Unlocking the future of medicine: CRISPR-Cas9 gene editing holds the key to transformation

Cansu Kizilkaya (Turkey)

The Biochemical Society identifies and celebrates outstanding science communication talent in molecular biosciences with its annual Science Communication Prize. Cansu Kizilkaya, from Turkey, won the second prize in the written category for students studying for A-level/T-level/BTEC National/Scottish Highers or equivalent qualifications. Cansu's piece is titled 'Unlocking the Future of Medicine: CRISPR-Cas9 Gene Editing Holds the Key to Transformation'.

Introduction from the author

In the timeless words of Mary Shelley's *Frankenstein*, we are reminded of the perils and promises of scientific exploration, where the pursuit of knowledge can lead to both awe-inspiring breakthroughs and moral dilemmas. In today's world, the echoing resonance of Shelley's cautionary tale finds a contemporary echo in the realm of CRISPR (clustered regularly interspaced short palindromic repeat)-Cas9, a revolutionary gene-editing technology. Just as Victor Frankenstein collected bones from charnel houses and delved into the forbidden secrets of life, modern scientists now manipulate the very building blocks of existence in laboratories reminiscent of his solitary chamber.

This essay explores the parallels between *Frankenstein* and CRISPR-Cas9, examining the ethical complexities and transformative potential of genetic engineering. As we venture further into the uncharted territory of genome editing, it is crucial to heed the lessons of the past and ensure that the power of scientific curiosity is tempered by a profound understanding of the consequences and a commitment to humanity's well-being. CRISPR-Cas9 represents a revolution in science, offering solutions to challenges in medicine, agriculture, and beyond. Yet, with this incredible power comes an equally incredible responsibility – to use it wisely and ethically in our pursuit of a brighter future.

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."[1] 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 ground-breaking technology of modern biosciences, it 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.[2] The name CRISPR refers to the unique organization of short, partially repeated DNA sequences found in the genomes of prokaryotes. CRISPR, and its associated protein, Cas9, is a method of adaptive immunity in prokaryotes to defend themselves against viruses or bacteriophages.[3] 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 remove, insert or modify genes within an organism's genetic code.

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.[4] 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.[4] 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 re-introducing 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.

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. Its future 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. ■

You can view all the winners from 2022 and 2023 on our website, including figures which accompany this article.

The Science Communication Prize will be back in summer 2024! Stay updated through our website and social media.

References

1. Shelley, M. *Frankenstein; or, The Modern Prometheus*. Finsbury Square, London: Lackington, Hughes, Harding, Mavor, Jones, 1818.
2. Porteus, M. Genome editing: a new approach to human Therapeutics. Annu. Rev. Pharmacol. Toxicol. 2016;56:163–90.
3. Asmamaw, M, Zawdie, B. Mechanisms and applications of Crispr/Cas-9- mediated genome editing. Biologics. 2021;15:353–61.
4. Demirci, S, Leonard, A, Haro-Mora, JJ. Crispr/Cas9 for sickle cell disease: applications, future possibilities, and challenges. Adv. Exp. Med. Biol. 2019;1144: 37–52.

Further Reading

- Hsu, P., Lander, E.S. and Zhang, F. (2014) Development and applications of CRISPR-Cas9 for genome engineering. *Cell*. **157**, 1262–1278. doi: 10.1016/j.cell.2014.05.010
- Bortesi, L. and Fischer, R. (2015) The CRISPR/Cas9 system for plant genome editing and beyond. *Biotechnol. Adv*. **33**, 41–52. doi: 10.1016/j.biotechadv.2014.12.006
- Soriano, V. (2017) Gene therapy with CRISPR/Cas9 coming to age for HIV cure. *AIDS Rev*. **19**, 167–172. PMID: 29019352.
- Khlidj, Y. (2023) What did CRISPR-Cas9 accomplish in its first 10 years? *Biochem. Med. (Zagreb)*. **33**, 030601. doi: 10.11613/BM.2023.030601

Author information

I entered the Science Communication Prize because I had a powerful belief that knowledge should be accessible to everyone in every age around the world. CRISPR-Cas9, with its transformative potential and ethical complexities, offered the perfect platform to bridge the gap between science and society, fostering understanding and engagement in this ground-breaking field of research.

