Press Release

GenSight Biologics to Host Key Opinion Leader Webcast on Outcomes Among Compassionate Use Patients Treated Bilaterally with LUMEVOQ® in the US

- December 2, 2021, at 8:00 am EST / 2:00 pm CET
- Connection details for live call and translation provided

Paris, France, November 30, 2021, 7:30 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 provided connection details for the webcast it will host with **Dr. Sean P. Donahue, MD, PhD,** of **Vanderbilt University** on outcomes among six Leber Hereditary Optic Neuropathy (LHON) patients treated bilaterally with LUMEVOQ® under an FDA-approved compassionate use protocol.

Dr. Donahue presented his findings at the 2021 Annual Meeting of the American Academy of Ophthalmology (AAO), held in New Orleans, Louisiana, USA, from November 12-15.

The clinical data of the patients, who had not qualified for the REFLECT Phase III trial of LUMEVOQ® because of age limitations or because enrollment had closed, indicated that bilateral intravitreal injection of the gene therapy in these adolescents and young adults resulted in "*a substantial improvement in visual function*" and preservation of retinal anatomy, while being associated with good tolerability of the drug. Dr. Donahue, the Coleman Professor and Vice Chair for Clinical Affairs, and Chief of the Pediatric Ophthalmology Department of the Vanderbilt Children's Hospital in Nashville, TN, further noted that the outcomes were "*not consistent with the published natural history of the 11778 [ND4] mutation*".

Thursday, December 2, 2021 (KOL Call)
8:00 - 9:00 am EST / 2:00 - 3:00 pm CET

Live webcast in English: https://bit.ly/31geZ2k
Simultaneous French translation: https://bit.ly/3lidNTf

Following his presentation, Dr. Donahue will be available for questions. The webcast will be **conducted in English, with a simultaneous French translation**. For those unable to attend the live broadcast, a recording will be accessible using the same links.

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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, to help preserve or restore vision in patients suffering from blinding retinal diseases. GenSight Biologics' lead product candidate, LUMEVOQ® (GS010; lenadogene nolparvovec), has been submitted for marketing approval in Europe for the treatment of Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease affecting primarily teens and young adults that leads to irreversible blindness. 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. The estimated incidence of LHON is approximately 800-1,200 new patients who lose their sight every year in the United States and the European Union.

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.