

## GenSight Biologics Announces LUMEVOQ® Scientific Updates at NANOS 2024

**Paris, France, Monday, March 4, 2024, 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 50<sup>th</sup> Annual Meeting of the North American Neuro-Ophthalmology Society (NANOS) in Honolulu, Hawaii (March 2-7, 2024).

Leading Leber Hereditary Optic Neuropathy (LHON) Key Opinion Leaders will provide updates on evidence related to the contralateral effect, on real-world experience with LUMEVOQ®, and on the comparison between LHON natural history, idebenone and LUMEVOQ®.

Poster presentation: “*Unilateral injection of lenadogene nolparvovec gene therapy induces successful transfection of retinal ganglion cells in both eyes*”

- Presenter: **Alfredo A. Sadun, MD, PhD**, Doheny Eye Centers-UCLA, Pasadena, California, USA
- Time: Sunday, March 3<sup>rd</sup>, 2024, from 12:00 - 1:00 pm HST
- Location: Lanai room

Poster presentation: “*In the real-life setting, lenadogene nolparvovec gene therapy improves visual acuity in patients with MT-ND4 LHON*”

- Presenter: **Chiara La Morgia, MD**, University of Bologna, Italy
- Time: Tuesday, March 5<sup>th</sup>, 2024, from 7:00 - 8:00 pm HST
- Location: Lanai room

Poster presentation: “*Lenadogene nolparvovec gene therapy improves visual recovery more than idebenone in m.11778G>A MT-ND4 LHON, and both treatments exceed the natural course of the disease*”

- Presenter: **Nancy J. Newman, MD**, Emory University School of Medicine, Atlanta, Georgia, USA
- Time: Tuesday, March 5<sup>th</sup>, 2024, from 8:00 - 9:30 pm HST
- Location: Lanai room

In addition, **Benson S. Chen, MBChB, MSc, FRACP**, Department of Clinical Neurosciences, University of Cambridge and Cambridge Eye Unit, Addenbrooke’s Hospital, United Kingdom, will present evidence on the quality of life of LHON patients in a platform presentation.

Platform presentation: “*Vision-Related Quality of Life in LHON Patients Treated with Lenadogene Nolparvovec Gene Therapy: Analysis of the VFQ-25 Using Rasch Measurement Theory*”

- Scientific Platform Session III
- Time: Tuesday, March 5<sup>th</sup>, 2024, from 11:15 - 11:30 am HST
- Location: Kauai/Maui (GS) room

## Contacts

### GenSight Biologics

Chief Financial Officer

Ivan Tortet

[itortet@GENSIGHT-BIOLOGICS.com](mailto:itortet@GENSIGHT-BIOLOGICS.com)

### LifeSci Advisors

Investor Relations

Guillaume van Renterghem

[gvanrenterghem@lifesciadvisors.com](mailto:gvanrenterghem@lifesciadvisors.com)

+41 (0)76 735 01 31

## 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 1<sup>st</sup> eye, with the 2<sup>nd</sup> 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 nolparovec)

LUMEVOQ® (GS010; lenadogene nolparovec) 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 nolparovec) by the European Medicines Agency (EMA) in October 2018.