Opinion

IMedPub Journals
www.imedpub.com

Cellular & Molecular Medicine: Open Access
ISSN: 2573-5365

2025
Vol. 11 No. 1:3

CRISPR-Cas9 Mediated Gene Editing in Cardiomyocytes: A Novel Approach to Correct Inherited Cardiac Disorders

Hiroshi Yamamoto*

Department of Gene Editing and Regenerative Medicine, University of Tokyo, Tokyo 113-8654, Japan

***Corresponding author:** Hiroshi Yamamoto, Department of Gene Editing and Regenerative Medicine, University of Tokyo, Tokyo 113-8654, Japan; E-mail: yamamotohiroshi09@u-tokyo.ac.jp

Received date: February 01, 2025, Manuscript No. ipcmm-25-20876; **Editor assigned date**: February 03, 2025, Pre QC No ipcmm-25-20876(PQ); **Reviewed date**: February 19, 2025, QC No ipcmm-25-20876; **Revised date:** February 26, 2025, Manuscript No.ipcmm-25-20876(R); **Published date:** March 5, 2025, DOI: 10.21767/2573-5365.11.1.3

Citation: Yamamoto H (2025) CRISPR-Cas9 Mediated Gene Editing in Cardiomyocytes: A Novel Approach to Correct Inherited Cardiac Disorders. J Cell Mol Med Vol.11 No.1:3

Introduction

Inherited cardiac disorders, such as hypertrophic cardiomyopathy, long QT syndrome, and dilated cardiomyopathy, represent a significant cause of morbidity and mortality worldwide. These conditions often arise from single-gene mutations that alter cardiac muscle structure or disrupt electrophysiological signaling pathways. Conventional therapeutic interventions, including pharmacological management and surgical correction, primarily focus on alleviating symptoms rather than addressing the underlying genetic defects. The emergence of genome-editing technologies, particularly the CRISPR-Cas9 (Clustered Regularly Interspaced Short Palindromic Repeats–CRISPR-associated protein 9) system, has revolutionized biomedical research by offering precise, efficient, and relatively cost-effective methods for correcting pathogenic mutations at their source. Within the field of cardiovascular medicine, CRISPR-Cas9 holds remarkable potential to modify defective genes in cardiomyocytes the contractile cells of the heart thereby restoring normal cellular and functional integrity. This breakthrough not only provides a potential cure for hereditary cardiac diseases but also opens new avenues for understanding cardiac biology and developing personalized therapeutic strategies [1].

Description

The CRISPR-Cas9 system operates as a molecular scissor guided by a synthetic RNA molecule (guide RNA) that directs the Cas9 enzyme to specific DNA sequences. Upon recognition, Cas9 induces a double-strand break, which can be repaired through either non-homologous end joining or homology-directed repair, depending on the desired outcome. In cardiomyocytes, this technique allows for precise correction of mutations in genes encoding key structural or regulatory proteins, such as MYBPC3, TNNT2, or LMNA, which are commonly implicated in inherited cardiomyopathies. Recent advances in delivery systems, including adeno-associated viruses (AAVs) and lipid nanoparticles, have enhanced the efficiency and specificity of CRISPR-mediated editing in cardiac tissues. Moreover, induced pluripotent stem cells (iPSCs) derived from Patients somatic cells can be genetically corrected ex vivo using CRISPR-Cas9 and then differentiated into functional cardiomyocytes [2].

This approach provides a dual advantage: it serves as a model for disease mechanism studies and as a potential source for autologous cell replacement therapies. Despite its promise, several technical and ethical challenges must be addressed before CRISPR-Cas9 can be translated into routine clinical therapy for cardiac disorders. Off-target mutations, which occur when Cas9 cleaves unintended genomic regions, remain a major safety concern due to their potential to trigger oncogenic or deleterious effects. Furthermore, the efficient delivery of CRISPR components to adult cardiomyocytes is challenging, as these cells are terminally differentiated and less amenable to genetic manipulation [3].

Immune responses to Cas9 proteins and viral vectors also pose barriers to long-term therapeutic success. However, ongoing research into high-fidelity Cas9 variants, base and prime editing systems, and transient delivery strategies is gradually mitigating these limitations. Ethical considerations surrounding germline editing and the potential for heritable modifications continue to fuel debate, emphasizing the importance of strict regulatory frameworks and transparent clinical governance [4,5].

Conclusion

Mitochondrial dysfunction and oxidative stress are intricately intertwined processes that lie at the heart of many chronic and degenerative diseases. Their bidirectional relationship creates a self-perpetuating cycle of cellular damage that compromises energy metabolism, signalling, and survival. As research continues to unravel the molecular underpinnings of mitochondrial pathology, it is increasingly evident that targeting mitochondrial health represents a promising therapeutic frontier. Interventions that restore mitochondrial function, enhance antioxidant defence, and maintain redox homeostasis could transform the management of diseases rooted in cellular dysfunction.

Acknowledgement

None

Conflicts of interest

None

References

1. Zhang Y, Huang Y, Li Z, Wu H, Zou B, et al. (2023) Exploring natural products as radioprotective agents for cancer therapy: Mechanisms, challenges and opportunities. Cancers 15: 3585

2. Barjaktarovic Z, Schmaltz D, Shyla A, Azimzadeh O, Schulz S, et al. (2011) Radiation–induced signaling results in mitochondrial impairment in mouse heart at 4 weeks after exposure to X-rays. PLoS ONE 6: e27811

3. Cheema AK, Pathak R, Zandkarimi F, Kaur P, Alkhalil L, et al. (2014) Liver metabolomics reveals increased oxidative stress and fibrogenic potential in Gfrp transgenic mice in response to ionizing radiation. J Proteome Res 13: 3065–3074

4. Fischer N, Seo EJ, Efferth T (2018) Prevention from radiation damage by natural products. Phytomedicine 47: 192–200

5. Zhang PY (2015) Cardioprotection by phytochemicals via antiplatelet effects and metabolism modulations. Cell Biochem Biophys 73: 369–379