Press Release

GenSight Biologics to present REVERSE data with GS010 at the 2018 Annual Meeting of the American Academy of Ophthalmology

Paris, France, October 12, 2018, 7.30 am CEST – 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, announced today that the 48-week top-line results from its REVERSE Phase III clinical trial of GS010 were accepted for an oral presentation at the 2018 Annual Meeting of the American Academy of Ophthalmology in Chicago, IL, October 27-30, 2018. Members of the management team will also present at the Ophthalmology Innovation Summit (OIS) on October 25 prior to the conference.

Ophthalmology Innovation Summit (OIS) @AAO
October 25, 2018 – Chicago (IL, USA)
Barrett Katz, MD, Chief Medical Officer, will present on October 25, 2018 at the Marriott Marquis Hotel in Chicago, IL, USA.

American Academy of Ophthalmology (AAO)
October 27-30, 2018 – Chicago (IL, USA)
"Efficacy of rAAV2/2-ND4, an Investigational Gene Therapy for ND4 LHON: Top-line Results of Phase 3 Clinical Trial REVERSE" will be presented by **Mark L. Moster, MD**, Neuro-Ophthalmology, Wills Eye Hospital and Professor of Neurology and Ophthalmology at Thomas Jefferson University, Philadelphia, PA (Investigator in REVERSE and RESCUE trials).

- *Oral Presentation*
- *Session: Retina, Vitreous*
- *Abstract Number: PA066*
- *Monday, October 29, 2018, 2:12 - 2:19 pm CDT*

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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 early Q1 2019.

ClinicalTrials.gov Identifiers:
REVERSE: NCT02652780
RESCUE: NCT02652767