epileptic seizures. The syndrome is due to repeat mutations in the FMR1 gene that prevents FMRP production, a protein essential for inter-neuronal communication. There is no current treatment for this disease that affects 1 in 4,000 boys and 1 in 8,000 girls.

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