GEG Tech
Genetic Engineering Technologies

UC Irvine

Press Release

January 20th, 2025

Retinal gene therapy: Visual cycle restored in blind mice

University of California, Irvine and GEG Tech announce results paving the way for high-precision gene therapy

Over the last few years, the field of gene editing witnessed a tremendous acceleration as new therapies were approved and treatment strategies started to make a measurable difference in patients' lives. Despite these advances, key technological challenges still hinder widespread adoption and its potential impacts. Finding the sweet spot between highly efficacious and equally safe technologies is a major disruption to the industry.

GEG Tech, a Paris-based Deeptech company, announced earlier this year the launch of **nanoparticle** technology which consists in messenger RNA vectors designed to deliver next-generation genome editing systems. These genome editors, known as base-editing and prime-editing are currently being tested by some of the world's leading genome editing research laboratories.

In partnership with Professor Krzysztof Palczewski, PhD, at the Center for Translational Vision Research from the University of California, Irvine (UC Irvine), has conducted a set of experiments using retinal degeneration mouse models. These experiments combined UC Irvine's genome editing technology and GEG Tech's mRNA vectorization technology.

The results showed two major innovations that can highly benefit this therapeutic field at both the **efficacy and the safety levels**:

- The recovery of more 50% of normal retinal electroretinography (ERG) function comparable to the best gene therapy approaches in similar animal models, based on DNA vectors.
- The delivery system used for the genome editor relies on RNA technology rather than a DNA vector, significantly **enhancing the biosafety of vectors**.

These data usher a new chapter of **safe and high precision gene therapy** treatments targeting not only retinal diseases but also a broad spectrum of genetic disorders.

UC Irvine's Professor Palczewski commented: *« Recent studies have demonstrated the power of our base editing technology to cure genetic retinal diseases in mice models. However, therapeutic and clinical applications will require a scalable, manufacturable, high biosafety vector that does not compromise efficiency in order to introduce genome editors into target cells and harness the full power of base editing »*

Mr. Antoine Duboscq, Co founder & CEO of GEG Tech added: "*GEG Tech is proud to pave the way for major therapeutic advances compatible with a high level of biosecurity »*

Dr. Nicolas Grandchamp, Scientific director of GEG Tech commented: *"These results validate GEG Tech's scientific vision, focused on biological engineering rather than exclusively chemical formulations. The development of highly precise and safe gene therapies is now a realistic goal for dozens of genetic pathologies »*

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The technology:

Next-generation genome editors, known as prime-editing and base-editing are regarded as cutting-edge advancements following the revolutionary **CRISPR** "molecular scissors." These technologies are being developed by some of the most advanced scientific teams globally, including the team led by Professor Krzysztof Palczewski at the University of California, Irvine.

To harness their potential for the **next generation of highly precise gene therapies**, new types **of mRNA vectors** capable of carrying these genome editors and "delivering" them to target cells must be developed.

Currently, the vectors available on the market lack the precision, efficiency, and safety required to deliver highly complex genome editors, such as those used in base-editing and prime-editing, for human health applications in gene therapy.

To address this challenge, GEG Tech has developed a novel mRNA vector technology capable of safely and precisely carrying these complex new base and prime-editing systems. Unlike most current solutions, which are based on chemical combinations, the vectors designed by GEG Tech leverage the power of molecular biology.

On October 10, 2024, GEG Tech announced *via* a press release the launch of experimental testing in collaboration with leading global genome editing laboratories, including UC Irvine.

Main Results

The mRNA vectors developed by GEG Tech were combined with the base-editing system used by UC Irvine to repair the defective *Rpe65* gene in *rd12* mice, which mimic a human genetic disorder responsible for a type of retinal blindness.

Mice treated with these vectors regained, on average**, over 50% of their retinal activity**, while untreated mice showed no retinal activity. These findings align with results published by UC Irvine on October 2020, in Nature Biomedical, which demonstrated retinal activity recovery in *rd12* mice using the same genome editor but delivered through an AAV DNA vectors, known for its lower level of biosafety.

These results show that it is possible to deliver such genome editors using a new generation of mRNA vectors, offering significantly higher biosafety levels compared to AAV DNA vectors**. This greatly enhances the risk-benefit balance.** These findings pave the way for precision gene therapy to restore proper vision in certain human conditions and have **broader implications for multiple other gene therapy applications**.

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About UC Irvine:

Founded in 1965, **UC Irvine** is a member of the prestigious Association of American Universities and is ranked among the nation's top 10 public universities by *U.S. News & World Report*. The campus has **produced five Nobel laureates** and is known for its academic achievement, premier research, innovation and anteater mascot. Professor Krzysztof Palczewski's laboratory studies inherited retinal diseases using biochemical methods and mouse models that replicate human blindness disorders. This research fosters the innovation of potential treatments for several of these diseases.

About GEG Tech:

GEG Tech is a French **Deeptech** company specializing in genetic engineering, developing innovative technologies for advanced therapies, including gene therapies, vaccines, and cancer immunotherapies. With 12 years of experience in genetic engineering, the company operates as a studio-lab, serving clients primarily in the United States and Europe.

GEG Tech's scientific team originates from the CNRS, and its laboratory is based on the **Saclay Plateau** near **Paris, France**.

Founded by the **adVentures** studio and supported by private investors, the company builds on the work initiated by the CNRS laboratory, a global pioneer in gene transfer to the brain since 1994. GEG Tech has developed a new generation of **biological mRNA vectors** that are far **more precise, efficient, and safer** than the currently available chemical or viral particles.



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