

GenSight Biologics Announces the Granting of Compassionate Use Authorization (CUA/AAC) for GS010/LUMEVOQ® in France

- French medicines agency ANSM has granted authorization for named patient early access program for GS010/LUMEVOQ®, a gene therapy indicated for the treatment of ND4-LHON.

Paris, France, December 22, 2025, 7:00 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 French medicines safety agency ANSM (*Agence nationale de sécurité du médicament et des produits de santé*) has granted compassionate use authorization (*Autorisation d'Accès Compassionnel*, or AAC) for the use of the GS010/LUMEVOQ® gene therapy¹.

The AAC Program in France is a national scheme that enables patients suffering from serious, rare or disabling diseases to benefit from a treatment that does not have marketing authorization, when there are an unmet medical need and no appropriate therapy. To be eligible for an AAC program, the candidate treatment must present a favorable benefit-risk ratio. Requests for AAC may be initiated only by healthcare professionals, who submit named patient requests to the ANSM, which then evaluates and authorizes the access requests. Patients for whom applications for treatment with GS010 are submitted must meet specific eligibility criteria, including the length of time since the onset of their vision loss.

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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¹ GS010/LUMEVOQ® has not received marketing authorization in any country and is not commercially available.



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. Thanks to its gene therapy-based approach, GenSight Biologics' candidates are designed to be administered as a single intravitreal injection per eye.

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.