

GenSight Biologics to Host KOL Event on June 12, 2018

Paris, France, May 21, 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, today announced that it will host a KOL Event on June 12, 2018 in New York City from 8 a.m. – 10:30 a.m. ET. Management and Key Opinion Leaders in the gene therapy and ophthalmology space will present full results and findings from the Phase III REVERSE study of GS010 for the treatment of Leber Hereditary Optic Neuropathy (LHON). Discussion will also focus on the future clinical and regulatory strategy for GS010. Topline results from the REVERSE study were reported in April 2018.

Speakers will include:

- **Nancy J. Newman, MD**, Director, Neuro-Ophthalmology, and LeoDelle Jolley Professor of Ophthalmology, Emory University School of Medicine, Atlanta, GA (*Investigator in REVERSE and RESCUE trials*)
- **José-Alain Sahel, MD**, Director of the Institut de la Vision (Sorbonne-Université/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 UPMC (University of Pittsburgh Medical Center)
- **Robert C. Sergott, MD**, Director, Neuro-Ophthalmology, Wills Eye Hospital; Director, William H. Annesley, Jr, EyeBrain Center, and Professor of Neurology and Ophthalmology at Thomas Jefferson University, Philadelphia, PA
- **Mark 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*)

The presentation will be webcast live at <https://www.gensight-biologics.com/category/events-presentations/>. For those not available to attend or listen to the live broadcast, a replay will be archived for 3 months and available at <https://www.gensight-biologics.com/category/events-presentations/>. For more information or to RSVP please contact Chad Rubin at crubin@troutgroup.com or Rebecca John at rjohn@troutgroup.com.

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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