Press Release

GenSight Biologics Announces Submission of LUMEVOQ® Dossier to ANSM to Prepare for Restart of Early Access Program in France

- Updated file will support medicines safety agency's review of individual applications for early access (AAC) use
- First injections expected in December 2024

Paris, France, November 13, 2024, 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 submission of the updated regulatory file for LUMEVOQ® gene therapy to the French medicines safety agency *Agence Nationale de Sécurité du Médicament et des Produits de Santé* (ANSM) to prepare for the restart of the early access (AAC) program in France.

The submission documents the successful manufacture of LUMEVOQ®, including the blending of two GMP drug substance batches to optimize the number of vials available for clinical use and the passing of all required quality control tests. LUMEVOQ® is being developed as a treatment for Leber Hereditary Optic Neuropathy (LHON) caused by a mutated *ND4* mitochondrial gene, a rare mitochondrial genetic disease that causes acute and usually irreversible loss of vision.

"*With the submission, we mark the successful transition from manufacturing to regulatory review*," commented **Laurence Rodriguez**, GenSight Biologics Chief Executive Officer. "*We join the LHON community in looking forward to the expeditious resumption of early access to LUMEVOQ by year's end*."

The updated regulatory file will support the ANSM's assessment of individual applications for compassionate use, which can now be submitted by healthcare professionals under the rules of the AAC program. GenSight Biologics anticipates a review period for these applications and will work closely with the ANSM to optimize the assessment timeline.

GenSight Biologics is preparing to initiate the supply of the drug to the designated treatment center, the Quinze-Vingts hospital in Paris, in mid-December. In parallel, the Company is working with the hospital's administrative and medical teams to enable the first injections to be administered by the end of December.

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About GenSight Biologics

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.

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 subjects 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.

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) has not been registered in any country at this stage.