Press Release

The *Quinze-Vingts* Hospital and GenSight Biologics announce a first Temporary Authorization for Use (ATU) for LUMEVOQ™ (GS010) in France

Paris, France, December 9, 2019, 7.30 am CET – The National Eye Hospital (CHNO) of the *Quinze-Vingts* in Paris and 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 the French Competent Authority, the National Drug Safety Agency (*Agence Nationale de Sécurité du Médicament* or ANSM), granted a named patient Temporary Authorization for Use ("ATU nominative") for LUMEVOQ™ (GS010) to the CHNO of the *Quinze-Vingts*. Dr Catherine Vignal, who as the prescribing physician originated the request, will be able to use LUMEVOQ™ to treat a patient recently affected by Leber Hereditary Optic Neuropathy (LHON). GenSight Biologics committed to provide the drug for a bilateral injection.

"*As we are preparing to file for Marketing Approval in Europe in 2020, this ATU testifies to the strong interest of the medical community for* LUMEVOQ™ *and to the impact on patients,*" said **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight. "*We are of course willing to provide available products if more requests are authorized*."

Leber Hereditary Optic Neuropathy (LHON) is a rare, maternally inherited mitochondrial genetic disease, characterized by the degeneration of retinal ganglion cells that results in precipitous and irreversible vision loss typically leading to legal blindness. The disease mainly affects adolescents and young adults. LHON is associated with painless, dramatic and sudden loss of central vision in the first eye, with the second eye becoming irreversibly involved shortly after. 97% of patients have bilateral involvement within one year of onset of vision loss, and in 25% of cases, vision loss occurs in both eyes simultaneously. LHON causes the blindness of an estimated 1,400 to 1,500 new patients each year in the United States and Europe.

"*France's ATU program is a powerful means of providing LHON patients with the ND4 mutation, with a therapeutic solution, LUMEVOQ gene therapy,*" said **Dr. Catherine Vignal**, Principal Investigator for LUMEVOQ™ trials at the Department of Ophthalmology at *Centre Hospitalier National d'Ophtalmologie des Quinze-Vingts* (Department led by Dr José-Alain Sahel) and Head of the department of Neuro-Ophthalmology at the Rothschild Foundation, Paris. *"To me as a clinician, it is important that I can now offer my patients a treatment for their condition, in close collaboration with the Regulatory Competent Authorities, while waiting for official marketing authorization."*

The temporary authorization is the outcome of a close partnership between physicians and pharmacists from the CHNO of the *Quinze-Vingts*, the "*Ouvrir les yeux*" (*Open the eyes*) patient advocacy group and GenSight Biologics, to the benefit of patients affected by LHON.

"*Access to treatment by gene therapy to people affected with LHON is the result a decade of research at the Institut de la Vision and a successful partnership with the teams at GenSight Biologics*." noted **Dr. José-Alain Sahel**, Director of the *Institut de la Vision* (Sorbonne-Université/Inserm/CNRS), Paris; Chairman of the Department of Ophthalmology at *Centre Hospitalier National d'Ophtalmologie des*

XV-XX, Paris; Professor and Chairman of the Department of Ophthalmology at University of Pittsburgh School of Medicine and UPMC (University of Pittsburgh Medical Center); and co-founder of GenSight.

"*Our association is deeply involved in all the steps that can improve the lives of patients with inherited neuropathy and their families. Gene therapy aims to bring about an improvement in one of the 3 main mutations of LHON,*" said **Maryse Roger**, President of the "*Ouvrir les yeux*" patient advocacy group. *"We hope for the rapid completion of the various ongoing research so that all patients can benefit from treatment as soon as possible. In the meantime, Ouvrir les yeux will continue to enable the voices of patients brutally struck by blindness to be heard."*

In France, use of pharmaceutical products not yet approved with a Marketing Authorization (AMM) and not recruiting for a clinical trial requires first obtaining an ATU from the ANSM.

Named patient ATUs are granted by the ANSM under the following conditions:

- The product is meant to treat, prevent or diagnose a severe or rare disease,
- No other appropriate treatment is available in France,
- The product's efficacy and safety are presumed in the state of scientific knowledge,
- The ATU is requested by and remains under the responsibility of the prescribing physician when the drug has the potential to benefit the patient.

"*The Quinze-Vingts Hospital, the world's oldest institution for fighting blindness, fully supports the innovation strategy led by the hospital's clinical teams and those of the Institut de la Vision,*" said **Jean-François Ségovia**, Director of the *Centre Hospitalier National d'Ophtalmologie* (National Eye Hospital) *des Quinze-Vingts.*

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About the *Centre hospitalier national d'ophtalmologie des Quinze-Vingts* (National Eye Hospital)

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.

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About GS010

GS010 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.