EURASIAN EXPERIMENT JOURNAL OF PUBLIC HEALTH (EEJPH)

ISSN: 2992-4081 ©EEJPH Publications

Volume 7 Issue 1 2025

Gene Editing Technologies: CRISPR and Its Medical Applications

Apio Christine

School of Nursing, Kampala International University, Uganda

ABSTRACT

Gene editing technologies have transformed the landscape of biotechnology, offering precise and efficient tools to modify genomes for diverse applications. Among these, CRISPR-Cas9 has emerged as a revolutionary tool due to its simplicity, versatility, and specificity. This paper examines the mechanisms of CRISPR-Cas9, its applications in medicine, and its potential to correct genetic disorders like cystic fibrosis and sickle cell anemia. Additionally, challenges such as off-target effects, ethical considerations, and regulatory hurdles are discussed. With ongoing advancements, CRISPR technology is poised to redefine genetic research, therapeutic strategies, and interdisciplinary biotechnological applications. The future of CRISPR promises innovative solutions for addressing global health challenges and advancing personalized medicine.

Keywords: CRISPR-Cas9, Gene editing, Biotechnology, Genetic disorders, Personalized medicine.

INTRODUCTION

Gene editing technologies are fast becoming a highly influential area of biotechnology. Earlier, biologists were able to use transgenic techniques; however, there was no technique to target genes that specifically require alteration for treatment or according to a patient's individuality. For a decade, protein-based methods were used with the help of cells, via the very old technique "zinc finger proteins" and later "transcriptional activator-like effector nucleases." There are several genes that are ZFN-related, such as HBB and CCR5. Gene editing technology has great potential in agriculture, the medicinal industry, and biotechnology; a tool that can manipulate genes for desired traits or to remove susceptible traits, such as P53, P21, and others [1, 2]. Gene editing technologies have changed the way that lab bench science works, and their impact across the medical and biotechnological fields is similarly revolutionary. In a gene editing experiment, the aim can range from answering very specific biological questions to major therapeutic goals. Gene editing can be used to study how genes and entire sets of genes are expressed, and how the regulation of particular genes may bring about or turn off disease. Shortfalls of traditional gene-knockout techniques have led to problems such as chimeric animals and the need for further crosses to obtain tissue-specific knockouts in different embryonic tissues, as well as animals for survival. This, along with the downsides of the process, made the ZFN, TALENs, and later the CRISPR systems revolutionary across life science. Nevertheless, sophisticated groups continue to use methods such as antisense, ribozyme, zebrafish/drosophila systems, and siRNA for their research. The less technically savvy may therefore wonder why to use gene editing when there are cheaper and easier ways to go about things. Within this, an overview of CRISPR and the present clinical landscape will be provided. The pioneers and key players in the field of gene editing will also be discussed. The aim is to make this special issue accessible to readers of all scientific backgrounds, including early career researchers [3, 4].

CRISPR: Mechanism and Applications

The Clustered Regularly Interspaced Short Palindromic Repeats, or CRISPR is a genome editing tool that can target specific stretches of genetic code and alter them. It directs an enzyme called Cas9 to cut

DNA at a specific place. Since its discovery, CRISPR has been in the spotlight due to its superior efficiency and specificity for editing almost any living organism [5, 6]. CRISPR functions using a relatively simple mechanism. Any CRISPR system generally involves two main components: guide RNA, which can bind to the Cas protein, leading it to a specific target site in human DNA, and Cas proteins, which act as molecular scissors that facilitate DNA cutting. The technology has been used to modify bacteria, plants, and animal genes, heralding its applications in the fields of medicine, agriculture, food industries, and biological research. As a gene editing tool, increasing evidence has shown that CRISPR can effectively modify targeted sequences. One of the most successful applications of CRISPR technology has been its ability to correct the cystic fibrosis transmembrane conductance gene, the gene responsible for cystic fibrosis in a patient's epithelial stem cells. The gene encoding the sickle hemoglobin protein that causes sickle cell anemia has also been corrected, and the edited stem cells were infused into the patient's bloodstream [7, 8]. The RNA-programmable CRISPR-Cas9 system offers unprecedented control over eukaryotic genome editing due to the ability to reprogram Cas9 to bind and cleave a desired target. Older gene editing tools relied on the use of programmable nucleases, such as zinc-finger nucleases and transcription activator-like effector nucleases. The evolution of these nucleases required a deep understanding of amino acids and nucleotide interactions to accurately design a targeting protein, limiting their accessibility to researchers. Given the CRISPR-Cas9 system's simple mechanism and broad utility, there has been rapid adoption of CRISPR technology, particularly for the correction of genetic diseases and gene editing of human embryos. CRISPR has the potential to revolutionize genetic research, therapy, and the treatment of various genetic diseases [9, 10].

Medical Applications of CRISPR

Medical Applications. Curative gene editing has been an important application of CRISPR technology and has found its use in several trials and has even been tested clinically in certain diseases. In particular, CRISPR has been used since 2016 to treat a variety of genetic diseases, such as diseases caused by a mutation in a single gene, including hereditary spherocytosis, tyrosinemia, transthyretin amyloidosis, and progeria. Gene editing strategies have been explored for more difficult targets than single-gene inherited diseases, including cystic fibrosis and sickle cell anemia [11, 12]. To date, the most common use of CRISPR is to treat disorders in hematology, where a patient's blood cells are removed, modified using CRISPR, and then reinfused back into the patient. In 2018, CRISPR was first used in humans in a clinical trial to treat end-stage cancer patients by eliminating certain immune genes in patients' T-cells, and the cells modified using CRISPR were then infused back into the patient. This approach is called "immunotherapy," and even more clinical trials using CRISPR-edited human cells have been conducted since using many personalized medicine protocols. If scientists understand a rare blood disorder caused by a certain genetic mutation, such as Wiskott-Aldrich syndrome, this makes each patient's disease explosive in medicine. Where single pediatric cases are examined, scientists can rapidly search for medications according to the exact genetic mutation implicated. By 2018, 31 CRISPR tests in humans had been completed or were awaiting approval, and it was both used in adult human patients and conducted without stem cells [13, 14].

Challenges and Ethical Considerations in CRISPR-Based Gene Editing

Using the CRISPR-Cas9 system for gene editing still represents major technical challenges, such as off-target effects, low delivery efficacy, and slow toxicity of in vivo treatments. In the context of precision medicine, potential foreseeable and unforeseeable side effects of gene editing from therapy or diagnostic tests are also issues up for discussion. Ethical frameworks guiding the application of CRISPR in research and health care highlight the importance of ethical issues including, but not limited to, the protection and well-being of research subjects and patients, risk-benefit ratio estimations, the start of clinical trials only after Phase 1 in healthy volunteers for nonlethal genetic modifications, respect for individuals participating in trials, obtaining and monitoring the informed consent of individuals participating in clinical trials, and the importance of linking R&D with a commitment to putting new genomic insights and techniques into medical practice [15, 1]. Several potentially harmful gene editing outcomes were identified including mosaicism, where edited and unedited cells exist side by side in an organism; unwanted targeted gene insertions; off-target effects; gene disruptions resulting in unpredictable multigenerational phenotypes; and the potential for cells that have their genes edited in unwanted ways to transform into cancer cells. Society envisions that with sufficient scientific understanding of this tool, and adequate regulatory approvals and oversight in place, gene editing can facilitate considerable medical advancements as long as the research is transparent and the broader public has trust in the research. It is important to safeguard innovation in science and establish a balance between regulation and the ability for biased political influences to stifle the evolution of new scientific techniques and advancements [16, 17].

Future Directions and Potential Impact of CRISPR Technology

Despite the current limitations of CRISPR, ongoing research is focused on using machine learning to improve its accuracy when cutting a desired position in the genome. There are also many ongoing seminal studies to take CRISPR beyond gene therapy for genetic disorders and cancer to one that may therapeutically tackle complex diseases without known single genes or treat infections and genetic and epigenetic syndromes. There are also ongoing efforts to develop crop and animal strains that may alleviate world hunger, improve health and food security in low- and middle-income countries, fight poverty, and address climate change. Gene drives have the potential to create diverse and precise ecological interventions that may prevent the spread of diseases and protect wildlife. Some biotechnologies may use CRISPR which may allow testing for physical and psychological resilience and provide synthetic genome-encoded memory for high-resolution data storage. Given the pace of developments in these and other areas, it is difficult to imagine what the regulatory landscape with regard to CRISPR would look like in five years, let alone a few decades. The same goes for the ethics of future technologies and the culture of societies that embrace them. The rate of acceleration of CRISPR-based research across interdisciplinary fields and applications underscores the numerous opportunities and capabilities that the field possesses. However, the full impact of CRISPR-based research will only be realized by further research that establishes effective and widespread use of the tool synergistically across disciplines and diseases. In the next 10 years, substantial changes and advancements are anticipated in biotechnological research activities that will transform the field of biotechnology as a whole as well as widely impact the healthcare sector [18, 19].

CONCLUSION

CRISPR-Cas9 technology has ushered in a new era of precision gene editing with significant implications for medicine, agriculture, and biotechnology. Its ability to target specific genetic sequences offers transformative potential for treating genetic disorders, developing targeted therapies, and addressing global challenges like food security and environmental sustainability. However, the technology is not without limitations, including off-target effects and ethical concerns, which necessitate rigorous research and regulatory oversight. As advancements in machine learning and interdisciplinary collaboration continue to enhance CRISPR's accuracy and efficiency, this technology is expected to redefine the boundaries of science and medicine. By fostering transparency, ethical practices, and public trust, CRISPR can pave the way for groundbreaking innovations that benefit humanity at large.

REFERENCES

1. Doudna JA. The promise and challenge of therapeutic genome editing. Nature. 2020 Feb 13;578(7794):229-36.
2. Delhove J, Osenk I, Prichard I, Donnelley M. Public acceptability of gene therapy and gene editing for human use: a systematic review. Human gene therapy. 2020 Jan 1;31(1-2):20-46.
3. Aljabali AA, El-Tanani M, Tambuwala MM. Principles of CRISPR-Cas9 technology: advancements in genome editing and emerging trends in drug delivery. Journal of Drug Delivery Science and Technology. 2024 Jan 6:105338. sciencedirect.com
4. Niazi SK. The Dawn of In Vivo Gene Editing Era: A Revolution in the Making. Biologics. 2023 Sep 25;3(4):253-95.
5. Bhattacharjee S, Bhowmick R, Kant L, Paul K. Strategic transgene-free approaches of CRISPR-based genome editing in plants. Molecular Genetics and Genomics. 2023 May;298(3):507-20. springer.com
6. Naik RA, Mir MN, Rajpoot R, Singh S, Singh K, Singh SK. Genome editing tools: principles, structures, and mechanisms. InGenome Editing for Neurodegenerative Diseases 2025 Jan 1 (pp. 47-67). Academic Press. [HTML]
7. Chen Q, Zhang Y, Yin H. Recent advances in chemical modifications of guide RNA, mRNA and donor template for CRISPR-mediated genome editing. Advanced Drug Delivery Reviews. 2021 Jan 1;168:246-58.
8. Kurt IC, Zhou R, Iyer S, Garcia SP, Miller BR, Langner LM, Grünewald J, Joung JK. CRISPR C-to-G base editors for inducing targeted DNA transversions in human cells. Nature biotechnology. 2021 Jan;39(1):41-6. nih.gov
9. Chen C, Wang Z, Qin Y. CRISPR/Cas9 system: recent applications in immuno-oncology and cancer immunotherapy. Experimental Hematology & Oncology. 2023 Nov 14;12(1):95.

10. Allemailem KS, Almatroudi A, Rahmani AH, Alrumaihi F, Alradhi AE, Alsubaiyel AM, Algahtani M, Almousa RM, Mahzari A, Sindi AA, Dobie G. Recent Updates of the CRISPR/Cas9 Genome Editing System: Novel Approaches to Regulate Its Spatiotemporal Control by Genetic and Physicochemical Strategies. International Journal of Nanomedicine. 2024 Dec 31:5335-63. tandfonline.com
11. Liu W, Li L, Jiang J, Wu M, Lin P. Applications and challenges of CRISPR-Cas gene-editing to disease treatment in clinics. Precision clinical medicine. 2021 Sep;4(3):179-91.
12. Cring MR, Sheffield VC. Gene therapy and gene correction: targets, progress, and challenges for treating human diseases. Gene therapy. 2022 Feb;29(1):3-12.
13. Humbert O, Samuelson C, Kiem HP. CRISPR/Cas9 for the treatment of haematological diseases: a journey from bacteria to the bedside. British journal of haematology. 2021 Jan;192(1):33-49. nih.gov
14. Zanganeh S, Zahedi AM, Bardsiri MS, Bazi A, Bastanifard M, Shool S, Kouhbananinejad SM, Farsinejad A, Afgar A, Shahabi A, Mirzaei-Parsa MJ. Recent advances and applications of the CRISPR-Cas system in the gene therapy of blood disorders. Gene. 2024 Aug 20:148865. [HTML]
15. Wang SW, Gao C, Zheng YM, Yi L, Lu JC, Huang XY, Cai JB, Zhang PF, Cui YH, Ke AW. Current applications and future perspective of CRISPR/Cas9 gene editing in cancer. Molecular cancer. 2022 Feb 21;21(1):57. springer.com
16. Hou Y, Ureña-Bailén G, Mohammadian Gol T, Gratz PG, Gratz HP, Roig-Merino A, Antony JS, Lamsfus-Calle A, Daniel-Moreno A, Handgretinger R, Mezger M. Challenges in Gene Therapy for Somatic Reverted Mosaicism in X-Linked Combined Immunodeficiency by CRISPR/Cas9 and Prime Editing. Genes. 2022 Dec 13;13(12):2348. mdpi.com
17. Capalbo A, Poli M, Rienzi L, Girardi L, Patassini C, Fabiani M, Cimadomo D, Benini F, Farcomeni A, Cuzzi J, Rubio C. Mosaic human preimplantation embryos and their developmental potential in a prospective, non-selection clinical trial. The American Journal of Human Genetics. 2021 Dec 2;108(12):2238-47. cell.com
18. Wang J, Zhang X, Cheng L, Luo Y. An overview and metanalysis of machine and deep learning-based CRISPR gRNA design tools. RNA biology. 2020 Jan 2;17(1):13-22.
19. Cui Z, Lin L, Zong Y, Chen Y, Wang S. Precision Gene Editing Using Deep Learning: A Case Study of the CRISPR-Cas9 Editor. Applied and Computational Engineering. 2024 May 15;64:134-41. researchgate.net

CITE AS: Apio Christine. (2025). Gene Editing Technologies: CRISPR and Its Medical Applications. EURASIAN EXPERIMENT JOURNAL OF PUBLIC HEALTH, 7(1):57-60