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Gene Therapy to Treat Inherited Retinal Diseases

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Since 1990s, more and more mutant genes that cause human diseases have been detected. At the same time, many naturally occurring animal models of the human diseases have also been found. Those animal models have similar gene mutations and show similar phenotypes as the human diseases. Those together with the great improvement of viral vectors have led to the development of a variety of therapeutic strategies and make more and more traditionally incurable inherited diseases becoming potential candidates for gene therapy.

For example, the eye has a combination of unique features, such as transparency and a highly compartmentalized anatomy, which make it particularly suitable as a target for gene therapy. After passing through transparent structures within the eye, light then reaches and interacts with the retina directly. This feature enables the visualization and accuracy of vector delivery and the subsequent non-invasive imaging and examination *in vivo*. The highly compartmentalized and enclosed anatomy of the eye makes the delivery of vectors to particular subsets of ocular cell types possible with minimal risk of vector dissemination to the rest of the body. The subretinal space between the retinal pigment epithelium (RPE) and photoreceptor has a relatively high degree of immunoprivilege and is thus being considered an ideal route for the delivery of vectors. Currently, the common vehicle to deliver the therapeutic gene into target retinal cells is adeno-associated viral vector (AAV). Because of the immune privilege of subretinal space, subretinal delivery of AAV vectors can efficiently target RPE and photoreceptor cells, in which most of the mutant genes locate. In recent years, different mouse models, especially naturally occurring mouse models of retinal diseases with recessive mutations have been treated with AAV vectors encoding therapeutic genes. AAV-mediated wild type gene expressions in targeted retinal cells of those models lead to obvious retinal rescues in many fields, including electrophysiology, morphology, biochemistry and behavior, proved by the current ongoing phase I/II Leber congenital Amaurosis type 2 (LCA2 with RPE65 mutation) gene therapy clinical trial. We concluded that gene therapy will be one of the focuses for the Journal of *Hereditary Genetics*.

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