Press Release

GenSight Biologics on Track to Submit LUMEVOQ® for European Approval in September 2020 Following Pre-Submission Meeting with EMA

Paris, France, April 15, 2020, 7.30 am CEST – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders, today announces the completion of the pre-submission meeting with the European Medicines Agency (EMA) for the Company's lead product LUMEVOQ® (GS010; lenadogene nolparvovec).

The EMA pre-submission meeting is a procedural milestone in the preparation of a marketing authorization application (MAA). Based on its successful conclusion, GenSight Biologics confirms its intention to submit the MAA for LUMEVOQ® in September 2020.

LUMEVOQ® is the gene therapy developed by the Company for the treatment of Leber Hereditary Optic Neuropathy (LHON), a rare blinding disease marked by sudden, irreversible vision loss. The pivotal trials for LUMEVOQ® in Europe, RESCUE and REVERSE, were completed in 2019; patients from those trials have been invited to participate in a long-term follow-up study. In addition, GenSight Biologics has been conducting a natural history study (REALITY) and mechanistic studies in animals to supplement the data from RESCUE and REVERSE.

In its meeting with GenSight Biologics, the EMA confirmed the information needed for the Company's September submission. The agency also provided updated guidance on various administrative topics, as well as advice on topics to be discussed in meetings between the Company and the Rapporteurs and Co-rapporteurs planned for June 2020.

"We are excited about our progress towards our regulatory filing of LUMEVOQ® *in Europe, as this brings us closer to making a novel, effective and safe treatment available to a large proportion of LHON patients,"* said **Bernard Gilly**, Co-founder and Chief Executive Officer of GenSight Biologics. "*We have been and continue to be extremely grateful for the support and guidance we have received from the European authorities."*

As a final step before actual submission, and as part of EMA's procedures, GenSight Biologics expects to re-confirm by July its intent to submit in September.

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders. GenSight Biologics' pipeline leverages two core technology platforms, the Mitochondrial Targeting Sequence (MTS) and optogenetics to

help preserve or restore vision in patients suffering from blinding retinal diseases. GenSight Biologics' lead product candidate, LUMEVOQ® (GS010; lenadogene nolparvovec), is in Phase III trials in Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible 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 the eye by intravitreal injection to offer patients a sustainable functional visual recovery.

About LUMEVOQ® (GS010; lenadogene nolparvovec)

LUMEVOQ® (GS010) targets Leber Hereditary Optic Neuropathy (LHON) by leveraging a mitochondrial targeting sequence (MTS) proprietary technology platform, arising from research 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. "LUMEVOQ" was accepted as the invented name for GS010 (lenadogene nolparvovec) by the European Medicines Agency (EMA) in October 2018.

About Leber Hereditary Optic Neuropathy (LHON)

Leber 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.

About RESCUE and REVERSE

RESCUE and REVERSE are two separate randomized, double-masked, sham-controlled 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' LogMAR (Logarithm of the Minimal Angle of Resolution) scores, which are derived from the number of letters patients 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 gain 15 ETDRS letters from baseline and nadir 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. Readouts for these endpoints are at 48, 72 and 96 weeks after injection.

The trials are conducted in parallel, in 37 subjects for REVERSE and 39 subjects for RESCUE, in 7 centers across the United States, the UK, France, Germany and Italy. Week 96 results were reported in 2019 for both trials, after which patients were transferred to a long-term follow-up study that will last for three years.

ClinicalTrials.gov Identifiers:
REVERSE: NCT02652780
RESCUE: NCT02652767

About REALITY

REALITY is a multi-country retrospective and cross-sectional observational study of affected LHON subjects, based on subjects' medical charts and the administration of surveys on Health-Related Quality of Life (HRQoL) and direct and indirect costs associated with the disease.

The study will recruit at least 50 subjects (both adult and pediatric) chiefly in the following countries: Spain, Italy, France, United Kingdom, and the United States.

The primary objectives for the REALITY study are: to describe the evolution of visual functional and structural changes and other associated symptoms in patients with LHON; understand the impact of LHON-related vision loss on the HRQoL; and understand the economic burden for patients and their families arising from direct and indirect costs associated with the disease. The secondary objective is to describe the relationship between genetic, lifestyle and/or environmental factors and the expression of the LHON phenotype.

The first subject was enrolled on 3 January 2018, and enrollment is targeted to be completed in early Q2 2020.

ClinicalTrials.gov Identifiers:
REALITY LHON Registry: NCT03295071