

GenSight Biologics Announces Positive Data Safety Monitoring Board Review of PIONEER Phase I/II Trial of GS030 Combining Gene Therapy and Optogenetics for the Treatment of Retinitis Pigmentosa

Paris, France, April 14, 2020, 7.30 am CEST – GenSight Biologics (Euronext : SIGHT, ISIN : FR0013183985, PEA-PME eligible), a biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders, today announced that the independent Data Safety Monitoring Board (DSMB) completed its second planned safety review of the ongoing PIONEER Phase I/II clinical trial of GS030, GenSight's novel product combining gene therapy and optogenetics for the treatment of Retinitis Pigmentosa (RP). The DSMB confirmed the absence of any safety issues for the second cohort of three subjects, who each received a single intravitreal injection of 1.5×10^{11} vg (viral genomes) and used a wearable optronic visual stimulation device after the injection. The DSMB recommended moving forward as planned without any modification in the protocol and recruiting the third cohort of three subjects who are to receive the maximal dose of 5×10^{11} vg.

"We are pleased to be able to move forward with our second program GS030, which has now treated 6 patients. We look forward to confirming the safety of GS030 at the highest dose and to demonstrate efficacy by showing signs of functional vision recovery in advanced stage RP patients," commented **Magali Taiel, MD**, Chief Medical Officer of GenSight Biologics.

The use of corticosteroids pre- and post-gene therapy injection, performed as part of the protocol to prevent and minimize inflammatory response, could potentially expose new patients to a higher risk of COVID-19 infection. Thus, to protect the safety of patients in the current COVID-19 situation, the Company and investigators have together decided to delay recruiting new patients into the third cohort until the public health situation has improved. As RP is a chronic disease, it does not require urgent treatment. In the interim, the six patients treated in the first two cohorts are being remotely monitored for safety aspects by investigators.

GenSight expects to complete enrollment in the second half of 2020. In the context of COVID-19, this will have to be more precisely assessed as soon as possible. Early findings from the first two cohorts may be available in the course of 2020.

About GS030 and the PIONEER Phase I/II trial

PIONEER is a first-in-man, multi-center, open label dose-escalation study to evaluate the safety and tolerability of GS030 in 18 subjects with Retinitis Pigmentosa. GS030 combines a gene therapy (GS030-DP) administered via a single intravitreal injection with a wearable optronic visual stimulation device (GS030-MD).

GS030 is based on a novel optogenetics technology platform developed by GenSight, which uses gene therapy to introduce a gene encoding for a light-sensitive protein into retinal ganglion cells by a single intravitreal injection, making them responsive to light and bypassing photoreceptors killed off by the disease. The optronic device works in conjunction with the gene therapy by activating the newly photo-sensitive retinal ganglion cells.



Eligible patients in the first three cohorts are those affected by end-stage non-syndromic RP with no light perception (NLP) or light perception (LP) levels of visual acuity. The extension cohort will include patients with hand motion (HM) and counting fingers (CF) levels of visual acuity.

As per protocol, three cohorts with three subjects each will be administered an increasing dose of GS030-DP via a single intravitreal injection in their worse affected eye. An extension cohort will receive the highest tolerated dose. The DSMB reviewed safety data of all treated subjects in each cohort and made recommendations before moving to the next dose.

The primary outcome analysis will be the safety and tolerability at one year post-injection.

GS030 has been granted Orphan Drug Designation in the United States and Europe. PIONEER is being conducted in three centers across the United Kingdom, France and the United States.

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders. GenSight Biologics' pipeline leverages two core technology platforms, the Mitochondrial Targeting Sequence (MTS) and optogenetics to help preserve or restore vision in patients suffering from blinding retinal diseases. GenSight Biologics' lead product candidate, LUMEVOQ® (GS010), is in Phase III trials in Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible 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.