Chapter 5

CRISPR and Gene Editing: A New Frontier in Healthcare

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Digvijay Singh[1] and Mohammed Ismail Iqbal[2]

Abstract

The potential for significant advancements in disease management is highlighted by this review, which looks at the application of CRISPR gene editing for treating allergies, particularly those related to allergens. It also evaluates the benefits and limitations of this technique in relation to existing treatment options. When it comes to treating medical illnesses like cancer, hepatitis B, cardiovascular disorders, and excessive cholesterol, CRISPR-Cas9 technology provides a rapid, precise, and effective way to manipulate DNA. The issue of reducing mismatches between sgRNA and genomic DNA caused by Cas9 is addressed. A wide range of genome-editing methods are discussed in this chapter, including as CRISPR/Cas9 systems, TALENs, and vector-based transfection. An overview of the prospective applications of gene modification techniques for the treatment of cardiovascular, hematological, neurological, viral, and cancer illnesses is also included, along with translational advances in ex vivo and in vivo studies. The roles of gene treatments, artificial intelligence (AI), and genetic testing in educational and medical settings are reviewed in this article, which also highlights moral concerns and emphasizes the necessity to develop novel legislative frameworks for their assessment and supervision. In order to emphasize the importance of further advancement, it also addresses situations in which these technologies exceed current ethical standards. The article discusses the developments in gene therapy, especially CRISPR-Cas9 and CAR-T cell therapy, as well as the constraints and critical thinking that must be addressed for oncology to proceed. It highlights the necessity of a multidisciplinary strategy, ethical supervision, and efforts to make things more affordable and accessible.

Keywords

Human Genomics, CRISPR/Cas, Genome Editing, Precision Medicine

[1]Centre of Excellence for Energy and Eco-Sustainability Research (CEER), Uttaranchal University, Dehradun, Uttarakhand, India. digvijaysingh019@gmail.com
[2]College of Engineering and Technology, University of technology and sciences, NIZWA. mohammed.iqbal@utas.edu.com

Corresponding author
Email id: mohammed.iqbal@utas.edu.com

1. Introduction

Although once futuristic concepts are now essential to contemporary scientific explorations, technological and medical advancements are efficiently altering our regulatory and ethical frameworks [1]. Personalized disease pathways are now the focus of public health decision-making, as a result of the emergence of precision medicine. Public health policies and procedures must evolve to ensure equitable and effective resource allocation, which will mitigate healthcare disparities and enhance population health [2]. To be able to treat illnesses, genome editing is designed to precisely remodel the human genome, while gene therapy transfers new genes into human cells. Almost all cell types, including human cells, may now undergo specific genomic sequence evolution through breakthroughs in human genomics, clinical medicine, molecular biology, and genetics [3]. Originally identified in bacteria and archaea, the CRISPR system is effective as an adaptive immune system at the genetic level. Its three stages of elimination of foreign genetic material are collecting, communication, and disruption. It does this by utilizing repetitive sequences, foreign DNA-derived spacers, and CRISPR-associated proteins [4]. In biomedicine, CRISPR/Cas systems are crucial for virus identification, clinical antiviral therapy, pharmaceutical and vaccine development, as well as hold enormous potential for the prevention and management of COVID-19 and other newly identified infectious illnesses in the near future [5]. With techniques including gene replacement immune modulation, editing, and silencing, gene therapy is a cutting-edge technique for curing cancer by targeting its genetic foundation. This offers a more effective and customized therapy alternative, but integrating it with conventional cancer therapies is highly challenging [6]. Gene therapy rectifies mutations by bringing a healthy gene into the body, whereas gene editing replaces a defective gene at the DNA level. Although the FDA is expected to accelerate the approval of multiple in-development in vivo gene editing products, few currently have obtained authorization. The potential for gene editing to treat illnesses is tremendous [7]. CRISPR-Cas have transformed several biological fields, including medicine. It is employed in a variety of applications, notably the engineering of bacteria, fighting against antibiotic resistance, the diagnosis and treatment of infections, genetic abnormalities, and tumors, as well as the investigation of antiviral medicines and gene functions [8].

2. CRISPR and Gene Editing

With innovative point-of-care diagnostic techniques, the CRISPR-associated protein system has transformed gene therapy and illness identification. In clinical research, it is utilized mostly in ex vivo therapy to enhance the quality of life for patients with incurable illnesses including cancer and blood complications [9]. Recent advancements have resulted in a beneficial biological tool for genetic engineering: the CRISPR-Cas9 genome editing system. This system, which originates from bacterial adaptive immunity techniques, could be leveraged to generate point mutations, knock-ins, and knock-outs [10]. Due to moral concerns, CRISPR-Cas9's relevance in vivo models is limited, despite its success in in vitro investigations employing animal germ cell lines. Experiments and clinical trials utilizing CRISPR gene knock-in techniques by sub-retinal injection have been conducted subsequently to target hereditary illnesses like Leber Congenital Amaurosis [11]. The process of "genome editing" involves manipulating genetic information with nucleic acids to permanently transform a specific gene. Employing a Cas protein and an extra RNA molecule to generate a double-stranded DNA break, the CRISPR/Cas system is an effective technique [12]. By eliminating allergy genes immediately, CRISPR technology offers a rare, more precise, efficient, and successful approach for genome modification. Initial investigations reveal promising results in wiping down allergenic proteins, and successful uses in clinical situations like sickle

cell disease and β-thalassemia demonstrate its potential [13]. Unlike previous genome editing techniques like zinc-finger nucleases and transcription activator-like effector nucleases, the CRISPR/Cas9 system efficiently targets specific genes. Although its application in the treatment of cardiovascular problems is still in its early phases, it has been extensively utilized in the prevention and treatment of cancers other peculiar diseases [14]. Genetic engineering is revolutionized by CRISPR technology, which makes utilization of Watson-Crick combination concepts that allow biologists to alter, insert, or eradicate particular genome segments. This technique opens up a wide range of applications in sectors that include biotechnology, medicine, and fundamental science [15]. Targeting different plant species for precise gene editing, CRISPR-Cas gene editing technology transformed genome exploration and manipulation. Science is concentrating on its structural characteristics, working mechanism, and latest advancements, although its application in medicinal plants is still in its early stages [16]. The eye is an appealing organ for clinical translational techniques since inherited retinal dystrophies (IRDs) are hereditary illnesses that affect vision. The CRISPR-Cas9 platform's advancements have made gene editing a feasible treatment option for IRDs, enabling the essential modifications [17]. For individuals with familial hypercholesterolemia, gene editing therapy offers substantial advantages beginning during early childhood by inactivating a crucial gene involved in cholesterol receptor control. It is a single-administration, potentially lifetime treatment [18]. By improving precision, efficiency, and cost-effectiveness, artificial intelligence (AI) has accelerated genome editing techniques for illnesses including Thalassemia and sickle cell anemia. By predicting the best gRNAs based on the genomic context, Cas protein type, intended mutation type, and possible consequences, AI models create RNAs for CRISPR-Cas systems [19]. High accuracy, lower material costs, and precise CAR insertion into genomic locations are made possible by gene-editing tools in synthetic biology for the development and manufacturing of CAR-T cells. This approach improves T cells' ability to treat patients and could eventually lead to the availability of readily available products [20].

3. Recommendations

After analyzing the literature available on current and past CRISPR and Gene Editing techniques, we propose following recommendations.

- Though current research and applied studies merely scratch the surface of CRISPR/Cas9's enormous potential, much more has to be done to fully fulfill this technology's promise in medical research.
- Despite being just recognized recently for its revolutionary medicinal benefits, CRISPR gene editing has the potential to completely transform how allergic diseases are managed and treated.
- The off-target impacts of CRISPR-Cas editing techniques have been mitigated employing AI models, affording a partial fix. However, further research is needed for predicting results beyond typical on-target effects or off-target expectations of different CRISPR-Cas9 applications, including as knock-ins and base modifications.
- The off-target impacts of CRISPR-Cas editing techniques have been mitigated employing AI models, affording a partial fix. However, further research is needed for predicting results beyond typical on-target effects or off-target expectations of different CRISPR-Cas9 applications, including as knock-ins and base modifications.
- Future advances in CRISPR-Cas have the potential to significantly impact the biotechnology industry by strengthening genome functional research, synthetic biology, genetic enhancement, and germ-plasm innovation in medicinal plants.

- It is anticipated that the initial CRISPR-Cas9 trial's safety will unlock the path for further research investigating a wider spectrum of diseases.
- Markerless gene editing and efficient transfection frameworks have been made achievable by CRISPR technology, which has also significantly boosted the knowledge of Plasmodium parasites. CRISPR/Cas9 has made significant advancements in Plasmodium falciparum by enabling long-lasting genomic modifications that modify invasion processes and gene expression.

Conclusion

With remarkable advantages, CRISPR-Cas9 technology is a cutting-edge targeted genome editing technique. Science's understanding of genetic variation and manipulation techniques has advanced, and it has contributed significant contributions to a variety of life science disciplines, including medicine, plant culture, and animal breeding. A multidisciplinary approach is necessary, as evidenced by the study of the intersection of gene editing, precision medicine, and ethical considerations. Despite persistent concerns regarding security and effectiveness, CRISPR-based genome editing is expected to become extensively utilized in therapeutic applications. In an effort to better understand allergy proteins, produce hypoallergenic food, and breed animals free of allergies, this paper explores potential applications of CRISPR technology. It implies the potential applications of CRISPR to identify novel allergens and modify immune responses to prevent the detection of allergenic proteins. The CRISPR/Cas system, a relatively new biomedicine tool, presents numerous opportunities in clinical settings. It is crucial to support emerging technologies, as it is expected to become indispensable in preventing and managing emerging infectious diseases through ongoing research and clinical verification.

ORCID iDs

Digvijay Singh https://orcid.org/0000-0003-3640-3891
Mohammed Ismail Iqbal https://orcid.org/0000-0001-6636-7014

References

1. Baldassarre, D., Piazza, O., & McConnell, P. (2023). Emerging ethical challenges in healthcare in the 21st century. *Anaesthesia & Intensive Care Medicine*.
2. Bilkey, G. A., Burns, B. L., Coles, E. P., Mahede, T., Baynam, G., & Nowak, K. J. (2019). Optimizing precision medicine for public health. *Frontiers in public health*, *7*, 42.
3. Dwivedi, S., Purohit, P., Vasudeva, A., Kumar, M., Agrawal, R., Sheikh, N. A. ... Misra, S. (2022). Gene therapy and gene editing in healthcare. In *Biotechnology in Healthcare* (pp. 147–175). Academic Press.
4. Moumaris, M. (2023). Revolutionizing Malaria Research: CRISPR unveils New Frontiers. *J, Biotechnology and Bioprocessing*, *4*(5), 2766–2314.
5. Ding, R., Long, J., Yuan, M., Jin, Y., Yang, H., Chen, M. ... Duan, G. (2021). CRISPR/Cas system: a potential technology for the prevention and control of COVID-19 and emerging infectious diseases. *Frontiers in cellular and infection microbiology*, *11*, 639108.
6. Ruikar, S. S., Masurkar, S. A., & Ghatage, A. A. (2024). Gene Therapy Innovations: A New Frontier in Cancer Biology Treatment. *NATURALISTA CAMPANO*, *28*(1), 29–35.
7. Niazi, S. K. (2023). In Vivo Gene Editing: A Healthcare Revolution in the Making.
8. Chawla, S., & Tyagi, K. (2024). The CRISPR-Cas technology: trends in healthcare. *In CRISPR-Cas System in Translational Biotechnology* (pp. 109–130). Academic Press.
9. Qiu, M., & Li, P. (2021). CRISPR/Cas-based diagnostics and gene therapy. *BIO Integration*, *2*(3), 121–129.

10. Tavakoli, K., Pour-Aboughadareh, A., Kianersi, F., Poczai, P., Etminan, A., & Shooshtari, L. (2021). Applications of CRISPR-Cas9 as an advanced genome editing system in life sciences. *BioTech*, *10*(3), 14.
11. Srivastava, A., Chauhan, S., & Ahuja, V. (2023). Recent advances in CRISPR/Cas9-assisted gene therapy. *Karbala International Journal of Modern Science*, *9*(4), 6.
12. Shademan, B., Nourazarian, A., Hajazimian, S., Isazadeh, A., Biray Avci, C., & Oskouee, M. A. (2022). CRISPR technology in gene-editing-based detection and treatment of SARS-CoV-2. *Frontiers in molecular biosciences*, *8*, 772788.
13. Brackett, N. F., Pomés, A., & Chapman, M. D. (2022). New frontiers: precise editing of allergen genes using CRISPR. *Frontiers in aller-gy*, *2*, 821107.
14. Saeed, S., Khan, S. U., Khan, W. U., Abdel-Maksoud, M. A., Mubarak, A. S., Aufy, M. ... Saleem, M. H. (2023). Genome editing technology: a new frontier for the treatment and prevention of cardiovascular diseases. *Current Problems in Cardiology*, *48*(7), 101692.
15. Zaib, S., Saleem, M. A., & Khan, I. (2022). CRISPR-Cas9 genome engineering: trends in medicine and health. *Mini reviews in medicinal chem-istry*, *22*(3), 410–421.
16. Guo, M., Chen, H., Dong, S., Zhang, Z., & Luo, H. (2022). CRISPR-Cas gene editing technology and its application prospect in medicinal plants. *Chinese medicine*, *17*(1), 33.
17. Hernández-Juárez, J., Rodríguez-Uribe, G., & Borooah, S. (2021). Toward the treatment of inherited diseases of the retina using CRISPR-based gene editing. *Frontiers in Medicine*, *8*, 698521.
18. Lewis, B. S. (2024). First-in-human trial of PCSK9 gene editing therapy for lowering cholesterol: a new frontier in cardiovascular pharmacotherapy? *News from AHA*.
19. Dixit, S., Kumar, A., Srinivasan, K., Vincent, P. D. R., & Krishnan, N. R. (2023). Advancing genome editing with artificial intelligence: opportunities, challenges, and future directions. *Frontiers in Bioengineering and Biotechnology*, *11*.
20. Wagner, D. L., Koehl, U., Chmielewski, M., Scheid, C., & Stripecke, R. (2022). sustainable clinical development of CAR-T cells–switching from viral transduction towards CRISPR-Cas gene editing. *Frontiers in immunology*, *13*, 865424.