LYSOGENE

LYSOGENE ANNOUNCES COMPLETE ENROLLMENT IN ITS SAF-301 PHASE I/II CLINICAL TRIAL OF INTRACEREBRAL GENE THERAPY IN CHILDREN WITH SANFILIPPO TYPE A SYNDROME.

Paris, France – June 14, 2012 - LYSOGENE announced today that the last planned patient in its phase I/II clinical trial in Sanfilippo Type A Syndrome (NCT01474343) had been treated with SAF-301, its investigational intracerebral gene therapy product.

SAF-301 aims at treating this pediatric life threatening disease with a high unmet medical need and currently no cure.

This open-label, single arm, monocentric, phase I/II SAF-301 clinical study is primarily designed to evaluate the tolerance and the safety of the intracerebral administration of SAF-301 performed in a single neurosurgical session. It is also designed to evaluate exploratory efficacy neuropsychological, radiological and biological endpoints from the perspective of future pivotal studies.

"Completing full enrollment and treatment in this phase I/II study is a crucial milestone in the development of a safe and efficacious gene therapy as what is intended to become the first line treatment for Sanfilippo Type A disease. It also sustains the extraordinary potential of gene delivery-based approaches for numerous other monogenic diseases with central nervous system involvement. Our hope is to bring significant clinical benefits and quality of live improvements to numerous patients and their families affected with such diseases worldwide", Karen Aiach, Founder and CEO of LYSOGENE said.

About Sanfilippo Syndrome and LSDs

Sanfilippo Syndrome or Mucopolysaccharidosis III (MPS-III) is a group of four rare autosomal recessive lysosomal storage diseases of which Type A accounts for approximately two thirds. Sanfilippo Syndrome is characterized with a heavy central nervous system involvement and an extremely severe phenotype, associated with a life expectancy reduced to the midst of the second decade.

Sanfilippo Type IIIA Syndrome is seen in approximately 1 in 100,000 live births and affects a few thousands patients worldwide.

MPS-III belongs to lysosomal storage diseases (LSDs), a group of over 50 inherited disorders, with a total combined incidence greater than 1 per 8,000 births, of which 70% have a central nervous system component.

About LYSOGENE

LYSOGENE is a platform biotechnology company specialized in the development of intra-cerebral gene therapy for the treatment of lysosomal diseases affecting the central nervous system, the main cause of mortality in childhood neurodegenerative diseases.

LYSOGENE brought its first product SAF-301 from bench to the bedside in less than five years.

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