Press release

GenSight Biologics receives FDA Orphan Drug Designation for GS030 in Retinitis Pigmentosa

Paris, France, January 31, 2017 – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company that discovers and develops innovative gene therapies for neurodegenerative retinal diseases and diseases of the central nervous system, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to the Company's product candidate GS030 for the treatment of retinitis pigmentosa.

"The Orphan Drug Designation both in Europe and in the United States, together with the Advanced Therapy Medicinal Product classification in Europe, fully recognize the urgent and unmet medical need for a safe and effective treatment for retinitis pigmentosa patients, and highlight the potential of optogenetics and GS030 to address it," commented **Bernard Gilly**, Chief Executive Officer of GenSight Biologics.

GS030 is currently undergoing a Good Laboratory Practices (GLP) regulatory toxicity study, and is expected to enter the clinic with a Phase I/II clinical trial in retinitis pigmentosa patients in Q3 2017, subject to toxicity results and future regulatory review.

The FDA grants orphan drug designation status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than 200,000 persons in the United States. The orphan drug designation provides GenSight with incentives and benefits in the US, including a 7-year period of market exclusivity if GS030 is approved for the treatment of retinitis pigmentosa patients.

GS030 had received both Orphan Drug Designation and Advanced Therapy Medicinal Product classification in Europe in September 2016.

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biotechnology company discovering and developing novel therapies for neurodegenerative retinal diseases and diseases of the central nervous system. GenSight Biologics' pipeline leverages two core technology platforms, the Mitochondrial Targeting Sequence (MTS) and optogenetics for retinitis pigmentosa, to help preserve or restore vision in patients suffering from severe degenerative retinal diseases. GenSight Biologics' lead product candidate, GS010, is in Phase III trials in Leber's Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible low vision and legal blindness in teens and young adults. Using its gene therapy-based approach, GenSight Biologics' product candidates are designed to be administered in a single treatment to each eye by intravitreal injection to offer patients a sustainable functional visual recovery.

About GS030

GS030 leverages GenSight's optogenetics technology platform, a novel approach to restore vision to patients by using gene therapy to introduce a gene encoding for light-sensitive protein into specific target cells in the retina by injection in order to make them responsive to light. An external wearable medical device to specifically stimulate the transduced cells is currently being developed to amplify the light signal and enable vision restoration. Patients will need to wear the external wearable device in order to enable restoration of visual function. Using this optogenetics technology platform, and with the support of the Vision Institute in Paris, GenSight is developing its second product candidate, GS030, to restore vision in patients suffering from Retinis Pigmentosa, or RP. RP is an orphan disease caused by multiple mutations in several genes involved in the visual cycle. GenSight's optogenetics technology platform is independent of the specific genetic mutations that lead to the disease. On average, RP patients begin experiencing vision loss in their young adult years, eventually turning blind around the age of 40 to 45. There is currently no existing treatment for RP. RP has an estimated prevalence of 1.5 million people throughout the world. It is expected that GS030 would benefit patients in the early stages of RP.

About Optogenetics

Optogenetics is a biological technique which involves the transfer of a gene encoding for a light sensitive protein to cause neuronal cells to respond to light stimulation. As a result, it is a neuromodulation method that can be used to modify or control the activities of individual neurons in living tissue and even in-vivo, with a very high spatial and temporal resolution. Optogenetics combines the use of gene therapy methods to transfer the gene into target neurons and the use of optics and optronics to deliver the light to the transduced cells. Optogenetics is widely used by research labs throughout the world and hold clinical promise in the field of vision impairment or neurological disorders.