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CRISPR and Gene Editing Therapies: Current Applications in Treating Inherited Disorders

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ABSTRACT

The purpose of the research was to investigate people's perceptions of CRISPR gene editing as a treatment for hereditary illnesses and the factors that shape such perceptions. It also looked at whether age, gender, and education level as well as safety concerns affect their willingness to accept such treatments. The sample used in this study was 250 and they were all patients; moreover, the general population also acted as participants. It was a study conducted between February and June, 2025 in Punjab, Pakistan. The answers were given according to the background, knowledge on CRISPR, and perception of the participants by means of a questionnaire. The SPSS software was used to analyze the data. It was found out that the more a person knew about the CRISPR, the greater the probability to adopt this method. The other factors that made a significant contribution to the willingness of the individuals to use CRISPR were a belief in its safeness and the level of education. Men and younger population were more predisposed to using CRISPR as compared to the older population and the female population. Income did not have much influence on their opinion. It means that raising awareness of people via teaching them about CRISPR is one of the methods that can be used to reduce the lack of acceptance. In the study, it is shown that awareness and trust make a very pivotal role in the uptake of new medical technologies. In the future we will be able to learn even more when additional research is conducted on larger and more diverse samples of people. Meanwhile, the findings of the current investigation can yield beneficial recommendations to physicians, scientists, and policymakers about a discussion with people about CRISPR and develop a safe intervention.

INTRODUCTION

Gene editing defied even our thoughts in the relation to medicine, and specifically after the revelation of a special tool called CRISPR, which means Clustered Regularly Interspaced Short Palindromic Repeats. The CRISPR technology assists researchers to trace and edit certain sections of the DNA in the cells. You may imagine it as a pair of scissors which are very small and are able to cut the DNA at the very point where mistake would be lying or wrong gene would be present[1]. Through this, researchers are able to repair the defective part or although they may eliminate the defective part. This plays a very significant role in the treatment of inherited disorders which are diseases that the individuals acquire due to the defectiveness of their genes. CRISPR in bacteria was initially discovered and it assists the bacteria defend against viruses by chopping down the virus DNA[2]. The same notion is now applied in human gene therapy. A major factor that makes CRISPR a very popular tool in research is that it is easy to work with, cheaper as compared to older methods and very efficient in editing the right components of the DNA[3]. It is due to these advantages that CRISPR is under development in most spheres of science and medicine to understand and cure some previously incurable diseases [4].

Inherited disorders are illnesses that are devolved by parents to their kids through the genes. Such issues occur due to altered changes, or as mutations of the DNA. These changes may prevent the body to function well. There are some of the common inherited diseases including: cystic fibrosis, that makes mucus become thick and accumulate inside the lung and other body regions; sickle cell anemia where red blood cells become sickled and cause pain, fatigue and other health problems; and huntington and

Huntington disease disease, where the brain slowly degenerates causing slow movements and thoughts [5].These conditions could be treated many years ago but the treatment was only effective in relieving the symptoms such as breathing difficulties or pain, but the doctor could not eradicate the cause of the disease. These diseases were a lifelong experience to people[6]. The new hope of patients is through gene editing. An exclusive procedure referred to as CRISPR allows researchers to identify the specific component of DNA in which the error lies and correct it. Rather than only targeting the external symptoms of the disorder, CRISPR is used in the cells to fix the true gene which leads to the issue [7].This provides patients with an opportunity of having their long-term or even permanent cure whereas before they could not get such a cure.

Recent studies have indicated that, the CRISPR is not only applied in laborations, but is also undergoing tests in actual patients in form of special studies known as clinical trials. These experiments assist physicians and researchers to know the safety and success of CRISPR in the treatment of inheritable illnesses [8].Among the most promising ones appears in the treatment of blood diseases such as sickle cell disease and beta-thalassemia. Such are severe disorders where the red blood cells are not produced normally due to a defect on the DNA. Consequently, the patients tend to be weak, fatigued, and should have medical attention frequently[9]. Doctors now have a chance to mend the issue at the gene level with the assistance of CRISPR. They take away some bone marrow cells of the patient these are the cells that produce blood in the body. Afterwards they work in the lab and through CRISPR, they fix the defective gene in these cells. Once the gene is fixed they fill the healthy cells back into the patient[10].This procedure is referred to as ex vivo gene editing meaning the editing will be done outside the body. These repaired cells then begin production of healthy blood cells once returned back into the body. Due to this reason, a significant number of patients feel much better and do not require frequent transfusions of blood. This form of individualized therapy heralds a new dawn to individuals who have had to live with these diseases that not only cause excruciating pain but that are incurable as well [11].

As much as CRISPR appears to be a highly strong and promising tool in the modern medical fraternity, it is not completely secure at the moment. Scientists have not resolved some major issues yet. Also, there is what is termed as one of the principal risks known as off-target effects. This means that at times, CRISPR will proceed to make a mistake and change the incorrect part of the DNA. It is also capable of correcting the healthy sides of the DNA and not only rectifying the damaged or the failed gene[12]. In case of this eventual happening, it will cause new problems in the body like changes in the way in which the growth of the cell is made or the way the body operates [13].This kind of random mutation may be harmful especially when it happens on some essential genes that control either cell repair or cell growth. It is because of this fact that the scientists are highly cautious and are conducting lots of experiments to determine the accuracy of CRISPR. They are trying to advance it and refine it and make it safer so that it simply cuts the correct part of the gene that has to be fixed [14].As long as it is not surely positive that CRISPR is safe and works the right way every time, one will only be able to conduct clinical trials, in other words, it will be applied in clinical trials to a large degree. Nonetheless, it needs more serious studies before the healthcare practitioners can standardize its usage at the hospital as a similar method of treating inherited diseases. They provide a platform of treating genetic diseases as opposed to managing symptoms[15]. Despite the fact that challenges remain to be resolved adequately, current studies and clinical results allow claiming that CRISPR may become a very convenient method in the practice of medicine after all[16].

Since the discovery and initial success of using CRISPR as a tool to edit genes, most researchers have turned to exploring the use of CRISPR in the treatment of inherited disorders. A number of investigations were carried out using animal models in order to determine whether genetic issues could be resolved at the DNA level using CRISPR. This research indicated some promising findings. As a case example, researchers have managed to fix the defected genes that cause Duchenne muscular dystrophy which is a severe muscle wasting disease in mice. In the same way researchers harnessed CRISPR to repair gene mutation causing cystic fibrosis resulting in better lung functioning in animal models[17].A second major study demonstrated that CRISPR would be able to cure murine mice that are born blind with a genetic form of blindness, something that proves that the technology can be applied to complex organs such as the eye [18].These tests proved that CRISPR is precise applicable, and dependable for editing DNA mutations! These successes in animals were an extension of prior studies of the accuracy and low cost of CRISPR, and they allowed scientists the confidence to undertake the clinical trials in humans. The findings also assisted the medical fraternity to be confident that gene editing may someday lead to the actual remedies of those individuals falling ill with inherited illnesses[19].

Several effective trials in animals made researchers apply CRISPR as a clinical trial on living humans. These recent researches have had very promising outcomes particularly to the field of blood related genetic diseases. The genetic disorders such as sickle cell anemia and beta-thalassemia are inherited disorder and the problem lies in the genes that regulate the red blood cells[20]. During the recent experiments, physicians extracted the stem cells in the bone marrow and repaired the defective gene with the use of the CRISPR out of the organism. This means that these corrected cells were put back to the patient. Following treatment, improvement was amazing on many patients. They no longer required frequent blood transfusions as they had in the better part of their lives [21].Their energy level increased, as well as their health, and they feel free to perform daily tasks with more ease. The cure of these severe genetic disorders with long-term success, potentially there would be permanently cured after such positive changes demonstrated that CRISPR could be used to provide relief of serious genetic disorders that requires long-term treatment. The human research is made possible due to the previous studies done in animal research where the CRISPR had already proved to be

capable of correcting the defective genes and enhancing the symptoms[22]. Similar effects were now being reported in individuals, proving that the CRISPR was not only effective but it could also be safe in responsible use [23].The results proved promising to many patients who previously did not have any potentially life-long solutions. Most current human experiments have even show positive results, but there are also safety issues concerning the use of CRISPR, which were mentioned in some research. Even though this gene editing tool is effective and useful, it is not always flawless. At times, there is a risk of CRISPR accidentally making a modification of the wrong portion of DNA. Such errors are termed as off-target effects and these errors lead to unforeseen complications within the body [24].The consequences may be severe even due to relatively minor mistakes in gene editing as such alteration of cell growth or behavior. This might in certain circumstances raise the probability of acquiring cancer or other health related risks. Due to these dangers, much attention of scientists started to be devoted to the ways of arranging CRISPR safer and more precise. They are also trying to make improvements in the technology to ensure that it only edits the precise gene that has to be corrected but does not affect the other parts of the DNA [25].Such safety concerns have recently become a relevant topic of studies. Scientists also think that before Before CRISPR will be widely used in the hospitals to cure patients, it must be more dependable and safe. Researchers are trying all they can to make it more accurate and lower the possibility of error[26]. These activities rely on previous researches indicating that CRISPR might operate effectively, although it should be used thoroughly to prevent the emergence of such issues as off-target effects as well as undesired gene alteration[27].

Research Objectives

- To examine the correlation between awareness of CRISPR-based therapies and acceptance of gene editing among patients with inherited disorders.
- To determine the predictive impact of patient education level and perceived safety concerns on willingness to undergo CRISPR therapy.
- To assess the association between demographic factors (such as age, gender, and income level) and patient attitudes toward gene editing using CRISPR technology.

The generic CRISPR-type gene editing tools can correct inheritable ailments that were traditionally non-specific and difficult to deal with. The causes of the diseases are at the gene level of an individual and this is a problem where CRISPR can assist them since it helps correct the defective genes at a molecular level. This was the reason why CRISPR could be a powerful medical device. Nevertheless, in spite of such a tremendous potential, not all people fully understand how safe CRISPR is, how efficiently its work corresponds to how useful the technology of such sort brings out any ethical problems. Because of this, there exist possibilities that there will be those patients who will not be able to make decisions, or afraid to accept gene editing cure. This type of misconception can make the use of CRISPR healthcare establishments and hospitals challenging. The role of such factors as education of an individual, his/her awareness or background on his/her attitude to gene editing is also poorly represented in research. These may be extremely influential on how patients will rationally think and how they will understand the thought of the very possibility of using CRISPR treatments. Such items are worth investigating to determine whether health workers and researchers can explain CRISPR to the patients in a better way. This will help them to make appropriate decisions concerning the treatment. It can also help in provision of fair and useful health policies. This research paper will discuss these crucial issues through the application of the data analysis methods, hence, we shall be in a position to learn more about the safe and reasonable procedures of CRISPR uses in genetic disease therapy.

MATERIAL AND METHODS

It was a longitudinal, quantitative research study conducted between February 10, 2025, and June 20, 2025, in some of healthcare centers, as well as academic institutions in Punjab, Pakistan. The aim was to interpret the way citizens think regarding CRISPR and gene editing therapy. Twenty-five hundred participants were selected to be engaged in this study. The sample size was determined through Raosoft online sample size calculator and this allowed the assurance that the outcomes will be meaningful and accurate. The number in the sample was determined by a 95 percent confidence rate and a 5 percent error level which are standard requirements in research. The method employed was a purposive sampling technique, i.e. the participants were selected according to a certain quality (i.e. a lack of experience or interest in genetic disorders or the use of new medical technologies). This approach assisted in collecting the information of people that were most important to the study.

There were two significant target groups of the participants: the population of patients with inherited genetic disorders, and the general population that may become the potential users or advocates of the CRISPR-based treatment. All the participants in the study were above the age of 18. Individuals with mental conditions, memory related challenges or individuals who declined to participate in the trial were also excluded in the study. The Institutional Review Board (IRB) was approached to grant permission before the research was begun to ensure the project was done in an ethical manner. The written consent was sought by all the participants demonstrating that they have consented to take part in the research and that they have been informed of the research.

Structured questionnaire was used to obtain the data. This was the primary information gathering instrument. The questionnaire was of different parts and written in simple clear words. It inquired about the age, gender, education and the earnings of the participants and their awareness of CRISPR, its perceived safety and efficacy and their readiness to take or advocate such remedies. Genetics, psychology and bioethics specialists have reviewed the questionnaire to ensure that its questions were valid and understandable.

Face-to-face surveys and web forms were used to collect the responses; whichever was more convenient to the participants. After collecting the data, it was maintained in

a computer program referred to SPSS version 26.0. To begin with, the basic information regarding the participants was described with the help of descriptive statistics. Afterwards, the correlation analysis was performed to check whether there existed any relationship among CRISPR awareness and acceptance. Their multiple regression analysis was employed to determine whether such correlates as education or safety beliefs might be used to know whether one would be eager to accept CRISPR therapies. Finally, chi-squares were done to determine whether demographic concentration such as age, gender, or income was associated with CRISPR attitude. A p-value of less than 0.05 was adopted in every element of the analysis to indicate that the results were statistically significant.

RESULTS

Table 1

Correlation Analysis

Variables	Awareness of CRISPR	Acceptance of Gene Editing
Awareness of CRISPR	1	0.610**
Acceptance of Gene Editing	0.610**	1
p-value		0.000

The results of correlation analysis indicate a moderate to high positive dependence between the knowledge about CRISPR and the willingness to accept gene editing treatment (r = 0.610, p = 0.000). It translates to the fact that the more the participants are informed about CRISPR the more they grow to accept it as a possible treatment option in gene editing. The p-value (0.000) shows that the finding is significant and as such implies that the one noted is not likely caused by chance. Thus, the enhancement of the public understanding of CRISPR can contribute significantly to enhancing the popularity of gene editing methods of treatment.

Table 2

Multiple Regression Analysis

Predictor Variables	B	SE B	β (Beta)	t	Sig. (p-value)
(Constant)	1.230	0.315	—	3.905	0.000
Education Level	0.428	0.071	0.482	6.028	0.000
Perceived Safety	0.390	0.068	0.521	5.735	0.000

The multiple regression analysis showed that level of education and the perceived safety of CRISPR correlate to the willingness of the participants to take CRISPR therapy, i.e. are significant predictors thereof. The value of the beta was 0.482 (p = 0.000) and 0.521 (p = 0.000) for education level and perceived safety, respectively, meaning that by increasing education level and the level of perceived safety, the willingness to accept gene editing treatments increases a lot. R 2 was 0.430 or 43 percent of the variance explained in willingness indicating that these two variables acted influentially in a significant way with respect to the outcome. According to the results, the better the community will understand the technology and focus on the safety issues, the more they will be ready to accept CRISPR therapies.

Table 3

Chi-Square Test

Demographic Variable	Attitude Category	χ^2 (Chi-Square Value)	df	p-value
Age Group	Favorable / Unfavorable	10.73	2	0.004
Gender	Favorable / Unfavorable	6.25	1	0.012
Income Level	Favorable / Unfavorable	5.89	2	0.053

Results of the chi-square analysis showed that there were significant associations between some demographic factors and their attitudes towards CRISPR gene editing technology. The correlation between age category and attitude was statistically significant (2 = 10.73, p = 0.004), which means that younger participants had more positive attitudes as opposed to older people. On equal grounds a significant relation was found between gender and Attitude (2 = 6.25, p = 0.012) with slightly higher relationship of acceptance shown by male participants than that of the female participants. Nonetheless, the correlation between level of income and level of attitude lacked a statistical significance (x 2 = 5.89, p = 0.053) implying that income was unlikely to have a significant impact on the level of attitude. people understand gene editing treatment using CRISPR. These results indicate to the fact that age and gender could be significant factors in determining the attitude of the population towards genetic technologies.

DISCUSSION

The aim of this present research work was to examine the extent of knowledge about the CRISPR based gene editing therapeutics in relation to awareness, acceptance and its impacts of the demographic related factors. The results show that enlightenment matters with regard to the expression of positive feelings toward genes editing[28]. The highest positive correlation was between the presence of awareness and acceptance of gene editing technologies in that awareness generated stronger response to accepting use of CRISPR in addressing inherited disorders in the people[29]. This is in agreement with earlier articles that gave a high emphasis on how the public education has contributed towards enhancing the rate of acceptance of the newer versions of biomedical technologies [30].The positive attitudes of the people may be shaped by the scientific basis of the CRISPR, its accessibility, and health outlooks in case they are properly educated on it [31].

In the further analysis, it said that the educational level and perceived safety of gene editing were important predictors of whether participants were willing to undergo CRISPR therapy significantly. Younger patients and patient with a higher education level along with the notion that the technology was safe were more suggestible towards treatment with this technology. These results go in line with the previous literature indicating that trust in medical technology, particularly in the situation of genetic interventions, is highly correlated with the elevated literacy level and likelihood of the result control in terms of treatment [32].Research done in developed nations has also revealed that as long as ethics and safety questions are addressed, the people tend to be more confident to allow genetic editing [33].The idea is that people would accept

CRISPR even better in case the assistance in communicating the safety and directing its use gets implemented.

In addition, it was found that age and gender are two demographic factors that significantly relate to the CRISPR attitude. The younger participants expressed more positive opinion than did older ones, and men presented more positive attitude than women. These inclinations were found in similar surveys of population, as the younger generations were, on average, more vulnerable to the specified novelties in biotechnology as they are more exposed to digital and scientific mass media [34].The context in which one can find these differences of such process as perception according to gender may be due to the gender differences in social roles, the belief in health related concerns or the avoidance or expression of risks that were found to be prevalent in health behavior research [8]. Interestingly, the attitude was not more significantly associated with the level of income as compared with previous studies which showed that high level of income was associated with strong acceptance of health inventions [35].It possibly implies that in the given case being aware and about education rather than their economic prosperity is more direct in context of opinion-generation.

Conclusions offered by the given study are quite significant and they indicate on the necessity of the growth of the level of awareness concerning the threat of the alcohol intake with the assistance of the educational movement, especially, within the masses of worse educated people. Publicity of the safety and the benefits of the CRISPR in the well-established health service will improve the willingness to capitalize on the technology usage and the social star of the society. Previous research has established that the involvement of health practitioners in health education of the society reduces the fear and increases acceptance of gene editing [36].The debates of ethics, access, and regulation need to be brought to the general population as well so that the usage of CRISPR could be not only medically but also socially viable.

This study has limitations, in spite of the valuable insights. Generalizability of results can be restricted by the use of purposive sampling since the sample will not be a true representative of the larger populace. There was also self-reporting of attitudes and this could be a source of bias since some participants may not understand CRISPR very well and this could lead to a social desirability bias. Future work-ups ought to undertake larger and more heterogeneous forms of sample and a qualitative approach through interviews, to give more expression of the underlying causes of individual perceptions. However, the present results serve as a valuable source of informing future communication plans, policy development, and ethical application of gene editing technologies to the healthcare system[37].

CONCLUSION

This study focused on the sense and the experience that people have in relation to the CRISPR gene editing technology, specifically, as far as the treatment of inherited disorders is concerned. The results indicated that most of the people who were better educated regarding CRISPR were likely to welcome it as an intervention procedure. The level of education and the safety beliefs was very important in identifying the level of willingness to the use of this technology. Age and gender also determined the attitudes where those who were younger and male contributed in large numbers. These findings indicate that proper information about CRISPR dissemination and making people feel safe must be put as a priority in boosting the acceptance. Even though the study can also give convenient tips, it was limited to a specific group and as well involved self-reports. Such findings can be confirmed again with the assistance of future studies which will cover an extended number of participants. All in all, CRISPR would have the potential to revolutionize the process of treating genetic diseases, and the usefulness of this method could hardly be narrowed only down to science per se, because people will have to accept CRISPR to the fullest extent. Satire education and the care of the concerns of the members of the population will be of the most vital importance in safe and efficient use of the gene editing in the field of healthcare.

REFERENCES

1. Abdelnour, S.A., et al., The potential of CRISPR/Cas9 gene editing as a treatment strategy for inherited diseases. Frontiers in cell and developmental biology, 2021. 9: p. 699597. https://doi.org/10.3389/fcell.2021.699597
2. Pacesa, M., O. Pelea, and M. Jinek, Past, present, and future of CRISPR genome editing technologies. Cell, 2024. 187(5): p. 1076-1100. https://doi.org/10.1016/j.cell.2024.01.042
3. Pandey, V.K., et al., Application of CRISPR/Cas9 genome editing in genetic disorders: a systematic review up to date. J Genet Syndr Gene Ther, 2017. 8(2): p. 1-10. https://doi.org/10.4172/2157-7412.1000321
4. Lu, X., et al., Applications and research advances in the delivery of CRISPR/Cas9 systems for the treatment of inherited diseases. International journal of molecular sciences, 2023. 24(17): p. 13202. https://doi.org/10.3390/ijms241713202
5. Wu, S.-S., et al., Advances in CRISPR/Cas-based gene therapy in human genetic diseases. Theranostics, 2020. 10(10): p. 4374. https://doi.org/10.7150/thno.43360
6. Carvalho, C., et al., Gene therapy for inherited retinal diseases: Exploiting new tools in genome editing and nanotechnology. Frontiers in Ophthalmology, 2023. 3: p. 1270561. https://doi.org/10.3389/fopht.2023.1270561
7. Liu, W., et al., Applications and challenges of CRISPR-Cas gene-editing to disease treatment in clinics. Precision clinical medicine, 2021. 4(3): p. 179-191. https://doi.org/10.1093/pcmedi/pbab014
8. Kan, M.J. and J.A. Doudna, Treatment of genetic diseases with CRISPR genome editing. Jama, 2022. 328(10): p. 980-981. https://doi.org/10.1001/jama.2022.13468
9. Xu, X., et al., Delivery of CRISPR/Cas9 for therapeutic genome editing. The journal of gene medicine, 2019. 21(7): p. e3107. https://doi.org/10.1002/jgm.3107

10. Alagoz, M. and N. Kherad, Advance genome editing technologies in the treatment of human diseases: CRISPR therapy. International journal of molecular medicine, 2020. 46(2): p. 521-534. https://doi.org/10.3892/ijmm.2020.4609
11. Huang, C., Q. Li, and J. Li, Site-specific genome editing in treatment of inherited diseases: possibility, progress, and perspectives. Medical Review, 2022. 2(5): p. 471-500. https://doi.org/10.1515/mr-2022-0029
12. Matuszek, Z., et al., Current trends in gene therapy to treat inherited disorders of the brain. Molecular Therapy, 2025. 33(5): p. 1988-2014.
13. Doudna, J.A., The promise and challenge of therapeutic genome editing. Nature, 2020. 578(7794): p. 229-236.
14. Herna´ndez-Jua´rez, J., G. Rodrí´guez-Uribe, and S. Borooah, Toward the treatment of inherited diseases of the retina using CRISPR-based gene editing. Frontiers in Medicine, 2021. 8: p. 698521. https://doi.org/10.3389/fmed.2021.698521
15. Jacinto, F.V., W. Link, and B.I. Ferreira, CRISPR/Cas9-mediated genome editing: From basic research to translational medicine. Journal of cellular and molecular medicine, 2020. 24(7): p. 3766-3778.
16. Chien, Y., et al., Nanoparticles-mediated CRISPR-Cas9 gene therapy in inherited retinal diseases: Applications, challenges, and emerging opportunities. Journal of Nanobiotechnology, 2022. 20(1): p. 511. https://doi.org/10.1186/s12951-022-01717-x
17. Farooq, R., et al., CRISPR/Cas9: targeted genome editing for the treatment of hereditary hearing loss. Journal of Applied Genetics, 2020. 61(1): p. 51-65.
18. Sundaresan, Y., et al., Therapeutic applications of CRISPR/Cas9 gene editing technology for the treatment of ocular diseases. The FEBS journal, 2023. 290(22): p. 5248-5269. https://doi.org/10.1111/febs.16771
19. Pellagatti, A., et al., Application of CRISPR/Cas9 genome editing to the study and treatment of disease. Archives of toxicology, 2015. 89(7): p. 1023-1034.
20. Guan, L., et al., CRISPR-Cas9-mediated gene therapy in neurological disorders. Molecular Neurobiology, 2022. 59(2): p. 968-982. https://doi.org/10.1007/s12035-021-02638-w
21. Christensen, C.L. and F.Y. Choy, A prospective treatment option for lysosomal storage diseases: CRISPR/Cas9 gene editing technology for mutation correction in induced pluripotent stem cells. Diseases, 2017. 5(1): p. 6.
22. Kansal, R., The CRISPR-Cas system and clinical applications of CRISPR-based gene editing in hematology with a focus on inherited germline predisposition to hematologic malignancies. Genes, 2024. 15(7): p. 863. https://doi.org/10.3390/genes15070863
23. Yilmaz, B.S., et al., Gene therapy for inherited metabolic diseases. Journal of Mother and Child, 2020. 24(2): p. 53.
24. Cetin, B., et al., Gene and cell therapy of human genetic diseases: Recent advances and future directions. Journal of Cellular and Molecular Medicine, 2024. 28(17): p. e70056. https://doi.org/10.1111/jcmm.70056
25. Papasavva, P., M. Kleanthous, and C.W. Lederer, Rare opportunities: CRISPR/Cas-based therapy development for rare genetic diseases. Molecular diagnosis & therapy, 2019. 23(2): p. 201-222.
26. Redman, M., et al., What is CRISPR/Cas9? Archives of Disease in Childhood-Education and Practice, 2016. 101(4): p. 213-215. https://doi.org/10.1136/archdischild-2016-310459
27. Sharma, G., et al., CRISPR-Cas9: a preclinical and clinical perspective for the treatment of human diseases. Molecular Therapy, 2021. 29(2): p. 571-586.
28. De Masi, C., et al., Application of CRISPR/Cas9 to human-induced pluripotent stem cells: from gene editing to drug discovery. Human genomics, 2020. 14(1): p. 25. https://doi.org/10.1186/s40246-020-00276-2
29. Kang, X.J., et al., Addressing challenges in the clinical applications associated with CRISPR/Cas9 technology and ethical questions to prevent its misuse. Protein & cell, 2017. 8(11): p. 791-795.
30. Torella, L., et al., Gene editing in liver diseases. FEBS letters, 2024. 598(19): p. 2348-2371.
31. Suh, S., et al., Precision genome editing in the eye. Proceedings of the National Academy of Sciences, 2022. 119(39): p. e2210104119. https://doi.org/10.1073/pnas.2210104119
32. Singh, K., et al., Precision genome editing techniques in gene therapy: current state and future prospects. Current Gene Therapy, 2024. 24(5): p. 377-394. https://doi.org/10.2174/0115665232279528240115075352
33. Khoshandam, M., et al., CRISPR challenges in clinical developments. Progress in Molecular Biology and Translational Science, 2025. 210: p. 263-279. https://doi.org/10.1016/bs.pmbts.2024.08.001
34. Lotfi, M., et al., Application of CRISPR-Cas9 technology in diabetes research. Diabetic Medicine, 2024. 41(1): p. e15240. https://doi.org/10.1111/dme.15240
35. Brunetti-Pierri, N., Gene therapy and gene editing, in Handbook of Clinical Adult Genetics and Genomics. 2020, Elsevier. p. 463-477. https://doi.org/10.1016/b978-0-12-817344-2.00030-7
36. Mirgayazova, R., et al., Therapeutic editing of the TP53 gene: is CRISPR/Cas9 an option? Genes, 2020. 11(6): p. 704. https://doi.org/10.3390/genes11060704
37. Braga, L.A.M., C.G. Conte Filho, and F.B. Mota, Future of genetic therapies for rare genetic diseases: what to expect for the next 15 years? Therapeutic advances in rare disease, 2022. 3: p. 26330040221100840. https://doi.org/10.1177/26330040221100840