



The Evolution of Gene Therapy in Ophthalmology – What Can the Future Hold?

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Inherited retinal dystrophies (IRD) have been always a challenge for everyone including the patients, the families and the ophthalmologists. Early diagnosis has aided early rehabilitation of the patients but still with no definite cure. However, the field of ophthalmology is now undergoing a notable transformation, due to the advancements in gene therapy that can hold the light at the end of the “dark” tunnel. As we stand at the crossroads of scientific innovation and clinical application, the potential for restoration of vision is more perceptible than ever.

Gene therapy in the “past” was just a dream, however, “now” is becoming reality; with the approval and successful application of the first FDA-approved gene therapy for inherited retinal diseases, voretigene neparvovec (Luxturna), designed for patients with biallelic RPE65 mutation-associated retinal dystrophy.

With the advent of CRISPR-based gene editing and adeno-associated virus (AAV) vectors, the precision and safety of these interventions continue to improve.

Despite the promise, several challenges remain an obstacle; long-term safety, immune response to viral vectors, and the cost-effectiveness of these therapies require more investigation. Furthermore, the accessibility of gene therapy to a larger patient population, particularly in developing countries, remains a challenge for global ophthalmic care.

The collaboration between ophthalmologists, geneticists and molecular biologists is highly needed.

The journey has just begun, and the future can hold the promise of vision restoration.

As the field progresses, Acta Scientific Ophthalmology remains committed to advancing discussions on integrating gene therapy into clinical practice, shaping the future of ophthalmic care.