



Press release

GenSight Biologics to Present Data on GS010 and GS030 at the Annual Meeting of ARVO

Paris, France, May 8, 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 five abstracts were accepted for one oral and four poster presentations at the 2017 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in Baltimore, MD, May 7-11, 2017.

GS010 - Phase I/II Clinical Trial Data after 78 Weeks of Follow-up

“Phase I/IIa Visual Acuity Outcomes 1.5-Years Post-Treatment with rAAV2/2-ND4, Investigational Gene Therapy for ND4 LHON” will be presented by Pr. Alain_José 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.

- *Poster Presentation*
- *Session: 452 Low Vision Populations, Services and Treatments*
- *Poster 4681 - B0588*
- *Wednesday, May 10, 2017, 11:00 am-12:45 pm*

GS010 - RESCUE and REVERSE Phase III Clinical Trials Baseline Characteristics

“Preliminary Baseline Characteristics of Patients with LHON Enrolled in RESCUE and REVERSE Gene Therapy Trials” will be presented by Pr. Alain_José 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.

- *Poster Presentation*
- *Session: 416 Optic Neuropathy*
- *Poster 3865 - A0036*
- *Wednesday, May 10, 2017, 8:30-10:15 am*

GS030 – Optogenetics

“Long term visual restoration using optogenetic engineering of retinal ganglion cells with AAV2.7m8 – ChrimsonR – tdTomato” will be presented by Grégory Gauvain, *Sorbonne Universités, UPMC Univ Paris 06, INSERM, CNRS, Institut de la Vision*, Paris, France.

- *Oral Presentation*
- *Session: 210 Diseases and Protection*
- *Presentation 1219*
- *Monday, May 8, 2017, 9:15-9:30 am*

“Visual acuity and optical flow in primate retinal ganglion cells treated with an optogenetic vision restoration strategy using ChrimsonR” will be presented by Himanshu Akolkar. *Sorbonne Universités, UPMC Univ Paris 06, INSERM, CNRS, Institut de la Vision*, Paris, France.

- *Poster Presentation*
- *Session: 545 Visual disease Models and Restoration*
- *Poster 5883 - B0475*
- *Thursday, May 11, 2017, 11:30 am-1:15 pm*

“*Modeling the dynamics of light-driven microbial opsin ChrimsonR*” will be presented by Quentin Sabatier, Sorbonne Universités, UPMC Univ Paris 06, INSERM, CNRS, Institut de la Vision, Paris, France.

- *Poster Presentation*
- *Session: 545 Visual disease Models and Restoration*
- *Poster 5873 - B0465*
- *Thursday, May 11, 2017, 11:30 am-1:15 pm*

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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.

About GS030

GS030 leverages GenSight's optogenetics technology platform, a novel approach to restore vision to patients by using gene therapy to introduce a gene encoding for light-sensitive protein into specific target cells in the retina by injection in order to make them responsive to light. An external wearable medical device to specifically stimulate the transduced cells is currently being developed to amplify the light signal and enable vision restoration. Patients will need to wear the external wearable device in order to enable restoration of visual function. Using this optogenetics technology platform, and with the support of the Vision Institute in Paris, GenSight is developing its second product candidate, GS030, to restore vision in patients suffering from Retinitis Pigmentosa, or RP. RP is an orphan disease caused by multiple mutations in several genes involved in the visual cycle. GenSight's optogenetics technology platform is independent of the specific genetic mutations that lead to the disease. On average, RP patients begin experiencing vision loss in their young adult years, eventually turning blind around the age of 40 to 45. There is currently no existing treatment for RP. RP has an estimated prevalence of 1.5 million people throughout the world. It is expected that GS030 would benefit patients in the early stages of RP.

About Optogenetics

Optogenetics is a biological technique which involves the transfer of a gene encoding for a light sensitive protein to cause neuronal cells to respond to light stimulation. As a result, it is a neuromodulation method that can be used to modify or control the activities of individual neurons in living tissue and even in-vivo, with a very high spatial and temporal resolution. Optogenetics combines the use of gene therapy methods to transfer the gene into target neurons and the use of optics and optronics to deliver the light to the transduced cells. Optogenetics is widely used by research labs throughout the world and hold clinical promise in the field of vision impairment or neurological disorders.

