Press release

GenSight Biologics to Present Data on GS010 at the 43rd Annual Meeting of NANOS

Paris, France, March 28, 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 three abstracts were accepted for one oral and two poster presentations at the 2017 North American Neuro-Ophthalmology Society (*NANOS*) 43rd Annual Meeting in Washington, D.C., April 1-6, 2017.

Phase I/II Clinical Trial Data after 78 Weeks of Follow-up

"Phase I/IIa Visual Acuity Outcomes 1.5-Years Post-Treatment with rAAV2/2-ND4, Investigational Gene Therapy for ND4 LHON" will be presented by Dr. Catherine Vignal, principal investigator of the study and Chief of the Department of Ophthalmology at the Rothschild Foundation Hospital in Paris, France.

- *Oral Presentation*
- *Platform Session II*
- *Tuesday, April 4, 2017, 10:00-10:15 am*

RESCUE and REVERSE Phase III Clinical Trials Baseline Characteristics

"Preliminary Baseline Characteristics of Patients with LHON Enrolled in RESCUE and REVERSE Gene Therapy Trials" will be presented by Dr. Patrick Yu-Wai-Man, international coordinating investigator of the REVERSE study and Honorary Consultant in Ophthalmology at the Moorfields Eye Hospital in London, and the Newcastle Hospital, United Kingdom.

- *Poster Presentation*
- *Poster Session II: Scientific Advancements in Neuro-Ophthalmology*
- *Category: Disorders of the Anterior Visual Pathway (Retina, Optic Nerve, and Chiasm)*
- *Poster #189*
- *Tuesday, April 4, 2017, 6:45-7:30 pm*

LHON Pediatric Literature Review

"Pediatric Leber Hereditary Optic Neuropathy (LHON): A Literature Review" will be presented by Dr. Catherine Vignal, Chief of the Department of Ophthalmology at the Rothschild Foundation Hospital in Paris, France.

- *Poster Presentation*
- *Poster Session II: Scientific Advancements in Neuro-Ophthalmology*
- *Category: Disorders of the Anterior Visual Pathway (Retina, Optic Nerve, and Chiasm)*
- *Poster #186*
- *Tuesday, April 4, 2017, 7:30-8:15 pm*

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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 at 48 weeks of both studies are expected in the first half of 2018.

ClinicalTrials.gov Identifiers:
REVERSE: NCT02652780
RESCUE: NCT02652767