INNOVATIONS IN MOLECULAR BIOTECHNOLOGY
2024, VOL. 2, pp. 19-21
https://doi.org/10.61577/imb.2024.100004

RESEAPRO JOURNALS

MINI REVIEW

OPEN ACCESS

Gene editing revolution: How CRISPR technology is transforming genetic science, medicine, and biotechnology for the future

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ABSTRACT

Gene editing tools like CRISPR-Cas9 have become ground-breaking resources in the fields of genetic research and biotechnology. In this discussion, we will examine the core mechanisms of CRISPR, emphasizing its extraordinary capability to accurately target and alter particular DNA sequences through the Cas9 protein. When juxtaposed with earlier gene-editing techniques, the straightforwardness and effectiveness of CRISPR have opened up numerous opportunities across various areas, including healthcare, agriculture, and biotechnology. In medicine, CRISPR presents significant potential for addressing genetic disorders such as sickle cell anemia, cystic fibrosis, and Duchenne muscular dystrophy. By enabling direct correction of the fundamental genetic mutations, CRISPR could transform the methods used to tackle these diseases. Additionally, researchers are investigating its applications in cancer therapy and regenerative medicine, which may lead to substantial advancements in cancer treatment and tissue healing approaches. Similarly, CRISPR's influence in biotechnology is noteworthy. It provides innovative avenues for improving crops, livestock, and microbial organisms, allowing for the development of genetically modified entities exhibiting favorable characteristics such as resistance to diseases, increased productivity, and adaptability to environmental changes. CRISPR presents significant ethical dilemmas, particularly in editing human germlines and creating genetically modified embryos, as its long-term implications and potential unintended consequences demand careful consideration.

KEYWORDS

Gene editing; CRISPR-Cas9; Biotechnology; Genetic disorders; Ethical considerations; Genetic science

ARTICLE HISTORY

Received 03 September 2024; Revised 23 September 2024; Accepted 01 October 2024

Introduction

CRISPR-Cas9 technology has transformed the realm of inheritable gene editing, providing unmatched precision and flexibility in modifying DNA sequences. Originally identified as a natural defense mechanism in bacteria, CRISPR collaborates with the Cas9 protein to locate and splice specific segments of DNA, enabling researchers to disrupt genes, insert new inheritable material, or correct faulty sequences. This innovation marks a significant advancement in inheritable engineering, exceeding earlier tools like zinc-finger nucleases (ZFNs) and transcription activator-like effector nucleases (TALENs) in terms of efficiency, precision, and user-friendliness [1].

The CRISPR breakthrough has initiated a fundamental change across numerous research areas and applications, especially in genetics, pharmaceuticals, and biotechnology. In pharmaceuticals, CRISPR holds great potential for addressing inheritable disorders at their source, including sickle cell anemia, cystic fibrosis, and muscular dystrophy [2]. By directly altering the faulty genes linked to these conditions, CRISPR paves the way for ground-breaking treatments, ushering in a new era of precise therapies.

Through the ability to modify crops to enhance yield, disease resistance, and environmental resilience, CRISPR could play a vital role in tackling global food security issues. Genetically engineered organisms developed using CRISPR may lead to a reduction in fungicide application, better crop adaptability to climate change, and enhanced nutritional value [3].

CRISPR Technology Overview

CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) technology, along with the Cas9 protein, has emerged as one of the most revolutionary breakthroughs in the arena of genetic editing [4]. The CRISPR-Cas9 system was initially identified in bacteria, where it serves as an adaptive defense mechanism, enabling bacteria to remember viruses they have previously encountered and protect against future infections. Scientists have leveraged this system to create a powerful tool that facilitates accurate modifications of DNA across a diverse range of organisms.

The effectiveness and affordability of CRISPR-Cas9 exceed those of earlier gene-editing methods such as ZFNs and TALENs. Both ZFNs and TALENs require intricate protein engineering to target specific DNA sequences, making their development lengthy and costly [5,6]. In contrast, CRISPR is easier to design and implement. This system utilizes a short RNA sequence to direct the Cas9 protein to the precise location on the DNA that requires alteration. Once there, the Cas9 protein creates a specific cut, allowing researchers to delete, insert, or modify the targeted gene with a high level of accuracy [7].

The straightforwardness and precision of CRISPR have rendered it a highly versatile platform for genetic manipulation.

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It can be employed to knock out genes, edit single nucleotides, or introduce entirely new genetic sequences. This ability to precisely target specific genes in a wide variety of organisms—ranging from bacteria to plants, animals, and humans—has established CRISPR as an essential tool in both research and applied sciences [8]. Its extensive applicability, combined with its user-friendliness, positions CRISPR as a vital resource for progressing genetic engineering in sectors such as medicine, agriculture, and biotechnology.

Applications in Genetic Science and Medicine

Genetic disease treatment

CRISPR technology has advanced significantly in addressing inheritable disorders by enabling the precise correction of faulty genes at their origin. Conditions such as sickle cell anemia, cystic fibrosis, and Duchenne muscular dystrophy, which result from mutations in a single gene, have become the target of numerous CRISPR-based treatments [9,10]. For instance, in the case of sickle cell anemia, researchers have utilized CRISPR to modify the genes that produce abnormal hemoglobin, leading to the deformation of red blood cells. By either inserting a healthy version of the gene or reactivating the natural production of fetal hemoglobin, scientists are working toward a potential cure for the disease [11]. Similarly, in cystic fibrosis, which arises from a mutation in the CFTR gene, CRISPR might be employed to correct the defect in lung cells, presenting a possibility for long-term treatment. Although these therapies remain in clinical trials, the ability to directly address the underlying genetic cause provides hope for permanent cures.

Cancer research

CRISPR is providing researchers with new instruments to investigate the genetic basis of cancer. By modifying genes associated with cancer in laboratory models, scientists are revealing the mutations that contribute to tumor formation, growth, and spread [12]. This knowledge is essential for creating targeted therapies that can selectively destroy cancer cells while reducing harm to healthy tissues. Additionally, CRISPR is being studied as a means to boost the immune system's ability to combat cancer more efficiently. One hopeful method involves altering immune cells, such as T cells, to better recognize and eliminate cancer cells [13]. This validated immunotherapy has shown promising results in early trials and may lead to innovative treatments for various cancer types.

Regenerative medicine

CRISPR's remarkable capabilities also hold significant promise for regenerative medicine, where it is being utilized to mend damaged tissues and organs. By modifying the DNA of stem cells, CRISPR enables researchers to create cells that can replace injured tissues, regenerate organs, and potentially cultivate organs in a laboratory setting. For example, CRISPR can be used to rectify hereditary disorders in stem cells, which can then generate healthy tissues for transplantation. Furthermore, CRISPR is being researched for its potential to heal injuries to organs such as the heart, liver, and nervous system, offering the possibility of regenerative therapies for conditions currently deemed untreatable. These advancements push the boundaries of medicine, with the ability to address a variety of degenerative diseases and enhance patients' quality of life [14,15].

Applications in Biotechnology

CRISPR has transformed biotechnology in agriculture, allowing for the creation of genetically modified plants that are more resistant to pests, various conditions, and environmental stressors [16]. These innovations are designed to enhance crop production and lessen the reliance on chemical fungicides, thereby supporting sustainable agriculture. In livestock management, CRISPR is utilized to improve beneficial traits such as disease resistance and growth rates, leading to better animal health and productivity [17]. Furthermore, the use of CRISPR in agriculture holds the potential to improve food security by developing crops and livestock that are more resilient to shifting climate conditions, while also minimizing agriculture's ecological impact [18].

Conclusions

CRISPR technology signifies a groundbreaking leap forward in genetic knowledge, unlocking unprecedented opportunities for modifying the genetic structure of organisms. Its accurate and efficient gene-editing abilities have the potential to transform numerous sectors, including medicine, agriculture, and biotechnology. In the field of medicine, CRISPR is currently being investigated as a method for addressing genetic disorders, such as sickle cell disease and cystic fibrosis, presenting the chance for lasting cures. This technology may also pave the way for new cancer treatments and advancements in personalized medicine. In agriculture, CRISPR is developing genetically modified crops that offer improved resistance to pests, harsh conditions, and environmental stresses, ultimately boosting food security and sustainability. It also shows promise in enhancing animal health and productivity, thereby reinforcing global food systems.

Nonetheless, the tremendous power of CRISPR presents important ethical and regulatory challenges. The ability to modify human genes, especially in embryos or germline cells, raises issues around the potential for unforeseen effects, designer babies, and genetic inequality. Regulatory frameworks need to adapt to tackle these concerns and ensure that the technology is employed responsibly and fairly. As CRISPR continues to progress, its future applications may transform medicine, agriculture, and biotechnology in ways once deemed impossible. However, it is crucial to proceed with care to strike a balance between advancement and ethical accountability.

Disclosure statement

No potential conflict of interest was reported by the authors.

References

1. Gaj T, Sirk SJ, Shui SL, Liu J. Genome-editing technologies: principles and applications. Cold Spring Har Perspect Biol. 2016;8(12):a023754. https://doi.org/10.1101/cshperspect.a023754
2. Luthra R, Kaur S, Bhandari K. Applications of CRISPR as a potential therapeutic. Life Sci. 2021;284:119908. https://doi.org/10.1016/j.lfs.2021.119908
3. Zaidi SS, Mahas A, Vanderschuren H, Mahfouz MM. Engineering crops of the future: CRISPR approaches to develop climate-resilient and disease-resistant plants. Genome Biol. 2020;21(1):289. https://doi.org/10.1186/s13059-020-02204-y
4. Wang H, La Russa M, Qi LS. CRISPR/Cas9 in genome editing and beyond. Annu Rev Biochem. 2016;85(1):227-264.

https://doi.org/10.61577/imb.2024.100004

https://doi.org/10.1146/annurev-biochem-060815-014607
5. Ahmadzadeh V, Farajnia S, Baghban R, Rahbarnia L, Zarredar H. CRISPR-Cas system: Toward a more efficient technology for genome editing and beyond. J Cell Biochem. 2019;120(10): 16379-16392. https://doi.org/10.1002/jcb.29140
6. Chandrasegaran S, Carroll D. Origins of programmable nucleases for genome engineering. J Mol Biol. 2016;428(5):963-989. https://doi.org/10.1016/j.jmb.2015.10.014
7. Ceasar SA, Rajan V, Prykhozhij SV, Berman JN, Ignacimuthu S. Insert, remove or replace: A highly advanced genome editing system using CRISPR/Cas9. Biochim Biophys Acta Mol Cell Res. 2016; 1863(9):2333-2344. https://doi.org/10.1016/j.bbamcr.2016.06.009
8. Li C, Brant E, Budak H, Zhang B. CRISPR/Cas: a Nobel Prize award-winning precise genome editing technology for gene therapy and crop improvement. J Zhejiang Univ Sci B. 2021;22(4):253-284. https://doi.org/10.1631/jzus.B2100009
9. Min YL, Bassel-Duby R, Olson EN. CRISPR correction of Duchenne muscular dystrophy. Annu Rev Med. 2019;70(1): 239-255. https://doi.org/10.1146/annurev-med-081117-010451
10. Chemello F, Bassel-Duby R, Olson EN. Correction of muscular dystrophies by CRISPR gene editing. J Clin Invest. 2020;130(6): 2766-2776. https://doi.org/10.1172/JCI136873
11. DeWitt MA, Magis W, Bray NL, Wang T, Berman JR, Urbinati F, et al. Selection-free genome editing of the sickle mutation in human adult hematopoietic stem/progenitor cells. Sci Transl Med. 2016;8(360): 360ra134-. https://doi.org/10.1126/scitranslmed.aaf9336
12. Guernet A, Grumolato L. CRISPR/Cas9 editing of the genome for cancer modeling. Methods. 2017;121:130-137. https://doi.org/10.1016/j.ymeth.2017.03.007
13. Elmas E, Saljoughian N, de Souza Fernandes Pereira M, Tullius BP, Sorathia K, Nakkula RJ, et al. CRISPR gene editing of human primary NK and T cells for cancer immunotherapy. Front Oncol. 2022;12:834002. https://doi.org/10.3389/fonc.2022.834002
14. Hsu MN, Chang YH, Truong VA, Lai PL, Nguyen TK, Hu YC. CRISPR technologies for stem cell engineering and regenerative medicine. Biotechnol Adv. 2019;37(8):107447. https://doi.org/10.1016/j.biotechadv.2019.107447
15. Loesch R, Desbois-Mouthon C, Colnot S. Potentials of CRISPR in liver research and therapy. Clin Res Hepatol Gastroenterol. 2019;43(1):5-11. https://doi.org/10.1016/j.clinre.2018.05.001
16. Ricroch A, Clairand P, Harwood W. Use of CRISPR systems in plant genome editing: toward new opportunities in agriculture. Emerg Top Life Sci. 2017;1(2):169-182. https://doi.org/10.1042/ETLS20170085
17. Tait-Burkard C, Doeschl-Wilson A, McGrew MJ, Archibald AL, Sang HM, Houston RD, et al. Livestock 2.0–genome editing for fitter, healthier, and more productive farmed animals. Genome Biol. 2018;19:1-1. https://doi.org/10.1186/s13059-018-1583-1
18. Kuiken T, Barrangou R, Grieger K. (Broken) promises of sustainable food and agriculture through new biotechnologies: the CRISPR case. CRISPR J. 2021;4(1):25-31. https://doi.org/10.1089/crispr.2020.0098

https://doi.org/10.61577/imb.2024.100004