Press Release

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

Paris, France, April 26, 2018, 7.30 am CET – 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 five abstracts were accepted at the 2018 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in Honolulu, Hawaii, April 29 – May 3, 2018. Also, Pr. José-Alain Sahel will present GS010 Phase I/II 1.5 years published results and REVERSE Phase III topline data at the 5th Annual Retinal Cell and Gene Therapy Innovation Summit taking place just prior to the ARVO.

GS010 - Leber Hereditary Optic Neuropathy (LHON)

Phase I/II Published Follow-up Data & REVERSE Phase III Topline Results

"Gene Therapy for Leber Hereditary Optic Neuropathy, 18-month Results from a Phase Ib-IIa Trial" will be presented by **Pr. José-Alain Sahel**, 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*
- *5th Annual Retinal Cell and Gene Therapy Innovation Summit*
- *Session 4: Gene Therapy II*
- *Friday, April 27, 2018, 4:40 - 4:55 pm*

Baseline Structure-Function relationship in RESCUE & REVERSE Phase III Clinical Trials

"Baseline Structural and Psychophysical Profiles of Subjects Enrolled in Phase 3 Trials with rAAV2/2-ND4, an Investigational Gene Therapy for ND4 LHON" will be presented by **Dr. Robert C. Sergott**, Director, Wills Eye Hospital, Neuro-Ophthalmology and Director, William H. Annesley, Jr, EyeBrain Center, Thomas Jefferson University, Philadelphia, PA, US.

- *Poster Presentation*
- *Poster Session: LV, VI - Profound Low Vision and Low-vision Clinical Trials*
- *Poster # 3901 – C0367*
- *Tuesday, May 1, 2018, 3:30 - 5:15 pm*

Phase I/II Clinical Trial Data Follow-up at 2.5 years

"Visual Acuity and Safety Outcomes 2.5 Years Post-Treatment with rAAV2/2-ND4, an Investigational Gene Therapy for ND4 LHON: Results of a Phase I/II Trial" will be presented by **Pr. José-Alain Sahel**, 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*

- *Poster Session: 442 - Ocular gene therapies and chemical therapeutics*
- *Poster # 4530 – A0045*
- *Wednesday, May 2, 2018, 11:15 am - 1:00 pm*

Bilateral intravitreal injection of GS010 in non-human primates

"Impact of sequential bilateral intravitreal injection of rAAV2/2-ND4 on ocular and systemic humoral immune status in non-human primates" will be presented by **Céline Bouquet**, Senior Preclinical Manager, GenSight Biologics, Paris, France.

- *Poster Presentation*
- *Poster Session: 442 - Ocular gene therapies and chemical therapeutics*
- *Poster # 4537 – A0052*
- *Wednesday, May 2, 2018, 11:15 am - 1:00 pm*

Phase I/II Clinical Trial immunology

"Intravitreal injection of rAAV2/2-ND4 in LHON: absence of correlation between ocular inflammation and humoral or cellular immune responses to AAV2" will be presented by **Céline Bouquet**, Senior Preclinical Manager, GenSight Biologics, Paris, France.

- *Poster Presentation*
- *Poster Session: 442 - Ocular gene therapies and chemical therapeutics*
- *Poster # 4531 – A0046*
- *Wednesday, May 2, 2018, 11:15 am - 1:00 pm*

GS030 – Optogenetics in Retinitis Pigmentosa (RP)

Safety of GS030 in blind rd1 mice

"Ocular Safety of AAV2.7m8-ChrimsonR-tdTomato (GS030-DP) following intravitreous injection and exposure to 595 nm LED light in blind rd1 mice" will be presented by **Brian J. Christian**, Covance Laboratories Inc., Madison, WI, US.

- *Poster Presentation*
- *Poster Session: 515 - Gene therapy, implants*
- *Poster # 5658 – A0377*
- *Thursday, May 3, 2018, 8:15 - 10:00 am*

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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, GS010, 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 each eye by intravitreal injection to offer patients a sustainable functional visual recovery.

About GS010

GS010 targets Leber Hereditary Optic Neuropathy (LHON) by leveraging 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 RESCUE and REVERSE

RESCUE and REVERSE are two separate randomized, double-masked, sham-controlled pivotal Phase III trials designed to evaluate the efficacy of a single intravitreal injection of GS010 (rAAV2/2-ND4) in subjects affected by LHON due to the G11778A mutation in the mitochondrial *ND4* gene.

The primary endpoint will measure the difference in efficacy of GS010 in treated eyes compared to sham-treated eyes based on Best Corrected Visual Acuity (BCVA), as measured with the ETDRS at 48 weeks post-injection. The patients' LogMAR (Logarithm of the Minimal Angle of Resolution) scores, which are derived from the number of letters patients read on the ETDRS chart, will be used for statistical purposes. Both trials have been adequately powered to evaluate a clinically relevant difference of at least 15 ETDRS letters between treated and untreated eyes adjusted to baseline.

The secondary endpoints will involve the application of the primary analysis to best seeing eyes that received GS010 compared to those receiving sham, and to worse seeing eyes that received GS010 compared to those that received sham. Additionally, a categorical evaluation with a responder analysis will be evaluated, including the proportion of patients who maintain vision (< ETDRS 15L loss), the proportion of patients who gain 15 ETDRS letters from baseline and the proportion of patients with Snellen acuity of >20/200. Complementary vision metrics will include automated visual fields, optical coherence tomography, and color and contrast sensitivity, in addition to quality of life scales, bio-dissemination and the time course of immune response.

The trials are conducted in parallel, in 37 subjects for REVERSE and 39 subjects for RESCUE, in 7 centers across the United States, the UK, France, Germany and Italy. Topline results of RESCUE at 48 weeks are expected in Q3 2018.

ClinicalTrials.gov Identifiers:
REVERSE: NCT02652780
RESCUE: NCT02652767

About GS030

GS030 leverages GenSight's optogenetics technology platform, a novel approach to restore vision in patients by using gene therapy to introduce a gene encoding for a light-sensitive protein into specifically targeted cells of the retina by a single injection in order to make them responsive to light. An external wearable medical device to specifically stimulate the transduced cells is developed to amplify the light signal and further enable vision. Patients will need to wear the external wearable device to enable optimal 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. GenSight's optogenetics technology platform is independent of the specific genetic mutations that lead to this family of disease. It is expected that GS030 would benefit patients from the early stages of RP. This technology offers the possibility of application to other diseases of the retina where photoreceptors have degenerated, and may be transferable to the dry form of Age Related Macular Degeneration (dry-AMD).