Press release

GenSight Biologics receives Orphan Drug Designation and Advanced Therapy Medicinal Product classification in Europe for GS030 in Retinitis Pigmentosa

Paris, September 1, 2016 – GenSight Biologics (Euronext: SIGHT, 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 European Commission, based on a favorable recommendation from the European Medicines Agency (EMA), has granted orphan drug designation (ODD) to the Company's product candidate GS030 for the treatment of retinitis pigmentosa. The EMA also granted Advanced Therapy Medicinal Product (ATMP) classification to GS030.

"We are very excited that GS030 has received both Orphan Drug Designation and Advanced Therapy Medicinal Product classification in Europe, recognizing the urgent and unmet medical need for a safe and effective treatment for retinitis pigmentosa patients, and the potential of GS030 to address it. This will allow us to optimize future steps to further advance GS030 toward regulatory approval", commented **Jean-Philippe Combal**, Chief Operating Officer of GenSight Biologics.

GS030 is currently undergoing preclinical development, and is expected to initiate in September 2016 a Good Laboratory Practices (GLP) regulatory toxicity study in non-human primates, prior to entering 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 European Commission 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 five in 10,000 persons in the European Union (EU). The orphan drug designation provides GenSight with incentives and benefits in the EU, including a period of market exclusivity if GS030 is approved for the treatment of retinitis pigmentosa patients.

Medicines classified as ATMP are deemed to offer groundbreaking innovative opportunities for the treatment of certain conditions with high unmet needs. The classification provides GenSight with scientific regulatory guidance from the EMA, notably on the applicable regulatory framework and pathway, as well as a centralized approval procedure in Europe.

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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, Mitochondrial Targeting Sequence (MTS) and optogenetics, 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 vision loss in teens and young adults. Using its gene therapy-based approach, GenSight Biologics' product candidates are designed to offer patients a sustainable functional visual recovery with a single treatment to each eye through an intravitreal injection.

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, 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.