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Commentary Article

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Recent Developments in the use of CRISPR-Cas9 for Targeted Therapy in Genetic Disorders

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DESCRIPTION

CRISPR-Cas9 technology has revolutionized the field of genetic engineering, offering unprecedented precision and versatility in the treatment of genetic disorders. Initially identified as a bacterial immune defense system, CRISPR-Cas9 has been repurposed into a gene-editing tool that enables targeted modifications to DNA sequences, with applications across a broad spectrum of genetic diseases. Recent developments in CRISPR-Cas9 for therapeutic purposes have focused on refining its precision, minimizing off-target effects and enhancing delivery mechanisms to improve safety and efficacy in human patients. CRISPR-Cas9 technology has great potential to heal diseases that were formerly thought to be incurable, ranging from complicated conditions like muscular dystrophy and some types of cancer to monogenic conditions like sickle cell anemia and cystic fibrosis.

Another major development in CRISPR-Cas9 technology for genetic therapy is the progress in gene delivery systems, essential for safely transporting the CRISPR components into the target cells. Viral vectors, such as Adeno-Associated Viruses (AAV), have been optimized to carry CRISPR-Cas9 machinery, especially for targeting specific organs or tissues, such as the liver and brain. Lipid nanoparticles, which are non-viral and biodegradable, have gained attention as an effective delivery system for CRISPR components, particularly for *in vivo* applications. These nanoparticles are a viable choice for systemic administration of CRISPR therapies because they can encapsulate the CRISPR-Cas9 ribonucleoprotein complex or mRNA, guaranteeing steady delivery and lowering immunogenic hazards.

Additionally, the recent development of biodegradable polymer-based nanoparticles has enhanced delivery efficiency and reduced toxicity, expanding the applicability of CRISPR-Cas9 to treat a wider range of genetic disorders. CRISPR-Cas9 applications in genetic disorders have been advancing from preclinical studies to clinical trials, showing encouraging results for various diseases. In particular, sickle cell anemia and β-thalassemia have become key areas of focus, as these blood disorders are caused by specific mutations in the HBB gene. Clinical studies using CRISPR-Cas9 have shown encouraging outcomes, with patients becoming transfusion-independent and seeing notable improvements in illness symptoms. The approach typically involves using CRISPR-Cas9 to reactivate fetal hemoglobin production in red blood cells, compensating for the defective adult hemoglobin and restoring proper blood function. This *ex vivo* approach involves editing patients' hematopoietic stem cells outside the body, followed by reinfusion, which reduces the risk of off-target effects and increases safety. Another noteworthy area is the application of CRISPR-Cas9 in Duchenne Muscular Dystrophy (DMD), a fatal muscle-wasting disease caused by mutations in the dystrophin gene. CRISPR-Cas9 has been used in animal models to correct the dystrophin gene, leading to functional improvement in muscle tissue. Although this approach has yet to reach human trials, the progress indicates potential for effective therapies in the future.

Inherited eye disorders are also a major target for CRISPR-Cas9, as the eye is an immune-privileged site, allowing for localized gene therapy with reduced immune response risks. In 2020, the first *in vivo* CRISPR-Cas9 human trial for an inherited retinal disorder, Leber Congenital Amaurosis 10 (LCA10), was initiated. LCA10 is caused by mutations in the *CEP290* gene, leading to progressive blindness. The trial, which delivered CRISPR-Cas9 directly into the retinal cells, aims to restore some degree of vision by correcting the genetic mutation *in situ*. Early outcomes suggest that CRISPR could be a viable treatment for genetic eye diseases, potentially extending to other conditions in the future. Neurodegenerative diseases, such as Huntington's Disease (HD) and Amyotrophic Lateral Sclerosis (ALS), are also being explored as targets for CRISPR-Cas9 therapy.

Ethical considerations have also become a central aspect of CRISPR-Cas9 applications, particularly with the prospect of germline editing, which could alter genetic material in future generations. The scientific community has largely agreed to limit CRISPR applications to somatic cells for therapeutic purposes, aiming to avoid the ethical and social implications of inheritable genetic modifications. Despite these ethical challenges, the continued refinement of CRISPR technology in therapeutic applications has shown that somatic gene editing could be a powerful tool in combating genetic disorders without affecting germline DNA.

In summary, the development of CRISPR-Cas9 for targeted therapy in genetic disorders represents a transformative approach in medical science, with the potential to cure or alleviate conditions that were previously untreatable. Advances in enhancing CRISPR’s specificity, safety and delivery methods have propelled it toward clinical application, with encouraging results in blood disorders, inherited eye diseases and muscular dystrophies.