Press Release

GenSight Biologics Announces LUMEVOQ® Scientific Updates at AAO 2024

Paris, France, Thursday, October 17, 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 2024 annual meeting of the American Academy of Ophthalmology (AAO) in Chicago, Illinois (October 18-21, 2024).

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

Poster: "*Meta-analysis of Treatment Outcomes for Patients with m.11778G>A MT-ND4 Leber Hereditary Optic Neuropathy*" (Poster PO004)

- Presenter: **Nancy J. Newman, MD**, Emory University School of Medicine, Atlanta, Georgia, USA
- Time: Saturday, October 19, 2024, from 9:15 - 10:15 AM CDT
- Location: Hall A, Poster Theater

Presentation: "*Efficacy and Safety of Lenadogene Nolparvovec Gene Therapy for LHON in Real-Life Settings*" (PA023)

- Presenter: **Valerie Biousse, MD**, Emory University School of Medicine, Atlanta, Georgia, USA
- Session: OP04 Neuro-Ophthalmology Original Papers
- Time: Saturday, October 19, 2024, from 2:48 - 3:00 PM CDT
- Location: S405

Poster: "*Post-Mortem Analyses with Histopathological and Molecular Assessments Following AAV2 Gene Therapy in LHON"* (Poster PO093)

- Presenter: **Alfredo A. Sadun, MD, PhD**, Doheny Eye Centers-UCLA, Pasadena, California, USA
- Time: Sunday, October 20, 2024, from 9:15 - 9:45 AM CDT
- Location: Station 2, Poster Discussion Lobby

Presentation: "*Four-Year Results of Bilateral Injection of Lenadogene Nolparvovec Gene Therapy for LHON*" (PA047)

- Presenter: **Patrick Yu-Wai-Man, FRCOphth, MBBS, PhD**, University of Cambridge, Cambridge, England
- Session: OP08 Retina, Vitreous Original Papers
- Time: Sunday, October 20, 2024, from 2:48 – 2:55 PM CDT
- Location: S405

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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.