Press Release

GenSight Biologics announces regulatory authorizations for Individual Patient Expanded Access treatment with GS010/LUMEVOQ® in the US

- FDA authorization and Institutional Review Board (IRB) approval followed by QP release for expanded access treatment of one patient in the US
- Treatment scheduled in November 2025 at the University of Pittsburgh School of Medicine
- Ongoing review of early access dossier by French agency ANSM
- Technology transfer to new manufacturing partner on track to produce new batches for 2026 milestones

Paris, France, October 30, 2025, 7:30 am CET – 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 announced that the Company's gene therapy GS010/LUMEVOQ® has been granted regulatory authorizations for an Individual Patient Expanded Access in the United States. The authorizations mark the first set of regulatory green lights for the candidate product since the Company's withdrawal of its European marketing authorization application in 2023.

FDA authorization was granted for expanded access treatment of one eligible patient, based on an application by a physician at the University of Pittsburgh School of Medicine (UPMC) to the agency. The required approval of the school's Institutional Review Board (IRB) was also granted. The patient is scheduled to be treated in November 2025.

GS010/LUMEVOQ® is in Phase III of its clinical development as a treatment for the rare blinding disease Leber Hereditary Optic Neuropathy (LHON) caused by a mutated *ND4* mitochondrial gene and has not received marketing authorization in any country.

Company Maintains Strategic Focus on Manufacturing and Clinical Development

"*We are very pleased to be able to provide GS010/LUMEVOQ for the individual patient expanded access treatment at UPMC,*" said **Laurence Rodriguez**, Chief Executive Officer of GenSight Biologics. "*At this time, the company's highest priority is on ensuring adequate supply for the study already under discussion with the ANSM and to support our discussions about an Early Access Program in France. These programs represent essential steps towards our ultimate goal of maximizing patient access to GS010/LUMEVOQ, given the high unmet need of those impacted by LHON.*"

The Company is currently completing the technology transfer to its new manufacturing partner, Catalent, which is expected to be finalized by year-end 2025. The transition will enable the production of new batches in 2026 to address the full scope of projected clinical and early access needs.

GenSight Biologics is preparing for the launch in H2 2026 of the pivotal Phase III study RECOVER for GS010/LUMEVOQ®, while engaging with the French medicines agency ANSM regarding a dose-ranging study the agency requested in connection with an Early Access Program (AAC) in France. The study protocol submitted in August 2025, is under review with well-defined regulatory timelines.

In parallel, the Company is pursuing opportunities to out-license GS010 in markets outside the USA and Europe, while exploring paid Early Access Programs worldwide.

About Leber Hereditary Optic Neuropathy (LHON)

LHON is a rare, maternally inherited mitochondrial genetic disease, characterized by the degeneration of retinal ganglion cells, which results in precipitous and usually irreversible vision loss and typically leads to legal blindness. The *ND4* mitochondrial mutation is the most common of the mutations that cause LHON and is associated with the worst prognosis among the leading mutations.

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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, GS010 (lenadogene nolparvovec) is in Phase III in Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible blindness in teens and young adults. GS010 is currently in clinical development, has not to date been granted marketing authorization in France or any other jurisdiction, and is therefore not available commercially. 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 GS010/LUMEVOQ® (lenadogene nolparvovec)

GS010/LUMEVOQ® (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 is then shuttled to the mitochondria through specific nucleotidic sequences in order to restore the missing or deficient mitochondrial function. GS010/LUMEVOQ® (lenadogene nolparvovec) is in Phase III of its clinical development. It has not been granted marketing authorization in any country and is not available commercially.