Press Release For Immediate Release

Genethon Joins U.S. Bespoke Gene Therapy Consortium Dedicated to Finding Treatments for Ultra-Rare Diseases

PARIS, FRANCE (March XX, 2022) – Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Telethon), announced today it has joined the U.S.-based Bespoke Gene Therapy Consortium (BGTC) launched in October 2021 by the Foundation for the National Institutes of Health (FNIH) as part of its Accelerating Medicines Partnership® (AMP®) program.

Genethon, a global leader in gene therapy development for rare and ultra-rare diseases, is the only European non-profit research organization in the BGTC. The consortium is a public-private partnership bringing together the resources of the U.S. National Institutes of Health (NIH) and Food and Drug Administration (FDA) with biopharmaceutical companies and other non-profit groups. The goal is to speed development of customized (or bespoke) gene therapies for millions of people worldwide suffering from ultra-rare diseases.

"The Bespoke Gene Therapy consortium is highly strategic as it aims to build an innovative pathway for gene therapy for ultra-rare diseases," said Frédéric Revah, Ph.D., Genethon's CEO. "At Genethon, for nearly 20 years, we have been devoting our efforts and our expertise to the development of these breakthrough therapies for rare diseases with results that today show it is possible to overcome diseases long considered incurable. We are proud to contribute to the research of the Bespoke Gene Therapy Consortium. It is an opportunity which we sincerely hope that Europe will also seize."

Among the 8,000 rare diseases worldwide, 85% are ultra-rare, meaning they have a prevalence of less than one person per million people and may affect fewer than several hundred or only dozens of patients. In addition, many of these diseases are linked to mutations or changes in a single gene, making gene therapy a potential curative treatment for patients that have long been excluded from all research.

However, ultra-rare diseases represent a major challenge in terms of development, clinical trial design and economic model. Traditional marketing mechanisms are unsuitable and drug makers who, in recent years, have been pioneers with these diseases, are gradually refocusing on the most frequent rare diseases or on common pathologies.

BGTC has defined the following objectives:

- Basic research: better understand the basic biology of adeno-associated viruses (AAV) in order to optimize bioproduction technologies and increase their therapeutic efficacy.
- Clinical research: create a development model to shorten the time between preclinical studies and human trials. 3 to 6 trials should be funded under this program.

- Bioproduction: design a standard set of analytical tests for the manufacture of viral vectors. Analyzes that are broadly applicable to different manufacturing methods could improve and speed up the manufacturing and production processes of genes and vectors.
- Regulatory requirements: Streamline regulatory requirements and processes to expedite approvals from health authorities, in this case the FDA.

Genethon is pleased to join this partnership that includes 31 partners spanning the public and private sectors. Additional details can be found here.

About Genethon

A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a unique non-profit organization created by a patient association, the AFM-Telethon. A first gene therapy drug, to which Genethon contributed, has obtained marketing for spinal muscular atrophy. With 200+ scientists and professionals, Genethon is pursuing its mission to bring life-changing therapies to patients suffering from rare genetic diseases. 12 products resulting from Genethon's research are in clinical trials for eye, liver, blood, immune system and muscle diseases. A further 7 products are in the preparation phase for clinical trials over the next five years. Find out more: genethon.com

About The Foundation for the National Institutes of Health

The FNIH creates and manages alliances with public and private institutions in support of the mission of the NIH. The FNIH works with its partners to accelerate biomedical research and strategies against diseases and health concerns in the United States and across the globe. Established by Congress in 1990, the FNIH is a not-for-profit 501(c)(3) charitable organization. For additional information about the FNIH, please visit https://fnih.org.

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