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PRESS RELEASE

Hansa Biopharma and Genethon announce collaboration to develop imlifidase as pre-treatment to gene therapy in Crigler-Najjar syndrome patients with anti-AAV antibodies

Lund, Sweden and Evry, France April 27, 2023. Hansa Biopharma AB, "Hansa", (Nasdaq Stockholm: HNSA), a pioneer in enzyme technology for rare immunological conditions, and Genethon, a pioneer and a leader in gene therapy research and development for rare genetic diseases, today announced they have entered a research and development collaboration.

The collaboration will, in a clinical study, evaluate the safety and efficacy of Hansa's antibody cleaving enzyme imlifidase as a pre-treatment prior to the administration of Genethon's gene therapy product candidate GNT-0003 in Crigler-Najjar syndrome in patients with pre-existing neutralizing antibodies (NAbs) to adeno-associated virus serotype 8 (AAV8). The presence of circulating NAbs today excludes patients from entering clinical studies with potentially curative gene therapy treatments and from future access to approved gene therapies.

Søren Tulstrup, President and CEO, Hansa Biopharma stated: "Genethon is a pioneer at the cutting-edge of research and development of gene therapies for rare diseases and we are thrilled to be collaborating with them. This research collaboration further validates Hansa's commitment in gene therapy and underscores the important role that our antibody-cleaving enzyme technology can play in ensuring that even more patients can benefit from life-saving gene therapies".

GNT-0003 is currently being evaluated in a pivotal clinical study in France, Italy, and the Netherlands and has received PRIME (PRIority MEdicines) status from the EMA. Through the collaboration announced today, patients with Crigler-Najjar and pre-formed antibodies to AAV8 will be enrolled in a study with similar design where imlifidase is evaluated as a pre-treatment to enable gene therapy treatment with GNT-0003. The outcome of the ongoing clinical study of GNT-0003 could potentially form the basis for a MAA or BLA application in Europe or the US.

Crigler-Najjar is a genetic disease-causing bilirubin accumulation which leads to irreversible neurological damage manifested as muscle weakness, lethargy, deafness, mental retardation, and eye movement paralysis. Crigler-Najjar syndrome is an ultra-rare disease affecting less than one case per one million people per year.[1]

Frédéric Revah, CEO, Genethon added: "Patients with pre-existing neutralizing antibodies against AAV vectors cannot today benefit from gene therapy. This collaboration with Hansa Biopharma is thus an important next step in the development of our gene therapy treatment for Crigler-Najjar syndrome, Hansa Biopharma's proven enzyme technology coupled with its scientific expertise will help us advance the critical research we are conducting in Crigler-Najjar and could enable gene therapy treatment for patients who are today not eligible because of their immunological status.".

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Notes to editors

About imlifidase

Imlifidase is a unique antibody-cleaving enzyme originating from Streptococcus pyogenes that specifically targets IgG and inhibits IgG-mediated immune response. It has a rapid onset of action, cleaving IgG-antibodies and inhibiting their activity within hours after administration. Imlifidase has conditional marketing approval in Europe and is marketed under the trade name Idefirix® for the desensitization treatment of highly sensitized adult kidney transplant patients with a positive crossmatch against an available deceased donor. Imlifidase is currently being evaluated as a pre-treatment to gene therapy in three indications: Duchenne Muscular Dystrophy and Limb-Girdle Muscular Dystrophy through a collaboration agreement with Sarepta Therapeutics; and in Pompe disease through a collaboration agreement with AskBio.

About Hansa Biopharma

Hansa Biopharma is a pioneering commercial-stage biopharmaceutical company on a mission to develop and commercialize innovative, lifesaving and life-altering treatments for patients with rare immunological conditions. Hansa has developed a first-in-class immunoglobulin G (IgG) antibody-cleaving enzyme therapy, which has been shown to enable kidney transplantation in highly sensitized patients. Hansa has a rich and expanding research and development program based on the Company's proprietary IgG-cleaving enzyme technology platform, to address serious unmet medical needs in transplantation, autoimmune diseases, gene therapy and cancer. Hansa Biopharma is based in Lund, Sweden, and has operations in Europe and the U.S. The Company is listed on Nasdaq Stockholm under the ticker HNSA. Find out more at www.hansabiopharma.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.
Find out more at www.genethon.com

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About Crigler-Najjar syndrome

Crigler-Najjar syndrome is a rare genetic liver disease characterized by abnormally high levels of bilirubin in the blood (hyperbilirubinemia). This 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, and can result in significant neurological damage and death if not treated quickly. At present, patients must undergo phototherapy for up to 12 hours a day to keep their bilirubin levels below the toxicity threshold.

References

1. https://www.genethon.com/our-pipeline/crigler-najjar-syndrome/