Press release

GenSight Biologics to Present Data on GS010 and GS030 at the 2017 Congress of the European Society of Gene & Cell Therapy

Paris, France, October 16, 2017, 7.30am 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 multiple data presentations at the Congress of the European Society of Gene and Cell Therapy (ESGCT) in Berlin, Germany, October 17-20, 2017.

GS010 – Leber's Hereditary Optic Neuropathy (LHON)

"78-week follow-up study results after intravitreal rAAV2/2-ND4 (GS010) injection in patients with vision loss due to G11778A ND4 Leber Hereditary Optic Neuropathy"

- *Anne Galy, GS010 Project Director, GenSight Biologics*
- *Poster P215*
- *Wednesday, October 18, 2017, 6:30 pm-9:00 pm*

"Humoral and cellular immune responses to AAV2 and ocular inflammation in patients after intravitreal injection of rAAV2/2-ND4 (GS010), an investigational gene therapy for the treatment of ND4 LHON"

- *Céline Bouquet, Senior Preclinical Manager, GenSight Biologics*
- *Poster P210*
- *Thursday, October 19, 2017, 6:45 pm-8:15 pm*

GS030 – Optogenetics in Retinitis Pigmentosa (RP)

"Efficacy and Safety of ocular AAV mediated optogenetic therapy for retinitis pigmentosa in rd1 mice and non-human primates support the First-in-Human clinical trial of GS030"

- *Anne Douar, GS030 Project Director, GenSight Biologics*
- *Oral Presentation OR07 – Session S1b: Ocular and CNS Gene and Cell Therapy I*
- *Wednesday, October 18, 2017, 8:30 am-10:40 am*

"Ocular tolerability of AAV2.7m8-ChrimsonR-tdTomato (GS030-DP) gene therapy product on blind rd1 mice injected intravitreously and exposed to 595 nm LED light"

- *Céline Bouquet, Senior Preclinical Manager, GenSight Biologics*
- *Poster P231*
- *Wednesday, October 18, 2017, 6:30 pm-9:00 pm*

"Toxicity, immunogenicity and biodistribution of AAV2.7m8-ChrimsonR-tdTomato (GS030-DP) gene therapy product after bilateral intravitreal administration in non-human primates"

- *Céline Bouquet, Senior Preclinical Manager, GenSight Biologics*
- *Poster P214*
- *Thursday, October 19, 2017, 6:45 pm-8:15 pm*

Contacts

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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 Retinis 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.