Genethon Pursues Different Strategies for Ensuring Patient Access to Gene Therapies for Rare Diseases

PARIS, FRANCE (February 29, 2024) – Genethon, a non-profit research organization focused on developing gene therapies for rare diseases, today marks International Rare Disease Day by highlighting its efforts to bring gene therapies to patients suffering from rare diseases such as limb girdle muscular dystrophies, Crigler Najjar syndrome and Duchenne muscular dystrophy.

Globally more than 300 million people, most of them children, are living with 7,000 rare diseases. Eighty percent are genetic diseases and nearly 95% have no effective therapies. This places a heavy burden on health care systems costing billions of dollars. Because patient populations are small, these diseases don't readily fit into the biopharma industry's business models.

"I am happy to say that thousands of patients around the world already are benefitting from our research through traditional licensing agreements such as the one that led to the development of the first gene therapy approved in 2019 for spinal muscular atrophy," said Frederic Revah, Ph.D., Genethon CEO.

"However, after 30 years of pioneering gene therapies, we realized that we had to create different development strategies beyond licensing out to biopharma the discoveries made by our more than 200 dedicated scientists."

One of those strategies involves creating its first spinout, Atamyo Therapeutics, in 2020. Atamyo is developing gene therapies for limb girdle muscular dystrophies (LGMD). The gene therapies were designed by researchers in Genethon's Progressive Dystrophies Laboratory under the leadership of Isabelle Richard, Ph.D., who is Atamyo's Co-Founder & Chief Scientific Officer and Head of Progressive Muscular Dystrophies Team at Genethon.

LGMD are a group of rare diseases and the fourth most common genetic cause of muscle weakness. It has more than 30 subtypes related to genetic mutations with an estimated worldwide prevalence of all forms ranging from one person in 14,500 to one person in 123,000.

Led by CEO Stephane Degove, Atamyo already is conducting clinical trials in Europe for LGMD2I/R9 and ready to initiate clinical trials for LGMD2C/R5. It has IND-enabling studies under way for a third, LGMD2A/R1; and the U.S. Food and Drug Administration has cleared Atamyo's IND for clinical trials of LGMD2I/R9.

Another strategy involves Genethon pursuing clinical development itself. Genethon is conducting pivotal trials for a gene therapy for Crigler Najjar syndrome, a life threatening liver disease. It would be the first-ever gene therapy for this ultra-rare disease which affects about 1 person (male or female) per 750,000 to 1 million people worldwide.

The pivotal trials of the Crigler-Najjar gene therapy will attempt to confirm positive data from Phase 1/2 trials. If successful, the results could support regulatory filing in Europe in two years.

A third strategy involves Genethon's partnership with Sarepta Therapeutics of Cambridge, MA, for co-development of a gene therapy for Duchenne muscular dystrophy, a genetic disease resulting in progressive muscular degeneration and weakness. The disease affects mostly males and occurs in about 6 children per 100,000 in Europe and North America.

Genethon's collaboration with Sarepta involves co-development of the clinical gene therapy program and, under the terms of agreement, Genethon is responsible for commercializing the product GNT0004 in Europe (excluding the UK) and Sarepta is responsible for the rest of the world.

Founded in 1990 by patients for patients, Genethon from the start has embraced a patient centric drug development approach based on applying the most advanced science with a pledge of ensuring affordability to gene therapy medicines.

"Despite challenges in generating interest with biopharma companies and investors, Genethon will never abandon patients suffering from rare and ultra-rare diseases," said Dr. Revah. "Gene therapies have proved their curative potential and we will continue to advance new clinical development and commercialization strategies to try to ensure all patients benefit from them."

About Genethon

As a pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit laboratory that was established by AFM-Telethon. A first gene therapy for spinal muscular atrophy to which Généthon contributed has obtained a product license. With more than 200 scientists and professional staff, Genethon is pursuing its aim to develop therapies which change the lives of patients suffering from rare genetic diseases. Thirteen products stemming from by Genethon's R&D or from collaborations are in clinical trial for diseases of the liver, blood, immune system, muscles and eyes. Seven other products could enter clinical trials over the next five years. More information at www.genethon.fr.