

Preface

The eye is an important sensory organ of the human body. The health of the eye is closely related to quality of life. However, a series of degenerative retinal diseases, such as hereditary retinitis pigmentosa, Leber's congenital amaurosis, as well as age-related retinal diseases, including but not limited to diabetic retinopathy and macular degeneration, seriously threaten ocular health and even cause irreversible visual impairments, hereby impose heavy burdens on patients, families, and society. When traditional therapeutic modalities are employed to treat these degenerative retinal diseases, repeated administration is required, side effects are obvious, yet the efficacy is limited. Therefore, it is particularly necessary to explore safer and more efficient therapeutic regimens. The emergence of gene therapy, in a generalized sense including viral or non-viral system-mediated delivery of therapeutic gene, RNA, protein or small molecule, has brought hope for treatment of the degenerative retinal diseases. Furthermore, the independence of the ocular immune system conferred by the blood-retinal barrier, the high proportion of single gene-mutation diseases in the eye, and the convenience for realtime monitoring of the treatment effects via noninvasive examinations, rendering the eye an ideal organ for gene therapy. As for the types of the systems for gene therapy, non-viral systems have become important for the treatment of the degenerative retinal diseases due to their excellent safety, simple preparation and modification process, low immunogenicity, and great cargo capacity, in spite of their currently low transfection efficiency.