Press Release For Immediate Release

Marking World Duchenne Muscular Dystrophy Day, Genethon Reaffirms Its Commitment to Helping Patients for 35 years and Provides an Update on Its Promising Gene Therapy Drug Candidate for This Fatal Disease

PARIS, FRANCE (September 6, 2024) – Genethon, a non-profit research organization and world leader in gene therapies for rare diseases, is marking World Duchenne Muscular Dystrophy Day on September 7, 2024, by reiterating its 35-year commitment to the families affected by Duchenne muscular dystrophy and its determination to offer patients a solution to overcome this fatal disease.

Genethon currently is developing a drug candidate that has demonstrated its efficacy in the first patients to be treated. Its characteristics make it the most promising gene therapy drug candidate currently in development to treat the thousands of children born each year with this disease.

Duchenne muscular dystrophy is one of the most common childhood neuromuscular diseases. Nearly 250,000 people - mostly children - are affected worldwide, and all are waiting for the treatment that will change their lives.

Today, gene therapy is the most promising therapeutic approach, because it tackles the origin of the disease, not just its symptoms. Genethon has developed GNT0004, a drug candidate combining a microdystrophin and an AAV8 vector, currently being evaluated in a gene therapy trial being conducted in France and the UK.

Safety and pharmacodynamic results from the first patients, presented at the Myology2024 congress in Paris in April, showed good tolerance of GNT0004, as well as efficacy data, both in terms of microdystrophin expression and functional improvement. Indeed, children treated at the therapeutic dose improved their ability to walk, even run, climb stairs and stand up on their own, with or without support.

On the strength of the positive opinion of the IDMC (Independant Data monitoring comitte), Genethon will begin the final phase of evaluation of the drug candidate with the dose that demonstrated its efficacy in the first phase of the trial. This dose is lower than those used in all other gene therapy trials currently being conducted for Duchenne muscular dystrophy and lower than the gene therapy product approved by FDA. This represents a major advantage in terms of patient safety (limiting side-effects), ease of production, and treatment cost.

"After years of research, during which Duchenne muscular dystrophy has taken so many lives, hope is now tangible for families, and particularly for those whose young children are now diagnosed. Seeing children, who should inexorably lose their strength, making manifest progress* - running, climbing stairs one leg at a time - is an incredible emotion. The fight goes on, stronger than ever! Our hearts go out to Genethon's researchers and experts in this

final stretch, and we look to the future with great optimism," said Laurence Tiennot-Herment, President of AFM-Telethon (French neuromuscular dystrophy association) and mother of Charles-Henri, who died of Duchenne muscular dystrophy in 2003.

"This program is a real scientific and technological adventure, but also a human one for us, born of the determination of families affected by Duchenne muscular dystrophy. We are very proud of the results obtained by our gene therapy GNT0004 in the first patients treated and look forward to starting the pivotal phase of the trial in Europe. Genethon teams are more determined than ever to succeed and offer families an effective and safe treatment that changes the lives of children suffering from Duchenne muscular dystrophy," said Frederic Revah, Chief Executive Officer of Genethon.

* *See more including patient testimonials on the web site of the AFM-Telethon*

About GNT0004

The GNT0004 gene therapy product is composed of an AAV8 (adeno-associated virus) vector and the optimized hMD1 transgene, a shortened but functional version of the gene encoding dystrophin, the protein deficient in people with Duchenne muscular dystrophy. This vector is designed to be expressed in muscle tissue and also in the heart, thanks to a tissue-specific Spc5-12 promoter sequence. GNT0004 is administered by a single intravenous injection. It was developed by Genethon, in partnership with the teams of Prof. Dickson (University of London, Royal Holloway) and the Institut de Myologie (Paris).

About Duchenne muscular dystrophy

Duchenne muscular dystrophy, which affects 1 in 5,000 boys, is a rare progressive genetic disease affecting all muscles in the body. It is linked to abnormalities in the gene responsible for the production of dystrophin, a structural protein essential for the stability of muscle fiber membranes and their metabolism. The absence of dystrophin leads to progressive degeneration of skeletal and cardiac muscles, loss of walking and respiratory capacity, cardiomyoapthy and death between the ages of 20 and 40.

About Genethon

A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a not-for-profit laboratory created by the AFM-Telethon. A first gene therapy drug to which Genethon contributed, was successfully marketed for spinal muscular atrophy. With over 220 scientists and professionals, Genethon aims to develop innovative therapies that change the lives of patients suffering from rare genetic diseases. Thirteen gene therapy products resulting from Genethon's research, or to which Genethon has contributed, are currently in clinical trials for diseases of the liver, blood, immune system, muscles and eyes. Seven other products are being prepared for clinical trials over the next five years. www.genethon.com

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