Press release

GenSight Biologics to Present GS010 at the 2017 Annual Meeting of the American Association of Neurology

Paris, France, April 20, 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 2017 Annual Meeting of the American Association of Neurology (*AAN*) in Boston, MA, April 22-28, 2017.

"Intravitreal rAAV2/2-ND4 (GS010): A gene therapy for vision loss in Leber's Hereditary Optic Neuropathy (LHON) caused by the G11778A ND4 mitochondrial mutation" 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*
- *S26 – Neuro-ophthalmology / Neuro-otology*
- *Tuesday, April 25, 2017, 4:18-4:30 pm (local time)*

Contacts

GenSight Biologics
Thomas Gidoin
Chief Financial Officer
tgidoin@gensight-biologics.com
+33 (0)1 76 21 72 20

RooneyPartners
Media Relations
Marion Janic
mjanic@rooneyco.com
+1-212-223-4017

The Trout Group
Investor Relations
Chad Rubin
crubin@troutgroup.com
+1-646-378-2947

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.