Press Release

GenSight Biologics to Host a Key Opinion Leader Webinar on the *Nature Medicine* Case Report: Visual Recovery after GS030 Optogenetic Treatment

Friday June 4th, 2021 – 8:00 am to 9:00 am EDT

Paris, France, May 31, 2021, 7.30 am CEST – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company focused on developing and commercializing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders, today announced that it will host a Key Opinion Leader (KOL) call on **June 4, 2021 from 8:00 am to 9:00 am EDT**. The webinar will feature presentations by KOLs **José-Alain Sahel, MD** (University of Pittsburgh School of Medicine) and **Botond Roska, MD, PhD** (Institute of Molecular and Clinical Ophthalmology Basel), who will discuss the *Nature Medicine* Case Report of partial recovery of visual function in a blind patient with late-stage retinitis pigmentosa (RP)., The subject is a participant in the ongoing PIONEER Phase I/II clinical trial of GenSight Biologics' GS030 optogenetic therapy. Following the formal presentations, Drs. Sahel and Roska will be available to answer questions.

In addition, GenSight's management team will discuss highlights from the *Nature Medicine* Case Report and provide an update on their pipeline candidate, GS030. Administered via intravitreal injection, GS030 uses an optimized viral vector (GS030-DP) to express the light-sensitive opsin ChrimsonR in retinal ganglion cells and proprietary light-stimulating goggles (GS030-MD) to project the right wavelength and intensity of light onto the treated retina.

The webinar will be webcast live at https://bit.ly/3uzvG1j. You will need to register in advance to get access to the webinar. For those unable to attend the live broadcast, a recording will be accessible using the same link.

The *Nature Medicine* Case Report can be found at https://www.nature.com/articles/s41591-021-01351-4. A video of the patient performing the tests, which was submitted as supplementary material to the publication, can be viewed at www.gensight-biologics.com.

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biopharma company focused on developing and commercializing 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. 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. Developed as a treatment for Leber Hereditary Optic Neuropathy (LHON), GenSight Biologics' lead product candidate, LUMEVOQ® (GS010; lenadogene nolparvovec), is currently in the review phase of its registration process in Europe, and in Phase III to move forward to a BLA filing in the U.S.

About GS030

GS030 leverages GenSight Biololgics' optogenetics technology platform, a novel approach to restore vision in blind patients using a combination of ocular gene therapy and tailored light-activation of treated retinal cells. The gene therapy, which is delivered via a single intravitreal injection, introduces a gene encoding for a light-sensitive protein (ChrimsonR-tdT) into retinal ganglion cells, making them responsive to light and bypassing photoreceptors killed off by diseases such as retinitis pigmentosa (RP). Because ChrimsonR-tdT is activated by high intensities of amber light, a wearable medical device is needed to stimulate the treated retina. The optronic light-stimulating goggles (GS030-MD) encode the visual scene in real-time and project a light beam with a specific wavelength and intensity onto the treated retina. Treatment with GS030 requires patients to wear the external wearable device in order to enable restoration of their visual function. With the support of the *Institut de la Vision* in Paris and the team of Dr. Botond Roska at the Friedrich Miescher Institute in Basel, GenSight is investigating GS030 as therapy to restore vision in patients suffering from late-stage RP. GenSight's optogenetics approach is independent of the specific genetic mutations causing blindness and has potential applications in other diseases of the retina in which photoreceptors degenerate, like dry age-related macular degeneration (dry-AMD). GS030 has been granted Orphan Drug Designation in the United States and Europe.

About Optogenetics

Optogenetics is a biological technique that involves the transfer of a gene encoding for a light sensitive protein to cause neuronal cells to respond to light stimulation. As a neuromodulation method, it can be used to modify or control the activities of individual neurons in living tissue and even in-vivo, with a very high spatial and temporal resolution. Optogenetics combines (1) the use of gene therapy methods to transfer a gene into target neurons with (2) the use of optics and electronics (optronics) to deliver the light to the transduced cells. Optogenetics holds clinical promise in the field of vision impairment or degenerative neurological disorders.

About Retinitis Pigmentosa

Retinitis pigmentosa (RP) is a family of orphan genetic diseases caused by multiple mutations in numerous genes involved in the visual cycle. Over 100 genetic defects have been implicated. RP patients generally begin experiencing vision loss in their young adult years, with progression to blindness by age 40. RP is the most widespread hereditary cause of blindness in developed nations, with a prevalence of about 1.5 million people throughout the world. In Europe and the United States, about 350,000 to 400,000 patients suffer from RP, and every year between 15,000 and 20,000 new patients with RP lose sight. There is currently no curative treatment for RP.

About the PIONEER Phase I/II trial

PIONEER is a first-in-man, multi-center, open label dose-escalation study to evaluate the safety and tolerability of GS030 in 12-18 subjects with late-stage retinitis pigmentosa. GS030 combines a gene therapy (GS030-DP) administered via a single intravitreal injection with a wearable optronic visual stimulation device (GS030-MD). Eligible patients in the first three cohorts are those affected by end-stage non-syndromic RP with no light perception (NLP) or light perception (LP) levels of visual acuity. The extension cohort will include patients with hand motion (HM) and counting fingers (CF) levels of visual acuity.

As per protocol, three cohorts with three subjects each will be administered an increasing dose of GS030-DP via a single intravitreal injection in their worse-seeing eye. An extension cohort will receive the highest tolerated dose. The DSMB will review the safety data of all treated subjects in each cohort and will make recommendations before a new cohort receives the next dose. The primary outcome analyses will be on the safety and tolerability at one year post-injection. PIONEER is being conducted in three centers in the United Kingdom, France and the United States.