

GenSight Biologics Announces Successful Completion of LUMEVOQ® Engineering Batch Validating Implemented Corrective Actions

- Successful engineering batch validates actions taken to correct low yields in previous runs
- Yield significantly improved to a level superior to any previous batches
- Committee for Medicinal Products for Human Use (CHMP) opinion still expected by Q3 2023

Paris, France, September 19, 2022, 7:30 am CEST – 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 successful completion of the first engineering batch incorporating refinements in the manufacturing process of LUMEVOQ®, the Company's gene therapy for Leber Hereditary Optic Neuropathy (LHON). The batch was the first to implement a set of targeted corrective measures around enhanced process control and strengthened on-site supervision that were identified by the Company and its manufacturing partner in the United States in April this year¹.

“The yield achieved by this engineering batch is extremely positive and gives us greater confidence in our ability to consistently manufacture LUMEVOQ at the best quality of execution going forward,” commented **Bernard Gilly**, Chief Executive Officer and Co-Founder of GenSight Biologics. *“While this manufacturing setback delayed the initial timeline, we want to reiterate to LHON patients and their physicians that we continue to work tirelessly to get LUMEVOQ approved to bring them a therapeutic solution as quickly and as safely as possible.”*

The successful engineering run generated drug substance whose viral genome titer achieved the acceptance threshold, thereby confirming that the manufacturing process of LUMEVOQ® is sound at the defined batch size. In addition, the results demonstrate that corrective actions have successfully fixed issues in the filtration steps of the downstream process, resulting in a significantly improved product yield that had not been achieved previously.

The Company is now moving forward with a renewed confidence to resume Early Access Program and clinical development and to prepare for commercialization. GenSight still expects to receive the opinion from the European Committee for Medicinal Products for Human Use (CHMP) by Q3 2023, to be followed by commercial launch by the end of 2023.

¹ In April 2022, GenSight announced a delay in the completion of the validation batches for the Company's product for Leber Hereditary Optic Neuropathy (LHON). This delay, granted by the European Medicines Agency (EMA), was required to implement necessary operational adjustments with its manufacturing partner to ensure a reliable execution of its process. The success of this first engineering run demonstrates that the adjustments implemented by GenSight in close collaboration with its manufacturing partner addressed the execution issues and even enhanced the execution of its manufacturing process.

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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), 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.