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# THE GENETIC CURE FOR BLINDNESS: ADVANCES IN RETINAL THERAPY

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**ABSTRACT**

**Background:** Gene therapy involves the transfer of genetic material to specific tissues or organs to achieve therapeutic effects, aiming to correct genetic abnormalities or diseases. This method utilizes cells as biological vehicles to deliver the therapeutic genes. Eye diseases, particularly those affecting the retina, have a significant hereditary component, which has prompted research into gene therapy as a potential treatment option. Macular diseases and retinal dystrophies, due to their genetic nature, are increasingly being targeted by innovative therapeutic strategies, including gene therapy.
**Objective:** The objective of this review is to explore the concept of gene therapy, the different types of vectors used for gene delivery, and the potential of gene therapy in treating retinal dystrophies.
**Methods:** This paper examines various studies, clinical trials, and scientific literature to assess the current advancements in gene therapy for retinal diseases. It will also discuss the types of gene therapy vectors, such as viral and non-viral vectors, that are used for efficient gene delivery in the eye.
**Results:** Gene therapy has emerged as a promising approach for the treatment of retinal dystrophies. Advancements in vector design and gene delivery methods have significantly improved the potential effectiveness of gene therapy. Studies have shown that gene therapy can potentially slow or halt the progression of retinal dystrophies by correcting genetic defects at the cellular level.
**Conclusion:** Gene therapy holds considerable promise as a treatment for retinal dystrophies, with ongoing research and clinical trials highlighting its potential. The choice of vector type plays a critical role in the success of gene therapy, and future research is necessary to refine these methods to maximize their therapeutic benefits for patients suffering from inherited retinal diseases.

## INTRODUCTION

Through the use of cells as a delivery vehicle, gene therapy is defined as the process of transferring genetic material to specific organs to produce therapeutic effects to correct genetic defects and/or diseases by restoring, adding, eliminating, or modifying gene expression to prevent or reduce the effects of the disease. This can be done directly, where the cell's genetic modification occurs inside the organism, or indirectly, when genetic manipulation occurs outside the organism, in a test tube (Abokyi and Tse 2025)

After research revealed that certain diseases are brought on by a person inheriting a defective gene, gene therapy was developed. Some methods for altering transfer processes include introducing a new gene to help combat the disease, inactivating a mutated gene that isn't working correctly, replacing a mutated gene with a functional copy, repairing an aberrant gene through selective reverse mutation, or controlling the level of activation or deactivation of a gene (Bereket, Kunter et al. 2025)

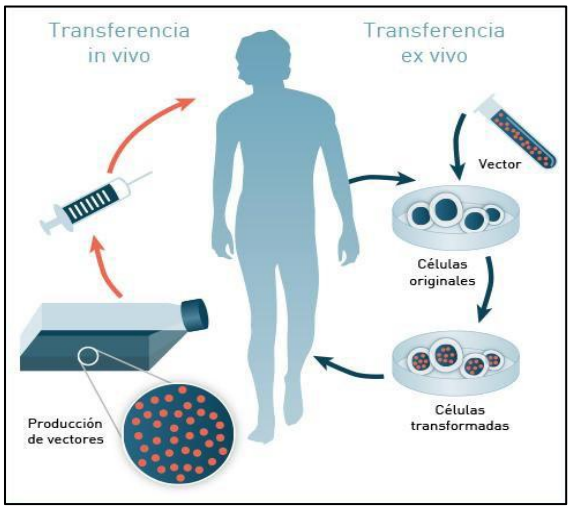


Figure 1. Release vehicles: In vivo, genetic modification within the organism.Ex vivo, genetic modification outside the organism.

This review paper presents ideas on gene therapy in treating retinal dystrophies. The scientific literature, found in Spanish and English books and the databases Science Direct, PubMed, Scielo, and Taylor and Francis, was thoroughly reviewed. The bibliography was published between 1999 and 2017. Considering the article "Evaluation of the Quality of Articles and Scientific Journals: proposal for a Weighted Impact Factor and a Quality Index," impact and prestige factors were applied to the reviewed articles (Darabuş, Dărăbuş et al. 2025).

## BRIEF HISTORY OF GENE THERAPY

After working with Frederick Griffith on studies on pneumonia caused by pneumococcus type I and type II, British researcher James Alloway discovered a "transforming factor" in 1928 that transformed "something" inside bacterial cells, changing their functions and modes of action. Because of further research on this "transforming factor," it was finally realized in 1944 that genes are made up of proteins. This led to further studies and even the Nobel Prize in Bacterial Genetics award (Ford and Petersen-Jones 2025).

Although gene therapy was conceived in the 1970s and 1980s, it wasn't implemented until the 1990s. This was due to the development of the transfer vector, understanding the human genome, and identifying genes and pathways essential to the body's operation. Similarly, an international research effort was initiated to document and comprehend the sequences that comprise human DNA. This program supports several molecular medicine goals, such as improving the knowledge and treatment of uncommon disorders and cancer (Harada, Guo et al. 2025).

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## TYPES OF GENE THERAPY AND VECTORS

Gene therapy comes in two varieties:

1. Somatic: This refers to the genetic modification of the somatic cells of a particular cell lineage. It is accomplished by altering a diseased gene by substituting it in the internal region of the cell of interest and then exchanging the modified gene for an unaltered one. Since this therapy is unique

to each individual, it is not passed on to the progeny.
2. The genetic endowment of the germ cells or gametes is altered and passed on to subsequent generations to prevent congenital disorders' growth.

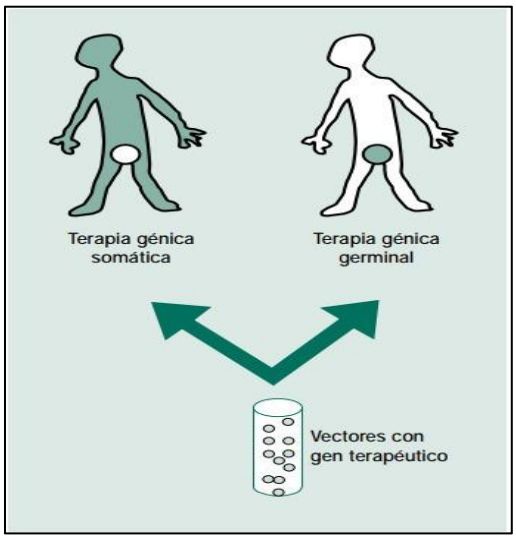


Figure 2: Because somatic therapy is individual-specific, it cannot be passed on to children. Germline therapy involves altering the genetic composition of gametes or germ cells.

The three parts of gene therapy are the vector, the construct, and the target cell.
The construct is transported into the target cell via the vector, which is a carrier. The therapeutic component, known as the construct, consists of the same gene, functional or absent protein for the target cell, a promoter, and a nucleic acid that may be small interfering RNA, microRNA, complementary DNA, or small hairpin shRNA.
The cell with the modified gene that has to be modified is known as the target cell (Mousavi, Naghshnejad et al. 2025).

However, administration vehicles are also referred to as vectors; these fall into two categories: viral and DNA. The earliest viruses were non-virals, whose DNA is in a plasmid separate from the host cell's genome. By quickly injecting a large volume of pDNA solution and momentarily creating holes in the cell membrane, plasmid DNA can be directly delivered in vivo using various hydrodynamic injection techniques, maximizing gene transfer efficiency in essential organs. Chemicals have been employed to enable negatively charged pDNA molecules to pass through hydrophobic cell membranes. pDNA is condensed into lipoplexes and polyplexes by microparticles containing cationic lipids and polymers (Oosten and ávan der Meer 2025).

Although viral vectors intentionally aim to reduce harm by eradicating as many viral genes as possible, they nonetheless capitalize on some viruses' contagiousness and gene-transmission potential. They are produced by substituting a therapeutic gene for one or more genes necessary for viral replication. In other words, the new virus is flawed so it can infect cells but not spread among them. It is important to note that the virus can be structurally changed using various methods and occupy a comparatively safe recombinant vector in this location, but only if the genes that cause its virulence and replication are swapped out for therapeutic genes that preserve its infectious potential.VVs have a high gene transfection efficiency, being able to infect target cells that, in some cases, reach up to 100% (Yassin, Wagner et al. 2025).

Some of the different types of viruses used as viral vectors for gene therapy are :

Retroviruses are members of a class of viruses that use the enzyme reverse transcriptase in the genome to create double-stranded DNA copies from their single-stranded RNA genetic material. The virus can also integrate into the host cell's chromosome by carrying an additional enzyme called integrase, which modifies the cell to contain a new gene. The primary drawback of this kind of vector is its ability to cause insertional mutagenesis, or the development of cancer, by inserting viral genetic material at any location in the host genome via the integrase enzyme. Nonetheless, one of the most effective uses of gene therapy is the clinical trial that uses the retroviral vector to treat severe combined immunological insufficiency associated with the X chromosome. As

researchers have become more confident, they have begun to inject altered retroviruses directly into the tissues where the corrected genes are needed (Yu, Dong et al. 2025).

Adenoviruses are larger and more complex than retroviruses; for this reason, they can infect a broader range of cells effectively, including lung cells. Some researchers have resorted to this class of viruses to avoid the problem of inserting genes in the wrong places; however, due to their composition of double-stranded linear DNA, they can cause respiratory, intestinal, and/or ocular infections. When an adenovirus infects a host cell, the genetic material of the virus does not integrate into the host cell's genetic material; on the contrary, the introduced DNA molecule remains free in the nucleus of the host cell, and the instruction in the extra DNA molecule is transcribed like any other gene (Abramovitch, Bick et al. 2025).

Their main drawback is that the patient's immune system is more likely to attack them, and the high viral concentrations needed for treatment can cause an unanticipated inflammatory reaction. Despite these disadvantages, these vectors have been utilized to treat ovarian and liver cancer. Gendicin, a p53 adenoviral product, is the first gene therapy product licensed to treat head and neck cancer (Bara-Ledesma, Viteri-Noel et al. 2025).

Since adeno-associated viruses [AAV] are tiny, non-autonomous, and naturally include only two genes, their payload is relatively restricted. They also have a single-stranded DNA genome and are non-pathogenic, which explains why they do not cause immune responses in patients. Since the virus directly inserts its genes into the DNA of the host cell, it may result in unintentional genetic harm. Recombinant DNA, or artificial DNA molecules produced in vitro, only contain the therapeutic gene and no viral genome. They do not integrate into the genome use at the end to form circular episomal forms that indicate the main factor influencing long-term gene expression {Pan, 2025 #80}.

It is currently used in preliminary studies to treat hereditary blood disease, hemophilia, music, and undereye disease. Clinical trials have also been initiated to use AAV vectors to deliver genes to the brain because the virus can infect non-dividing cells, such as neurons in which its genome is expressed long-term (Specht, Klimezak et al. 2025).

The primary function of the neurotropic virus Herpes simplex virus [HSV] is gene transfer into the brain system. Because of its vast genome, numerous therapeutic genes can be inserted into a single virugainingng latent infections in the host cell over an extended period. Numerous tissues, including lungs, muscles, pancreas, and liver cells, are susceptible to HSV infection. The Herpes

Humans frequently have antibodies against HSV; vascular seen-related consequences are uncommon. Since these viruses are linked to lymphoproliferative diseases, living with these genes and menem humans is vital, as is choosing only those that permit virus multiplication and viral plasmid maintenance (Philippidis 2025)

Both viral and non-viral vectors can directly deliver genes into the human body.

**Table 1. Vectors used for gene therapy with advantages and disadvantages**

| Vector | Advantages | Inconveniences |
|---|---|---|
| **Retrovirus** | - Stable integration - Easy handling - No provoked immune response - Efficient transduction | - Insertional mutation possible - Transgene extinction in vivo - Requires cell proliferation |
| **Adenovirus** | - Can infect resting or replicating cells<br>- Episomal (non-integrating) - Stable in vivo - High | - Triggers immune/inflammatory response - Difficult directionality - Difficult to handle |

| Vector | Advantages | Inconveniences |
|---|---|---|
| | titers | |
| **Adeno-Associated Virus (AAV)** | - Can infect resting or replicating cells - Possible specific integration - Low immunogenicity - High titers - Not pathogenic in humans | - Short transgene capacity - Difficult large-scale production - Limited in vivo cell tropism - Possible insertional mutation |
| **Herpes Simplex Virus (HSV)** | - Can infect resting cells - Episomal - Suitable for nervous system applications | - Pathogenicity concerns - Difficult to handle |

## GENE THERAPY IN EYE DISEASES

Due to the significant genetic component of eye diseases, feweaudienceses have proposed alternative treatments, including gene therapy, for their management. These studies have found that gene therapy is a novel and promising therapeutic approach that may offer a more effective means of treating these conditions (Tang and Yokota 2025).

The ocular diseases that have been studied the most from a molecular biology perspective, genetics, and gene therapy include choroidal and retinal neovascularizations like diabetic retinopathy, retinopathy of prematurity, and age-related macular degeneration, as well as other immunological gene factors with antiangiogenic properties in addition to the well-known vascular endothelial growth factor, which has shown excellent results in experiments with ischemic-induced retinas in mice. The eye is an easily accessible organ with immunological privilege, making it an ideal therapeutic target (Theodore, Imventarza et al. 2025).

Also, studies in diseases that involve degeneration of the optic nerve, ganglion cells, and photoreceptors, such as Retinitis Pigmentosa, show that the release of vectors with modified genes for the treatment of the disease increases visual sensitivity in the neurons of the superior colliculus, these results are precise to the Prph2 gene, a gene with which other research has been carried out where even protective functions of the external segments of photoreceptors have been found (An, Zhang et al. 2025).

Regarding glaucoma, factors such as brain-derived neurotrophic factor, ciliary neurotrophic factor, and ciliary cell neurotrophic factor have been found that, after being transmitted with adeno-associated vectors in mice, demonstrate neuroprotective properties and survival of retinal ganglion cells. Some studies have pointed to the reduction of intraocular pressure through gene therapy as an alternative treatment for glaucoma. However, the vectors have had to be reconsidered due to unwanted inflammatory responses (Azadeh, Neghab et al.).

Postnatal implantation of the PAX6 gene in mice has been demonstrated to restore electrical activity in the retina, according to studies on other illnesses such as aniridia. Through the antiapoptotic mechanisms of the RB1 gene, which prevent its growth, gene therapy has reduced and arrested mass growth in retinoblastoma; however, the research has remained in the experimental stages due to severe inflammatory effects induced by the type of vector utilized (Bakir, Robertson et al. 2025).

Studies on choroidal melanoma, corneal neovascularization, and other congenital eye abnormalities have shown encouraging outcomes. Since reduced vision and blindness are associated with all these diseases, gene therapy is an alternative for managing and preventing these conditions (Branham, Samarakoon et al. 2025).

## GENE THERAPY IN RETINAL DYSTROPHIES

Retinal dystrophies are one of the leading causes of severe visual impairment.

The retina is responsible for detailed central vision and peripheral vision for the movement and adaptation to lighting conditions; it is made up of various layers of photoreceptors called cones and rods, ganglion cells and pigments xanthophylls, lutein and zeaxanthin; the alteration of retinal function causes the loss of reading skills, face recognition, perception of movement, light, and colors. A classification of retinal dystrophies is found in Table 2. Table 2. Classification of retinal layers and the dystrophies that each one presents (Chichan, Aldujaly et al. 2025)

| LAYERS OF THE RETINA | DYSTROPHIES |
|---|---|
| Layers of fibers are nervous. | • Juvenile retinoschisis X-linked<br>• Achromatopsia.<br>• Cone-rod dystrophy.<br>• -Disease of Stargardt |
| EPR and photoreceptors | • -LCA.<br>• Progressive.atrophic macular dystrophy<br>• Vitelliform dystrophy.<br>• Flavimaculatus.<br>• Butterfly pigment |
| EPR | • dystrophy, or pattern dystrophy.<br>• Reticular dystrophy.<br>• Macular dystrophy dominant cystoid<br>• Dystrophy |
| Bruch's Membrane | • Pseudoinflammatory.<br>• AMD. |
| Choroid | • Centralareolar choroidal dystrophy. |

The most prevalent juvenile retinal degeneration, Stargardt disease, is defined by rapid central vision impairment brought on by various mutations in the ABCA4 gene. It is extensive and has a high degree of polymorphic heterogeneity, which leads to a wide range of clinical variability. Lentiviruses are the most effective vectors for transducing photoreceptor genes with the ABCA4 gene in macaques and rabbits without causing adverse effects. Previous findings indicate that human clinical trials have previously been conducted. The only report to date is at the first dose level, mentioning that eight patients have been treated with subretinal injection using the lentivirus vector carrying the ABCA4 gene without any serious adverse effects and that the next dose level will be increased (He, Fu et al. 2025). However, its efficacy is yet to be proven, and it could mainly benefit from stopping the progression of the disease.

**Leber congenital amaurosis** is caused by mutations in the RPE65 gene classified as LCA2. Patients with LCA2 present nyctalopia, nystagmus, and poor vision before age one. Finding improvement in vision towards adolescence and progressive decline between the third and fifth decade. In murine models, the safety and efficacy of subretinal gene therapy were demonstrated using the recombinant adeno-associated virus 2 carrying the RPE65 gene, finding a restoration of visual function (Hermankova, Javorkova et al. 2025).

Given the significant success of gene therapy in RPE65-deficient animal models, researchers decided to conduct clinical trials in human eyes. The patients studied underwent vitrectomy followed by subretinal injections of human RPE65 via the rAAV2 vector in the eye with poorer vision. Transduction tolerance was demonstrated, with no serious adverse effects related to the treatment. The most frequent adverse effects were mainly related to the surgical procedure, such as subconjunctival hemorrhage and ocular hyperemia. The study found an increase in best-corrected visual acuity in the treated eye in 5 patients, improvement in the kinetic visual field area, improvement in the total volume of the entire island of vision, and the central 30° portion of the island of vision. Only one subject showed decreased visual acuity, and two patients demonstrated decreased kinetic visual field area (Lv, Fan et al. 2025).

Although an ideal animal model of AMD has not yet been conducted, vascular endothelial growth factor has been identified as the proangiogenic factor that promotes

choroidal neovascularization in Age-related Macular Degeneration wet. Laser disruption of Bruch's basement membrane or local implantation of proangiogenic cytokines, such as VEGF and basic fibroblast growth factor, have been shown to induce choroidal neovascularization.

Following intravitreal injections of adeno-associated vectors connected to a novel soluble chimeric protein fms-like tyrosine kinase-1, which inhibits endogenously generated VEGF by ligating and neutralizing VEGF-A, neovascularization was markedly reduced. Nonhuman primates were used in this investigation. Retroviral-lentiviral systems are alternative vectors for gene therapy in choroidal neovascularization.

However, the development of a panel of anti-VEGF short hairpin RNAs and the use of microRNA hairpins derived from lentiviruses or adeno-associated vectors based on the most effective shRNAs have greatly improved the results of treating exudative AMD since the introduction of anti-VEGF gene therapy (Mei 2025).

**The cone dystrophies**

They are a broad category of clinically diverse disorders primarily affecting the RPE or cone photoreceptors. The estimated prevalence of achromatopsia is 1:40,000, and it is solely inherited in an autosomal recessive fashion. Its genetic etiology is nearly fully understood; 93% of cases in the Caucasian population are explained by mutations in the five genes linked to the disease, which encode crucial proteins in the cone phototransduction cascade. Achromatopsia is a candidate disease for gene therapy due to the small number of related genes; however, these treatments are only effective when the retina's cones are still present. The genes CNGB3, CNGA3, PDE6C, GNAT2, and PDE6H are linked to this illness (Nash, Turner et al. 2025).

After therapy with the human CNCB3-cDNA gene and a human cone arrestin promoter through the adenoviral-associated vector rAAV2/8, studies conducted in mice with a deficient CNGB3 gene demonstrated an increase in cone density and survival, improved structure of the external segments of the cone, and adequate subcellular compartmentalization of cone opsins. This study represents the most extensive restoration of visual function in animal models of achromatopsia using a human construct, and it now has a high chance of being used in clinical trials.

Studies with the GNGA3 gene have been performed in sheep with a mutation of the same, performing gene therapy under the control of a red-green opsin promoter and the associated adenovirus vector AAV5, finding a marked improvement in the response of the cones through electroretinogram. There are no conclusions from studies, including those on the PDE6C, GNAT2, and PDE6H genes in experimental stages and animal methods (Saute, Picanço-Castro et al. 2025).

In addition to photophobia or hemeralopia, patients with cone dystrophy exhibit severe loss of visual acuity and changes in color vision between the first and second decade of life. At first, they show normal cone function. When the RPGR gene's ORF15 region is mutated, the initial symptom is hemeralopia, which is followed by decreased visual acuity. Myopic refractive errors of six or more diopters may also be present. Its phenotypic can include RPE atrophy, bull's-eye patterns, or healthy retinas. Its most prevalent Mendelian inheritance pattern is autosomal recessive, and its estimated prevalence ranges from 1:30,000 to 40,000 (Stöhr and Weber 2025).

When the adeno-associated virus AAV5 or AAV8 encodes the RPGRIP1 gene and is injected into dogs, studies reveal that the photoreceptors in the transduced retinal regions survive better, resulting in increased retinal function. Vitelliform Macular Dystrophy's Best The BEST1 gene, which codes for the protein bestrophin-1, an active calcium chloride channel found in the basolateral membrane of RPE cells, is mutated autosomally dominantly. There are significant differences in the onset age of

central vision loss between the first and sixth decades. Studies of potential animal models using adeno-associated ribozyme-mediated vectors are currently available. However, they have not yet produced any conclusive results .

Additional retinal dystrophies include central areolar choroidal dystrophy, butterfly pattern pigmentary dystrophy, lattice dystrophy, cissoid dominant macular dystrophy, neuroinflammatory dystrophy, and X-linked juvenile retinoschisis. They have not taken gene therapy research seriously and are instead studying their molecular biology (Yu, Zhou et al. 2025).

## CONCLUSIONS

Gene therapy for managing retinal dystrophies is a novel and promising alternative. Currently, there are no effective treatments for this type of genetic disease, and individuals with these conditions remain classified as visually impaired. More research is needed in ocular genetics to expand management options for this population.

The published clinical trials demonstrate that the scientific community is getting closer every day to preventing these diseases and preventing progression in patients who already have them. Side effects are one of the weaknesses of this therapy; however, the long-term effects of successful treatments prove that we are very close to preventing many types of blindness.

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