Gene therapy of inherited retinal diseases with AAV vectors

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The last 20 years have seen a tremendous advancement in the field of *in vivo* retinal gene transfer, through the development of vectors able to efficiently and safely transfer genetic information to various cell types in the retina. Among the vectors with the highest potential for retinal gene therapy are those derived from the small, non-pathogenic adeno-associated virus (AAV). Proof-of-principle studies have indicated that strategies like gene replacement in recessive forms of retina degenerations, gene silencing in dominant forms, or gene supply of neurotrophic molecules in both, can significantly prevent/halt photoreceptor degeneration in animal models. These studies have allowed to move gene therapy for inherited retinal degenerations from bench to bedside. To date, more than 20 patients with Leber congenital amaurosis, a childhood inherited blinding disease, have been treated subretinally with AAV encoding for the therapeutic gene. The safety and efficacy results from patients that were treated over 3 years ago appear very promising, suggesting that gene therapy can be succefully applied to this disease as well as other blinding conditions.