Press Release

GenSight Biologics Announces Publication on Matching-Adjusted Indirect Comparison of Leber Hereditary Optic Neuropathy Treatments

Paris, France, Monday, May 11, 2026, 6:30 pm CEST – 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 the publication of a Matching Adjusted Indirect Comparison (MAIC) of outcomes from the clinical trials of GS010/LUMEVOQ®, the Company's gene therapy investigational product for the treatment of *ND4*-LHON (Leber Hereditary Optic Neuropathy),[1] and idebenone, the only approved treatment for LHON. The study was published in the April 2026 issue of the journal *British Journal of Ophthalmology*.

The full article, named "*Efficacy of lenadogene nolparvovec gene therapy versus idebenone in Leber hereditary optic neuropathy due to the m.11778G>A MT- ND4 variant: two matching adjusted indirect comparisons*", is available online on **this link**.

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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. GS030, in a Phase I/II clinical trial, is an optogenetic treatment that is being investigated as a mutation-agnostic treatment for late stage Retinitis Pigmentosa, the leading cause of blindness in the world. 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 Leber Hereditary Optic Neuropathy (LHON)

Leber Hereditary Optic Neuropathy (LHON) is a rare maternally inherited mitochondrial genetic disease, characterized by the degeneration of retinal ganglion cells that results in brutal and irreversible vision loss that can lead to legal blindness, and mainly affects adolescents and young adults. LHON is associated with painless, sudden loss of central vision in the 1st eye, with the 2nd eye sequentially impaired. It is a symmetric disease with poor functional visual recovery. 97% of patients have bilateral involvement at less than one year of onset of vision loss, and in 25% of cases, vision loss occurs in both eyes simultaneously.

[1] GS010/LUMEVOQ® has not, to date, received marketing authorization in any country and is therefore not available commercially.

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) has not been registered in any country at this stage.