Press release

GenSight Biologics receives approval to include teenage patients in RESCUE and REVERSE Phase III trials with GS010 in Leber's Hereditary Optic Neuropathy

Paris, France, September 8, 2016 – GenSight Biologics (Euronext: SIGHT, 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 regulatory agencies and ethics committees in the United States, France and the United Kingdom, have authorized a protocol amendment to include teenage patients (15-18 years) in RESCUE and REVERSE, two ongoing Phase III clinical trials of GS010 in the treatment of Leber's Hereditary Optic Neuropathy (LHON).

The Investigational New Drug (IND) Application had been cleared by the U.S. Food and Drug Administration (FDA) in August 2015, and Clinical Trial Applications (CTAs) had been accepted in France, Germany, Italy and the United Kingdom by national agencies in the first half of 2016, allowing GenSight Biologics to initiate the two Phase III clinical trials in the US and in Europe. The initial protocols were designed to enroll adult patients, whereas this amendment now allows inclusion of patients from the age of 15.

Bernard Gilly, Chief Executive Officer of GenSight Biologics, commented *"Given the brutality of the onset of vision loss and the neurodegenerative nature of the disease, we are thrilled to be able to consider all patients irrespective of their age."*

"Considering that affected adolescents with LHON present in a similar fashion to adults with the disease, and the current good safety profile and potential prospect for benefit, it is extremely important to be able to include these teenage patients with LHON," also commented **Pr. Nancy J. Newman, MD**, Director of the Section of Neuro-Ophthalmology, Emory University School of Medicine, Atlanta, Georgia (USA).

Available epidemiologic data suggest that teenage patients (15-18 years) may represent between 14 to 22% of all LHON patients (all mutations included).

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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, Mitochondrial Targeting Sequence (MTS) and optogenetics, 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 vision loss in teens and young adults. Using its gene therapy-based approach, GenSight Biologics' product candidates are designed to offer patients a sustainable functional visual recovery with a single treatment to each eye through an intravitreal injection.

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 7 centers across the United States, the UK, France, Germany and Italy. Topline results at 48 weeks are expected early 2018.

ClinicalTrials.gov Identifiers:
REVERSE: NCT02652780
RESCUE: NCT02652767