Press release

GenSight Biologics Completes Enrollment of RESCUE Phase III Study of GS010 in the treatment of Leber's Hereditary Optic Neuropathy

Paris, France, August 1, 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 enrollment in RESCUE, a Phase III clinical trial of GS010 for the treatment of Leber's Hereditary Optic Neuropathy (LHON), has been successfully completed.

RESCUE is one of two parallel randomized, double-masked, sham-controlled, multi-center 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. RESCUE enrolled 37 patients with an onset of vision loss of less than 6 months. REVERSE, the second trial, completed enrollment in February 2017 of 36 patients with an onset of vision of 7-12 months. Both studies are being conducted in 7 centers in Europe and in the United States.

Bernard Gilly, CEO and co-founder of GenSight Biologics, commented, *" The completion of enrollment in our RESCUE Phase III Study of GS010 is a significant accomplishment for GenSight and for the LHON Community. We have now completed enrollment in both of our ongoing Phase III studies and look forward to reporting data in the first half of 2018. "*

"We are very excited to be part of the RESCUE and REVERSE trials with GS010. Safety and pharmacodynamics results seen in the Phase I/II study are particularly encouraging, and if confirmed in these Phase III trials, GS010 could be a potentially transformative treatment for LHON, and a fantastic hope for patients and their families," commented **Dr. Mark Moster, MD**, investigator in the study and neuro-ophthalmologist, Wills Eye Hospital, Philadelphia, Pennsylvania (USA).

Topline results of REVERSE at 48 weeks of follow-up are expected in Q2 2018, while RESCUE is expected to read out in Q3 2018.

GS010 has been granted Orphan Drug Designation both in the United States and in Europe.

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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 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' Log of the Minimal Angle of Resolution, or LogMAR, scores, which are derived from the number of letters they 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 36 patients each, in 7 centers across the United States, the UK, France, Germany and Italy. Topline results of REVERSE at 48 weeks are expected in Q2 2018, while RESCUE is expected to read out in Q3 2018.

ClinicalTrials.gov Identifiers:
REVERSE: NCT02652780
RESCUE: NCT02652767