PRESS RELEASE

MEDESIS PHARMA: the development plan of its drug candidate for the treatment of Huntington's Disease receives a positive opinion from the European Medicines Agency

Montpellier (France), June 3, 2021 - 8:00am – Medesis Pharma (ISIN: FR0010844464, Ticker: ALMDP), a pharmaceutical biotechnology company developing drug candidates with its proprietary oral nanodroplet active ingredient administration technology, is announcing today that the development program for its drug candidate NanosiRNA HD, for the treatment of Huntington's Disease, received a positive opinion at the end of May from the European Medicines Agency's Scientific Committee. Submitted beginning of March 2021, the development plan covers the pharmaceutical, preclinical and clinical milestones for NanosiRNA HD. NanosiRNA HD is one of the four drug candidates currently under development by Medesis Pharma.

Huntington's Disease (HD) is a neurodegenerative disease affecting the central nervous system and characterized by involuntary chorea movements, behavioral disorders and psychiatric disorders. HD is an orphan disease with a prevalence among the Caucasian population estimated at 1/10,000 to 1/20,000. Disease progression has significant impact on quality of life, with loss of autonomy requiring lifetime daily assistance.

Innovative approach further strengthened with the proprietary Aonys® technology for the buccal administration of active ingredients in a microemulsion

Huntington's Disease is caused by CAG triplet repeat expansions (to 36 repeats or higher) on the short arm of chromosome 4(4p16.3) in the huntingtin HTT gene. A curative treatment must block the expression of the abnormal gene.

The therapeutic approaches developed to date by the scientific community have been discontinued. They targeted the huntingtin gene by blocking both the wild huntingtin (paramount for normal brain function) and the mutant huntingtin. The tested candidates were administered either intrathecally (into the cerebrospinal fluid) or directly into the brain. There is no curative treatment to date.

Medesis Pharma's drug candidate specifically targets the abnormal part of the gene. The Aonys technology enables a non-invasive buccal administration and crosses the blood–brain barrier (BBB) for an intracerebral delivery of the active ingredient. The specific targeting of solely the mutant allele represents a major challenge: mutant and wild sequences are identical, with the exception of their number of CAG trinucleotide repeats. The HTT gene contains some single-nucleotide polymorphisms (SNPs) with allele frequencies among the human population. It is therefore possible to specifically target the mutant ARNms with a specific ARNi for the allele targeting the SNPs. As the relationship between the individual SNPs and the CAG repeats depends on each patient's genetic information, the patient's HTT locus will need to be sequenced before choosing the siRNAs. This type of personalized medicine is now possible thanks to the development of human genome sequencing, making NanosiRNA HD a completely innovative project.

Medesis Pharma's development plan for NanosiRNA HD, reviewed by the EMA, covers an initial CMC (chemical, manufacturing and control) and preclinical development phase for 9 to 12 months, followed by about a year of clinical development in several European countries. The clinical results could be known by mid-2023, with a possibility to quickly get market access worldwide offering a new treatment option for HD patients.

About the European Medicines Agency's scientific advice

The European Medicines Agency (EMA) can provide medicine developers with advice on the most appropriate way to generate robust evidence on a medicine's benefits and risks. The EMA provides scientific advice to support the timely and sound development of high-quality, effective and safe medicines, for the benefit of patients.

Scientific advice and protocol assistance are particularly useful to medicine developers when they are developing an innovative medicine and there appears to be no or insufficient relevant detail in EU guidelines or guidance documents, or in Pharmacopoeia monographs, including draft documents or monographs released for consultation.

Scientific advice helps to ensure that developers perform the appropriate tests and studies, so that no major objections regarding the design of the tests are likely to be raised during the evaluation of the marketing authorization application. This also helps avoid patients taking part in studies that will not produce useful evidence.

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 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 nine 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).

Learn more at
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