New report illustrates potential of precision genome editing in treating inherited retinal diseases
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In a new paper, University of California, Irvine researchers explain how precision genome editing agents have enabled precise gene correction and disease rescue in inherited retinal diseases (IRDs). The study, titled, "Precision genome editing in the eye," was published this week in the Proceedings of the National Academy of Sciences.

The paper describes progress toward using genome editing for treating IRDs and important considerations for robust clinical translation.

"More and more CRISPR-based treatment approaches are being tested in clinical trials," said Palczewski. "We believe that there will be an increasing number of clinical trials for targeting IRDs and that any mutation that causes them will be amenable to treatment with this approach."

Programmable CRISPR-Cas nucleases are effective tools for gene disruption, but they are poorly suited for precisely correcting pathogenic mutations in most therapeutic settings. Improvements are needed for clinical translation.

Inherited retinal diseases (IRDs) are a genetically heterogeneous group of blinding disorders characterized by a progressive degeneration of the photoreceptors as well as the retinal pigment epithelium (RPE). These disorders affect ?1 in 3,000 individuals worldwide and profoundly impact patients' quality of life. IRDs are caused by mutations in genes that are critical for development and/or function of the retina or RPE, and more than 270 causative genes have been identified.

Over the past two decades, major advances in gene therapy have engendered new hopes for successful treatment of these IRDs. Most recently, precision genome editing agents, including base editors (BEs) and prime editors (PEs), developed by The Liu Lab, have enabled efficient and precise target gene correction, rather than gene disruption, in various therapeutic settings, including mouse models of IRDs. Precise target gene correction greatly expands the potential therapeutic applications of genome editing technologies, since most genetic disorders cannot be treated by gene disruption.
"Precision medicine for IRDs has a promising outlook, as basic science has consistently led to the development of therapeutic tools to target patient-specific genetic mutations," said Palczewski. "The results of initial clinical trials, that use in vivo gene editing to treat IRDs, will be essential for informing the design and translation of future precision genome editing therapies."


Provided by University of California, Irvine