Press Release

GenSight Biologics Provides 2021 Operations Update in the Context of COVID-19

Paris, France, May 14, 2021, 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 provides an update on the impact of COVID-19 on its operations in 2021.

"It has been slightly over a year since we had to adjust our operations to the COVID-19 situation and provided you with an update on our activities. Although conditions have improved in many regions, the burden of the COVID-19 crisis continues to impact the medical and regulatory ecosystems. GenSight is preparing for the commercialization of LUMEVOQ first in Europe in 2022, then in North America a year later, despite the US Defense Production Act introducing a slight delay in manufacturing our validation batches," said **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight Biologics. "*Our team will be ready in due time to deliver LUMEVOQ to patients affected with this highly debilitating disease and continue to work tirelessly to develop alternative strategies to minimize the impact of COVID-19 on our operations."*

LUMEVOQ® Commercial Launch in Europe Still Expected in H1 2022

The **REVERSE and RESCUE** Phase III trials of LUMEVOQ® (GS010) for the treatment of Leber Hereditary Optic Neuropathy (LHON) are completed, and patients have been transferred to long-term follow-up, the RESTORE study, for an additional three-year period. The sustained efficacy of LUMEVOQ® three years after injection was previously reported. Patients are now followed-up annually, and given the follow-up nature of these visits and the stability of patients with no safety concern, delaying some of these visits has been an acceptable precautionary measure, which should have no impact on the conduct of the trial, and will be properly documented and reported to regulators.

The **strategic manufacturing partner (CDMO)** for LUMEVOQ®, ThermoFischer Scientific (TFS) in Boston, USA, is maintaining its operations and is due to manufacture three validation batches to support the MAA filing with the European Medicines Agency (EMA) in Europe. TFS informed the Company that, as a consequence of the US Defense Production Act (DPA), American suppliers have had to redirect certain consumables towards the manufacture of COVID vaccines in the US. It is our understanding that this is an Industry issue impacting many manufacturers, biotech and pharmaceutical companies in particular. This has resulted in extended timelines for the availability of some items required for manufacturing LUMEVOQ®. Accordingly, the Company anticipated delays in providing data from the planned validation batches to the EMA and, after discussions with the European Agency, agreed upon an extended clock-stop period. Responses to the Agency D120 questions are therefore now due by January 2022 instead of the previously anticipated August 2021. Based on this new timeline the Company now expects EMA approval for LUMEVOQ® to shift from Q4 2021 to H1 2022. The revised timeline will be confirmed as soon as there is greater clarity from TFS on material availability. The timing for commercialization remains unchanged and the Company will continue to build the European commercial platform during 2021 to prepare the commercial launch of LUMEVOQ® in Europe, still expected in H1 2022.

LUMEVOQ® Regulatory Pathway in the US: REFLECT Phase III read-out in June 2021; BLA Submission Now Expected Q2 2022

The **REFLECT** Phase III trial of LUMEVOQ® is fully recruited with a primary endpoint at 78 weeks. Although some on-site visits had to be postponed due to COVID-19 travel restrictions, the Company closely partnered with clinical sites and properly documented and reported delays to regulators, as well as pre-specified them in the Statistical Analysis Plan (SAP), in agreement with biostatisticians, before database lock. Consequently, GenSight Biologics was able to collect data from 95 out of 98 patients with no consequence on the primary endpoint, other than a delay in the 78-week read out from Q1 initially to June 2021. The Company expects to meet with the U.S. Food and Drug Administration (FDA) for a pre-BLA meeting in Q3 2021. Due to the impact of the US DPA on the manufacturing of LUMEVOQ's validation batches, the regulatory submission target in the US is now Q2 2022.

PIONEER Phase I/II Clinical Trial of GS030 in Retinitis Pigmentosa (RP)

In order to protect patients, the Company and investigators together decided to delay recruiting new patients into the 3rd cohort of the **PIONEER** Phase I/II clinical trial of GS030 until the COVID-19 situation had improved, as RP is a chronic disease and does not require urgent treatment. The use of corticosteroids pre- and post-gene therapy injection, performed as part of the protocol to minimize inflammatory response, was deemed by GenSight and investigators to expose patients to a higher risk of COVID-19 infection. In the interim, the six patients in the first two cohorts were remotely monitored for safety aspects by investigators. Consequently, recruitment took longer than originally planned.

PIONEER, combining gene therapy and optogenetics for the treatment of RP, has now fully completed recruitment of the 3rd cohort. The Data Safety Monitoring Board (DSMB) is expected to make a recommendation on the optimal dose to use in the extension cohort in the coming weeks. GenSight Biologics expects to complete the recruitment of the extension cohort by the end of 2021. In the meantime, the Company expects to report early findings shortly in Q2 2021 and more preliminary results later in the second half of the year.

LUMEVOQ® Temporary Authorization for Use, Compassionate Use and Early Access Programs

Additional patients were treated with LUMEVOQ® in France in Q1 2021 under a **nominative Temporary Authorization for Use (ATU)** granted by the French National Drug Safety Agency (*Agence Nationale de Sécurité du Médicament* or ANSM). Additional ATUs have been requested by the CHNO of the Quinze-Vingts in Paris.

GenSight is committed to providing the drug, subject to available stock. For now, the Company does not foresee any shortage due to the impact of the DPA on TFS in the US and is closely monitoring the situation. Bilateral injections are priced at €700,000 per patient and are expected to generate revenues prior to regulatory approval and official reimbursement in France. In addition, the Company has submitted to the French ANSM an application for a cohort ATU to further facilitate access to LUMEVOQ® for patients in France. The application is under review and patients can benefit from nominative ATUs in the meantime.

Compassionate use in Italy was granted with some patients already treated in Q1 2021. A compassionate use program in Germany is under review by competent authorities. Patients have also been treated in the United States under an Expanded Access Program granted by the FDA. For all these programs, LUMEVOQ® is provided free of charge to requesting physicians.

GenSight continues to implement measures to protect its staff against COVID-19 by putting in place telecommuting for all employees. These measures have not affected activities carried out at its Paris headquarters.

The Company is financed until at least the end of Q2 2023 and is able to face any evolution of the COVID-19 situation with as much flexibility and foresight as required.

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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.