Press Release

GenSight Biologics Announces Publication of LUMEVOQ® Safety Data over 5-Year Period in renowned Peer-Reviewed *American Journal of Ophthalmology*

- Pooled analysis of safety data from 5 clinical studies in 189 patients constituting the largest ever *ND4*-LHON cohort treated with gene therapy
- Confirmation of the long-term safety profile of intravitreal injections of LUMEVOQ® with data covering a 5-year period following administration
- Comparable safety outcomes among bilaterally treated patients and those receiving one unilateral injection

Paris, France, March 9, 2023, 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 the publication of a peer-reviewed article in the renowned *American Journal of Ophthalmology* focusing on pooled safety data from the 5 clinical studies with lenadogene nolparvovec (LUMEVOQ®). This analysis constitutes the largest cohort of *ND4*-LHON patients studied after gene therapy treatment. It confirms the good overall safety profile of the product in terms of systemic and ocular tolerability, humoral and cellular immune response and highlights a comparable safety profile for unilaterally and bilaterally treated patients.

"The publication of this safety analysis is a key milestone, combining data from all 5 clinical studies of LUMEVOQ® and representing the largest cohort of ND4-LHON patients ever treated with gene therapy," said **Dr. Catherine Vignal-Clermont, MD,** Department of Neuro Ophthalmology and Emergencies at the *Rothschild Foundation Hospital* in Paris and lead author of the article. *"We are very pleased to see our work published in such a prestigious journal, which highlights the significance of our findings. Given the good safety profile demonstrated, we believe lenadogene nolparvovec is proving itself as a potential new treatment solution for ND4-LHON patients as a clinically relevant patient benefit has been established with minimal safety risks."*

The article, entitled *"Safety of lenadogene nolparvovec gene therapy over 5 years in 189 patients with Leber hereditary optic neuropathy"*, analyzes safety data collected from *ND4*-LHON patients who received single unilateral or bilateral intravitreal injections of lenadogene nolparvovec across 5 clinical studies (REVEAL, RESCUE, REVERSE, RESTORE and REFLECT) who were followed over five years after treatment. Almost all patients (95%) received lenadogene nolparvovec at a dose of 9×10^{10} viral genomes per eye and 88% had at least 2 years of follow-up.

Systemic adverse events (AE) were mainly mild in intensity and were exceptionally related to lenadogene nolparvovec or the study procedure. The absence of systemic issues related to lenadogene nolparvovec is mainly supported by the limited bio-dissemination of the product. Furthermore, the systemic humoral and cellular immune response was limited, acknowledging the local ocular nature of the immune response. Also, ocular AE were mostly mild and were responsive to conventional ophthalmologic treatments.

This safety profile was consistent across patients, whether treated bilaterally or unilaterally, in terms of AE, bio-dissemination and immunogenicity. No significant differences in the frequency, nature or severity of systemic and ocular AE were found between these two groups of patients.

The article is available in print and online via this link.

Contacts

GenSight Biologics
Corporate Communications Director
Clothilde Caillet
ccaillet@gensight-biologics.com

RooneyPartners
Media Relations
Jeanene Timberlake
jtimberlake@rooneypartners.com
+1 646-770-8858

LifeSci Advisors
Investor Relations
Guillaume van Renterghem
gvanrenterghem@lifesciadvisors.com
+41 (0)76 735 01 31

Orpheon Finance
Retail Investors
James Palmer
j.palmer@orpheonfinance.com
+33 (0)7 60 92 77 74

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), is an investigational compound and has not been registered in any country at this stage; a marketing authorization application is currently under review by the EMA 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 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), is an investigational compound and has not been registered in any country at this stage; a marketing authorization application is currently under review by the EMA.