

Unlocking The Future Of Medicine: Crispr-Cas9 Gene Editing Holds The Key To Transformation

"I collected bones from charnel-houses and disturbed, with profane fingers, the tremendous secrets of the human frame. In a solitary chamber, or rather cell, at the top of the house, and separated from all the other apartments by a gallery and staircase, I kept my workshop of filthy creation." (Shelley, 1818)

Does this paragraph look familiar to you? It does come from Mary Shelley's one and only Frankenstein. Frankenstein serves as a cautionary tale that resonates with the ethical problems surrounding contemporary gene editing technology due to Victor Frankenstein's obsessive quest of scientific discovery and his manipulation of human remains to create life. Similar to how Victor explored the forbidden and collected bones from charnel homes; scientists today are delving into the complex field of genetic engineering, a groundbreaking technology of modern biosciences is making waves and capturing the imagination of all the scientists and public alike.

The CRISPR-Cas9 gene editing system, a ground-breaking development in biotechnology, enables scientists to precisely manipulate DNA. Laboratories now house state-of-the-art equipment where scientists painstakingly orchestrate genetic modifications at the cellular level, much like Victor's secluded chamber where he performed his "filthy creation." However, just as Victor's actions had unexpected consequences and moral conundrums, so too does CRISPR-Cas9's exceptional potential raise a number of concerns regarding the responsible and moral use of this revolutionary tool. The lessons from "Frankenstein" remind us of the need to use such power wisely as we navigate this bright new world of genetic manipulation, making sure that scientific curiosity is restrained by a profound grasp of the repercussions and a commitment to the welfare of humanity. CRISPR-Cas9, known as the revolutionary gene-editing tool has the ability to change how we think about genetics, medicine, and agriculture.

Genome editing is a type of genetic engineering in which DNA is deliberately inserted, removed, or modified in living cells. (Porteus, 2016) The name CRISPR (Clustered Regularly Interspaced Short Palindromic Repeat) refers to the unique organization of short, partially repeated DNA sequences found in the genomes of prokaryotes. CRISPR and its associated protein, Cas-9, is a method of adaptive immunity in prokaryotes to defend themselves against viruses or bacteriophages. (Asmaw & Zawdie, 2021) This technology conceals a simple yet

immensely powerful technique. CRISPR acts as a pair of molecular scissors, capable of precisely cutting DNA at specific locations. This ability allows scientists to either remove, insert, or modify genes within an organism's genetic code.

Figure 1

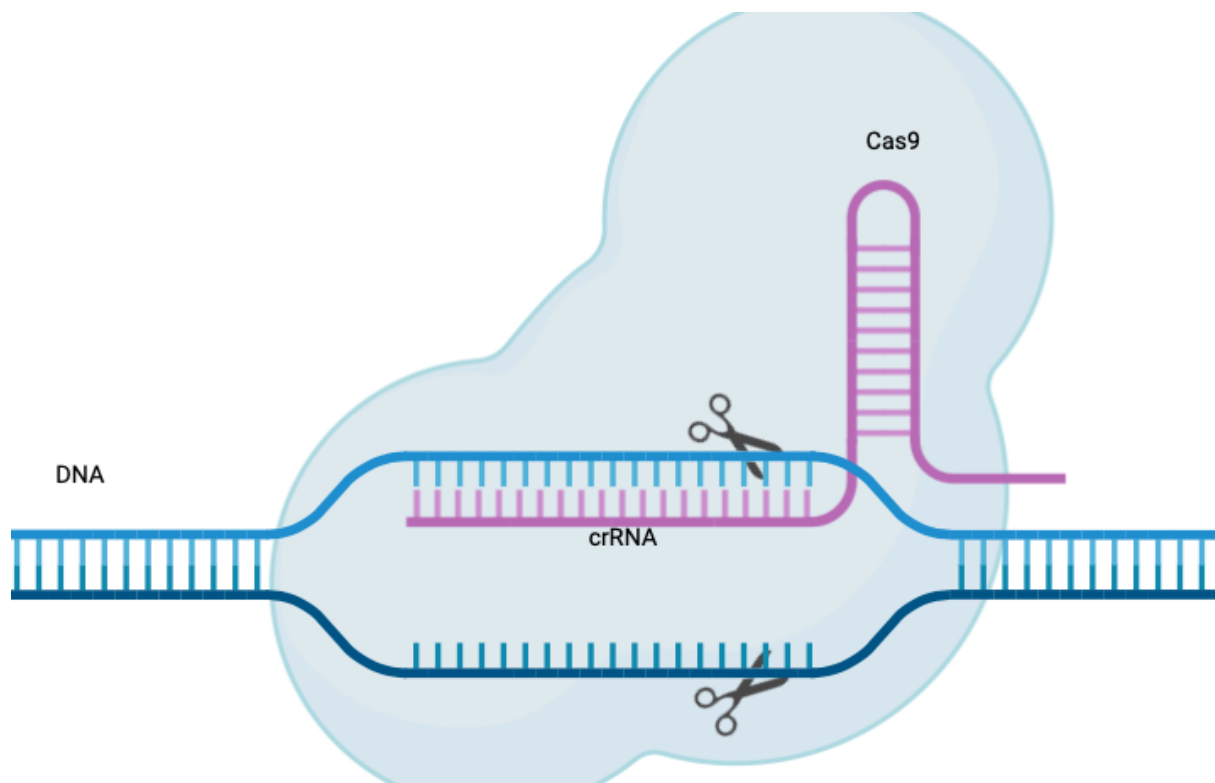


Figure 1: The diagram of how CRISPR-CAS9 mainly Works. Figure made with BioRender.

One of the most exciting prospects of CRISPR is its potential to treat genetic disorders. Imagine a future where conditions like sickle cell anemia or muscular dystrophy are no longer in the picture. CRISPR could be used to correct the genetic mutations responsible for these diseases, offering hope for many patients and their families. Researchers have made progress in recent years utilizing CRISPR to alter cancer cells to self-destruct by targeting them. This novel strategy might transform cancer treatment by providing a less intrusive and potentially more tailored treatment alternative. Sickle cell anemia is the major example that I want to give. Sickle cell disease (SCD) is an inherited monogenic disorder resulting in serious mortality and morbidity worldwide. (Demirci and friends, 2019) Rapid and substantial progress in genome editing approaches have proven valuable as a curative option given plausibility to either correct

the underlying mutation in patient-derived hematopoietic stem/progenitor cells (HSPCs), induce fetal hemoglobin expression to circumvent sickling of red blood cells (RBCs), or create corrected induced pluripotent stem cells (iPSCs) among other approaches. (Demirci and friends, 2019) The recent discovery of CRISPR/Cas9 has revolutionized genome engineering and opened up the prospect of making these ideas a reality with clinical significance. Here, we discuss CRISPR/Cas9 genome engineering approaches, highlighting their drawbacks and potential applications as a treatment for SCD. The process involves extracting stem cells from the patient's bone marrow, using CRISPR-Cas9 to edit the HBB gene to correct the mutation, and then reintroducing the edited cells back into the patient's body. The utilization of CRISPR-Cas9 in treating sickle cell anemia represents a compelling strategy to generate healthy red blood cells, thereby mitigating the debilitating symptoms of this genetic disorder.

Figure

2

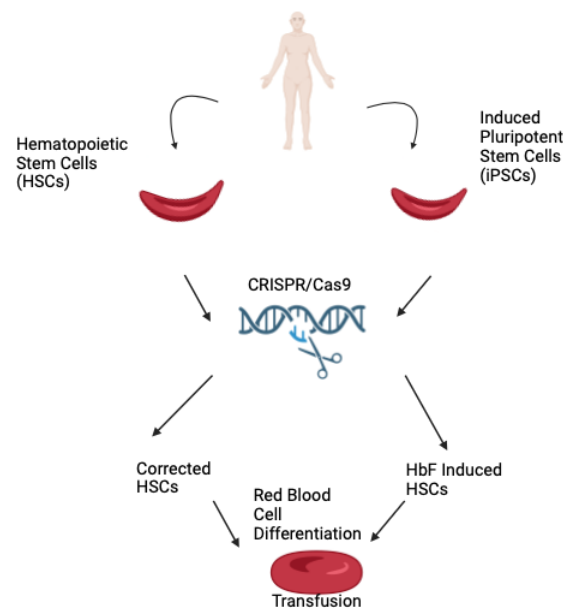


Figure 2: The diagram of the process of how SCD is treated with CRISPR-Cas9. Figure made with BioRender.

Early laboratory investigations and initial forays into clinical trials have demonstrated encouraging outcomes. These preliminary studies have shown the potential of CRISPR-Cas9 to precisely edit the genetic mutations responsible for sickle cell anemia, offering a glimmer of hope for patients and the medical community. However, it's imperative to emphasize that while these initial findings are promising, a comprehensive and rigorous process of further research and testing is essential to ascertain the safety, long-term viability, and overall effectiveness of this therapeutic approach. The complexities of human biology and the intricacies of genetic editing warrant thorough investigation before CRISPR-based treatments for sickle cell anemia can be considered a mainstream medical intervention. As scientists continue to refine and

validate this methodology, the trajectory of progress holds the potential to transform the lives of those afflicted by this challenging condition.

CRISPR-Cas9 has ushered in a new era of possibility and potential in the biosciences. The future of it is both exciting and uncertain. Its applications span from medicine to agriculture, offering solutions to some of humanity's most pressing challenges. However, as we tread further into this uncharted territory, it is imperative that we proceed with caution, guided by ethics and a shared commitment to the betterment of society. As regulations continue to evolve, scientists are pushing the limits of what this technology can achieve. Possibilities include the treatment of genetic disorders we once said untreatable, as well as breakthroughs in our understanding of complex diseases like Alzheimer's and HIV. CRISPR-Cas9 invites us to reimagine the boundaries of human ingenuity and invites us all to take part in shaping the future of science and medicine.

Futher Reading

Futher reading on the topic "*CRISPR-Cas9*"

- Hsu, P., Lander, E.S., Zhang, F. Development and Applications of CRISPR-Cas9 for Genome Engineering. *Cell*. 2014 Jun 5; 157(6): 1262–1278.
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- Bortesi, L., Fischer, R. The CRISPR/Cas9 system for plant genome editing and beyond. Elsevier Inc. 2015 Jan-Feb;33(1):41-52.
- Soriano V. Gene Therapy with CRISPR/Cas9 Coming to Age for HIV Cure. *AIDS Rev*. 2017 Oct-Dec;19(3):167-172. PMID: 29019352.
- Khlidj Y. What did CRISPR-Cas9 accomplish in its first 10 years? *Biochem Med (Zagreb)*. 2023 Oct 15;33(3):030601. doi: 10.11613/BM.2023.030601. Epub 2023 Aug 5. PMID: 37545694; PMCID: PMC10373057.

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3. Asmamaw, M., Zawdie, B. 2021. Mechanisms and Applications of CRISPR/Cas-9-Mediated Genome Editing.
4. Demirci, S., Leonard, A., Haro-Mora, J. J., Uchida, N., Tisdale, J.F. 2019. CRISPR/Cas9 for Sickle Cell Disease: Applications, Future Possibilities, and Challenges.
5. Demirci, S., Leonard, A., Haro-Mora, J. J., Uchida, N., Tisdale, J.F. 2019. CRISPR/Cas9 for Sickle Cell Disease: Applications, Future Possibilities, and Challenges.