

The Promises and Challenges of Developing Drugs with CRISPR Technology

Ying Jie*

Department of Molecular Biology and Biotechnology, Jomo Kenyatta University of Chemical Technology, Juja, Kenya

INTRODUCTION

In the quest to develop new therapies for a myriad of diseases, the emergence of CRISPR technology has sparked excitement and anticipation among researchers and clinicians. This revolutionary tool, with its unparalleled precision and versatility in genome editing, holds immense potential for transforming the landscape of drug discovery and development. As we embark on this journey of harnessing CRISPR for drug development, we encounter a multitude of promises and challenges that shape the trajectory of this transformative approach.

DESCRIPTION

At its core, CRISPR technology offers a powerful platform for elucidating the genetic underpinnings of disease and identifying novel drug targets with unprecedented precision. By enabling researchers to manipulate specific genes and observe the resulting phenotypic changes, CRISPR facilitates the systematic interrogation of gene function, pathway analysis and target validation. This fundamental understanding of disease mechanisms provides a solid foundation for the rational design of therapeutic interventions, paving the way for more effective and targeted treatments.

One of the most promising applications of CRISPR in drug development lies in the realm of precision medicine, where therapies are tailored to individual patients' genetic profiles. By leveraging CRISPR to correct disease-causing genetic mutations or modulate aberrant gene expression, researchers can envision a future where personalized treatments offer superior efficacy and safety compared to traditional one-size-fits-all approaches. CRISPR-based therapies hold the potential to address a wide range of genetic disorders, from monogenic diseases like cystic fibrosis and sickle cell anemia to complex conditions such as cancer and cardiovascular diseases.

Moreover, CRISPR technology has revolutionized the process of target identification and validation, accelerating the pace of drug discovery and development. By enabling high-throughput screening of candidate genes and drug targets, CRISPR streamlines the identification of promising therapeutic leads, significantly reducing the time and resources required for

preclinical research. This efficiency in target validation not only expedites the translation of basic science into clinical applications but also increases the success rate of drug development programs, minimizing the risk of late-stage failures and attrition.

Furthermore, CRISPR technology has the potential to revolutionize traditional drug modalities, opening up new avenues for therapeutic innovation. Beyond conventional small molecules and biologics, CRISPR enables the development of gene therapies, RNA-based therapeutics and cell-based therapies with unprecedented precision and efficacy. From correcting disease-causing mutations at the DNA level to modulating gene expression or editing RNA transcripts, CRISPR offers a versatile toolkit for addressing a wide range of diseases, including those that have been historically challenging to target with traditional approaches.

However, the translation of CRISPR technology into viable drug therapies is not without its challenges and limitations. One of the primary hurdles is the efficient and precise delivery of CRISPR components to target cells and tissues *in vivo*. Strategies for delivering CRISPR-Cas9 machinery, such as viral vectors, lipid nanoparticles or cell-penetrating peptides, must overcome barriers related to immunogenicity, off-target effects and limited payload capacity, posing significant technical and safety challenges for clinical implementation.

Moreover, concerns about off-target effects and unintended genomic alterations raise safety considerations that must be addressed rigorously in the development of CRISPR-based therapies. While advances in CRISPR engineering, such as enhanced specificity variants and novel delivery systems, have mitigated some of these concerns, comprehensive preclinical safety assessments and risk mitigation strategies are essential to ensure the safety and efficacy of CRISPR-mediated interventions in human patients.

Furthermore, regulatory and ethical considerations surrounding the use of CRISPR in drug development necessitate careful navigation and stakeholder engagement. Regulatory agencies must establish clear guidelines and standards for the development and evaluation of CRISPR-based therapies,

Correspondence to: Ying Jie, Department of Molecular Biology and Biotechnology, Jomo Kenyatta University of Chemical Technology, Juja, Kenya; E-mail: jieying@a.com

Received: 20-May-2024, Manuscript No. EOED-24-31566; **Editor assigned:** 23-May-2024, PreQC No. EOED-24-31566(PQ); **Reviewed:** 06-Jun-2024, QC No. EOED-24-31566; **Revised:** 16-Jun-2025, Manuscript No. EOED-24-31566 (R); **Published:** 23-Jun-2025, DOI: 10.35248/2329-6631.25.14.242

Citation: Jie Y (2025) The Promises and Challenges of Developing Drugs with CRISPR Technology. J Develop Drugs. 14:242.

Copyright: © 2025 Jie Y. This is an open-access article distributed under the terms of the Creative Commons Attribution License, which permits unrestricted use, distribution, and reproduction in any medium, provided the original author and source are credited.

balancing the imperative to promote innovation with the need to safeguard patient safety and public trust. Ethical concerns related to germline editing, equitable access and informed consent also underscore the importance of robust ethical frameworks and public dialogue to guide the responsible use of CRISPR technology in medicine.

CONCLUSION

In conclusion, the development of drugs with CRISPR technology holds immense promise for revolutionizing the treatment of diverse diseases and advancing the field of

precision medicine. By leveraging the precision and versatility of CRISPR, researchers can elucidate disease mechanisms, identify novel drug targets and develop personalized therapies tailored to individual patients' genetic profiles. However, realizing the full potential of CRISPR in drug development requires overcoming technical challenges, addressing safety concerns and navigating regulatory and ethical considerations with diligence and foresight. As we continue to harness the power of CRISPR technology for therapeutic innovation, it is essential to uphold the highest standards of scientific rigor, ethical integrity and patient-centered care to ensure the translation of CRISPR-based therapies from the laboratory to the clinic.