



REGENX BIOSCIENCES AND LYSOGENE ENTER INTO EXCLUSIVE LICENSE AGREEMENT FOR DEVELOPMENT OF TREATMENTS FOR SERIOUS, RARE LYSOSOMAL STORAGE DISORDER USING NAV® rAAVrh10 VECTORS

WASHINGTON, DC and PARIS, FRANCE December 5, 2013 – REGENX Biosciences, LLC (REGENX) and LYSOGENE SAS (LYSOGENE) announce that they have entered into an agreement enabling the development and commercialization of products to treat mucopolysaccharidosis type IIIA (MPS IIIA or Sanfilippo syndrome Type A) using **NAV** rAAVrh10.

Under the terms of the Agreement, REGENX granted LYSOGENE an exclusive worldwide license, with rights to sublicense, to REGENX's **NAV** rAAVrh10 vectors for treatment of MPS IIIA in humans. In return for these rights, REGENX receives payments in the form of an up-front payment, certain milestone fees and royalties on net sales of products incorporating **NAV** rAAVrh10.

“We believe this exclusive license agreement will enable LYSOGENE to advance the development of its **NAV** based treatment for patients with MPS IIIA,” said Ken Mills, President and CEO of REGENX. “As a leader in gene therapy, we are pleased to be formally collaborating with the LYSOGENE team that, by the successful completion of a recent Phase I/II trial, demonstrates outstanding expertise, resources, and commitment to patients. Providing partners with access to our **NAV** technology further advances REGENX's mission to enable the development of successful new AAV therapeutics.”

“LYSOGENE is a leading clinical stage gene therapy company committed to the development of breakthrough therapies in rare diseases. The company successfully completed a phase I/II study (NCT01474343/EudraCT 2010-019962-10) using the **NAV** rAAVrh10 technology in Sanfilippo syndrome. We are very pleased to enter into this agreement with REGENX, which we believe offers us

the best path to expeditiously advance the clinical development and commercialization of our lead product for patients with Sanfilippo syndrome,” said Karen Aiach, Founder, President and CEO of LYSOGENE.

“Sanfilippo syndrome is a complete unmet medical need and our clinical study using **NAV** rAAVrh10 indicates that gene therapy may become an outstanding treatment option,” said Olivier Danos PhD, Co-founder and Senior Scientific Advisor to LYSOGENE.

About MPS III A (Sanfilippo syndrome Type A)

Sanfilippo syndrome is a lethal, rare, autosomal recessive condition characterized by rapid neurodegeneration, severe and invasive behavioral disorders, and mild peripheral symptoms. Patients with Sanfilippo syndrome generally do not live beyond their second decade. There is currently no treatment. Sanfilippo syndrome is caused by mutations in a gene that encodes N-sulfoglucosamine sulfohydrolase (*sulfamidase*) which is needed to break down glycoaminoglycans - used in a number of biological functions. It affects approximately 1 in 100,000 births, and is still largely underdiagnosed.

About REGENX Biosciences

REGENX Biosciences is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary **NAV** vector technology platform, for a range of severe diseases with serious unmet needs. **NAV** vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Its treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, and Audentes Therapeutics. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding REGENX, please visit www.regenxbio.com.

About LYSOGENE

LYSOGENE is a clinical stage biotechnology company committed to the development of innovative therapies for patients affected with rare disorders and high unmet medical needs. LYSOGENE's team translated its rAAVrh10 lead product for Sanfilippo from bench to bedside in an unprecedented fashion over the last few years. Its lead product is for Sanfilippo syndrome, a neurodegenerative lysosomal storage disorder considered to be a perfect model for gene therapy. LYSOGENE is currently expanding its pipeline to additional diseases with high unmet medical needs.

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

###

Contacts:

REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio.com

LYSOGENE
Karen Aiach
karen.aiach@lysogene.com