



Lysogene Announces Workshop at National Institutes of Health (NIH) on CNS Immunogenicity Considerations for AAV-mediated Gene Therapy

Paris, France, - 6 June 2019, at 06:00 pm CEST — Lysogene (FR0013233475 – LYS), a leading, biopharmaceutical company pioneering gene therapy technologies to treat central nervous system diseases, today announced the organization of a workshop on CNS immunogenicity considerations for adeno-associated virus (AAV)-mediated Gene Therapy on June 11, 2019 from 8:30 a.m. to 5 p.m. ET at the National Institutes of Health (NIH).

Lysogene played a major role in the planning and organization of this workshop. The purpose of the meeting is to bring together the scientific and medical expert community for AAV-mediated therapies to focus on issues related to the central nervous system (CNS) administration and immunogenicity of these products.

AAV gene therapy holds the promise to transform CNS disorders by providing a durable therapeutic effect following a single intervention. However, transgene expression and overall efficacy of gene therapy strategies in humans can be impaired by undesirable humoral and cellular host immune response against the viral capsid protein and/or against the protein expressed from the administered gene. How to prevent immune reactions, and how to evaluate their consequences on efficacy and safety of AAV gene delivery, are crucial questions.

“By sharing immunogenicity findings from AAV clinical trials and bringing together the main experts in the field, this workshop holds the promise to provide guidance for the advancement of CNS gene therapy,” said Samantha Parker, Chief Patient Officer at Lysogene, member of the Organizing Committee for this event.

“It is crucially important to discuss and share our current understanding with experts in the field and to reach consensus on best treatment practices”, continued Sophie Olivier, Chief Medical Officer at Lysogene.

Individuals, who wish to view the workshop, can register to the live videocast on ncats.nih.gov/events.

About Lysogene

Lysogene is a gene therapy company focused on the treatment of orphan 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 in partnership with Sarepta Therapeutics, Inc. is ongoing and a phase 1-2 clinical trial in GM1 Gangliosidosis is in preparation. In accordance with the agreements signed between Lysogene and Sarepta Therapeutics, Inc., Sarepta Therapeutics, Inc. will hold exclusive commercial rights to LYS-SAF302 in the United States and markets outside Europe; and Lysogene will maintain commercial exclusivity of LYS-SAF302 in Europe. Lysogene is also collaborating with an academic partner to define the development strategy for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

Contacts

Mathilde Bohin / Emmanuel Huynh
NewCap
lysogene@newcap.eu
+ 33 1 44 71 94 95

Lysogene's forward-looking statements

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