Press Release

GenSight Biologics reports long-term positive safety and visual acuity results at Week 96 in Phase I/II Study of GS010 for the treatment of Leber's Hereditary Optic Neuropathy (LHON)

Sustained clinically significant improvement of visual acuity in LHON patients with less than 2 years of visual loss

Confirmation of the long-term safety profile of GS010

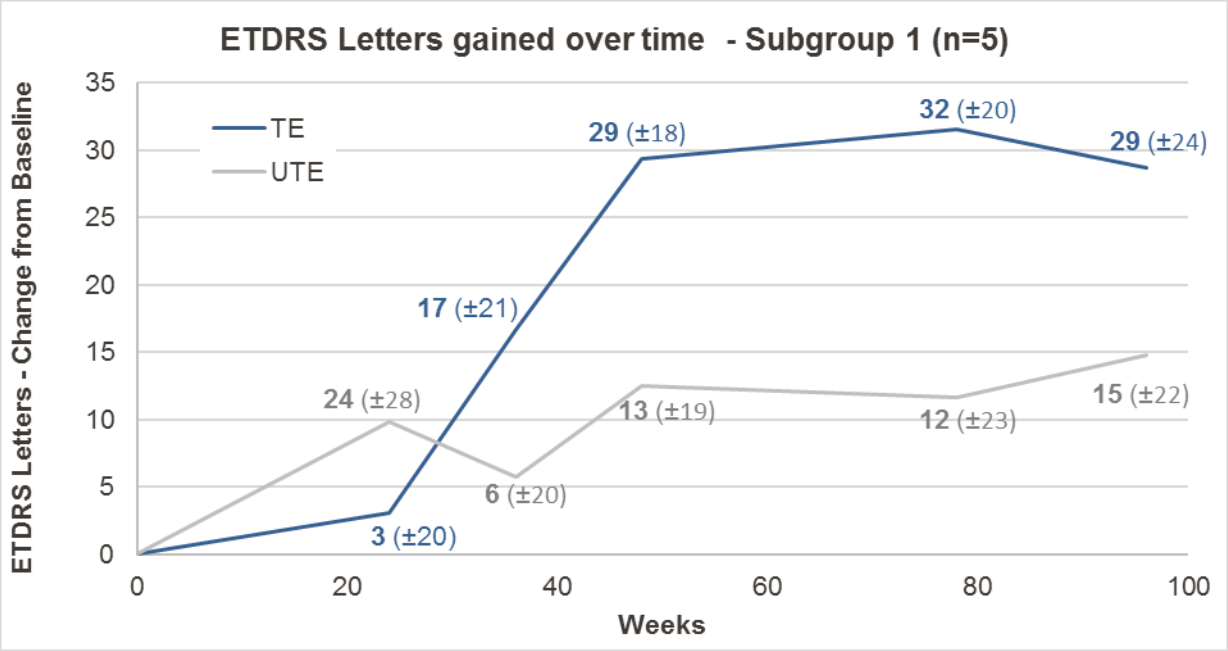
Paris, France, June 14, 2017 – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company that discovers and develops innovative gene therapies for neurodegenerative retinal diseases and diseases of the central nervous system, today reported additional promising clinical trial results with GS010 after 96 weeks of follow-up in its Phase I/II study. These results confirm the long-term positive sustained visual acuity gain after 2 years with a single intravitreal injection of GS010 in patients with Leber's Hereditary Optic Neuropathy (LHON), especially in those with less than 2 years of disease onset**.**

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 (TE) and untreated (UTE) eyes had an off-chart median visual acuity[1].

At week 96 post-injection, in patients with an onset of vision loss of less than 2 years and relatively better vision (<2.79 LogMAR) at the time of treatment, a mean gain of **+29 ETDRS letters (-0.57 LogMAR)** was observed in TE compared to baseline, with a mean gain of **+15 ETDRS letters (-0.30 LogMAR)** in UTE, resulting in a difference of +14 ETDRS letters in favor of TE. This improvement is clinically significant, and the magnitude of improvement similar to the observed trend at week 48 and week 78.

[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).

Evolution of visual acuity in the treated eye vs. untreated eye at Week 96 of follow-up in patients with less than 2 years of vision loss and baseline < 2.79 LogMAR*



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

The patient group with an onset of vision loss of 2 years or less and relatively better vision (<2.79 LogMAR) at the time of injection (n=5) demonstrated a treatment effect in favor of the treated eye of increasing magnitude from week 36 onwards with **60.0% of patients showing clinically significant ≥ 15 letters gain** on EDTRS visual charts[2]. The characteristics of this patient group are similar to the baseline characteristics of LHON patients enrolled in ongoing Phase III REVERSE and RESCUE clinical trials[3].

The mean change of visual acuity from baseline in TE of all patients (n=14) showed an improvement of +21 ETDRS letters (-0.41 LogMAR) and was statistically significant (p = 0.0159). There was no observed difference with the mean change from baseline in the UTE. There was a linear correlation between the TE and UTE in the change from baseline to week 96 that reached 0.67 and was highly significant (p = 0.0067). Based on all subjects except one, the improvement in UTE was only observed when there was an improvement in TE, which was significantly larger than the natural visual recovery[4].

Lastly, the results confirmed the long-term good safety and tolerability of GS010 with no reported vision worsening or ocular sequelae, no serious treatment-emergent adverse events (TEAEs) nor systemic adverse events (AEs) related to study drug or procedure. As expected, mostly mild, well-tolerated, and completely reversible ocular AEs that were responsive to standard therapy occurred.

Bernard Gilly, CEO and co-founder of GenSight, commented, "We continue to see a sustained clinical benefit for patients in this long-term follow up after 2 years with a single injection, and this is very encouraging. Furthermore, 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 are now less than a year away

[2] Clinical significance being defined as a change from baseline of at least 0.3 LogMAR decrease (≥15 ETDRS letters gain).

[3] One patient had an onset of vision loss of 9 months (eligible to REVERSE, 6 to 12 months), and 4 patients had an onset of vision loss > 1 year and < 2 years. None would have been eligible to RESCUE (<6 months).

[4] Lam BL, Feuer WJ, Schiffman JC, et al. JAMA Ophthalmol. 2014 Apr 1;132(4):428-36.

from Phase III efficacy data, and more than ever committed to find a cure for patients and their families affected by 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 safety and continued positive trends after 2 years of follow-up confirm significant hope for patients suffering from LHON. It is worth noting that the observed improvement in some of the untreated eyes was consistent with several prior studies in neuroretinal degenerative diseases. 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."

The full data from the Phase I/II up to week 96 analyses is pending submission as a peer-reviewed manuscript.

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 of REVERSE was completed in February 2017, while RESCUE is expected to be completed by the end of July 2017. Topline results at 48 weeks of both studies are expected in the second and third quarters of 2018, respectively.

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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) by leveraging 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 Leber's Hereditary Optic Neuropathy (LHON)

Leber's Hereditary Optic Neuropathy (LHON) is 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. LHON is associated with painless, sudden loss of central vision in the 1st eye, with the 2nd eye sequentially impaired. It is a symmetric disease with poor functional visual recovery. 97% of patients have bilateral involvement at less than one year of onset of vision loss, and in 25% of cases, vision loss occurs in both eyes simultaneously. The estimated incidence of LHON is approximately 1,400 to 1,500 new patients who lose their sight every year in the United States and Europe.