



## **GENETHON and ESTEVE announce agreement to manufacture the gene therapy for the treatment of Sanfilippo Syndrome**

**Evry, France – March 11, 2014** –ESTEVE, a Spanish pharmaceutical company devoted to the research, development, manufacturing and commercialization of novel medicines and Genethon, a non-profit organization dedicated to the research and development of gene therapies for orphan genetic diseases, announce that they have entered into an agreement to manufacture Esteve's investigational gene therapy for the treatment of Sanfilippo A Syndrome, AAV9-hsulfamidase, under GMP.

“We are very happy to contribute to the development of the treatment for Sanfillippo A developed by Esteve. The quality of the project and of the teams both academic and industrial participating in the program is outstanding and it is our goal to support clinical development with our biomanufacturing expertise, in the most efficient way, and in the best interest of patients” explained Frédéric Revah, Chief Executive Officer of Genethon.

“The signature of this agreement with Genethon is a new milestone for ESTEVE, as it enables us to advance the development of our gene therapeutic for Sanfilippo A towards clinical trials. We are very pleased to collaborate with Genethon, a reference center in manufacturing of gene therapies for rare diseases. Our mission at ESTEVE R&D is the development of innovative products to meet patient needs and that is why this is one of our highest priority projects today.” said Albert Esteve, CEO of ESTEVE.

ESTEVE is developing the Sanfilippo project in a Public and Private Partnership (PPP) with the Universitat Autònoma de Barcelona (UAB) for the development of gene therapies for mucopolysaccharidoses. The program relies on state-of-the art science developed at the CBATEG (Center of Animal Biotechnology and Gene Therapy) of the UAB. The most advanced project in this program is the development of a novel gene therapy treatment for Sanfilippo A Syndrome.

### **About MPS III A (Sanfilippo syndrome Type A)**

Sanfilippo A Syndrome is a devastating disease that leads to progressive and significant deterioration in mental status of children who rarely live beyond their twenties. Diagnosis of many rare diseases are lengthy and time consuming and is only initiated once the symptoms have begun to appear like the Sanfilippo Syndrome, a lysosomal storage disease caused by the loss of the activity of the enzyme sulfamidase. It affects approximately 1 in 100,000 births and is still largely underdiagnosed.

### **About Genethon**

Genethon ([www.genethon.fr](http://www.genethon.fr)) located in Evry, France, is a non-profit R&D organization dedicated to the development of biotherapeutics for orphan genetic diseases, from research to clinical validation. Genethon is specialized in the discovery and development of gene therapy drugs and has multiple ongoing programs at clinical, preclinical and research stages for neuromuscular, blood, immune system, liver, and eye diseases. To support clinical



development of gene therapy drugs, Genethon has built one of the largest facilities worldwide for the production of clinical-grade gene therapy vectors.

### **About ESTEVE**

ESTEVE ([www.esteve.com](http://www.esteve.com)) is a leading pharmaceutical chemical group based on Barcelona, Spain. Since it was founded in 1929, ESTEVE has been firmly committed to excellence in healthcare, dedicating efforts to innovative R&D of new medicines for unmet medical needs and focusing on high science and evidence-based research. ESTEVE has a strong partnership approach to drug discovery, development and commercialization. The company works both independently and in collaboration to bring new, differentiated best-in-class treatments to patients who need them. The company currently employs 2,300 professionals and has subsidiaries and production facilities in several European countries, USA, China and Mexico.

### **Genethon contact info**

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