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Gene Editing Technologies: Crispr-Cas9 and Its Therapeutic Applications

Pratibha Sahu[1*]

***Corresponding Author E-mail: pharma.pratibha23@gmail.com**

[1]**SRSIP, Kumhari, Durg, Chhattisgarh, India**

ABSTRACT

The study looks into how CRISPR-Cas9 technology is developing and how it might be useful for therapeutic purposes and in biomedical science. A quantitative research design was used and information was collected from 200 people who are researchers, medical practitioners, genetic counselors and healthcare workers in India. It appears that people understand that CRISPR-Cas9 is precise and can improve genetic conditions, mainly in conditions that have hereditary causes and medical treatment based on personal needs. Also, participants shared that there are troubles with its clinical use such as side effects on cells other than the intended ones and ethics-related questions. The use of inferential statistics showed that people's ideas about ethics varied by profession and education level, proving the value of specific education and ethical responsibility. It is determined in the study that while CRISPR-Cas9 shows promise, its use in healthcare should proceed carefully by involving policy, special training and open conversations among the public.

Key Words:

CRISPR-Cas9, Gene Editing, Therapeutic Applications, Personalized Medicine, Ethical Concerns

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1.INTRODUCTION

Novel findings in molecular biology are changing the way medicine is practiced today [1]. One of the greatest technologies of this century is gene editing. Gene editing could make it possible to correct the causes of genetic illnesses which might give patients a way to be healed instead of only receiving treatment for their symptoms [2]. Researchers are now talking more about the value of the CRISPR-Cas9 system because of how precise, efficient and accessible it is. To truly value this new approach, we must learn about the science behind it and what it can do for medicine [3].

1.1.Background Information:

Genetic engineering moving so quickly has brought a true revolution to biomedical science and gene editing is the most important aspect of it [4]. An important advancement is the CRISPR-Cas9 system found in bacteria to help them resist viruses. Ever since it was developed as a gene-editing tool in 2012, most people have appreciated CRISPR-Cas9 for its simple operation, low cost and its exact pinpointing and adjustment of sequences in DNA [5]. With this method, scientists can add, remove or switch DNA parts at particular points in the genome which is helpful for correcting mutations that cause illnesses [6].

CRISPR-Cas9 is flexible, it has made it possible to develop new treatments for inherited diseases, cancer, viruses and several chronic illnesses [7]. CRISPR has proved very useful, from fixing problems with genes related to diseases like cystic fibrosis and sickle cell anemia to boosting immunotherapies for cancer therapies. The use of mesenchymal stem cells is also being considered for treating diseases of the nervous system, including Huntington's and Alzheimer's which emphasizes their wide clinical importance.

1.2.Statement of the Problem:

Still, using CRISPR-Cas9 in clinical settings deals with many technical, biological and ethical concerns [8]. One main problem is that off-target effects can result in wrong genetic changes that may harm the body's important genes. Patient safety and results for the long-term are at risk because of these issues. Besides, the issue of modifying gene laws in ways that can be passed down to future generations has led to disputes worldwide about whether such actions are right [9].

There are no standard delivery methods to make sure gene editing works efficiently on different kinds of cells. Additionally, the legal rules are unclear in many places which makes it difficult to take research into clinical use [10]. Because research in this field is swift, it is important to assess the helpful parts and the issues that stop us from using CRISPR properly in medical practice.

1.3.Research Objectives

The main goal of this study is to give a clear picture of CRISPR-Cas9 and assess what its use in medicine involves. The main objectives include:

1. To explore the origin, mechanism, and components of CRISPR-Cas9 technology, emphasizing its role as a gene-editing tool in biomedical science.
2. To analyze current therapeutic applications of CRISPR-Cas9 in the treatment of various human genetic disorders and diseases.
3. To examine the major technical challenges, risks, and ethical concerns associated with its clinical use.
4. To assess the future prospects of CRISPR-Cas9 in advancing personalized and precision medicine.

2. METHODOLOGY

The study thoroughly examines CRISPR-Cas9 technology and its medical applications by using an organized and planned method. The fast-changing process of gene editing, together with different opinions in biomedical and healthcare fields, makes it necessary to use reliable data for measuring knowledge, attitudes and perceptions. The study aims to get results that are statistically significant by examining responses from a clear sample and use those outcomes to influence research and policy in CRISPR-Cas9 technology.

2.1.Description of Research Design

This study aims to study professionals from biomedical science, healthcare and genetics by using a quantitative approach to assess their knowledge and opinions of CRISPR-Cas9 and its therapeutic uses. In order to determine conclusions from the research objectives, a descriptive survey method is applied to gather information that can be statistically analyzed.

2.2.Sample Details

A purposive sampling method was selected and 200 people were chosen from the population to make sure the study related to the topic. People from research, medical, genetic, biotechnology and healthcare fields took part in the event. According to the eligibility conditions, applicants should have at least a graduate degree in genetics or in biomedical sciences.

2.3.Instruments and Materials Used

This study collected data by using a questionnaire made especially for it. Participants were asked questions that were both closed-ended and on a Likert scale, all about four key topics:

1. Basic knowledge of CRISPR-Cas9 mechanisms.
2. Awareness of its current therapeutic applications.
3. Perceptions of associated risks and ethical concerns.
4. Expectations regarding its future use in personalized medicine.

A set of subject matter experts checked the questionnaire's validity and a small group tested it to guarantee it was clear and reliable.

2.4.Procedure and Data Collection Methods

The study participants were asked to get involved through email and the professional networks they belong to. The questionnaire was distributed online through Google Forms which made it easy and allowed a larger number of people from a wider area to participate. Those taking part in the survey were told about the aim of the study and promised that their information would be kept private and anonymous. Collection of data lasted four weeks and messages were sent out periodically to boost the rate of replies.

2.5.Data Analysis Techniques

Once all necessary data was collected, it was then coded, put into Microsoft Excel and finally transferred to SPSS version 26 to conduct the analysis. Statistics such as frequencies, percentages and means were employed to sum up the data. The Chi-square tests and ANOVA

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statistical methods were also used to analyze how demographic variables were linked to the participants' answers about the four research objectives. To identify whether the results were statistically important, the significance level was established as $p < 0.05$.

3. RESULTS

In this section, findings based on research using 200 participants' quantitative data are discussed which look at their knowledge, views and expectations of CRISPR-Cas9 and its medical applications. Descriptive stats help summarize the responses given by study participants, while inferential stats study the relationship of demographic details to the main research variables.

3.1 Demographic Profile of Participants

The demographic information of the 200 participants in the study on CRISPR-Cas9 is shown in the following table 1. People are sorted by gender, their profession and what level of education they have. The table is intended to show the variety and importance of the group involved, since every member was chosen from the biomedical sciences field.

Table 1: Demographic Profile of Participants

Demographic Variable	Categories	Frequency (n)	Percentage (%)
Gender	Male	110	55.0
	Female	90	45.0
Profession	Researcher	60	30.0
	Medical Practitioner	50	25.0
	Genetic Counselor	40	20.0
	Biotechnology Student	30	15.0
	Healthcare Professional	20	10.0
Education Level	Graduate	50	25.0
	Postgraduate	100	50.0
	Doctorate	50	25.0

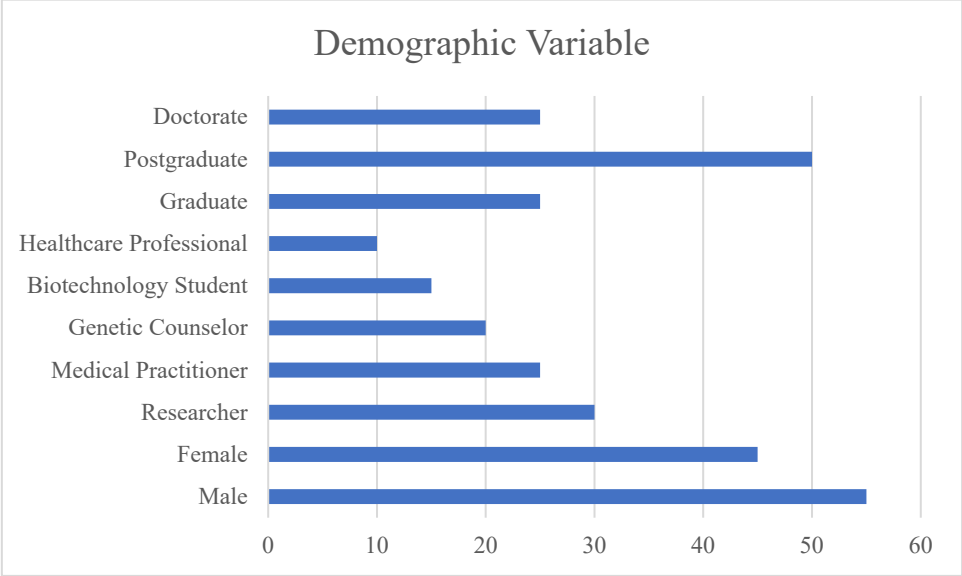

Figure 1: Graphical Representation of Demographic Profile of Participants

The data demonstrates that the sample is gender-balanced, since males and females each comprise 55% and 45% of the group. With regards to their professional backgrounds, researchers were the most numerous group (30%), followed by medical practitioners (25%) and genetic counselors (20%), making sure different biomedical specialties were heard. 15% of the answers were from biotechnology students and healthcare professionals provided 10% of the feedback. Half of the surveyed audience held a postgraduate degree, among them both graduate and doctorate degree holders represented 25% each. Such of spread demonstrates that the researchers are qualified, adding value and reliability to the data about awareness and opinions about CRISPR-Cas9.

3.2 Awareness and Knowledge of CRISPR-Cas9 Technology

The information presented in table 2 reveals participants' views about CRISPR-Cas9 technology. They are also asked to share their ideas about how accurate the gene-editing procedure is, how helpful it may be for fixing faulty genes and if it is thought to be used only in laboratories. People were asked to respond using the three options: Agree, Neutral and Disagree.

Table 2: CRISPR-Cas9 Knowledge Overview

Knowledge Statement	Agree n (%)	Neutral n (%)	Disagree n (%)
CRISPR-Cas9 is a precise gene-editing technology.	156 (78%)	24 (12%)	20 (10%)
CRISPR-Cas9 can correct genetic mutations in diseases.	148 (74%)	30 (15%)	22 (11%)
CRISPR-Cas9 is limited to laboratory research only.	42 (21%)	38 (19%)	120 (60%)

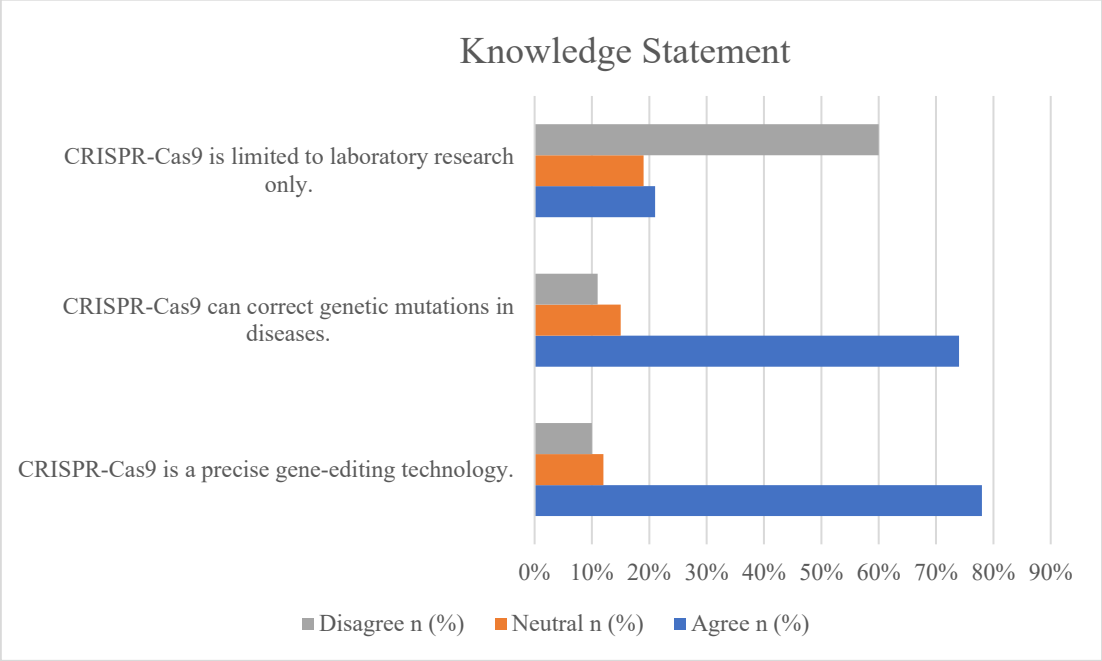

Figure 2: Graphical Representation on CRISPR-Cas9 Knowledge Overview

The study demonstrates that people recognize the important abilities of CRISPR-Cas9. Some 78% and 74% of the respondents agreed that CRISPR-Cas9 is a precise method of changing genes and that it is important in correcting genetic problems. A total of 60% of the group disagreed that CRISPR-Cas9 only applies to laboratory science and appeared to know about its progress in clinical and therapy areas. This proves that the medical experts studied are informed about how CRISPR technology is being and could be applied in the field of medicine.

3.3 Perception of Therapeutic Applications

In table 3, you can see how participants think about the use of CRISPR-Cas9 technology in therapy. It discusses what role it may play in treating hereditary diseases and how much it is applied by doctors today. People's views were grouped as Agree, Neutral or Disagree.

Table 3: Perception of CRISPR-Cas9 Therapeutic Applications

Therapeutic Application Statement	Agree n (%)	Neutral n (%)	Disagree n (%)
CRISPR-Cas9 has potential to treat hereditary diseases.	140 (70%)	36 (18%)	24 (12%)
CRISPR-Cas9 is widely used in clinical settings today.	56 (28%)	44 (22%)	100 (50%)

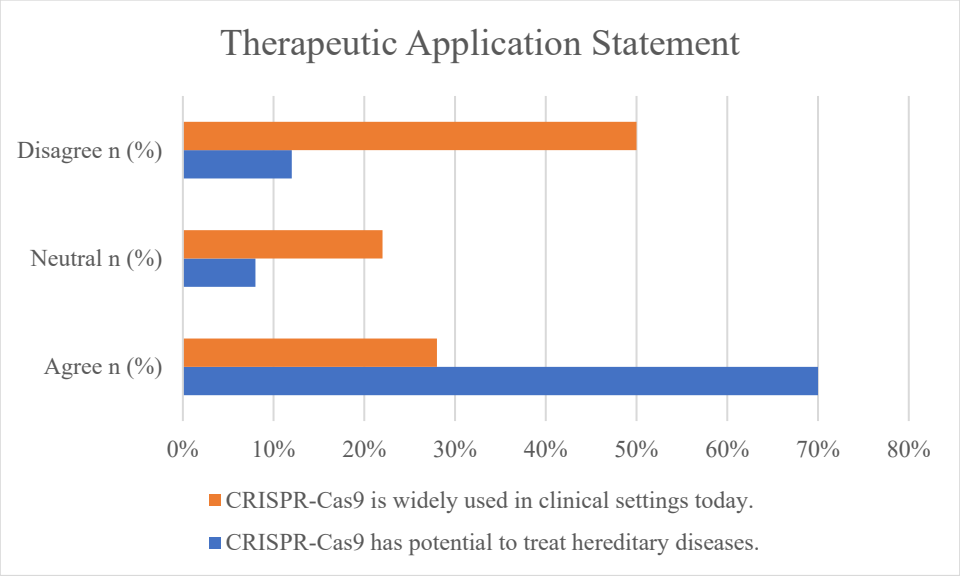

Figure 3: Graphical Representation on Perception of CRISPR-Cas9 Therapeutic Applications

About three out of four participants (70%) agreed that CRISPR-Cas9 is likely to treat hereditary diseases which shows there is hope and knowledge about its importance in genetics. Just 28% see it as a tool that is widely practiced in medicine and 50% do not agree with this. It means most believe CRISPR-Cas9 can offer big healthcare breakthroughs, although it is only in its research and limited application phase. The study points out that what people think future science will involve is different from what is being used in practice nowadays.

3.4 Perceptions of Risks and Ethical Concerns

Participants' opinions on major risks and ethical topics connected to CRISPR-Cas9 are shown in table 4. In particular, it looks at how off-target changes can happen and considers the role of ethical issues in using it for patients. People are divided into three groups: those who agree, are neutral or disagree.

Table 4: Ethical and Risk Views on CRISPR

Risk and Ethics Statement	Agree n (%)	Neutral n (%)	Disagree n (%)
Off-target effects are a major challenge in CRISPR use.	164 (82%)	22 (11%)	14 (7%)
Ethical concerns limit the use of CRISPR in humans.	150 (75%)	30 (15%)	20 (10%)

It is clear from the data that people involved are aware of the problems and ethical concerns related to CRISPR-Cas9. The majority of respondents (82%) pointed out that off-target causes are a major issue, showing they understand what the technology needs to improve. Even more, three-fourths of people acknowledged that ethical issues prevent the CRISPR technique from being used in human medicine. It is apparent from these responses that people are aware of the benefits of CRISPR-Cas9 and are also concerned about issues that come with it.

3.5 Expectations for Future Use

Participants expect that CRISPR-Cas9 will be a key technology for personalized medicine in the upcoming years as shown in table 5. The responses say if individuals consider CRISPR-Cas9 to be an important advance in personalized medicine.

Table 5: Expectations for CRISPR-Cas9 in Personalized Medicine

Future Prospects Statement	Agree n (%)	Neutral n (%)	Disagree n (%)
CRISPR-Cas9 will revolutionize personalized medicine.	170 (85%)	20 (10%)	10 (5%)

The results show that more than 85% of the respondents think that personalized medicine will experience a major revolution through CRISPR-Cas9. Out of the students, 10% said they were neutral and only 5% opposed the view. Because a large majority believe that CRISPR-Cas9 can help tailor treatments to a person's genes, it is seen as a foundation for the future of healthcare.

3.6 Inferential Statistical Analysis

The table displays the outcomes of Chi-square and ANOVA tests performed to check whether there are any associations or differences among the participants' replies. The Chi-square was used to investigate if knowing about CRISPR-Cas9 is related to a person's profession and ANOVA evaluated differences in ethics according to the level of education. It was decided that $p < 0.05$ would be a statistically significant result.

Table 6: Inferential Analysis of Awareness and Ethical Perception Variables

Test Type	Variable Tested	Test Statistic	Degrees of Freedom	p-value	Interpretation
Chi-square (χ^2)	Awareness by Profession	12.68	4	0.013*	Significant association between profession and awareness
ANOVA	Ethical Concerns Perception by Education Level	4.52	2, 197	0.012*	Significant difference in ethical concerns among education levels

***Significant at $p < 0.05$**

The Chi-square test points out that there is a significant link between profession and knowing about CRISPR-Cas9 ($\chi^2 = 12.68$, $p = 0.013$), revealing that some groups such as researchers and medical practitioners know more than others. It was also seen on the ANOVA test that there was a significant distinction in ethical concerns based on education level ($F = 4.52$, $p = 0.012$), as doctorate holders showed more concern than graduates and postgraduates. According to these results, what someone learned before using CRISPR-Cas9 can guide what they think and know about the approach. It appears that biomedical and healthcare professionals in general are aware and favor the use of CRISPR-Cas9 technology. It is clear that people think highly of

gene therapy, but questions about its off-target effects and ethics are still widely considered. Because people in different sectors view and understand this technology differently, it is necessary for more targeted talks about education and policies as the technology advances.

4.DISCUSSION

This part critically studies the leading findings from the study, compares them to what is already known, illustrates how they can affect the field and policies and suggests possible future directions for research.

4.1. Interpretation of Results

The research demonstrates that a majority of biomedical and healthcare professionals in India have knowledge about CRISPR-Cas9. Most people understood that using CRISPR-Cas9 can correct errors in DNA and contribute to medicine. But while most scientists expressed optimism that it could address hereditary diseases (70 percent), only a quarter (28 percent) felt it is broadly used in real treatments which indicates that theory and practice do not always match.

A lot of participants were also concerned about ethics and safety issues. Most scientists (82%) saw that off-target reactions are a main issue in genomics and three-quarters (75%) felt that ethical issues make it difficult to apply genetic research to humans. They show that doctors should handle this new technology thoughtfully since it still has some drawbacks.

Further review indicated that professionals differed in their knowledge and researchers and doctors had the highest scores. Doctorate holders also mentioned ethical issues more often than others which suggests that how much one has learned and practiced influences views on CRISPR-Cas9.

4.2. Comparison with Existing Studies

It demonstrates the similarities and differences between this study's conclusions and similar conclusions drawn from previous studies about CRISPR-Cas9, mostly concerning its use in medicine, ethics, the clinic and education.

Table 7: CRISPR-Cas9 Insights – Present vs. Past Studies

Study	Focus Area	CRISPR Components / Delivery	Key Findings	Relevance to Present Study
Rein, Yang & Chao (2018) [11]	Cellular therapies	Gene editing in hematopoietic stem cells	Demonstrated clinical potential of gene editing in immune-based treatments	Supports observed optimism among respondents about CRISPR's future clinical role
Uddin, Rudin & Sen	Therapeutic applications	General CRISPR-Cas9	Discussed ethical, safety, and	Corroborates concerns raised by participants about

(2020) [12]	and limitations	application framework	delivery challenges	off-target effects and ethics
Xu et al. (2019) [13]	CRISPR delivery systems	Nanoparticles, viral vectors, electroporation	Identified delivery as a key barrier to therapeutic use	Aligns with participant view that CRISPR is not yet widely used clinically
Zhang et al. (2021) [14]	Cancer therapy and diagnostics	CRISPR in gene knockout and detection assays	Demonstrated success in oncology applications	Reinforces participant belief in CRISPR's future role in personalized medicine
Zhang, Li & Jin (2024) [15]	Disease modeling and gene therapy	CRISPR-based human disease models	Emphasized CRISPR's role in precision medicine and translational research	Echoes high agreement among respondents on revolutionizing personalized healthcare

4.3. Implications of Findings

The implications of the research will affect the development of gene-editing in India. First, because CRISPR-Cas9 is seen to have great promise in personalized medicine, professionals are eager to use it once the necessary approval is granted. In addition, because people's knowledge and ethics vary a lot by their education and career, custom programs, workshops and curriculum updates should be designed to ensure all users are responsible.

Furthermore, many people believe CRISPR will shape the future of personalized medicine (85%), so policy makers and healthcare planners have a solid reason to improve their infrastructure, develop regulations and work on involving the public to ensure its ethical application.

4.4. Limitations of the Study

This research is strong in using numbers, but it still has some weaknesses. Even so, using the purposive sampling method helps gather information from relevant people, but this kind of sampling makes it difficult to generalize the findings for the general public. Third, getting data from people themselves may lead to biased replies, especially when dealing with controversial issues. Furthermore, the study did not look into people's continuing adjustments of their attitudes after actually encountering CRISPR research and its outcomes.

4.5. Suggestions for Future Research

Future research ought to concentrate on qualitative research to study the deeper ethical consequences and emotions that gene editing generates. Researchers can use longitudinal

studies to see how people's opinions change as CRISPR goes from testing to being used in patients. Moreover, conducting comparative studies between cultures may help detect regional differences in how aware people are and their ethical views. In addition, involving both patients and members of the general public in studies would help understand the society's overall preparedness for gene-editing technologies.

5. CONCLUSION

In this section, the main results of the study are explained, its wider importance is emphasized and suggestions are given for future integration and ongoing research on CRISPR-Cas9.

5.1. Summary of Key Findings

The study reviewed CRISPR-Cas9 technology and its clinical uses by talking to 200 experts involved in biomedical and medical care in India. The study reveals that most people are aware of CRISPR-Cas9's high precision and its ability to fix genetic flaws. Although most are positive about its role in hereditary diseases and personalized care, there is agreement that its medical use is still underdeveloped. Significant concerns were shown about using them in the wrong place and the ethical issues, mostly by doctorate holders and some professional groups. It was found through inferential statistical analysis that knowledge and perception related to the technology are affected by both a person's profession and education level.

5.2. Significance of the Study

The research adds key evidence to the gene editing discussion by looking at a professionally significant group from India. It points out that scientists are both encouraged and worried by the use of CRISPR-Cas9 in clinical and research fields. The outcomes confirm that proper use of gene-editing relies on specialized learning, awareness of ethics and being actively involved in policy. In addition, the similarity of this study's outcomes to previous findings shows that biomedical researchers everywhere consider it trustworthy and relevant.

5.3. Final Thoughts or Recommendations

The wider use of CRISPR-Cas9 in medicine will largely depend on achieving advancement in science, winning public trust and having clear ethical guidelines and responsible laws. To overcome knowledge gaps and deal with ethical matters, institutions need to make sure interdisciplinary training and consult with stakeholders are their first priorities. Those who make health policies should coordinate with scientists and doctors to ensure the guidelines support new treatments without increasing risks. Research should also focus on various groups, track individuals over a long time and involve patients to make sure gene-editing technologies benefit all parts of the population.

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