Lysogene creates a Medical and Regulatory Department

Soraya Bekkali, M.D. appointed Senior Vice President, Chief Medical Officer, based in Paris

Sean O'Bryan appointed Vice President, Regulatory Affairs & Quality Assurance, based in Cambridge, Massachusetts U.S.

FOR IMMEDIATE RELEASE

PARIS, FRANCE—February 1, 2016—Lysogene, a health biotechnology company specializing in gene therapy technology applied to central nervous system diseases, is progressing its clinical development programs in Mucopolysaccharidosis Type A (also known as Sanfilippo A) and GM1 Gangliosidosis. To expedite this development, Lysogene is expanding its in-house resources and expertise with a medical and regulatory affairs department to be led by Soraya Bekkali.

Soraya Bekkali is appointed Senior Vice President, Chief Medical Officer. A graduate from Paris University Medical School, she is specialized in clinical research and biostatistics. Dr. Bekkali started her career in academia before joining Aventis Pharma, followed by Orphan Europe, a pharmaceutical company dedicated to the development of treatments for rare diseases, before joining Sanofi in 2007. At Sanofi, Dr. Bekkali held successively increasing leadership positions including Vice President and Global Ophthalmology Unit Head. Her extensive industry experience spans from early development to ensuring market access, with an important focus on viral and non-viral gene therapy products. She has a breadth of experience across a variety of therapeutic areas. Dr Bekkali will be based in Lysogene's Paris office.

Reporting to the CMO, Sean O'Bryan is appointed Vice President, Regulatory Affairs & Quality Assurance. A graduate of Boston University, Mr. O'Bryan spent 8 years at Genzyme/Sanofi with progressively senior regulatory positions and ultimately held the role of Product Regulatory Affairs lead for Cell Therapy and Regenerative Medicine. He then joined Bluebird Bio as Director of Regulatory Affairs to lead the company's regulatory efforts on the treatment of the rare disease CCALD (Childhood Cerebral Adrenoleukodystroph) with their gene therapy. He also supported their Regulatory Affairs activities across several therapeutic areas. Mr. O'Bryan will be based at Lysogene's U.S. office in Cambridge, Massachusetts.

"We are delighted to welcome Soraya and Sean to Lysogene's management team," said Karen Aiach, Founder and Chief Executive Officer. "Thanks to their impressive expertise and capabilities, Lysogene will now be able to accelerate our development programs in Mucopolysaccharidosis Type A and GM1 Gangliosidosis and also advance pioneering, new, gene therapy programs for individuals suffering from rare diseases."

About Mucopolysaccharidosis Type A (also known as Sanfilippo A) and GM1 Gangliosidosis.

MPS IIIA is a lysosomal disease caused by an autosomal recessive defect of the SGSH gene and affecting approximately 1:100,000 live births. MPS IIIA presents in early childhood, causing progressive neurodegeneration associated with intractable behavioral problems and developmental regression. Life span is shortened and there is currently no treatment.

GM1-gangliosidosis is a rare inherited neurodegenerative disorder characterized by severe cognitive and motor developmental delays resulting in early death. It is caused by mutations in the GLB1 gene, which encodes an enzyme called beta-galactosidase necessary for recycling the GM1-ganglioside molecule in neurons. This brain lipid is essential for normal function, but its accumulation causes neurodegeneration, resulting in severe neurological symptoms. There is currently no treatment.

About Lysogene

Lysogene is a clinical stage biotechnology company pioneering the basic research and clinical development of AAV gene therapy for CNS disorders with a high unmet medical need. Since 2009, Lysogene has established a unique platform and network, with lead products in Sanfilippo A and GM1 Gangliosidosis, to become a global leader in orphan CNS diseases.

For more information www.lysogene.com.

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