Press Release

GenSight Biologics reports validation of LUMEVOQ® Marketing Authorisation Application (MAA) by European Medicines Agency

Paris, France, Tuesday, November 3, 2020, 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 reported that the LUMEVOQ® Marketing Authorisation Application (MAA) passed the validation checks required for submissions to the European Medicines Agency (EMA), triggering the official start of the MAA review procedure. The application for use of LUMEVOQ® gene therapy to treat vision loss in patients with Leber Hereditary Optic Neuropathy (LHON) due to a mutated *ND4* mitochondrial gene was submitted in September, and the procedure was formally initiated on October 29.

"We are delighted that the formal review is now underway and look forward to clarifying any questions that the EMA's scientific committees may have," commented **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight. *"Meanwhile, we continue apace with our commercial preparations to ensure that LUMEVOQ® becomes available to LHON patients at the earliest possible time."*

LHON is a rare, mitochondrial genetic disease, mainly affecting young males. The *ND4* mutation results in the worst visual outcomes, with most patients becoming legally blind. There continues to be a high unmet medical need for the 800-1200 new *ND4* LHON patients in Europe and the U.S. each year, particularly those who are struck blind in their prime working years.

Lenadogene nolparvovec (tradename: LUMEVOQ®) is a recombinant adeno-associated viral vector, serotype 2 (rAAV2/2), containing a cDNA encoding the human wild-type mitochondrial NADH dehydrogenase 4 protein (ND4), which was specifically developed for the treatment of LHON associated with mutation in the *ND4* gene. GenSight submitted the MAA based on the benefit-risk balance established by results from a Phase I/IIa study (CLIN-01), two pivotal Phase III efficacy studies (CLIN-03A: RESCUE, and CLIN-03B: REVERSE) and the long-term follow up study of RESCUE and REVERSE (CLIN 06 - readout at Year 3 post injection), supported by a statistics-based indirect comparison methodology to establish the gene therapy's efficacy compared to natural history.

The next major administrative milestone step in the review is a pause at Day 120, at which point the Committee for Advanced Therapies will issue a first round of questions and the Company responds as well as submitting information that had been pre-agreed with the agency.

The Company is also working towards submitting LUMEVOQ®'s Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) in H2 2021.

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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. 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. Developed as a treatment for Leber Hereditary Optic Neuropathy (LHON), GenSight Biologics' lead product candidate, LUMEVOQ® (GS010; lenadogene nolparvovec), is currently in the review phase of its registration process in Europe, and in Phase III to move forward to a BLA filing in the U.S.

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® is currently in the review phase of its registration process in Europe, and in Phase III to move forward to a BLA filing in the U.S.

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 1,400 to 1,500 new patients who lose their sight every year in the United States and Europe.