Press Release

GenSight Biologics Announces Approval of the LUMEVOQ® Cohort Temporary Authorization for Use (ATUc) in France

Paris, France, July 5, 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 announced that the French Competent Authority, the National Agency for Medicines and Health Products Safety (*Agence Nationale de Sécurité du Médicament et des produits de santé* or ANSM), granted a Cohort Temporary Authorization for Use ("*ATU de Cohorte*" or ATUc) for LUMEVOQ® in the treatment of Leber Hereditary Optic Neuropathy (LHON) caused by a mutated *ND4* gene.

LUMEVOQ® was first approved for early access in France in December 2019 when the ANSM authorized a Named Patient ATU ("*ATU Nominative*" or ATUn) for the *CHNO des Quinze-Vingts* Hospital in Paris. To date, 18 patients have been treated under an ATUn. Under Named Patient ATUs, physicians have to submit individual requests to the ANSM for each patient. The Cohort ATU greatly simplifies the process by which patients gain access to LUMEVOQ® prior to EU marketing authorization expected in H1 2022. French hospital-based physicians, including those practicing outside the *Quinze-Vingts* Hospital in Paris, will now be able to request treatment for eligible patients directly from GenSight Biologics. The ATUc also allows the Company to monitor patients more systematically and to collect data that would allow the safety and efficacy of LUMEVOQ® to be assessed for these patients. Under the ATUc, GenSight Biologics will provide LUMEVOQ® to hospitals at a price similar to that in the current ATUn.

"*The decision of the French ANSM to authorize LUMEVOQ to be administered under a Cohort ATU will facilitate early access to treatment for patients with LHON and indeed attests to the safety and efficacy of LUMEVOQ,*" said **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight. "*The compassionate use and expanded access programs already running in Europe and the US will also allow GenSight to collect additional data that will bolster the already impressive evidence on LUMEVOQ's clinical benefit and the safety profile and support our drive to obtain marketing authorization in Europe and North America*."

Mr. Gilly further noted, "*GenSight's achievement of the Cohort ATU highlights the importance of several initiatives contained in the Health Innovation 2030 plan that was recently announced by the French President to support the growth of innovative healthcare startups*."

LHON is a rare, maternally inherited mitochondrial genetic disease, characterized by the degeneration of retinal ganglion cells that results in precipitous and irreversible vision loss typically leading to legal blindness. The disease mainly affects adolescents and young adults, striking patients suddenly and painlessly. LHON causes the blindness of an estimated 1,200 to 1,500 new patients each year in the United States and Europe. The *ND4* mitochondrial mutation, which LUMEVOQ® targets, is associated with the most severe clinical form of LHON, with poor overall visual outcomes.[1]

Under the Cohort ATU, LUMEVOQ® will be administered as a bilateral intravitreal injection to patients with vision loss due to LHON caused by a confirmed G11778A mutation in the *ND4* mitochondrial gene.

In France, pharmaceutical products not yet granted Marketing Authorization and not recruiting for a clinical trial can only be used if the ANSM authorizes an ATU. ATUs are reserved for products whose efficacy and safety are "strongly presumed" based on clinical trial data and whose therapeutic indication targets a serious, rare or disabling disease lacking appropriate treatment. The products are innovative, and the company filing the application must commit to submitting a Marketing Authorisation Application within one year from the approval of the Cohort ATU.

A Cohort ATU is granted only after a company submits a successful application to the ANSM, detailing how patients will be treated and monitored in a Protocol for Therapeutic Use ("*Protocole d'Utilisation Thérapeutique*", or PUT). The authorization takes effect after the agency completes its review of the PUT and annexes submitted in the application. The granting of a Cohort ATU usually precedes the Marketing Authorisation.

Reference:

1. Newman NJ, Carelli V, Taiel M, Yu-Wai-Man P. Visual outcomes in Leber hereditary optic neuropathy subjects with the m.11778G>A (MTND4) mitochondrial dna mutation. *J Neuroophthalmol.* (2020) 40:547–57. doi: 10.1097/WNO.0000000000001045.

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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), has been submitted for marketing approval in Europe 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 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 1,200-1,500 new patients who lose their sight every year in the United States and the European Union.

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.