



Turning Science into a Cure

ALLIANCE SANFILIPPO (ASF) is a non-profit organisation of parents committed to accelerating the pace of biomedical research and developing clinical trials in Sanfilippo syndrome/MPS III. Founded in 2005 by parents with affected children, the organisation aims at becoming a leading provider of support for MPSIII research and resources throughout Europe.

The projects sustained by the organisation are featured on the ASF website: www.alliancesanfilippo.com.

One project is the ASF state-of-the-art report, an international scientific audit mission, to be released in late Spring 2006. This audit, conducted with the support of a neurobiologist specialised in neurodegenerative diseases, is the base for ASF strategies and programmes.

One major programme has been launched based on the read-through of stop codons mechanisms. A proof of concept funded by ASF is due to start in coming days.

Other core projects focus on the following axes:

- Strongly advocating in favour of clinical trials for the use of Zavesca® (actelion) in Sanfilippo syndrome;
- Developing and intensifying the relationship with Shire PLC and Shire Human Genetics Therapies (ex TKTx) to accelerate the sulfamidase programme (direct intracerebral ERT);
- Encouraging gene therapies programmes;
- Encouraging MPS III natural history programmes.

“Traditionally, for many reasons, MPS III families have not been in a position to access the following three worlds: the world of scientific and medical research, the world of industry, and the world of regulatory affairs. However, all of these are absolutely crucial to therapeutic developments. We want MPS III families to be provided with an accessible source of added-value information, both in English and French, on research projects, therapeutic approaches to be encouraged to fight against MPS III, and eventually the numerous actions families can perform to reach our unique goal: turning science into a cure for our children”, says Karen Aiach, President of ASF.

An MPS III father concurs: *“Today, the rare disease world has changed. Tremendous improvements have been made in the influence of patient organisations in biomedical research, clinical trials (protocol design, definition of endpoints...) and regulatory affairs. We as parents have the duty to utilise every single tool that we now have in hand to help find a cure for our children. And we will do so”.*

In May 2006, Eurordis, the European Organisation for Rare Diseases, launched the Sanfilippo syndrome electronic discussion forum intended for families and hosted by Medicalistes: (<http://eurordis.medicalistes.org/article27.html>).

Families from all over Europe and worldwide will have the opportunity to communicate amongst themselves in privacy, provided with a place to exchange information on the progress of the course of a treatment or hospitalisation or clinical trial, on side effects a child may be experiencing, or on any clue that may bring improvement to some specific problem affecting the quality of life. The forum will be animated by a member of ASF. This is another step designed to sustain the actions of the MPS III community.