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Families with children affected by SANFILIPPO Syndrome are expecting the beginning of the first gene therapy trial on MPSIIIA

For years, patient associations around the Sanfilippo disease are waiting for this moment: the first gene therapy clinical trials on MPSIIIA. For many of them, hope has come out to finally reach a treatment of this incurable disease that combines rarity, severity and life expectancy extremely shortened for affected children.

Sanfilippo syndrome is a lysosomal storage disease extremely severe and debilitating, fatal in childhood with not any existing treatment. This disease is caused by a malfunction of the lysosome-related part of a gene. The enzyme deficiency causes an overload of waste produced by the cells in organs.

In France, Sanfilippo disease affects 1/135 000 births. For these patients' families' hope arrived when the gene therapy program Sanfilippo was born in 2006. Supported by the Alliance SANFILIPPO, by the AFM-Telethon Foundation and by the SANFILIPPO foundation in Switzerland, the program brings together scientific, medical, toxicological, regulatory, and clinical expertise recognized in this kind of very specific orphan diseases.

Based on solid preclinical studies in terms of efficiency and safety, three years after a validating proof of principle on animals, SAF-301 program obtained the agreement of the Persons Protection Committee in November 2010. The program is actually in the final validation phase within the competent agency for clinical trials authorization in France.
The objective of this first clinical trial is to evaluate the clinical, radiological, biological safety of this treatment and to collect essential data to prepare of the subsequent phase, which will, meanwhile, mainly dedicated to the treatment effectiveness evaluation.

About Alliance Sanfilippo:
Alliance Sanfilippo is a patient association created in 2005 fully dedicated to support affected patients (with Sanfilippo syndrome) and their families.
Alliance Sanfilippo is committed to promote, encourage and finance research and development of curative treatments for Sanfilippo Syndrome. The association facilitates exchanges between families with one or more affected children and believes in the interest of sharing experience to improve their life quality.

Alliance Sanfilippo is in the deepest respect for ethics and professionalism with families, physicians, researchers, industrialists and government institutions, both nationally and internationally.