

# Gene therapy for inherited diseases: Correcting genetic aberrations at the source.

Shuixi Chen\*

Department of Ophthalmology, National University Health System, Singapore

## Introduction

The blueprint of life resides within our genes, the intricate code that shapes our biology and governs our existence. However, for some individuals, this code carries errors—genetic aberrations inherited from their parents that can lead to a myriad of inherited diseases. In recent years, the field of gene therapy has emerged as a beacon of hope, offering a revolutionary approach to correct genetic abnormalities at their very source. This article explores the landscape of gene therapy for inherited diseases, delving into the science, successes, and promises that this transformative field holds [1].

Inherited diseases, often referred to as genetic or hereditary disorders, result from mutations or alterations in an individual's DNA. These mutations can manifest as single-gene disorders, where a mutation in a single gene leads to a specific disease, or as complex disorders influenced by multiple genetic factors. Examples range from cystic fibrosis and sickle cell anemia to muscular dystrophy and haemophilia [2].

The impact of inherited diseases is profound, affecting individuals from birth and often leading to lifelong challenges. Traditional treatments for these conditions have focused on managing symptoms rather than addressing the root cause—the genetic aberrations encoded within the individual's DNA [3].

Gene therapy represents a paradigm shift in medical intervention by aiming to correct genetic abnormalities rather than merely alleviate symptoms. At its core, gene therapy involves the introduction, removal, or alteration of genetic material within a patient's cells to treat or prevent disease. The overarching goal is to restore normal gene function, offering a potential cure rather than a lifelong management strategy [4].

One of the key approaches in gene therapy is to replace a faulty gene with a functional one. This is achieved by introducing a healthy copy of the gene into the patient's cells, often utilizing viral vectors as vehicles to deliver the corrected genetic material. Another strategy involves editing the existing genetic code using technologies like CRISPR-Cas9, which allows for precise modifications at the molecular level [5].

Gene therapy has already yielded notable successes, particularly in the treatment of rare monogenic disorders. One

groundbreaking example is the use of gene therapy to treat severe combined immunodeficiency (SCID), often referred to as "bubble boy" disease. By introducing a functional copy of the defective gene responsible for SCID, researchers have successfully restored immune function in affected individuals [6].

Similarly, gene therapy has shown promise in the treatment of certain forms of inherited blindness. In these cases, introducing a functional gene into the retina has resulted in improved vision, demonstrating the potential of gene therapy to address conditions that were once considered untreatable [7].

The field has also witnessed advancements in addressing more complex genetic disorders. Ongoing research is exploring the use of gene therapy for conditions such as muscular dystrophy, where multiple genes contribute to the disease phenotype. While challenges remain, the progress made suggests that gene therapy may eventually offer solutions for a broader spectrum of inherited diseases [8].

Despite the remarkable strides in gene therapy, challenges persist. Delivery mechanisms, ensuring the targeted and efficient delivery of corrected genes to the relevant cells, remain a focal point of research. Additionally, concerns about the long-term safety and unintended consequences of gene editing technologies underscore the need for rigorous testing and ethical considerations [9].

The accessibility and affordability of gene therapies also pose challenges, particularly in the context of rare diseases where development costs may be high, and patient populations are small. Balancing the ethical imperative to provide innovative therapies with the practicalities of healthcare economics is an ongoing conversation in the field [10].

## Conclusion

Gene therapy for inherited diseases stands at the forefront of biomedical innovation, offering a glimpse into a future where genetic aberrations need not define an individual's life. The ability to correct the underlying genetic code holds profound implications for medicine, transforming the treatment landscape for a multitude of conditions.

As research advances, the hope is that gene therapy will become more widely applicable, addressing a diverse range

---

\*Correspondence to: Shuixi Chen, Department of Ophthalmology, National University Health System, Singapore, E-mail: zeshui@263.net

Received: 01-Dec-2023, Manuscript No. AABB-23-123756; Editor assigned: 03-Dec-2023, Pre QC No. AABB-23-123756(PQ); Reviewed: 17-Dec-2023, QC No. AABB-23-123756;

Revised: 21-Dec-2023, Manuscript No. AABB-23-123756(R); Published: 29-Dec-2023, DOI:10.35841/aabb-6.6.177

---

Citation: Chen S. Gene therapy for inherited diseases: Correcting genetic aberrations at the source. *J Biochem Biotech* 2023; 6(6):177

of inherited diseases with greater precision and efficacy. From monogenic disorders to complex genetic conditions, the potential for gene therapy to rewrite the narrative of inherited diseases is both exhilarating and transformative.

In navigating the complexities of gene therapy, a commitment to ethical considerations and responsible innovation is paramount. The successes witnessed in recent years underscore the power of genetic interventions, yet they also underscore the importance of cautious progress and comprehensive evaluation.

In the journey toward widespread implementation, collaboration among researchers, healthcare professionals, policymakers, and ethicists is essential. By collectively addressing challenges, refining techniques, and ensuring equitable access, the promise of gene therapy for inherited diseases can become a reality for individuals facing the challenges of their genetic legacy. Gene therapy stands not only as a scientific endeavor but as a beacon of hope, illuminating a path toward a future where the source code of life itself becomes a tool for healing.

## References

1. Fischer A, Cavazzana-Calvo M. Gene therapy of inherited diseases. *Lancet*. 2008;371(9629):2044-7.
2. Nuzbrokh Y, Ragi SD, Tsang SH. Gene therapy for inherited retinal diseases. *Ann Transl Med*. 2021;9(15).
3. Wolfe JH. Recent progress in gene therapy for inherited diseases. *Curr Opin Pediatr*. 1994;6(2):213-8.
4. Curiel DT, Pilewski JM, Albelda SM. Gene therapy approaches for inherited and acquired lung diseases. *Am J Respir Cell Mol*. 1996; 14(1):1-8.
5. Hu ML, Edwards TL, O'Hare F, et al. Gene therapy for inherited retinal diseases: progress and possibilities. *Clin Exp Optom*. 2021; 104(4):444-54.
6. Yilmaz BS, Gurung S, Perocheau D, et al. Gene therapy for inherited metabolic diseases. *J Mother Child Health*. 2020; 24(2):53-64.
7. Trapani I, Puppo A, Auricchio A. Vector platforms for gene therapy of inherited retinopathies. *Prog Retin Eye Res*. 2014; 43: 108-28.
8. Boudes PF. Gene therapy as a new treatment option for inherited monogenic diseases. *Eur J Intern. Med*. 2014; 25(1):31-6.
9. Pelletier R, Caron SO, Puymirat J. RNA based gene therapy for dominantly inherited diseases. *Curr Gene Ther*. 2006;6(1):131-46.
10. Herzog RW, Cao O, Hagstrom JN, et al. Gene therapy for treatment of inherited haematological disorders. *Expert Opin Biol Ther*. 2006;6(5):509-22.

**Citation:** Chen S. Gene therapy for inherited diseases: Correcting genetic aberrations at the source. *J Biochem Biotech* 2023; 6(6):177