Press Release

GenSight Biologics Announces Successful Manufacture of LUMEVOQ® GMP Batch

- Confirms corrections implemented in remediation plan
- All protocols successfully followed during manufacture of first GMP (Good Manufacturing Practice) batch
- Batch may provide product supply in Q1 2024 for potential resumption of early access program and initiation of a possible new clinical trial, as well as additional data for planned regulatory submission
- Manufacturing of a second GMP batch underway to provide additional product and data; results expected in October 2023

Paris, France, September 18, 2023, 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 that the Company's manufacturing partner in the US has successfully manufactured the drug substance (DS) for LUMEVOQ®, the Company's gene therapy for Leber Hereditary Optic Neuropathy (LHON), compliant with Good Manufacturing Practice (GMP) standards.

Because the batch was manufactured according to GMP manufacturing protocols, which are the required standards for commercial batches, the gene therapy may be eligible for use with patients after passing all quality control tests and pending discussions with regulatory bodies. The manufacturing of a second GMP DS batch, which will increase the amount that can be made available to patients, is already underway, with vg titer results expected in October 2023.

"I would like to congratulate the entire GenSight team, as well as our manufacturing partner, for all the hard work that enabled us to cross this important milestone," said **Bernard Gilly,** Chief Executive Officer and Co-Founder of GenSight Biologics. *"With this achievement, we confirm that our robust manufacturing process can be executed at the best level of quality as required by authorities. LUMEVOQ should once again be available to patients and physicians in early 2024. The greater clarity in our timelines is great news for LHON patients struck blind by the disease."*

GenSight is planning to manufacture at least 3 GMP batches at commercial scale outside the context of a validation campaign[1], to generate more process data for a future Marketing Authorisation Application (MAA) submission, to provide more experience of the manufacturing process to the operating teams, and to fulfil the immediate requirement of supplying product for a possible new clinical trial and for the potential resumption of an early access program for patients in Q1 2024.

[1] A validation campaign, or Process Performance Qualification (PPQ) campaign, consists of at least 3 successful GMP (Good Manufacturing Practice, required standards for human use outside of a clinical trial) batches manufactured sequentially to demonstrate and document the robustness, control, consistency and reproducibility of the commercial manufacturing process at the designated commercial facility. This exercise is required only as part of a Marketing Authorisation Application with the EMA and a Biologics License Application (BLA) with the FDA.

Contacts

GenSight Biologics
Chief Financial Officer
Thomas Gidoin
tgidoin@gensight-biologics.com

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

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.