Press Release

GenSight Biologics Submits EU Marketing Authorisation Application for LUMEVOQ® Gene Therapy to Treat Vision Loss due to Leber Hereditary Optic Neuropathy (LHON)

- EMA decision expected in H2 2021
- First Marketing Authorisation Application for a gene therapy treating a mitochondrial disease
- U.S. FDA submission on track for H2 2021

Paris, France, September 15, 2020, 7.30 am CEST – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders, today announced that it has submitted the Marketing Authorisation Application (MAA) for its lead product LUMEVOQ® to the European Medicines Agency (EMA), seeking approval for the treatment of patients with vision loss due to Leber Hereditary Optic Neuropathy (LHON) caused by mutation in the *ND4* mitochondrial gene.

"This first regulatory submission for GenSight is a major milestone in our progression from a pure research organization to one with commercial capabilities. It validates a technology platform that has the potential to address the high unmet medical needs of patients suffering from a range of rare diseases. I would like to thank all GenSight employees and partners whose motivation, focus and effort made this submission possible," said **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight Biologics.

LHON is a rare, mitochondrial genetic disease, mainly affecting young males. The *ND4* mutation results in the worst visual outcomes, with most patients becoming legally blind. There continues to be a high unmet medical need for the 800-1200 new LHON patients in Europe and the U.S. each year, particularly those who are struck blind in their prime working years.

Lenadogene nolparvovec (tradename: LUMEVOQ®) is a recombinant adeno-associated viral vector, serotype 2 (rAAV2/2), containing a cDNA encoding the human wild-type mitochondrial NADH dehydrogenase 4 protein (ND4), which has been specifically developed for the treatment of LHON associated with mutation in the *ND4* gene. It received orphan drug designation status for the treatment of LHON from the EMA in 2011 and from the U.S. Food and Drug Administration (FDA) in 2013.

GenSight submitted the MAA based on the benefit-risk balance established by results from a Phase-I/IIa study (CLIN-01), two pivotal Phase-III efficacy studies (CLIN-03A: RESCUE, and CLIN-03B: REVERSE) and the long-term follow up study of RESCUE and REVERSE (CLIN 06 - readout at Year 3 post injection). To demonstrate the efficacy of LUMEVOQ® in the context of a contralateral effect, the Company used a statistics-based indirect comparison methodology to assess the visual outcomes in LUMEVOQ®-treated patients (from LUMEVOQ® efficacy studies) against those in untreated patients from Natural History studies and GenSight's REALITY Natural History Registry.

GenSight expects to submit the Biologics License Application (BLA) for LUMEVOQ® to the FDA in H2 2021. First-in-human data from GenSight's second clinical stage program, GS030, are expected to be available in H2 2021.

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biopharma company focused on discovering and developing 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 in Phase III trials in Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible blindness in teens and young adults. Using its gene therapy-based approach, GenSight Biologics' product candidates are designed to be administered in a single treatment to the eye by intravitreal injection to offer patients a sustainable functional visual recovery.