

GenSight Biologics and the 15-20 National Hospital Announce *NEJM* Publication on GS030 Phase 1/2 Trial Results in Late-Stage Retinitis Pigmentosa

Paris, France, Thursday, October 8, 2026, 8:30 am CET – 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, and the 15-20 National Hospital (l'Hôpital national des 15-20) in Paris today announced the publication of the results of the GS030 PIONEER trial in the prestigious medical journal *The New England Journal of Medicine* (*NEJM*).

Available in the October 8, 2026 issue of the *NEJM*, the article reports the trial's primary safety findings and exploratory visual function assessments evaluating GS030, GenSight Biologics' candidate optogenetics treatment for late-stage retinitis pigmentosa (RP). GS030 uses an intravitreal injection of an AAV-based gene therapy that encodes light-sensitive proteins in the intact retinal ganglion cells of one eye, coupled with light-stimulating goggles to activate the light-sensitive proteins.¹

Published a few days after the awarding of the 2026 Nobel Prize for Physiology or Medicine to Karl Deisseroth, Peter Hegemann and Georg Nagel for their foundational discoveries concerning light-gated ion channels and optogenetics, the *NEJM* article illustrates the therapeutic potential of the scientific approach recognized by the prize.

In the field of vision, the publication builds on more than two decades of research into vision restoration through optogenetics, to which Professors José-Alain Sahel and Botond Roska, co-founders of GenSight Biologics, have made major contributions, ranging from fundamental scientific breakthroughs to clinical applications. In recent years, their work in vision and neuroscience has been recognized with several of the world's most prestigious awards. Teams from the Institut de la Vision and GenSight Biologics have played a key role in advancing the program from the laboratory to the clinic.

Teams from the 15-20 National Hospital, notably within the REFERET rare disease reference center coordinated by Prof. Isabelle Audo, took part in the clinical evaluation of the optogenetic approach alongside leading international centers, including the UPMC Vision Institute in Pittsburgh, Moorfields Eye Hospital in London and Streetlab (www.streetlab-vision.com). This involvement underscores the hospital's ability to contribute to world firsts in innovative treatments for rare retinal diseases.

"The results mark an important step in the development of optogenetics as an approach to restore vision," said **Prof. José-Alain Sahel**, founder and former director of the Institut de la Vision and the FOReSIGHT University Hospital Institute, Exceptional Class Professor Emeritus of Ophthalmology at Sorbonne University, Distinguished Professor in the Department of Ophthalmology at the University of Pittsburgh, Director of the UPMC Vision Institute, and Eye & Ear Foundation Chair of Ophthalmology. *"They show that measurable visual responses can be achieved in patients with advanced retinitis pigmentosa, using an approach that bypasses the loss of photoreceptors. These results remain exploratory and will need to be confirmed and further investigated, but they open up a particularly promising avenue for patients who currently have very limited treatment options."*

¹ GS030 has not received marketing authorization in any jurisdiction.

Encouraging exploratory findings in a study primarily focused on safety

Most ocular adverse events were mild or moderate, with the single event classified as severe resolving within minutes after instillation of lopidine. There was no systemic adverse event determined to be related to the study drug or to the intravitreal injection.

The study was not designed to establish the efficacy of GS030, but the authors highlight several results that merit further research into the efficacy of GS030.

- In six of the ten participants, exploratory assessments showed a clinically meaningful improvement in light sensitivity, as assessed with full field stimulus threshold (FST) testing.
- Four of the 8 participants who completed visual behavioral testing showed positive changes in the ability to perform certain tasks while using the light-stimulating goggles: detecting, localizing or touching objects, or determining the orientation of bars.
- Exploratory high-density EEG recordings in a subset of participants showed changes consistent with the transmission of optogenetically evoked visual signals to the visual cortex, supporting the interpretation that the observed behavioral responses reflected changes in visual processing rather than being an artifact.

"For patients with advanced retinitis pigmentosa, who still have very limited treatment options, an approach that could restore some form of visual perception represents an especially important prospect," said Prof. Isabelle Audo, coordinator of the REFERET reference center at the 15-20 National Hospital, team leader in the Department of Genetics and Deputy Director of the Institut de la Vision. "This study also demonstrates the value of patient-centered clinical research conducted through close collaboration among research teams and innovation partners, brought together within the FOReSIGHT University Hospital Institute."

About retinitis pigmentosa (RP)

Retinitis pigmentosa (RP) is a group of inherited retinal diseases caused by mutations in more than 100 different genes. The mutations cause photoreceptors to degenerate, leading to vision loss that typically begins with night blindness and progresses irreversibly, leaving many patients legally blind by middle age. RP is the most common inherited retinal disease, affecting about 1 in 4,000 people, or more than 1.5 million people worldwide.² There is currently no approved treatment for most people with RP.

The abstract for the article, titled "Optogenetic Therapy for Restoring Aspects of Visual Function", is available on [this link](#). Access to the full article requires an account to be created.

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² Suleman N. Current understanding on Retinitis Pigmentosa: a literature review. Front Ophthalmol. 2025;5:1600283 (DOI:[10.3389/fopht.2025.1600283](https://doi.org/10.3389/fopht.2025.1600283))

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 develop novel approaches for restoring vision in patients with inherited retinal neurodegenerative diseases. GenSight Biologics' lead product candidate, GS010, is in Phase 3 clinical development in Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible blindness in teens and young adults. The company's second product candidate, GS030, is in Phase 1/2 clinical development as a mutation-agnostic optogenetic candidate therapy for late-stage Retinitis Pigmentosa, a leading cause of blindness in the world. Using gene therapy-based approaches, GenSight Biologics' product candidates are designed to be administered as one-time intravitreal injections with the potential to provide sustained functional visual recovery.

About the Hôpital national des 15-20 (the 15-20 National Hospital)

The 15-20 National Hospital is the leading French hospital in ophthalmology and the fight against vision pathologies. A hospital with national standing and university teaching, the 15-20 National Hospital brings together the best medical and care teams and provides dedicated care for visual diseases. In 2018, the hospital founded, with the Institut de la Vision, the University Hospital Institute (IHU) FOrReSIGHT, whose mission is to promote fundamental research to develop tomorrow's ophthalmology care and provide access to therapeutic innovations through international partnerships and the development of companies involved in preventing and treating eye diseases.

www.15-20.fr

About GS030

GS030 leverages GenSight Biologics' optogenetics technology platform, a novel approach for addressing photoreceptor degeneration 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. GS030 is in clinical development as a potential therapy in patients suffering from late-stage RP. GS030 has not been granted marketing authorization in any country.

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. GenSight Biologics' optogenetics approach 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. This optogenetics approach is independent of the specific genetic mutations causing blindness and may have potential applications in a number of retinal diseases in which photoreceptors degenerate, such as retinitis pigmentosa (RP) and dry age-related macular degeneration (dry-AMD).

About the PIONEER Phase 1/2 trial

PIONEER (NCT03326336) is a first-in-human, multi-center, open-label dose-escalation study designed to evaluate the safety and tolerability of GS030 in 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 PIONEER are those affected by late-stage non-syndromic RP with no light perception (NLP) or light perception (LP) levels of visual acuity. Three cohorts of three patients each were administered one of three doses of GS030-DP (5e10 vg; 1.5e11 vg; 5e11 vg) via a single intravitreal injection in their worse-seeing eye. Based on the safety profile of GS030 in all treated subjects, the Data Safety Monitoring Board (DSMB) approved an extension cohort and recommended selecting the highest dose (5e11 vg) for the cohort, for which one patient was enrolled. The primary outcome analyses are on the safety and tolerability with a follow up to 5-year post-injection. The long-term follow-up of PIONEER is being conducted in three centers in the United Kingdom, France and the United States.