Press Release

GenSight Biologics Announces Publication of a Study of the impact of LHON disease on the lives of Patients and Relatives in *Journal of Neuro-Ophthalmology*

- Study is first of its kind to explore the impact of LHON on patients and their relatives in four different countries
- Study determined that the impact of LHON extends beyond vision-related activity limitations

Paris, France, May 19, 2022, 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 announced that the highly-regarded *Journal of Neuro-Ophthalmology* has published a qualitative study to explore the impact of Leber Hereditary Optic Neuropathy (LHON) on patients and their relatives. The study was featured in a paper published on the journal's website last month titled, *"The Impact of Leber Hereditary Optic Neuropathy on the Quality of Life of Patients and Their Relatives: A Qualitative* Study". It is the first of its kind to explore this condition and its impact for patients and relatives in four different countries.

The study determined that the impact of LHON extends beyond vision-related activity limitations, while addressing its psychosocial impact. It concluded that helping patients and their relatives adapt and cope with vision loss is vital. An accurate and timely diagnosis for patients is also crucial to address these issues and also to allow for early intervention.

"*This study is the first to describe the impact of LHON on the families of affected individuals. Partners and families of affected individuals take on many responsibilities and shoulder some of the burden of LHON. The impact that LHON has on partners and families has not been reported previously and it is vital that we acknowledge the support and care that they provide*," explained **Patrick Yu-Wai-Man**, MD, PhD, Moorfields Eye Hospital and Department of Clinical Neurosciences, University of Cambridge, United Kingdom.

Participants reported feeling devastated by the diagnosis of LHON after a lengthy and worrisome diagnostic journey. They were also frustrated by the loss of autonomy that affected their relatives. Participants described challenges across several domains: physical capabilities, emotional well-being, interpersonal relationships, work and studies, finances, and recreational activities.

Additionally, the study determined that despite residing in different countries, LHON patients and their relatives described similar experiences in the four areas of focus in this study. These areas include (1) experience leading to the point of diagnosis; (2) impact of their condition on various aspects of life; (3) perceptions about treatment; and (4) expectations toward future therapies.

"*This study confirms what we have known all along in the eye clinic - that LHON impacts every aspect of quality of life, not just activities that rely on vision. Understanding how LHON affects individuals who*

develop vision loss enables doctors to intervene early and provide care that will improve the quality of life of affected individuals," commented **Benson Chen**, MD, Department of Clinical Neurosciences, University of Cambridge, United Kingdom. *"This might include different kinds of assessments in the eye clinic that measure the emotional and psychological impact of LHON or developing the referral pathways that enable affected individuals to access psychological support and work or skills re-training*," he added.

LHON is an inherited mitochondrial disease characterized by severe bilateral vision loss and chronic visual impairment. The objective of this study was to comprehensively explore the impact of LHON on the lives of patients and their relatives at the time of diagnosis and now.

The qualitative study design encompassed eight focus group interviews conducted in France, Germany, the United Kingdom, and the United States, involving 17 individuals with m.11778G>A mutation and their relatives. Separate focus groups for patients and their relatives were facilitated by a moderator in French, German, or English. Neuro-ophthalmologists in the four countries who participated helped to identify additional patients who fulfilled under-represented sampling criteria. The four countries were selected because all have established networks of individuals with LHON and were places where previous LHON studies have been conducted.

Focus group interviews were conducted as part of a market research study sponsored by GenSight Biologics, and independently designed and conducted by groupH, a health care market research and analytics firm. The design and conduct of the study complied with the European Pharmaceutical Market Research Association and British Healthcare Business Intelligence Association guidelines.

"*We, at GenSight, have long been convinced that by providing an innovative therapeutic solution for LHON we would offer much more than an improved vision. Documenting the impact of LHON experienced by all study participants highlights the fact that we could empower patients and improve their ability to enjoy a fulfilling life, while easing the demands on their loved ones. This is precisely why we are dedicated to developing a long-lasting therapeutic solution for patients with LHON,*" explained **Magali Taiel**, Chief Medical Officer of GenSight. "*We are hopeful that we may one day help patients to recover not only their vision, but also their sense of autonomy and well-being,"* she added.

Full text versions of this article are available on the journal's website: https://journals.lww.com/jneuro-ophthalmology/Abstract/9900/The_Impact_of_Leber_Hereditary_Optic_Neuropathy_on.77.aspx.

***About the paper:**

The Impact of Leber Hereditary Optic Neuropathy on the Quality of Life of Patients and Their Relatives: A Qualitative Study

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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.