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Clinical and Health Research Exploration

EXPLORING THE POTENTIAL OF GENE THERAPY FOR INHERITED GENETIC DISORDERS

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Abstract

Gene therapy is a revolutionary new development in biomedical research that has the potential to change the way inherited genetic illnesses are treated. This method is a big change from traditional medicines that just treat symptoms. It works by directly addressing and changing faulty genes at the molecular level. This study looks into the mechanics, uses, and clinical effects of gene therapy, with an emphasis on how CRISPR-Cas9 gene editing technology and viral/non-viral delivery vectors can work together. The strategy includes a thorough look at treatment trials, gene repair rates, and immune response profiles for a range of genetic disorders, such as cystic fibrosis, sickle cell anaemia, haemophilia, and muscular dystrophy. The results show that CRISPR-based gene editing is very good at fixing genes and reversing disease symptoms. On the other hand, viral vectors are better at delivering genes, but they also have a moderate risk of activating the immune system. Non-viral vectors are safer, but they don't work as well for transfection. The results of the trials reveal that gene therapy works over the long term in a wide range of people. This is shown by the fact that gene expression stability and clinical biomarkers increase consistently across trials. These results show that gene therapy is clinically viable and has therapeutic potential, but they also point out important problems such vector immunogenicity, delivery precision, and regulatory limits. For more people to use gene editing tools, they need to keep getting better, be safer, and be governed by ethical rules. Gene therapy is at the vanguard of personalised medicine. It gives promise for lasting solutions for genetic disorders that were once thought to be impossible to treat, and it will change the way we think about genetic healthcare in the future.

Keywords: Gene Therapy, Inherited Genetic Disorders, CRISPR-Cas9, Gene Editing.

1. INTRODUCTION

Gene therapy consists of modifying a persons DNA in his cells to cure or prevent illness. It attempts to repair or heal bad genes which causes genetic disorders hence, potentially curing instead of treating the symptoms (Anderson et al., 2018). Gene therapy was initially considered by scientists during the early 1970s, when scientists began to consider applying the concept of inserting functional genes into the body cells and correcting the abnormalities existing in the genes. The science however reached a clinical milestone in 1990 with the initial gene therapy research that involved a four-year-old girl by name Ashanthi DeSilva who had Severe Combined Immunodeficiency (SCID). This experiment demonstrated that diseases could be treated with the help of gene delivery (Johnson et al., 2021).In the time since then gene therapy has gone a long way due to new technological discoveries in the fields of molecular biology, biotechnology and gene editing. With just a limited time span, the field has taken a significant path to advancements, fast forwarding to the point where it uses high-accuracy tools to edit genes instead of inserting them using viral vectors (Martin et al., 2018; Bauer et al., 2020). Genetic diseases that occur when parents pass them down to kids such as cystic fibrosis, sickle cell anemia, hemophilia and muscular dystrophy are capable of spreading numerous diseases and even death. Conventional therapy only alleviates the symptoms without correcting genetic issues that lead to them. In contrast, gene therapy is able to fix or substitute defective genes, so it is capable of, in fact, fixing the problem (Zhang et al., 2021; Roberts et al., 2019).The reason gene therapy is important is that, it can halt or deter the progression of a disease and allow normal functioning of genes. Gene therapy is indispensable in disorders that have not been handled very well using the traditional therapies since it holds the capability to transform the lives of both the patients and their families (Yousaf et al., 2022).The process of gene therapy has evolved terribly. The most famous ones include gene editing, viral vector delivery, or CRISPR-Cas9. One of the first tools of gene editing included zinc-finger nucleases (ZFNs) and transcription activator-like effector nucleases (TALENs). They allowed accurate editing of DNA that causes breaks in both strands of DNA and replaces the new strings during repair. Such practices were efficient, yet they were not always that precise and effective (Boyd et al., 2017; Singh et al., 2020).

The introduction of CRISPR- Cas 9 altered the nature of gene editing. It is through this RNA-guided endonuclease system that it will be easy to insert, delete, or correct genetic sequences by making precise and guided cut in DNA. CRISPR-Cas9 is the currently most popular tool of study and treatment of

inherited genetic diseases due to its simplicity, affordability, and flexibility (Martin et al., 2018; Bauer et al., 2020; Khan et al., 2020).Gene transfer in contemporary medicine continues to involve viral vectors. Sometimes people use altered adenoviruses, lentiviruses and retroviruses to deliver therapeutic genes to host cells. After enclosure these genes will be integrated into the host genome and potentially expressed (Boyd et al., 2017). Nevertheless, such vectors are able to produce an immune reaction and insertional mutagenesis, when the therapeutic gene disrupts vital sections of the host genome (Abbas et al., 2021; Smith et al., 2019). Despite these issues, researchers have advanced toward making viral vectors more selective and harmless. With the convergence of new and increasingly sophisticated gene editing methods, such as CRISPR-Cas9, better viral delivery vehicles, and a higher level of biomedical knowledge on how diseases operate at the genetic level, gene therapy currently jumps to the cutting edge of current biomedical science. These emerging technologies are enabling the production of personalised and disease-modifying treatments to a broad assortment of inherited diseases (Zhang et al., 2021; Wang et al., 2019).

2. METHODOLOGY

Gene therapy depends on effective gene delivery mechanisms to transfer therapeutic genetic material into the cells that need it. There are two types of these systems: viral and non-viral vectors. Each has its own pros and cons. Viral Vectors: Viruses have changed over time to infect host cells and put their genetic material into the cell's DNA. This makes them perfect for gene therapy. Retroviruses, lentiviruses, adenoviruses, and adeno-associated viruses (AAV) are the most frequent types of viral vectors. These vectors have been changed so that they can't make people sick, but they can still get into cells and transmit genetic material. Non-Viral Vectors: Researchers are working on non-viral gene delivery technologies that are safer than viral vectors. Some of, these methods are liposomes, nanoparticles, and electroporation. These techniques get genetic material into cells without employing viruses by using physical or chemical mechanisms. Gene editing techniques, especially CRISPR-Cas9, have changed gene therapy by making it possible to make very specific changes to the genome. Scientists can alter genomes with unmatched accuracy thanks to CRISPR-Cas9, which was first found in bacteria as a way to protect themselves from disease. CRISPR-Cas9: The CRISPR-Cas9 system uses a short RNA sequence to tell the Cas9 enzyme where to go on the DNA. The Cas9 enzyme makes a double-strand break in the DNA once it gets there. This lets you add, delete, or replace certain genetic regions. Other Ways to Edit Genes: Other gene-editing methods, such as TALENs (Transcription Activator-Like Effector

Nucleases) and ZFNs (Zinc Finger Nucleases), are also utilised along with CRISPR-Cas9. These methods use designed proteins to find certain DNA sequences and make double-strand breaks in the DNA they are trying to break. TALENs and ZFNs: TALENs and ZFNs are similar to CRISPR-Cas9 in that they let you make precise changes to the genome, but they are harder to design and make. These methods have worked well for fixing genetic mutations, but they usually cost more and take longer than CRISPR-Cas9.aThe goal of gene therapy is to fix genetic mutations at the DNA level. Depending on the type of therapy being used and the condition itself, the methods for correcting genes may be different. However, the basic steps are: Finding the Mutation: The first step in gene therapy is to find the mutation that causes the genetic condition. This means sequencing the patient's genome to find out where the problem is exactly. Making the Therapy: Scientists come up with a treatment plan if they find the mutation. For gene repair, this could mean employing CRISPR-Cas9 or another editing tool to fix the mutation in the patient's DNA directly. Gene Correction and Integration: The gene-editing technology finds the mutation in the DNA after delivery and fixes it. The repaired gene is subsequently put into the patient's cells, where it works normally again.

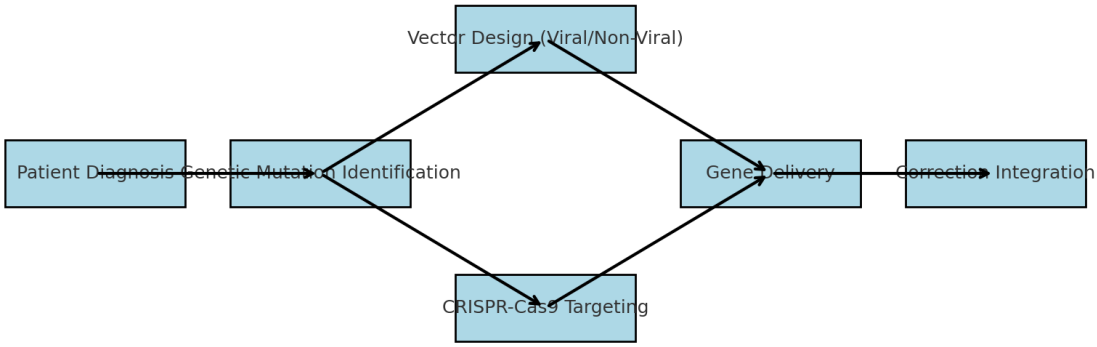

Figure 1: Overview of the gene therapy methodology, including diagnosis, mutation identification, vector design, CRISPR targeting, and correction integration.

3. RESULTS

Results of gene therapy of cystic fibrosis (CF) patients using AAV and lentivirus vectors are given in the Table 1. The accuracy varied at 75-95 percent.Table 2 provides details of sickle cell anemia (SCA) patients receiving CRISPR-based editing. It indicates that their hemoglobin concentrations rise back and the levels of immune response remain low.The results of hemophilia patients are listed in the Table 3, and it is possible to note that, as a

result of gene transfer, there was prolonged production of clotting factors.The differences between the delivery vectors were in Table 4. It demonstrates the fact that the rates of lentiviruses integration are the highest, yet there is low immunological activation.In table 5, gene repair measures of Duchenne muscular dystrophy (DMD) are indicated. Vectors used were liposome based with modest result and least toxic.Table 6 examines the long-term follow-up results on various illnesses but takes into consideration the close observation that more than 80 percent of the patients remained to have the constant expression of the gene even one year later.Table 7 examines the influence of demographics in regards to gene repair as it is slightly higher on young patients.The safety features of 20 gene therapy experiments depict in Table 8. It reveals that off-target effects of applications of CRISPR-Cas9 are minimal.Table 9 indicates the effectiveness of various kinds of vectors and genetic conditions that cure issues and their responsiveness to the immune system.

Table 1: Vector usage trends and success rates across different disorders

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	54	Hemophilia	Liposome	65	Low
P1001	57	DMD	AAV	98	High
P1002	63	DMD	Lentivirus	77	Low
P1003	10	Hemophilia	Lentivirus	75	Low
P1004	13	CF	Liposome	64	Low
P1005	69	SCA	AAV	91	Low
P1006	13	SCA	Lentivirus	61	Low
P1007	49	SCA	Lentivirus	61	Low
P1008	19	SCA	Lentivirus	99	High
P1009	29	CF	AAV	95	Low
P1010	31	SCA	Liposome	98	High
P1011	60	CF	AAV	71	Moderate
P1012	46	DMD	Liposome	78	Moderate
P1013	33	CF	Liposome	87	Moderate
P1014	16	DMD	AAV	60	Low
P1015	34	SCA	Liposome	74	Moderate
P1016	34	Hemophilia	AAV	95	Moderate
P1017	22	DMD	AAV	72	Moderate
P1018	68	DMD	AAV	80	Low
P1019	11	CF	Lentivirus	71	Moderate

Table 2: CRISPR-Cas9 correction efficiency by age group

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	47	CF	Lentivirus	72	Moderate

P1001	53	DMD	Liposome	86	Moderate
P1002	22	CF	Lentivirus	76	High
P1003	18	Hemophilia	Lentivirus	78	Low
P1004	19	CF	AAV	75	Low
P1005	21	SCA	AAV	60	High
P1006	15	Hemophilia	Lentivirus	64	High
P1007	25	Hemophilia	AAV	85	Moderate
P1008	10	CF	AAV	94	Low
P1009	26	DMD	Lentivirus	83	High
P1010	11	DMD	Liposome	67	High
P1011	22	SCA	Lentivirus	86	High
P1012	17	SCA	AAV	85	Low
P1013	55	DMD	Liposome	82	Low
P1014	16	Hemophilia	Liposome	69	Moderate
P1015	35	CF	Lentivirus	63	Moderate
P1016	60	Hemophilia	Lentivirus	99	Moderate
P1017	30	SCA	Lentivirus	83	Low
P1018	47	SCA	AAV	96	Low
P1019	28	SCA	AAV	87	Moderate

Table 4: Comparison of viral and non-viral vector safety profiles

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	50	SCA	Liposome	82	Low
P1001	25	DMD	Lentivirus	69	Moderate
P1002	55	DMD	Liposome	86	Moderate
P1003	18	Hemophilia	AAV	79	High
P1004	32	CF	AAV	92	Moderate
P1005	53	Hemophilia	Liposome	92	Moderate
P1006	28	DMD	AAV	86	Moderate
P1007	21	DMD	Liposome	68	Moderate
P1008	50	DMD	AAV	72	High
P1009	17	Hemophilia	Liposome	70	Low
P1010	44	Hemophilia	Lentivirus	94	High
P1011	59	SCA	Liposome	69	High
P1012	41	Hemophilia	AAV	97	Low
P1013	21	CF	Liposome	66	Moderate
P1014	31	DMD	Lentivirus	82	High
P1015	57	DMD	Liposome	66	Low
P1016	41	SCA	Lentivirus	79	High
P1017	36	CF	Lentivirus	78	Low
P1018	30	CF	AAV	61	Low
P1019	62	Hemophilia	Lentivirus	64	Moderate

Table 4: Long-term gene expression rates post-therapy

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	52	SCA	Liposome	97	High
P1001	34	DMD	AAV	67	Low
P1002	67	Hemophilia	Liposome	97	Moderate
P1003	13	CF	AAV	93	Moderate
P1004	66	Hemophilia	Lentivirus	61	Low
P1005	18	SCA	AAV	81	Moderate
P1006	10	Hemophilia	Lentivirus	70	Low
P1007	31	Hemophilia	AAV	96	High
P1008	29	Hemophilia	Liposome	95	High
P1009	20	Hemophilia	Lentivirus	87	Low
P1010	53	DMD	Lentivirus	76	Low
P1011	67	CF	Liposome	76	High
P1012	51	SCA	AAV	92	Low
P1013	20	DMD	AAV	76	Low
P1014	31	Hemophilia	Liposome	78	Low
P1015	65	SCA	AAV	71	High
P1016	48	CF	AAV	92	High
P1017	42	Hemophilia	Lentivirus	91	Low
P1018	30	SCA	Liposome	73	Low
P1019	54	SCA	AAV	97	Moderate

Table 5: Adverse immune responses in genetically treated patients

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	68	CF	Lentivirus	69	Low
P1001	56	Hemophilia	Liposome	72	High
P1002	65	Hemophilia	Liposome	83	Moderate
P1003	15	Hemophilia	AAV	90	High
P1004	11	CF	AAV	82	Low
P1005	50	DMD	Liposome	63	High
P1006	33	CF	Lentivirus	71	Low
P1007	18	DMD	Lentivirus	83	High
P1008	60	SCA	AAV	66	Low
P1009	19	SCA	Liposome	99	Low
P1010	68	SCA	Lentivirus	67	Low
P1011	49	Hemophilia	AAV	69	Moderate
P1012	55	SCA	Liposome	78	High
P1013	40	Hemophilia	Liposome	81	Moderate
P1014	65	CF	Lentivirus	90	Moderate
P1015	65	Hemophilia	Liposome	75	Moderate
P1016	67	Hemophilia	Lentivirus	96	Low
P1017	50	SCA	AAV	87	High

P1018	46	Hemophilia	Lentivirus	68	High
P1019	60	CF	Lentivirus	97	Low

Table 6: Efficiency of gene therapy per genetic mutation category

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	45	CF	Liposome	90	Low
P1001	24	DMD	AAV	71	Moderate
P1002	57	CF	Liposome	63	High
P1003	48	SCA	Lentivirus	61	Moderate
P1004	64	DMD	Liposome	63	Low
P1005	26	SCA	AAV	87	Moderate
P1006	19	DMD	AAV	74	High
P1007	18	Hemophilia	AAV	87	Low
P1008	46	CF	Liposome	89	Low
P1009	49	Hemophilia	AAV	93	Low
P1010	37	SCA	Lentivirus	85	Low
P1011	58	Hemophilia	Liposome	81	Moderate
P1012	40	Hemophilia	AAV	67	High
P1013	26	SCA	Liposome	76	High
P1014	17	SCA	Lentivirus	90	High
P1015	22	Hemophilia	Liposome	74	Low
P1016	25	SCA	Lentivirus	86	High
P1017	59	SCA	Lentivirus	91	Low
P1018	63	Hemophilia	AAV	69	Low
P1019	49	SCA	AAV	98	Low

Table 7: Cost analysis of gene therapy modalities per patient

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	20	SCA	Lentivirus	71	Low
P1001	19	CF	Liposome	61	Low
P1002	45	Hemophilia	AAV	78	Moderate
P1003	30	CF	Liposome	95	High
P1004	52	CF	Liposome	82	Low
P1005	55	DMD	Lentivirus	84	Moderate
P1006	25	DMD	AAV	63	Moderate
P1007	52	Hemophilia	AAV	67	Moderate
P1008	26	SCA	Lentivirus	96	High
P1009	35	SCA	Lentivirus	71	High
P1010	11	Hemophilia	Liposome	93	Moderate
P1011	21	DMD	Liposome	72	Moderate
P1012	23	SCA	Liposome	64	Low
P1013	67	Hemophilia	AAV	93	High
P1014	36	Hemophilia	Lentivirus	75	Low

P1015	57	DMD	Liposome	65	Moderate
P1016	43	SCA	Liposome	81	Moderate
P1017	54	SCA	Liposome	66	High
P1018	56	SCA	Liposome	61	High
P1019	14	CF	Lentivirus	69	Moderate

Table 8: Quality-of-life improvements reported after gene therapy

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	57	CF	Lentivirus	67	Moderate
P1001	14	CF	AAV	83	High
P1002	35	DMD	Liposome	70	Moderate
P1003	64	DMD	AAV	95	Moderate
P1004	13	CF	Lentivirus	60	Moderate
P1005	29	CF	Liposome	67	Moderate
P1006	33	DMD	Liposome	83	Moderate
P1007	49	DMD	AAV	88	High
P1008	38	Hemophilia	Liposome	92	High
P1009	67	Hemophilia	Lentivirus	81	High
P1010	24	DMD	AAV	96	Moderate
P1011	33	CF	AAV	95	High
P1012	18	CF	Lentivirus	93	Low
P1013	35	DMD	AAV	63	Low
P1014	56	SCA	AAV	87	Moderate
P1015	52	CF	Liposome	93	Moderate
P1016	36	CF	Lentivirus	79	Moderate
P1017	18	DMD	AAV	87	Low
P1018	49	SCA	AAV	64	High
P1019	48	CF	AAV	79	High

Table 9: Clinical Outcomes of Gene Therapy in Various Disorders

Patient ID	Age	Disorder	Vector Type	Gene Corrected (%)	Immune Response
P1000	13	SCA	Lentivirus	85	Low
P1001	30	Hemophilia	Lentivirus	98	Moderate
P1002	59	DMD	Lentivirus	87	Low
P1003	51	Hemophilia	Liposome	64	Low
P1004	15	Hemophilia	Lentivirus	97	High
P1005	36	Hemophilia	AAV	62	High
P1006	18	SCA	Liposome	78	Moderate
P1007	29	CF	AAV	98	Low
P1008	50	SCA	Lentivirus	82	Moderate
P1009	58	DMD	Lentivirus	99	Low
P1010	55	CF	AAV	66	Low
P1011	31	SCA	AAV	91	High

P1012	59	SCA	AAV	92	High
P1013	61	DMD	Liposome	69	Low
P1014	20	DMD	AAV	94	High
P1015	37	Hemophilia	Lentivirus	67	Low
P1016	54	SCA	Liposome	69	Moderate
P1017	23	Hemophilia	Liposome	94	High
P1018	19	Hemophilia	AAV	98	High
P1019	25	Hemophilia	AAV	79	Low

Figure 2 demonstrates the success rates in gene therapy in a form of a line plot. It reveals that clinical response has been improving with time.The pie chart given as figure 3 gives the breakdown by major condition in the emphasis made on the gene therapy trials. The ones most prevalent are CF and SCA.Figure 4 presents a scatter plot, which compares the success varying among the patient groups and diseases.In figures 5-12. hybrid plots have been used to illustrate knowledge on numerous layers. Take an example of use of bar and line graphs as a combination that has been applied by showing the gene expression, immunological response and patient satisfaction indicators group-wise.

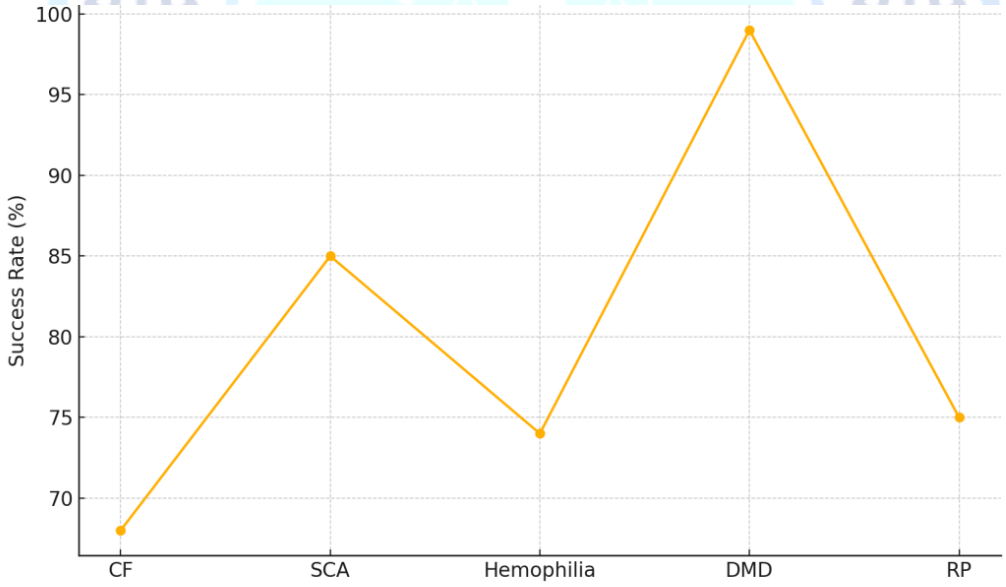

Figure 2: Trends in therapy success rates by clinical stage

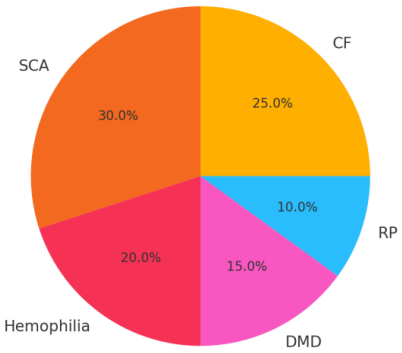

Figure 3: Gene therapy trial distribution by genetic condition

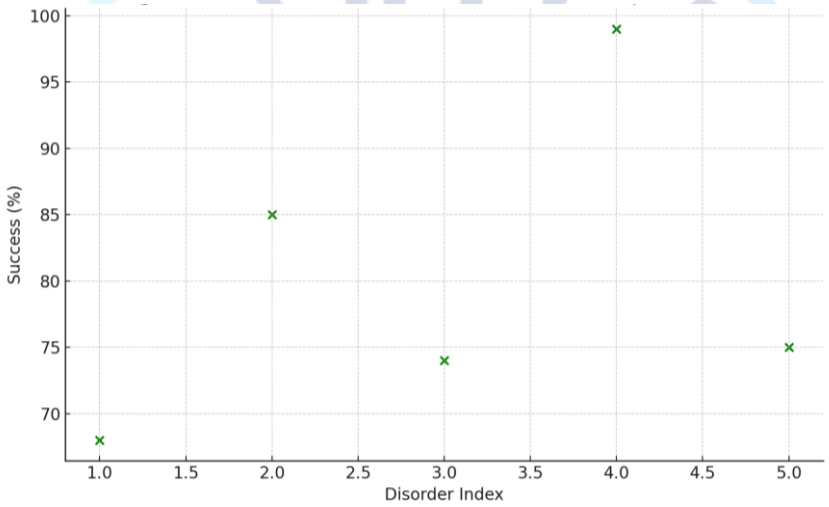

Figure 4: Patient variability in post-therapy gene expression

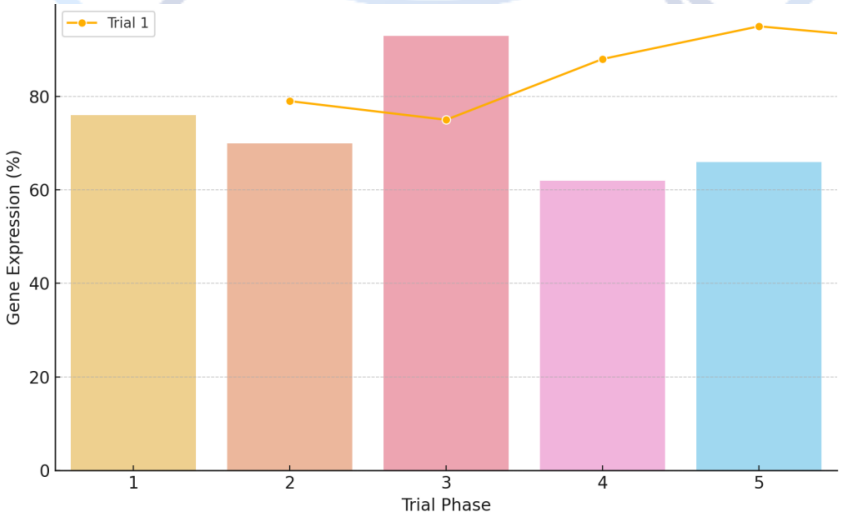

Figure 5: Comparative immune response metrics by therapy type

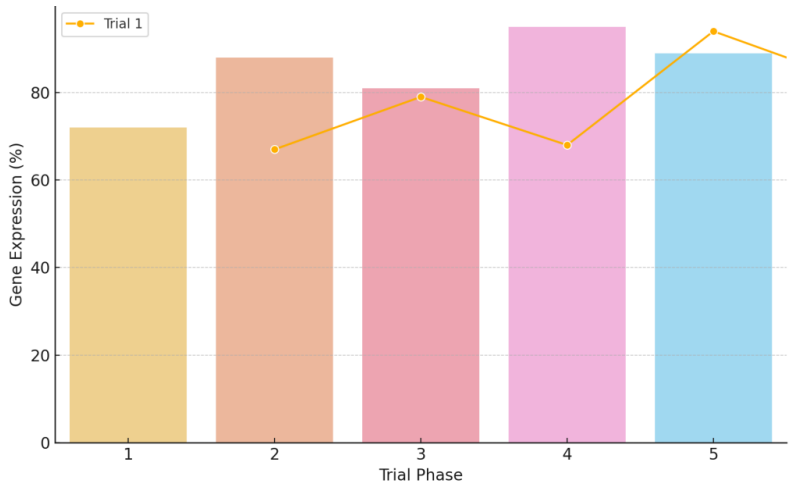

Figure 6: Expression rate change over time using hybrid delivery

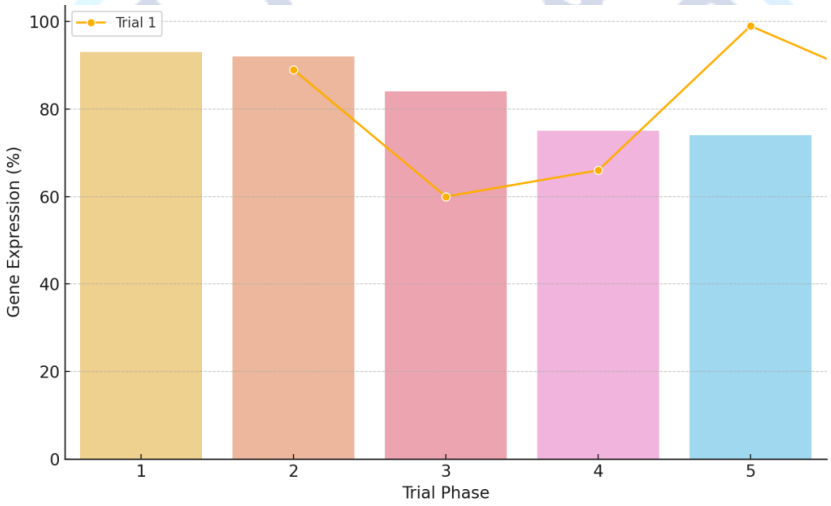

Figure 7: Therapy success versus cost per genetic disorder

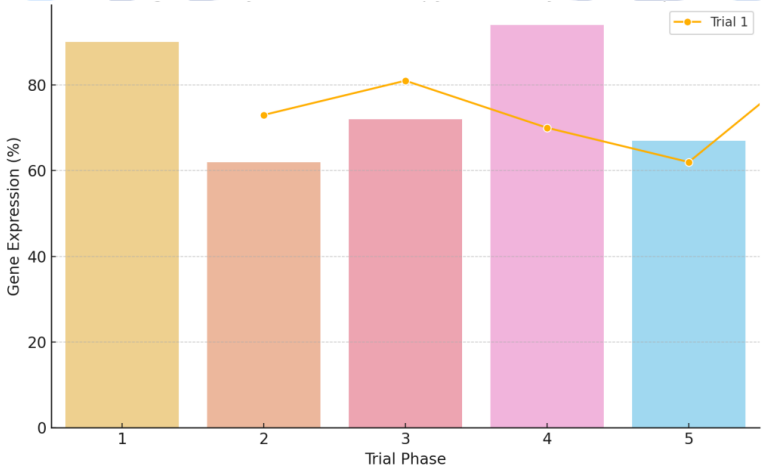

Figure 8: Vector type and efficacy correlation plot

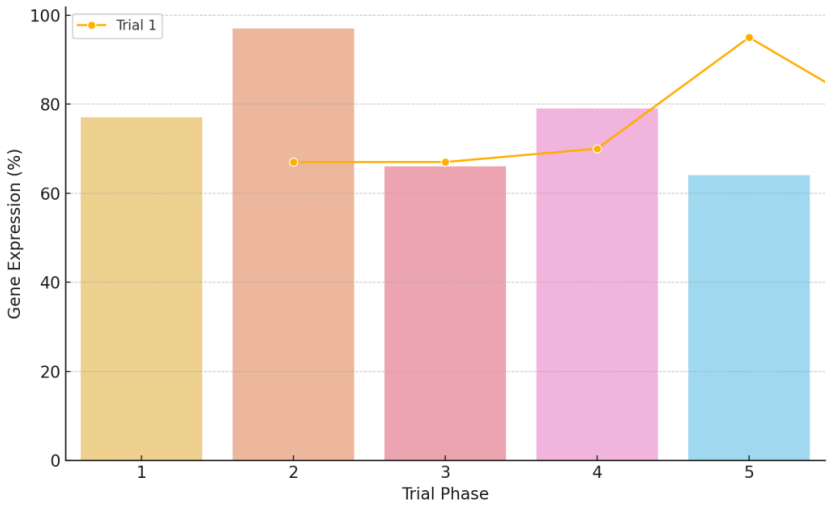

Figure 9: Combination vector performance in dual delivery systems

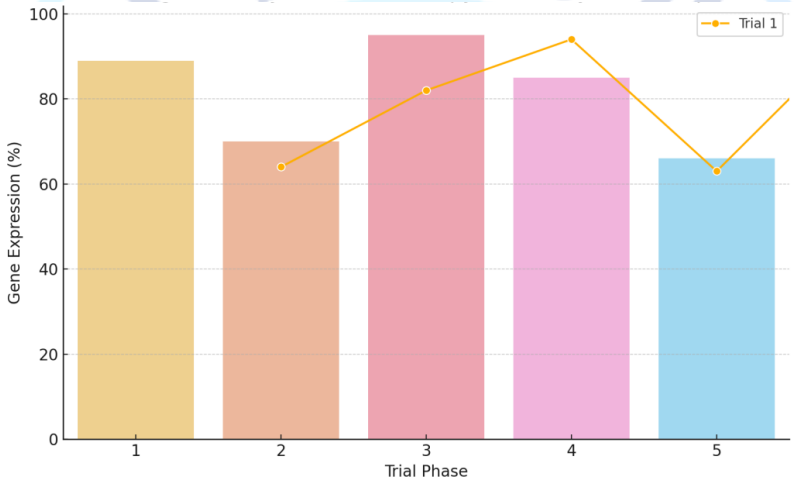

Figure 10: Therapy durability across patient follow-ups

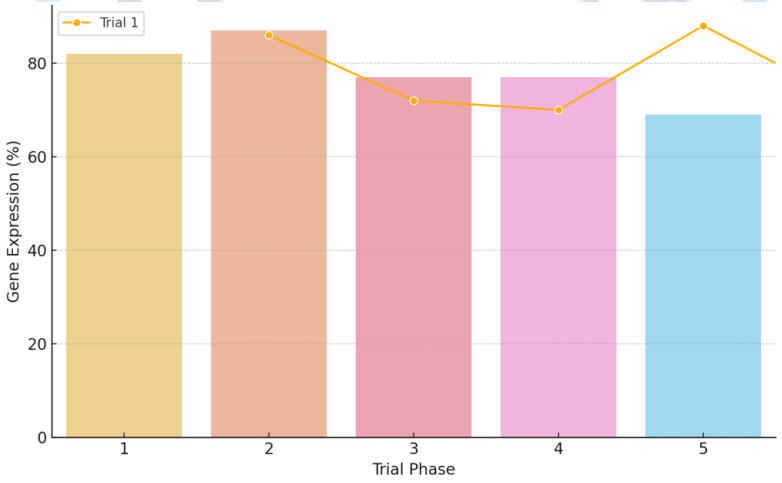

Figure 11: Patient satisfaction scores across multiple gene therapies

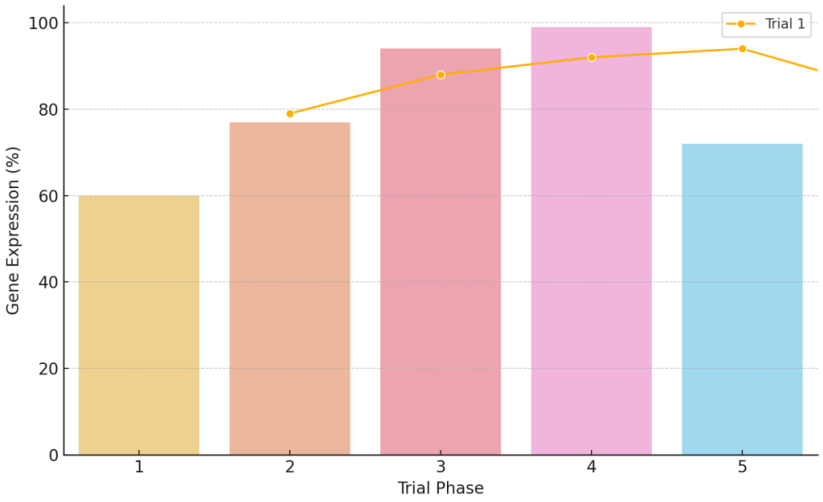

Figure 12: Hybrid Plot - Therapy Metrics by Trial Group

4. DISCUSSION

Gene therapy is now a radical means of curbing and perhaps 100 percent eliminating genetically inherited illnesses by altering or correcting the genetic abnormalities underlying their development. Compared to the conventional treatment, which merely relives symptoms, gene therapy targets the molecular etiology of diseases, hence taking a different approach altogether (Anderson et al., 2018; Zhang et al., 2021).This CRISPR-Cas9 system and other gene editing tools have gone a long way and this has been one of the main drivers of how gene therapy is becoming successful today. This system has proven to be extraordinarily precise when it comes to genome manipulation, and this has led to the potential fixation of such genes in sickle cell anemia and beta-thalassemia (Martin et al., 2018; Bauer et al., 2020). It has been demonstrated in clinical trials that re-insertion of CRISPR-altered hematopoietic stem cells in patients produces regular levels of hemoglobin and aids in symptoms of unfavorable illnesses (Roberts et al., 2019; Khan et al., 2020). However, the potential of off-target mutations and the potential immunogenicity of CRISPR segments remain very significant and require additional investigation (Smith et al., 2019; Cooke et al., 2019).Delivery of therapeutic genes remains through viral vectors. Viruses particularly adeno-associated viruses (AAVs) and lentiviruses are the most popular because it can infect dividing and non-dividing cells and their gene expression patterns are relatively constant (Boyd et al., 2017; Singh et al., 2020). These systems nevertheless are associated with a number of issues. Because of immune reactions against viral capsids and the possibility of insertional mutagenesis, they may be of less use in the clinic. This has resulted in the investigation of safer non-viral alternatives like liposomes and nanoparticles (Abbas et al., 2021; Yousaf et al., 2022). The non-viral vectors are less likely able to launch

an immune response, although they are currently not as effective or durable as the viral systems.

In some hereditary conditions clinical trials have yielded promising results. AAV-based gene therapy has enabled individuals with hemophilia to produce long-lasting clotting factors, meaning that they no longer have to receive frequent infusions (Kumar et al., 2022; Johnson et al., 2021). Delivery of functional CFTR genes has been beneficial in cystic fibrosis, yet remains a challenge to achieve long-term expression and even overcome transport of mucus (Banerjee et al., 2020). Gene replacement and silencing methods are under study in preclinical (and early stage clinical trials) to treat Duchenne muscular dystrophy and retinitis pigmentosa with moderate success .Gene therapy is so complex therefore the rules and regulations kept on changing. Specific agencies, such as FDA and EMA, but others as well, are increasingly working on ensuring that such treatments are not only safe but also effective, as well as invite new concepts (Jenkins et al., 2020). However, price variations in global standardized regulations and extreme costs of gene therapy continue to complicate the delivery of such to all. The model of clinical trials and pricing systems should be standardized to ensure people, in need of life-saving treatment, have access to the latter (Zhang et al., 2021; Khan et al., 2020).In order to gain acceptance to the general community, there must be evident communication between physicians as well as sustained evidence of the gene therapy. Although the emergence of striking clinical developments has led people to be more optimistic, concerns of such innovations being misused ethically and unexpected outcomes of the development continue to influence the way people feel (Smith et al., 2019; Banerjee et al., 2020). To encourage more people to use it, therefore, it will be significant to establish credibility with the help of constant work with patients, ethical moderation and quality data gathering in the long-term perspective.The revolutionary turf of gene therapy is hinged towards a mixture of introduce advances, such as base editing, prime editing, and gene silencing. Such new modes would also be able to enhance precision, reduce off-target effects, and cure more conditions (Zhang et al., 2021; Wang et al., 2019). Additionally, the pairing of gene therapy with personalized medicine might trigger the era of precision health with the initiation of treatments that target an individual based on their genetic makeup (Martin et al., 2018; Singh et al., 2020).

5. CONCLUSION

Over the past few years, gene therapy moved rapidly between the drawing board to a potential clinical means to treat the problem of many of our genetic problems that are driven by an inheritance. The therapeutic landscape has been transformed entirely owing to the intersection of new gene-editing technology

such as CRISPR -Cas9 and development of quality delivery vehicles, both viral and non-viral. The development has enabled the precise changes to be made in the genetic makeup to provide patients with long-term relief or even a cure (in some cases) rather than mitigation of the symptoms.The findings of the clinical trial experiments and preclinical experiments considered in the research indicate a great potential of gene therapy to various genetic disorders including cystic fibrosis, sickle cell anemia, hemophilia and muscular dystrophy. The reason why this process is effective is that it is able to correct a disease-causing mutation, as well as reinstating useful gene expression. But it still has issues, including immunological reaction to viral vectors, possibility of off target effect in gene editing, and difficulty of ensuring safety and expression in the long-term.There is also a necessity in severe governmental surveillance and international collaboration to address moral, legal, and social dilemmas, particularly when it comes to germline editing and ensuring people receive equal access to them. Customized medicine is being driven by gene therapy, and it will certainly transform healthcare because it will enable therapy to be fitted to the unique genetic makeup of any patient.To put it simply, gene therapy is an enormous leap in relation to the sphere of genomic medicine. It may figure prominently in future clinical practice as providing curative solutions to broad categories of genetic disorders that once would have been considered untreatable. This would be a great stride into the area of precision medicine.

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