Press Release

GenSight Biologics reports sustained visual acuity gain at 78 weeks in its Phase I/II Study with GS010 for the treatment of Leber's Hereditary Optic Neuropathy (LHON)

- **Confirmation of the good safety and tolerability profile of GS010**
- **Sustained improvement of visual acuity in patients with vision loss for less than 2 years**

Paris, France, 20 December 2016 – 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 reported additional promising results after 78 weeks of follow-up in its Phase I/II clinical trial. These results confirm the favorable safety and tolerability profile of GS010, while demonstrating sustainable visual acuity improvement in patients with Leber's Hereditary Optic Neuropathy (LHON).

Each cohort of three patients was administered an increasing dose of GS010 through a single intravitreal injection in the eye most severely affected by the disease. Recruitment was completed in April 2015 and long-term follow-up is ongoing. These patients had an average onset of disease of 6 years at the time of treatment. At baseline, both treated and untreated eyes had an off-chart median visual acuity[1].

At 78 weeks post-injection, the mean change of visual acuity from baseline in the treated eyes of all patients* was -0.61 LogMAR ($p<0.001$), equivalent to a mean improvement of +30 ETDRS letters. For all untreated eyes at week 78, the mean change from baseline was -0.31 LogMAR ($p=0.0866$), equivalent to a mean improvement of +15 ETDRS letters. This provides a treatment effect (mean difference between treated worse-seeing and untreated best-seeing eyes) of +15 letters ($p=0.11$) in favor of treated worse-seeing eyes.

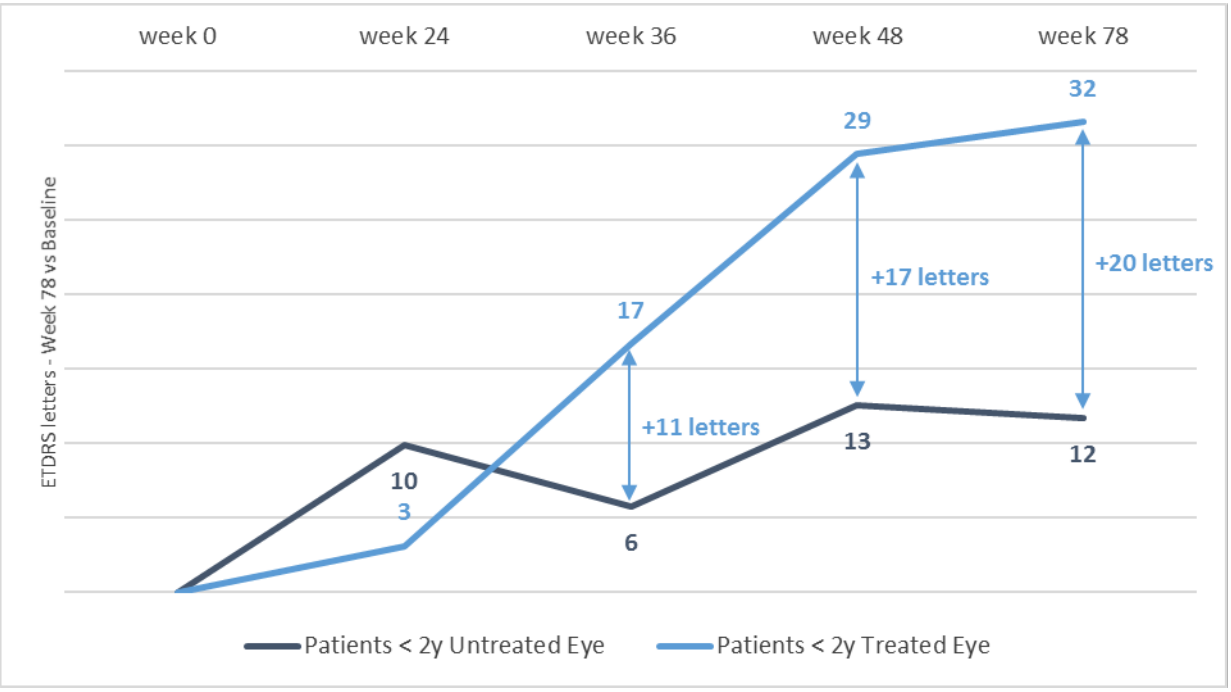
ETDRS letters (LogMAR) Visual Acuity change from baseline to week 78	Treated Eye (TE) mean change (LogMAR)	Untreated Eye (UTE) mean change (LogMAR)	Outcomes in Visual Acuity **TE vs UTE**
All patients (n=14)	+30 letters (-0.61)	+15 letters (-0.31)	+15 letters (-0.30 LogMAR)
Patients with ≤ 2y disease duration (n=5)*	+32 letters (-0.63)	+12 letters (-0.23)	**+20 letters (-0.40 LogMAR)**

Note (*) Excludes "hand motion" patients, in accordance with the Phase III protocol.
Source: Company

[1] At baseline, treated worse-seeing eyes had a median visual acuity of 2.79 LogMAR (approximately equivalent to hand motion at 1m) and untreated better-seeing eyes had a median acuity of 2.01 LogMAR (approximately equivalent to counting fingers at 50cm).

More interestingly, in patients with an onset of vision loss of less than 2 years at the time of treatment, a mean gain of +32 ETDRS letters (-0.63 LogMAR) was observed in treated eyes, while a mean gain of +12 ETDRS letters (-0.23 LogMAR) was observed in untreated eyes, resulting in a difference of 20 ETDRS letters in favor of treated eyes.

The patient group with vision loss for 2 years or less at the time of injection demonstrated a treatment effect in favor of the treated eye of increasing magnitude from week 36 onwards.



Note (*) Excludes "hand motion" patients, in accordance with the Phase III protocol.
Source: Company

Evolution of visual acuity in the treated eye vs. untreated eye at week 78 of follow-up in patients with less than 2 years of vision loss symptoms
(in equivalent number of letters read; n=5)*

Bernard Gilly, CEO and co-founder of GenSight, commented, "We are very encouraged by the latest results indicating a sustainable clinical benefit for patients in this long-term follow up. This data continues to support the design of our two Phase III studies with GS010 for the treatment of Leber's Hereditary Optic Neuropathy, which are currently ongoing in the U.S. and Europe, addressing patients with an early onset of vision loss. We continue to be thankful for the participation of our clinical trials' patients and their families, which ultimately will help us find a cure for this devastating condition."

Dr. Catherine Vignal, investigator of the study and Chief of the Department of Ophthalmology at the Rothschild Foundation Hospital in Paris, added, "The confirmatory trends of week 48 data after 1.5 years of follow-up confirm significant hope for patients suffering from LHON. The insights gained from this and forthcoming data will be tremendously helpful as GenSight works to develop a therapy for this very severe disease with no existing curative treatment."

GenSight Biologics is currently conducting two Phase III clinical studies (RESCUE and REVERSE) in Europe and the United States to assess the efficacy of GS010 in patients affected with LHON due to the *ND4* mutation, with vision loss up to one year at the time of treatment. Recruitment is expected to be completed by Q1 2017 for both studies.

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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.