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GenSight Biologics Receives IND Acceptance from FDA to Enter Phase III with GS010

GenSight Biologics to initiate two Phase III Clinical Trials with GS010 in Leber's Hereditary Optic Neuropathy (LHON)

Paris, France, September 10, 2015 – GenSight Biologics S.A. (**GenSight**), a clinical-stage biotechnology company discovering and developing novel gene therapies for mitochondrial and neurodegenerative diseases of the eye, and in the future, of the central nervous system, today announced that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) Application for two Phase III clinical trials of GS010 in the treatment of Leber's Hereditary Optic Neuropathy (LHON).

GenSight has also submitted a Clinical Trial Application (CTA) in certain individual European Union countries.

Bernard Gilly, PhD, Chairman & Chief Executive Officer of GenSight, commented: *"I am thrilled that GenSight has reached this important regulatory milestone for GS010, following the successful completion of the Phase I/II in France, and we look forward to rolling out these trials both in the US and in Europe."*

Pr. Nancy J. Newman, MD, Director of the Section of Neuro-Ophthalmology, Emory University School of Medicine, Atlanta, USA, also commented: "*Patients are desperately waiting for a safe treatment for LHON. GS010 carries promise and could pave the way towards a novel therapy. We are eager to start enrolling patients in the US."*

GS010 is a gene therapy-based single intravitreal injection in the eye targeting LHON due to the ND4 gene mutation, a rare mitochondrial genetic disease. GS010 uses GenSight's Mitochondrial Targeting Sequence (MTS) proprietary technology platform, which permits missing mitochondrial proteins to be shuttled into the mitochondrion, enabling the restoration of mitochondrial function.

The clinical trials are designed to demonstrate efficacy in subjects with LHON due to the G11778A *ND4* mutation with up to one year onset of vision loss. The two trials will be conducted in parallel and will include patients with an onset of vision loss up to 6 months, and between 6 and 12 months respectively. The two trials are described as follows:

- **RESCUE** Study: *"A Randomized, DoublE-Masked, Sham-Controlled, Pivotal Clinical Trial to Evaluate the Efficacy of a Single Intravitreal Injection of GS010 (rAAV2/2-ND4) in Subjects Affected for 6 Months or Less by Leber's Hereditary Optic NeUropathy Due to the G11778A Mutation in the Mitochondrial NADH Dehydrogenase 4 GEne"*

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- **REVERSE** Study: *"A Randomized, DoublE-Masked, Sham-Controlled, PiVotal Clinical Trial to Evaluate the Efficacy of a Single Intravitreal Injection of GS010 (rAAV2/2-ND4) in Subjects Affected for More Than 6 Months To 12 Months by Leber's Hereditary Optic NeuRopathy Due to the G11778A Mutation in the Mitochondrial NADH DehydrogenaSe 4 GEne"*

Each of the trials is expected to randomize 36 subjects, and will be conducted at 7 sites (1 each in France, Germany, Italy, and the United Kingdom and 3 in the United States) upon full regulatory and ethics committee approvals. The first patient, First Patient First Visit (FPFV), to a trial site is expected by the end of 2015.

The primary objective of the RESCUE and REVERSE trials is to evaluate the efficacy of GS010 versus sham injection, in patients with up to one year onset of vision loss, measuring visual acuity change from baseline to week 48.

More information

About Leber's Hereditary Optic Neuropathy (LHON)

LHON is a maternally-inherited genetic disease that causes the onset of irreversible and severe loss of sight leading to blindness and disability in teens and young adults. LHON greatly alters the patient's ability to perform daily life activities, reduces their autonomy and, in particular, affects their ability to read, drive and recognize facial features and expressions. The quality of life of patients with LHON is generally poor. LHON is estimated to affect approximately 1,400 to 1,500 new patients who lose their sight every year in the United States and Europe.

LHON is caused by defects in mitochondrial genes encoding for proteins called NADH dehydrogenase. These proteins are part of a large enzyme complex responsible for driving the production of adenosine triphosphate (ATP), which is the main source of energy within the cell. Three different genes encoding for four NADH dehydrogenases have been linked to LHON and are considered to be the primary mutations for the disease to manifest. The ND4 mutation accounts for over 75% of them.

About GenSight Biologics

GenSight Biologics S.A. (**GenSight**) is a clinical-stage biotechnology company discovering and developing novel therapies for mitochondrial and neurodegenerative diseases of the eye, and in the future, of the central nervous system. GenSight's pipeline leverages two core technology platforms, Mitochondrial Targeting Sequence (MTS) and optogenetics, to help preserve or restore vision in patients suffering from severe retinal diseases. GenSight's lead product, GS010, is entering a pivotal Phase III trial for vision loss in Leber's Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible sight loss in teens and young adults. Using its gene therapy-based approach, GenSight's product candidates are designed to be administered in a single treatment to each eye by intravitreal or subretinal injection in order to provide patients with a long-lasting functional cure, potentially for the rest of their lives.

For more information: www.gensight-biologics.com

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