

Press Release - May 27, 2019

## **Genethon welcomes the first FDA-approved gene therapy for a neuromuscular disease and emphasizes its decisive role in this historic step**

On May 24, the Food and Drug Administration approved Zolgensma®, a gene therapy drug for the treatment of spinal muscular atrophy developed by AveXis (Novartis).

Genethon, the AFM-Telethon laboratory, played a decisive role in the design of both the product and the route of administration of this first gene therapy for a neuromuscular disease, thanks to the pioneering work lead by Martine Barkats and her team. In fact, the French researcher demonstrated that a recombinant AAV9 vector made it possible to cross the blood-brain barrier and thus reaching motor neurons, whose dysfunction is responsible for spinal muscular atrophy, and that such a vector carrying the *smn1* gene extended the life span of mouse models of spinal muscular atrophy. These discoveries had been the subject of patents filed as early as 2007 and scientific publications in 2009 in *Molecular Therapy*, and in 2011 in *Human Gene Therapy*. In 2018, Genethon granted AveXis a license to patents in the U.S., Europe and Japan, for the AAV9 SMN product and in vivo gene therapy delivery of AAV9 vector into the CNS using intrathecal or intravenous routes of administration for the treatment of SMA.

After having played a pioneering role in the deciphering of the human genome (first maps published between 1992 and 1996), Genethon confirms, once again, its essential role in research and development of gene therapy treatments for rare diseases in the world. Eight gene therapy products, resulting from Genethon's research or developed in collaboration with partners, are currently in clinical trials in Europe and the United States for myotubular myopathy, immune deficiencies, blood diseases or vision diseases....

***"The approval of this first gene therapy for spinal muscular atrophy represents a major breakthrough for patients and their families. We are very pleased and proud to have made a decisive scientific contribution to the development of this treatment, thanks to the determined commitment of AFM-Telethon, donors and our researchers. This further, demonstrates Genethon's capability to develop effective first-in-class treatments and the excellence of our translational research driven by the commitment to treat patients living with rare diseases"*** says Frederic Revah, CEO of Genethon.

**Press contacts:**

Stéphanie Bardon: 06 79 34 15 68