Genethon Celebrates Rare Disease Day by Highlighting New Technologies to Advance Gene Therapies and Bring Hope to Millions of Patients Worldwide

PARIS, FRANCE (February 27, 2025) – Genethon, a pioneering gene therapy research organization created by the AFM-Telethon, marks Rare Disease Day February 28, 2025, by highlighting recent technological breakthroughs signaling a next generation of safer, more effective and lower cost gene therapies to expand applications of these life-saving medicines and make them more accessible for patients across the globe.

Gene therapy has demonstrated its effectiveness and full potential in treating rare diseases, sometimes being the only therapeutic option for highly complex genetic disorders. Genethon is working to enhance its efficacy through breakthrough technologies aimed at improving vector efficiency, treating more patients by bypassing the natural immunity (40% of patients are naturally immune to certain vectors) and reducing production costs.

"These scientific advances underline the potential of our R&D and technology developments for designing, developing and producing innovative and potent gene therapy treatments, and place our science at the forefront of innovation for millions of patients," said Frederic Revah, Ph.D., Genethon CEO.

More efficient vectors through artificial intelligence
Among the scientific advances in design and production of Adeno-Associated Virus-based vectors (AAV) is the application of artificial intelligence to create a next generation of AAV capsids specifically targeting skeletal muscle for more efficient, safer and more effective treatment of neuromuscular diseases. Some of these diseases require large doses of natural AAVs, many of which do not reach their target cells and can cause adverse effects. Read more.

A combination of vectors for maximum effectiveness
The development of a dual AAV vector for gene therapy is another breakthrough. It increases the packing capacity for delivering normal copies of mutated genes whose coding sequence is greater in length than 4.5 kilobase pairs, the maximum capacity of one AAV. Delivering a longer copy close to the normal gene increases the potential of therapeutic benefit for many diseases, such as Duchene muscular dystrophy, certain limb-girdle muscular dystrophies, Pompe disease as well as non-muscle-related diseases such as retinopathies. Read more.

Plant technology to reduce production costs
A third scientific advance aims to tackle the thorny issue of the current high production cost for gene therapies which leads to high prices for patients. Genethon is collaborating with Samabriva in applying its plant-based technology to produce AAV vectors to significantly

reduce bioproduction costs and help decrease the price of gene therapy treatments. Read more.

“In its 30-year history as an organization founded by patients for patients, Genethon has led the medical scientific field in pioneering gene therapy for rare diseases, and continues to innovate to develop novel-generation gene therapies,” observed Dr. Revah. “Genethon remains more committed than ever to the development of these innovative drugs for the benefit of patients.

Thousands of patients around the world already are benefitting from Genethon’s research, including the first gene therapy approved for spinal muscular atrophy. Thirteen other products are in clinical trials for diseases of the liver, blood, immune system, muscles and eyes.

Genethon also recently presented positive results from the Phase 1/2 dose escalation part of an international multicenter all-in-one Phase 1/2/3 trial evaluating its gene therapy, GNT-0004, for Duchenne muscular dystrophy (DMD). Based on positive safety and efficacy data Genethon expects to launch pivotal trials in Europe this year and the US.

Link to Genethon’s pipeline

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 Genethon contributed has obtained a product license. With more than 220 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 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.

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