

Advancements in Gene Therapy for Cystic Fibrosis

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Abstract. This article explores the recent advancements in gene therapy as a treatment for cystic fibrosis, focusing on the development of new vectors for gene delivery. It discusses the potential of CRISPR-Cas9 and other gene-editing technologies to correct genetic mutations associated with the disease. Furthermore, the article evaluates the clinical trials conducted in recent years, highlighting the improvements in patient outcomes and the challenges that remain in making gene therapy a standard treatment for cystic fibrosis.

Keywords: Gene Therapy, Cystic Fibrosis, CRISPR-Cas9, Genetic Mutations, Clinical Trials

Introduction

Gene therapy has emerged as a promising approach to treat genetic disorders like cystic fibrosis, which is caused by mutations in the CFTR gene. Recent advancements in the field have focused on developing efficient gene delivery vectors and leveraging technologies such as CRISPR-Cas9 to correct these mutations at the genetic level. This article reviews the progress made in clinical trials and the technological breakthroughs that have improved the efficacy and safety of gene therapy for cystic fibrosis. Despite these advancements, challenges remain, including the delivery of the therapeutic gene to the appropriate tissues and the potential for immune responses. Continued research is crucial to overcome these hurdles and establish gene therapy as a viable treatment option.

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