NEWS RELEASE

GenSight Biologics Provides Update on European Medicines Agency Scientific Advice for LUMEVOQ®

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- Two-arm Phase III trial with sham control arm vs. LUMEVOQ® bilateral injection considered acceptable for assessing efficacy
- Open-label provision to provide LUMEVOQ® bilateral treatment to sham subjects after positive primary analysis
- GenSight to continue discussions with EMA and other regulatory authorities to define the optimal path to marketing authorization for LUMEVOQ®

PARIS--(BUSINESS WIRE)-- Regulatory News:

GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company focused on developing and commercializing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders, today provided an update on the scientific advice it received from the European Medicines Agency (EMA) regarding the design of a new Phase III trial for LUMEVOQ®, the Company's gene therapy for LHON caused by a mutated ND4 mitochondrial gene.

The new study, which will be called RECOVER, will be a randomized controlled trial with a two-arm design: a sham control arm, in which a sham procedure mimics an injection into each eye but no substance is injected into the eye, and a treatment arm, in which subjects will be given bilateral intravitreal injections of LUMEVOQ® (also known as GS010) The Agency found that the "proposed study design with bilateral administration appears acceptable to assess the benefits of GS010 in patients who require both eyes to be treated". The proposed study design also contains an open-label provision, in which subjects in the sham arm will be eligible to receive LUMEVOQ® bilateral injection if the primary endpoint is met.

The Agency provided further guidance on planned statistical analyses and advised on topics that the Company will consider as it refines and finalizes the study design.

The RECOVER study is designed to address the questions raised by the EMA's Committee for Advanced Therapies (CAT) when it reviewed the MAA filed in 2020. The Company decided to withdraw the dossier in April 2023 to be able to discuss the Agency's concerns more fully. RECOVER will be able to begin recruiting once the design is finalized; the product is manufactured and released for human use; and approval is obtained from local competent authorities and ethics committees. GenSight expects to initiate the study in Q2 2024 and to have it completed by H2 2026.

"We are grateful that key aspects of RECOVER have been endorsed so that we can work on refining and optimizing the design," said Bernard Gilly, Chief Executive Officer and Co-Founder of GenSight Biologics. "We continue to keep in sight the goal of providing access as quickly as possible for LHON patients, and so we will strive to provide evidence that meets regulators' expectations."

GenSight also plans to share key aspects of RECOVER's design with other regulatory authorities such as the UK's Medicines and Healthcare products Regulatory Agency (MHRA) and the U.S. Food and Drug Administration (FDA). An initial discussion with the MHRA is scheduled to take place in November 2023. The Company plans to engage with the FDA in the coming months, so that RECOVER results will be accepted by all major regulatory authorities.

About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biopharma company focused on developing and commercializing 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; lenadogene nolparvovec), is an investigational compound and has not been registered in any country at this stage; a marketing authorization application is currently under review by the EMA for the treatment of Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease affecting primarily teens and young adults that leads to irreversible blindness. 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 LUMEVOQ ® (GS010 ; lenadogene nolparvovec)

LUMEVOQ® (GS010; lenadogene nolparvovec) targets Leber Hereditary Optic Neuropathy (LHON) by leveraging a mitochondrial targeting sequence (MTS) proprietary technology platform, arising from research conducted at the

Institut de la Vision in Paris, which, when associated with the gene of interest, allows the platform to specifically address defects inside the mitochondria using an AAV vector (Adeno-Associated Virus). The gene of interest is transferred into the cell to be expressed and produces the functional protein, which will then be shuttled to the mitochondria through specific nucleotidic sequences in order to restore the missing or deficient mitochondrial function. “LUMEVOQ” was accepted as the invented name for GS010 (lenadogene nolparvovec) by the European Medicines Agency (EMA) in October 2018. LUMEVOQ® (GS010; lenadogene nolparvovec), is an investigational compound and has not been registered in any country at this stage.

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