

GenSight Biologics Announces LUMEVOQ® Scientific Updates at NANOS 2025

Paris, France, Monday, March 17, 2025, 7:30 am CET – 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 new scientific data and analyses on the gene therapy LUMEVOQ® will be presented at the 51st Annual Meeting of the North American Neuro-Ophthalmology Society (NANOS) in Tucson, Arizona, USA (March 15-20, 2025).

Leading Leber Hereditary Optic Neuropathy (LHON) Key Opinion Leaders will share new data on predictive factors of response to LUMEVOQ® treatment; on a comparison of the treatment outcomes from idebenone and LUMEVOQ®; on real-world experience with LUMEVOQ®; and on long-term outcomes from bilateral treatment with the gene therapy.

Poster presentation: “*Predictive Factors of Improved Final Visual Outcome in Patients with Leber Hereditary Optic Neuropathy Treated with Lenadogene Nolparvovec Gene Therapy*”

- Presenter: **Robert C. Sergott, MD**, Wills Eye Hospital, Philadelphia, USA
- Poster Number 209
- Time: Monday, March 17th, 2025, 5:00 pm – 6:00 pm (MDT)
- Location: Arizona Ballroom 1-6

Poster presentation: “*Efficacy of Lenadogene Nolparvovec Gene Therapy Versus Idebenone: Two Matched Adjusted Indirect Comparisons*”

- Presenter: **Patrick Yu-Wai-Man, MD, PhD**, University of Cambridge, Moorfields Eye Hospital, and the UCL Institute of Ophthalmology, UK
- Poster Number 186
- Time: Monday, March 17th, 2025, 6:00 pm – 7:00 pm (MDT)
- Location: Arizona Ballroom 1-6

Poster presentation: “*Efficacy and Safety of Lenadogene Nolparvovec Gene Therapy for Leber Hereditary Optic Neuropathy in the Real-Life Setting*”

- Presenter: **Mark L. Moster, MD**, Wills Eye Hospital, Philadelphia, USA
- Poster Number 12
- Time: Sunday, March 16th, 2025, 2:00 pm – 3:00 pm (MDT)
- Location: Arizona Ballroom 1-6

Platform presentation: “*Long-Term Outcomes of Bilateral Injection of Lenadogene Nolparvovec Gene Therapy for Leber Hereditary Optic Neuropathy*”

- Presenter: **Nancy J. Newman, MD**, Emory University School of Medicine, Atlanta, USA
- Scientific Platform Session I
- Time: Monday, March 17th, 2025, 11:45 am – 12:00 pm (MDT)
- Location: Tucson Ballroom

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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 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 subjects 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.

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.