Press Release For Immediate Release

Genethon Given PRIME Status by EMA for Gene Therapy To Treat Crigler-Najjar Syndrome, a Rare Liver Disease.

PARIS, FRANCE (March 6, 2023)—Genethon, a unique non-profit gene therapy R &D organisation founded by the French Muscular Dystrophy Association (AFM-Telethon), today announced that the European Medicines Agency (EMA) has granted PRIME (Priority Medicines) status to the gene therapy GNT-0003 currently in clinical trials for Crigler-Najjar syndrome, a rare liver disease. This status, granted only to drug candidates with major therapeutic potential, follows the successful completion of the first phases of the clinical trials.

"We're excited about the EMA's PRIME recognition of GNT-0003," said Genethon CEO Frederic Revah. "If successful, GNT-0003 would be the first gene therapy for Crigler-Najjar syndrome. The PRIME status is similar to the U.S. FDA's fast track and breakthrough designations. The EMA's program was started in 2016 and in the first five years, only 25% of eligible drug candidates received the PRIME designation."

PRIME status was granted to GNT-0003 following promising results from early phases of the European trial currently underway in collaboration with the CureCN consortium, and sponsored by Genethon. The trial is taking place in three countries: France, Italy and the Netherlands.

Crigler-Najjar syndrome is a rare genetic liver disease characterized by abnormally high levels of bilirubin in the blood (hyperbilirubinemia). Accumulation of bilirubin is caused by a deficiency of the UGT1A1 enzyme, responsible for transforming bilirubin into a substance that can be eliminated by the body.

High levels of bilirubin can result in significant neurological damage and death if not treated quickly. Symptoms of the most severe form of the disease become apparent shortly after birth. At present, patients must undergo phototherapy for up to 12 hours a day to keep their bilirubin levels below the toxicity threshold.

GNT-0003 combines normal copies of the UGT1A1 gene coding for the bilirubin metabolizing enzyme with an AAV vector. The gene therapy, administered intravenously, was designed by Genethon's Immunology and Gene Therapy of Liver Diseases team, led by Dr. Giuseppe Ronzitti.

In the early phases of the GNT-0003 clinical trials, the treatment of five adult patients demonstrated the safety and good tolerance of the gene therapy as well as a dose effect. In three patients treated at the highest dose, the bilirubin level decreased sufficiently to stop phototherapy for at least one year. The trial has now entered its pivotal phase, with the objective of confirming the efficacy of this dose in additional patients including children.

The EMA established the PRIME program to support the development of drugs that may benefit treatment-naive patients or offer a major therapeutic advantage over existing treatments. With this status, the developer of the drug candidate benefits from early and proactive support from the EMA throughout the clinical development process, which allows the drug candidate to be evaluated and made available to patients as soon as possible.

More information on the clinical trial : Crigler-Najjar syndrome (genethon.com)

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. Thirteen products resulting from Genethon's research are in clinical trials for liver, blood, immune system, muscle and eye, diseases. A further six products are in the preparation phase for clinical trials over the next five year.

US Contact:
Charles Craig
404-245-0591

EU Contact
Stephanie Bardon
communication@genethon.fr