PRESS RELEASE

MEDESIS PHARMA receives a grant from AFM-TELETHON to finance the first phases of the development of NanosiRNA-HD RNA interference therapy for Huntington's disease

Montpellier, France, July 20, 2022 at 8:00am CET – MEDESIS PHARMA, a pharmaceutical biotechnology company developing drug candidates based on its proprietary Aonys® technology for the administration of active ingredients in nano micelles by oral route, announces the decision of AFM-Téléthon to contribute 300,000 euros over 24 months to the first phases of preclinical development of its treatment for Huntington's disease.

The AFM-Téléthon, French Association against Myopathies has decided to participate in the financing of the preclinical program developed by MEDESIS PHARMA.

The Board of Directors of AFM-Téléthon, at its July 7, 2022 meeting, gave an agreement in principle for the financing of the NanosiRNA-HD project "Use of NanosiRNA for the treatment of Huntington's disease" for a duration of 24 months and a total amount of 300,000 euros excluding VAT, corresponding to 1/3 of the total cost of preclinical development.

The current project and the subject of the financing is a 24-month preclinical development (July 2022 - July 2024) with a pharmacokinetic / pharmaco distribution study, a pharmacological efficacy study and a toxicology study.

The positive results of these development phases will allow the implementation of the phase Ib/IIa clinical study on patients in several European countries, in accordance with the program validated by the European Medicines Agency.

This project would validate the first disease modifying therapy for Huntington's disease and would open new perspectives for the development of treatments based on RNA interference.

Huntington'Disease

Huntington's disease is a rare and hereditary neurodegenerative disease-causing motor, cognitive and psychiatric disorders that gradually worsen until a bedridden state and severe intellectual deterioration. Fatal outcome occurs on average twenty years after the onset of symptoms.

The disease is due to mutations consisting in an increased number of the CAG trinucleotide repeats in the HTT gene. Alleles with low (< 27) numbers of repeats are wild type; alleles with larger numbers of repeats are mutant (with phenotypic severity correlating with the number of repeats). A genetic test (analysis of the huntingtin gene from a blood sample) confirms the diagnosis.

The prevalence of Huntington's disease is approximately 8 cases per 100,000 individuals worldwide; the number of patients is estimated at around 100,000 and the number of carriers of the abnormal gene at 200,000. There is no treatment for Huntington's disease, current treatments are strictly symptomatic.

MEDESIS PHARMA is developing a genetic treatment program for Huntington's disease combining RNA interference technologies and its active ingredient delivery platform, Aonys.

Huntington patients are heterozygous: they have a mutant, dominant allele, and a wild-type allele. Because the mutant allele is dominant, efficient therapies will have to be repressive. But, because the HTT gene is essential, it will be important to not repress the wild-type allele, efficient therapies will have to be selective. The two alleles can be differentiated by the length of the CAG repeats but also by the presence of certain different nucleotides (single nucleotide polymorphism).

Medesis Pharma uses small interfering RNAs specifically targeting a different nucleotide on the mutated gene, which makes it possible to specifically reduce the production of the mutated protein and therefore its deleterious consequences. The first targeted nucleotide could be used in 50% of patients in the EU and North America.

Aonys, the active ingredient administration platform developed by MEDESIS PHARMA, makes it possible to administer these unmodified interfering RNAs by the buccal route by protecting them from their administration by a deposition in the mouth, loading In HDL lipoproteins until their delivery in all the cells of the body including the brain (passage of the Blood-Brain Barrier).

About Medesis Pharma

To advance the treatment of serious diseases without effective treatments, Medesis Pharma creates drug candidates based on its proprietary Aonys® technology for the oral administration of active ingredients in nanodroplet form, enabling active ingredients to be effectively delivered to all cells, with passage through the blood–brain barrier (BBB). This innovative approach is being applied for future drugs to treat major diseases that do not have effective treatments: Alzheimer's Disease, Huntington's Disease, certain resistant cancers and severe respiratory inflammations such as those linked to COVID-19. Medesis Pharma is also developing dedicated treatments for people contaminated or irradiated following a civil or military nuclear accident.

Medesis Pharma, a French biopharmaceutical company based near Montpellier, has a track record of 15 scientific publications, holds 11 patents family and 71 patents, reflecting 17 years of research, and is focused specifically on four projects that are moving into Clinical Phase II for neurodegenerative diseases and the treatment of Covid-19. Building on its world-renowned positions, Medesis Pharma is also working on new applications for its technology in partnership with public research laboratories (CNRS, CEA, IRBA), major teaching hospital centers in France, Canada and the United States, as well as private structures such as Transgene.

Medesis Pharma's shares are listed on Euronext Growth Paris (FR0010844464 – ALMDP

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About the AFM-Téléthon

The AFM-Téléthon is an association of patients and parents of patients committed to the fight against the disease. Thanks to Telethon donations, it has become a major player in biomedical research for rare diseases in France and around the world. Today, it supports clinical trials concerning genetic diseases of sight, blood, brain, immune system and muscle. Through its laboratories, it is also an atypical association capable of designing, producing and testing its own innovative therapy drugs.