Press Release

UK MHRA grants GenSight Biologics' LUMEVOQ® ophthalmic gene therapy *Promising Innovative Medicine* designation

Paris, France, September 6, 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 its gene therapy LUMEVOQ® has been granted Promising Innovative Medicine (PIM) designation by the UK's Medicines and Healthcare products Regulatory Agency (MHRA) for the treatment of vision loss due to Leber Hereditary Optic Neuropathy (LHON) caused by a confirmed G11778A mutation in the *ND4* mitochondrial gene.

"*This is great news for those in the UK affected by LHON, as they currently have limited treatment options,*" said **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight. "*This marks another important milestone in our preparations to make LUMEVOQ available to LHON patients as soon as possible.*"

PIM designation is given to a medicinal product that is likely to offer a major advantage for patients. For the MHRA to grant a PIM designation, the product must meet each of the following three criteria:

- The condition should be life-threatening or seriously debilitating with high unmet need, meaning there is no method of treatment, diagnosis or prevention available, or existing methods have serious limitations
- The medicinal product is likely to offer major advantage over methods currently used in the UK; preliminary evidence should be submitted based on both non-clinical and clinical data
- The potential adverse effects of the medicinal product are likely to be outweighed by the benefits, allowing for the reasonable expectation of a positive benefit-risk balance

PIM designation is also an early indication that a medicine is a promising candidate for the MHRA's Early Access to Medicines Scheme (EAMS) in the treatment, diagnosis or prevention of life-threatening or seriously debilitating conditions with an unmet need. The EAMS is similar to the Cohort ATU (*Autorisation Temporaire d'Utilisation de Cohorte* [Cohort Temporary Authorisation for use]) program in France, which simplifies the process by which patients could be treated with promising treatments ahead of market authorization. LUMEVOQ® was approved for a French Cohort ATU in July 2021.

LHON is a rare genetic disease that results in severe and irreversible vision loss that mainly affects adolescents and young adults. The *ND4* mutation is the most common and results in the worst visual outcomes, with most patients becoming legally blind.[1] Treatment options for LHON are limited.[2]

LUMEVOQ® is not yet licensed in the EU or in the UK for the treatment of patients with vision loss due to Leber Hereditary Optic Neuropathy (LHON). Its Marketing Authorisation Application is currently under review by the European Medicines Agency, with a decision expected in H1 2022.

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[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.

[2] Sundaramurthy, S., SelvaKumar, A., Ching, J., et al. Leber hereditary optic neuropathy—new insights and old challenges. Graefes Arch. Clin. Exp. Ophthalmol. (2020). doi:10.1007/s00417-020-04993-1.

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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.

About the Early Access to Medicines Scheme (EAMS)

The Early Access to Medicines Scheme (EAMS) aims to provide earlier availability of promising new unlicensed medicines to UK patients with high unmet clinical need. A Promising Innovative Medicine (PIM) designation is an early indication that a medicine is a potential candidate for the EAMS scheme. A PIM designation should not be regarded as a future commitment by the MHRA to license such a medicine.