Press Release

GenSight Biologics announces positive Data Safety Monitoring Board review and continuation of PIONEER Phase I/II clinical trial of GS030 combining gene therapy and Optogenetics for the treatment of Retinitis Pigmentosa

Paris, France, May 7, 2019, 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 first safety review of the ongoing PIONEER Phase I/II clinical trial of GS030 combining gene therapy and optogenetics for the treatment of Retinitis Pigmentosa. The DSMB confirmed the absence of any safety issues for the first cohort of three subjects who received a single intravitreal injection of 5e10 vg combined with a wearable optronic visual stimulation device. The DSMB recommended moving forward as planned without any modification in the protocol and recruiting the second cohort of three subjects receiving an escalating dose of 1.5e11 vg.

"We are pleased to be able to move forward GS030, our second clinical stage program. We look forward to confirm the safety of GS030 at higher doses and to demonstrate efficacy in restoring useful visual functions in RP patients," commented **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight.

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 the 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 disease-destroyed photoreceptors.

Eligible patients in the first three cohorts will be those affected by end-stage non-syndromic Retinitis Pigmentosa 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 of 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 will review safety data of all treated subjects in each cohort and make recommendations before moving to the next dose.

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

GS030 was 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.

GenSight expects to complete enrollment in the first half of 2020. Early findings may be available and released in the second half of 2019, and preliminary results are expected in the fourth quarter of 2020.

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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, 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.

About GS030

GS030 leverages GenSight's optogenetics technology platform, a novel approach to restore vision in patients by using gene therapy to introduce a gene encoding for a light-sensitive protein into specifically targeted cells of the retina by a single injection, thereby making them responsive to light. An external wearable medical device to specifically stimulate the transduced cells is developed to amplify the light signal and further enable vision. Patients will need to wear the external wearable device to enable optimal restoration of visual function. Using this optogenetics technology platform, and with the support of the Vision Institute in Paris and the team of Dr. Botond Roska at the Friedrich Miescher Institute in Basel, GenSight is developing its second product candidate, GS030, to restore vision in patients suffering from Retinitis Pigmentosa, or RP. GenSight's optogenetics technology platform is independent of the specific genetic mutations that lead to this family of disease. It is expected that GS030 would benefit patients from the early stages of RP. This technology offers the possibility of application to other diseases of the retina where photoreceptors have degenerated, and may be transferable to the dry form of Age Related Macular Degeneration (dry-AMD).

About Optogenetics

Optogenetics is a biological technique that 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 a gene into target neurons with the use of optics and electronics (optronics) to deliver the light to the transduced cells. Optogenetics is widely used by research laboratories throughout the world and holds clinical promise in the field of vision impairment or degenerative neurological disorders.

About Retinitis Pigmentosa (RP)

Retinitis Pigmentosa (RP) is a family of orphan genetic diseases caused by multiple mutations in numerous genes involved in the visual cycle. Over 100 genetic defects have been implicated. RP patients generally begin experiencing vision loss in their young adult years, with progression to blindness by age 40. RP is the most widespread hereditary cause of blindness in developed nations, with a prevalence of about 1.5 million people throughout the world. In Europe and the United States, about 350,000 to 400,000 patients suffer from RP, and every year between 15,000 and 20,000 new patients with RP lose sight. There is currently no existing curative treatment for RP.