

Media Release

Optogenetic therapy is safe and allows blind people to detect objects

Five years after a landmark result in one patient, the first treated cohort shows that optogenetic vision restoration works beyond a single case.

Basel, 8 October 2026 – An international research team has reported new results from the first clinical cohort of blind people treated with an optogenetic therapy for advanced retinitis pigmentosa. The approach combines gene therapy to make surviving retinal ganglion cells sensitive to light with special goggles that stimulate these cells. The study was led by José-Alain Sahel from the University of Pittsburgh and Botond Roska from the Institute of Molecular and Clinical Ophthalmology Basel (IOB). It involved GenSight Biologics (Euronext: SIGHT) and an international team of researchers. The same international collaboration performed the 2021 proof-of-concept study.

Key findings:

- **Safety was the primary endpoint.** Within the limits of the study, the treatment was considered safe. Most eye-related adverse events were mild or moderate. One severe event occurred immediately after injection and resolved within minutes after treatment.
- **Seven of ten patients became more sensitive to light.** Six reached the predefined threshold for a clinically meaningful improvement.
- **Four of eight patients who completed visual behavioural testing improved** in tasks such as detecting or locating objects. Even more patients improved in finding a doorway, or following a line while wearing the goggles.

Stefan Futterknecht, second author of the study, who played a major role in analyzing the data states: “This study represents an important milestone for optogenetic vision restoration. It demonstrates that the effects first observed in a single patient can be reproduced across several patients and provides a foundation for developing more sensitive and effective future therapies.”

Giving surviving retinal ganglion cells a new task

Retinitis pigmentosa is an inherited disease in which the retina's light-sensing cells, the photoreceptors, gradually lose their ability to respond to light. Mutations in more than 100 different genes can cause the disease. Unlike treatments directed at a specific genetic defect, optogenetic therapy takes a different approach: It gives surviving retinal ganglion cells a new sensitivity to light.

In this study, each participant received a single injection into their worse-seeing eye carrying the genetic instructions for ChrimsonR, a light-sensitive protein that responds to amber light. The patients then used special goggles equipped with a camera.

The goggles convert changes in the visual scene into pulses of amber light that activate ChrimsonR in the treated retina. Put simply, the gene therapy makes surviving retinal cells sensitive to light, and the goggles provide the light signal they need.

Learning to use a new visual signal

The study shows that several patients were able to detect or locate objects, find a doorway or follow a line while wearing the goggles. Measurements of brain activity also showed signals consistent with visual information reaching the visual areas of the brain.

Training played an important role. Patients who spent more time learning to use the goggles tended to perform better on object-detection tests, suggesting that rehabilitation may be an important component of optogenetic therapy.

While the treatment did not restore normal sight and the patients still could not read or recognize faces, the study marks an important milestone for optogenetic vision restoration. Within the limits of the study, the researchers concluded that the combination of gene therapy and light-stimulating goggles was safe. The findings show that even at a very advanced stage of blindness, surviving retinal ganglion cells can be made responsive to light and transmit visual information to the brain. This provides a foundation for developing more sensitive and effective optogenetic therapies in the future.

The full article, “*Optogenetic Therapy for Restoring Aspects of Visual Function*” is available in the *New England Journal of Medicine (NEJM)* at <https://doi.org/10.1056/NEJMoa2602215>.

About IOB

At the Institute of Molecular and Clinical Ophthalmology Basel (IOB), basic researchers and clinicians work hand in hand to advance the understanding of vision and its diseases, and to develop new therapies for vision loss. IOB started its operations in 2018. The institute is constituted as a foundation, granting academic freedom to its scientists. Founding partners are the University Hospital Basel, the University of Basel and Novartis. The Canton of Basel-Stadt has granted the institute substantial financial support.

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