LYSOGENE receives US ORPHAN DESIGNATION FOR ITS LEAD INTRACEREBRAL GENE THERAPY PRODUCT SAF-301.

Paris, France – May 13, 2013 - LYSOGENE announced today that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to its lead gene therapy product SAF-301 for the treatment of Sanfilippo type A syndrome (also named mucopolysaccharidosis type IIIA), a rare, genetic and life-threatening disease affecting children (http://www.accessdata.fda.gov).

SAF-301 is an adeno-associated viral vector serotype rh.10 carrying the human SGSH and SUMF1 cDNAs currently under investigation in a Phase I/II clinical trial (P1-SAF-301) conducted in France. In September 2010, the European Commission granted SAF-301 orphan designation for the same indication in the European Union.

“We are very pleased to receive FDA orphan drug designation for SAF-301. This designation is an important strategic milestone in the development of our program”, Karen Aiach, MBA, Founder and Chief Executive Officer of LYSOGENE, said. “It represents another advance that our company is making in the area of gene therapy and rare diseases and it is extremely encouraging and promising. Through the development of SAF-301, we hope to address the significant unmet medical need for patients who suffer from Sanfilippo type A syndrome.”

Orphan Drug Designation is granted by the FDA Office of Orphan Drug Products to drugs intended to treat rare disease or condition affecting fewer than 200,000 people in the U.S. This designation confers special incentives to the drug developer, including tax credits on the clinical development costs, prescription drug user fee waivers and may entitle a period of seven year market exclusivity in the US upon FDA approval. “Orphan drug designation also provides regulatory support by regulatory Agencies in development activities such as protocol assistance in Europe and fast track procedure and priority review in the US”, Fanny Vincent, PhD, Director of Regulatory Affairs, added. “This designation is the first step of a productive exchange with the FDA”.

About the P1-SAF-301 clinical trial
The P1-SAF-301 clinical trial is an open-label, single arm, monocentric, phase I/II clinical study evaluating the tolerance and the safety of intracerebral administration of adeno-associated viral vector serotype rh.10 carrying the human SGSH and SUMF1 cDNAs for the treatment of Sanfilippo type A syndrome. The Principal Investigator is Professor Marc Tardieu, Head of Unit, Neuropediatry, CHU Bicêtre, Le Kremlin-Bicêtre (France).


About Sanfilippo Syndrome and LSDs
Sanfilippo syndrome type A (or MPSIIIA) is a rare and fatal lysosomal storage disease due to an enzyme deficiency of the protein N-sulfohydrolase sulfoglycosamine (SGSH). The main clinical symptom is a degenerative neurological condemning afflicted child with a life expectancy of between 10 and 20 years old. Lysosomal storage disease (LSDs) are a group of over 50 inherited disorders typically characterized by a deficiency in one or more enzymes that degrade glycans, the carbohydrate chains of glycoproteins, proteoglycans and glycolipids. LSDs are the commonest cause of pediatric neurodegenerative disease, their total combined incidence is greater than 1 per 8,000 births.
Further information about Sanfilippo Syndrome and LSDs is available at:

**About LYSOGENE**

Founded in 2009 by Karen Aiach, LYSOGENE is a leading biotechnology company specialized in the development of intracerebral gene therapy aimed at treating pathologies affecting the central nervous system, which is the main cause of neuro-degenerative disease mortality in children. Further information about LYSOGENE is available at http://www.lysogene.com.

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