



Press release

GenSight Biologics to Present GS010 at the 4th Annual Retinal Cell & Gene Therapy Innovation Summit

Paris, France, April 27, 2017, 7.30 CET – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company that discovers and develops innovative gene therapies for neurodegenerative retinal diseases and diseases of the central nervous system, today announced that an abstract was accepted for an oral presentation at the 4th Annual Retinal Cell & Gene Therapy Innovation Summit in Baltimore, MD, May 4-5, 2017.

“*Gene therapy for Leber hereditary optic neuropathy and neuroprotection of cone photoreceptors in rod-cone dystrophies*” will be presented by **Pr. José-Alain Sahel, MD**, Director of the *Institut de la Vision (Sorbonne-Universités/Inserm/CNRS)*, Paris, Chairman of the Department of Ophthalmology at *Centre Hospitalier National d’Ophtalmologie des XV-XX*, Paris, Professor and Chairman of the Department of Ophthalmology at *University of Pittsburgh School of Medicine and Medical Center*, and co-founder of GenSight Biologics.

- *Oral Presentation*
- *Session 2 – Clinical Gene Therapy*
- *Friday, May 5, 2017, 11:10-11:30 am (local time)*

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biotechnology company discovering and developing novel therapies for neurodegenerative retinal diseases and diseases of the central nervous system. GenSight Biologics’ pipeline leverages two core technology platforms, the Mitochondrial Targeting Sequence (MTS) and optogenetics for retinitis pigmentosa, to help preserve or restore vision in patients suffering from severe degenerative retinal diseases. GenSight Biologics’ lead product candidate, GS010, is in Phase III trials in Leber’s Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible low vision and legal 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 each eye by intravitreal injection to offer patients a sustainable functional visual recovery.

About GS010

GS010 targets Leber’s Hereditary Optic Neuropathy (LHON), 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. GS010 leverages a mitochondrial targeting sequence (MTS) proprietary technology platform, arising from research works 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.