Engineering CRISPR, Stem Cells, and CAR T to Transform Existing Biology into Disease Cures

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Abstract -

In recent years, strides have been made in medical technology that may shift the paradigm of the field as a whole. Complex treatments like CRISPR therapy, stem cell therapy, and CAR T-cell therapy have been made more effective and more easily accessible. With so many ailments afflicting people across the world, one of these three modern medicine technologies may act as a cure. This paper is a literature review, detailing both the benefits and current drawbacks of each form of treatment. In addition, the long term potentials of these treatments are discussed. Following a description of each of the technologies and their respective therapies, ongoing trials combining them in an attempt to be more effective overall are discussed. All three of these treatments are useful in different cases, and when used together, they have the potential to help a lot of people.

Section 1 - Introduction

Incurable genetic diseases have afflicted millions of people worldwide (Stark & Scott, 2023). Medicine has come a long way in a short amount of time, but many still suffer as cures to their ailments have not yet been discovered. However, recent technological strides have allowed scientists to develop new treatments such as CRISPR therapy, stem cell therapy, and CAR T-cell therapy. This paper will explore these innovations in medicine, and discuss their potential to revolutionize the field of medicine as a whole and improve the lives of millions. While these technologies each carry their own unique risks, the reward of curing millions of their illnesses is too important to ignore. To ameliorate these risks, the treatments may be used in unison to facilitate greater safety and greater effectiveness overall.

Section 2 - CRISPR

Clustered Regularly Interspaced Short Palindromic Repeats, commonly known as CRISPR, was discovered in 1987 by Japanese scientist Yoshizumi Ishino. Over the next decade, Jennifer Doudna and Emmanuelle Charpentier began learning more about CRISPR (Ozkan, 2021). They learned that CRISPR is integrated into a bacterial immune system, providing resistance against viruses and plasmids. In 2012, researchers realized they could repurpose CRISPR as a gene editing tool by using it to cut viral DNA (Ozkan, 2021). This research would later earn Doudna and Charpentier the 2020 Nobel Prize in Chemistry. This breakthrough demonstrated the unparalleled potential to efficiently edit genes in plants, animals and humans. Compared to the previous generation of gene editing, CRISPR is inexpensive, precise with a high success rate, and thus makes it a lot easier to edit genes.

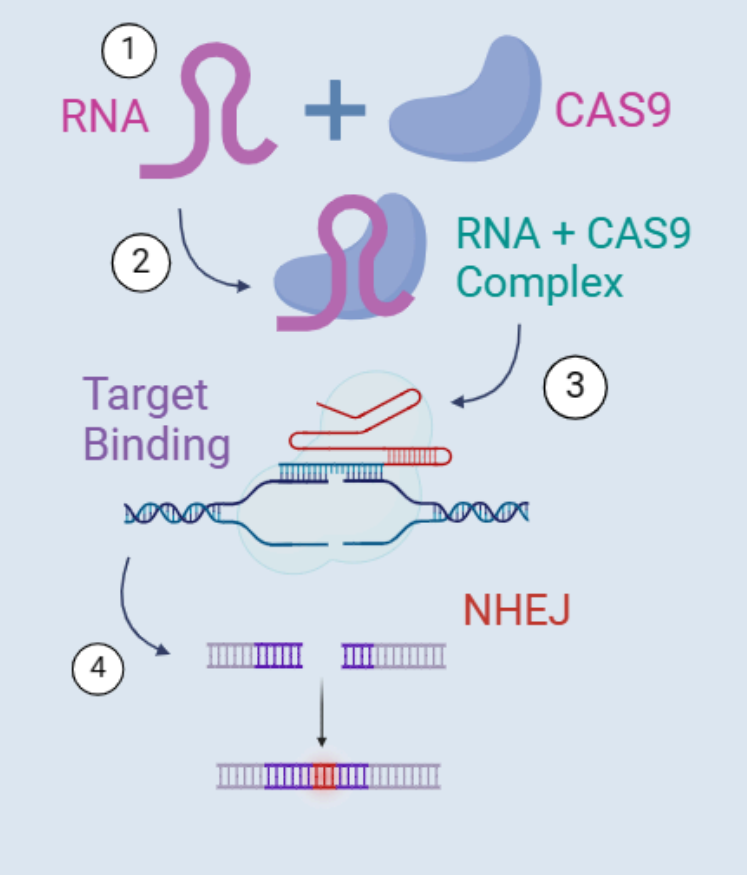

Figure 1: Schematic of Cas9 Gene Editing. A single-stranded RNA molecule, known as the guide RNA, (1) combines with the Cas9 enzyme (a nuclease that can break apart DNA strands) (2), and this complex moves to the target DNA site, creating a double-strand break (3). After CRISPR makes the break, in the non-homologous end joining (NHEJ) pathway, enzymes fill in gaps and ligases seal strands (4). Created using Biorender.

The CRISPR system involves repeating sequences of DNA and short palindromic repeat sequences that act as a guide. As shown in Figure 1, guide RNA leads to the Cas9 enzyme to target the DNA sequence. Cas9 cuts the DNA, allowing insertions, deletions or other modifications of genes (Kim et al., 2017).

When the CRISPR system makes this double-strand break in the DNA, the cell has two main pathways to repair the break. In the non-homologous end joining (NHEJ) pathway, enzymes like polymerases find and fill in the gaps, while ligases seal the strands back together. This process is error-prone because no template DNA strand is being used, and thus it can result in insertions or deletions of bases (Kozovska et al., 2021). Bioengineers can exploit this to intentionally disrupt genes, like those involved in sickle cell anemia or hemophilia, where disrupting the defective gene can allow healthy gene variants to be expressed or prevent the production of harmful proteins.

The alternative is homology-directed repair (HDR), in which a template DNA strand is used to precisely edit the sequence during the repair process (Kozovska et al., 2021). In HDR, another homologous sequence from a sister chromatid acts as your guide to fix double stranded

breaks. This prevents mutations but can fail if the template or repair machinery is defective, leading to errors or cancer development. Natural defects in HDR include BRCA mutations which can lead to breast cancer.

CRISPR is at the forefront of gene modification technology. The technologies that preceded its use, such as ZFNs (zinc finger nucleases) and TALENs (transcription-activator-like-effector nucleases), have shortcomings that CRISPR is able to ameliorate. For instance, ZFNs and TALENs require specific protein engineering for each individual target site (Krishna & Kantipudi, 2019). CRISPR, on the other hand, only needs a guide RNA to instruct Cas9 on where to make a cut, and can target multiple sites using multiple guide RNAs. This capability is particularly significant because most genetic diseases involve mutations on different nucleotides across various chromosomes. For ZFNs and TALENs, another protein would be needed to target each additional gene, while CRISPR can target various genes using the same Cas9 protein. This makes CRISPR a more effective tool for addressing complex genetic disorders compared to its predecessors.

CRISPR has primarily been used in gene and cell engineering in biological research, as it is often employed to modify cells for disease modeling. However, its applications extend beyond medical research. CRISPR has also been utilized in plant gene editing, enhancing traits that are beneficial to crops, such as resistance to specific diseases and pests (Wang & Doudna, 2023). In recent years, scientists have turned their attention to addressing the challenges posed by climate change on agriculture. Rising temperatures can cause significant damage to plant cell structures, potentially leading to crop failure and subsequent food shortages (Li et al., 2022). To combat this issue, researchers are exploring innovative applications of CRISPR technology to develop crops with increased heat tolerance, allowing plants to withstand higher temperatures and maintain productivity in changing climates (Li et al., 2022). This research not only aims to ensure food security but also addresses the potential increase in pest populations that often accompanies warmer conditions. By creating more resilient crops, scientists hope to mitigate the risks of crop loss and potential food shortages in the face of global climate change, preventing food insecurity.

In medicine, CRISPR has been used to produce T-cells that are used to help people fight cancer (NCI Staff, 2020). Furthermore, in pre-clinical tests, CRISPR has even been used to edit neurons that may assist patients suffering from Huntington's disease (Liu et al., 2021). CRISPR has been used to correct disease-causing mutations in cell lines and animal models of genetic diseases like Duchenne muscular dystrophy, hemophilia, and sickle cell anemia. CRISPR has also been used to disrupt viral DNA and genes from HIV (Liu et al., 2021). Most notably, in December 2023, the U.S. Food and Drug Administration approved Casgevy, the first CRISPR-based gene therapy for treating sickle cell disease in patients 12 years and older. This approval represents not only a breakthrough treatment for the approximately 100,000 Americans living with sickle cell disease, but also marks the first FDA-approved therapy utilizing CRISPR/Cas9 genome editing technology (FDA, 2023).

In the future, CRISPR has a variety of use cases that may help both people and plants. By editing mutations, CRISPR may be used to treat an even wider range of diseases than it currently is used for. Additionally, more complex diseases such as heart disease and diabetes may be ameliorated by CRISPR as the technology develops. For parasitic insect populations, CRISPR may be utilized in gene drives, a genetic engineering technique that increases the likelihood of a specific gene being inherited, to keep the insect population count in check (YAN et al., 2023).

While CRISPR is a groundbreaking technology with the potential to help millions, if not billions, of people, there are potential risks and side effects to consider with its use. CRISPR is incredibly precise, but there is always the risk of editing unintended gene targets, leading to undesirable consequences (Kozovska et al., 2021). For instance, in some animal trials, researchers have found that CRISPR caused large chromosome rearrangements and even complete deletions (Rezza et al., 2019). However, researchers could modify the protein itself to prevent unwanted edits or improve delivery methods for Cas9. As with any new technology, especially those that edit the genes of humans, we currently do not know the potential long term side effects of CRISPR treatment.

While CRISPR is thought of as being a potential treatment for cancer, if a gene cut occurs in a tumor suppressor gene, it could exacerbate the cancer or cancer could develop in a cancer-free patient. More complex edits like inserting longer gene sequences into a patient's DNA could unintentionally disrupt other genes. There is a reason why CRISPR is undergoing extensive review before its widespread adoption. Scientists are looking to improve CRISPR's specificity to curtail any potential risks to patients. The potential for short term and long term side effects from CRISPR must be monitored as research further continues on the gene-editing technology.

However, one controversial potential use of CRISPR, one that has been debated by many, is its potential to be used for the enhancement of human embryos and fetuses to facilitate desirable traits (Waddington et al., 2016). The editing of human embryos and fetuses is one concern ethicists have regarding the further advancement of CRISPR technology. Some may argue that by using CRISPR technology to create "perfect" offspring, scientists and parents are, in a sense, playing "god" (Locke, 2020).

Of course, the enhancement of humans in this sense may also undermine the work that the scientists currently working on CRISPR technology are setting out to do. CRISPR may be used to treat a variety of diseases, making it a therapeutic gene editing tool. By utilizing CRISPR for the sake of enhancing humans, the purpose of CRISPR shifts from being therapeutic to being vain. This gene editing of unborn humans could even undermine what it means to be human, as engineering "designer" babies could compromise the individual qualities that contribute to the uniqueness of each human being.

Section 3 - Stem Cells

Stem cells are non-specialized cells that possess the capacity for self-renewal and differentiation into specialized cell types (Aly, 2020). Both adults and embryos contain stem cells, but there are multiple stages of specialization (Zakrzewski et al., 2019). Stem cells can be classified based on their potency- the range of cell types they can differentiate into. Pluripotent stem cells, found only in embryos, have the ability to differentiate into any of the three germ layers that make up the entire embryo and developing fetus (Volarevic et al., 2018). These pluripotent cells are the most versatile and can potentially form any cell type in the body, from heart cells to brain cells. A step below pluripotent are multipotent stem cells, which can differentiate into a related family of cells, such as blood cells arising from hematopoietic stem cells in bone marrow (Zakrzewski et al., 2019). Unipotent can only differentiate into one type of cell (Zakrzewski et al., 2019).

The key difference between stem cells in younger humans as opposed to older humans is the number of stem cells available. Stem cells are more plentiful earlier in development. As development proceeds, the number of stem cells available drop in quantity and quality (HSCI,

2020). This can also be seen with tissue regeneration in younger people. Due to their more robust stem cell pool, younger people tend to heal quicker than more mature adults (Ahmed et al., 2017). Overall, stem cells are useful in forming tissue and developing the bodies of younger humans, but for adults the stem cells essentially go into a “maintenance” mode, where they primarily focus on replacing damaged cells.

Currently, stem cells can be effectively used in a variety of ways. Researchers are constantly tinkering with stem cells and their biology to develop new stem cell therapies for patients. Additionally, the reactive nature of stem cells can be used in research that could further help humans outside of stem cell therapy. Stem cells can be used to model diseases in isolated dishes, and these same stem cells can be used to test new medications before trial on humans (Moradi et al., 2019).

There is a large variety of stem cell therapies already available to the public. Stem cell therapy has been used in bone marrow transplants, gene therapy, and cell replacement therapy for some serious ailments, such as Parkinson’s disease (Yamanaka, 2020). So far, stem cells have been successful for trials in which they have been employed. Stem cell therapy-based tissue regeneration for such conditions as burn injuries has proven to be quite effective (Wang et al., 2021). Hematopoietic stem cell transplants are often used to treat blood disorders in patients (Yamanaka, 2020). There are promising signs of stem cells’ potential in the future too. Stem cells are being used in trials to treat vision and heart disease ailments, but these therapies are likely years away (Yamanaka, 2020). Stem cells have been very effective thus far in treating patients and will definitely be further developed in the future.

The most common type of stem cells used in stem cell therapy are adult stem cells (Zakrzewski et al., 2019). These stem cells exist in various tissues throughout the human body, even into adulthood. Because of the nature of who they are being extracted from, these stem cells harbor less ethical concerns than embryonic stem cells. Adult stem cells are typically used to repair human tissue throughout the body. These adult stem cells have proven useful for treating conditions like leukemia, in which damaged blood cells can be replaced with healthy stem cell transplants from the patient's own marrow or a matched donor (Yamanaka, 2020). A limitation of adult stem cells is their low differentiation potential, meaning they can only differentiate into a limited number of cell types compared to embryonic stem cells, which have high differentiation potential and can become almost any cell type in the body (Zakrzewski et al., 2019). This isn’t a roadblock for leukemia because only hemetopoietic stem cells are mutated or dysregulated, and these are precisely the cells that stem cell therapy aims to replace. Other diseases like blood or liver disorders require more than one cell type.

Embryonic stem cells are a more controversial type of stem cell being employed in stem cell therapy. As the name suggests, these stem cells are derived from embryos, which has drawn the ire of many activists (Aly, 2020). These stem cells are desirable because of their unlimited differentiation potential, which is due to their pluripotent nature (Moradi et al., 2019). Embryonic stem cells are typically used to treat a variety of conditions, and they are quite malleable overall. However, embryonic stem cells do come with ethical concerns and drawbacks. This type of research involves the destruction of human embryos, which consequently lose their potential to develop into a human being (Lo & Parham, 2009). Furthermore, direct implantation of embryonic stem cells has a higher risk of causing tumors in patients (Volarevic et al., 2018).

Induced pluripotent stem cells (iPSCs) have the same defining characteristic as embryonic stem cells, which is their pluripotent nature. These stem cells may share the same

benefit as embryonic stem cells, but they avoid the aforementioned ethical concerns because of how they are derived. To create these stem cells, scientists take mature somatic cells, such as skin or blood cells, and reprogram them into a pluripotent state (Moradi et al., 2019). Because of this, scientists can actually take the cells of a specific patient, modify them, and then use them in stem cell therapy. This could help reduce negative immune response because the cells being transplanted originally came from the patient themself. As opposed to adult stem cells though, induced pluripotent stem cells (IPSCs) carry a higher risk of causing tumors in patients if not properly differentiated before therapeutic use. This is because IPSCs are pluripotent and can become any cell type, so researchers must first direct their differentiation into the desired cell type (such as a heart cell or neuron) before administering them to patients. If undifferentiated IPSCs are injected, they may continue dividing and developing into a tumor mass (Volarevic et al., 2018).

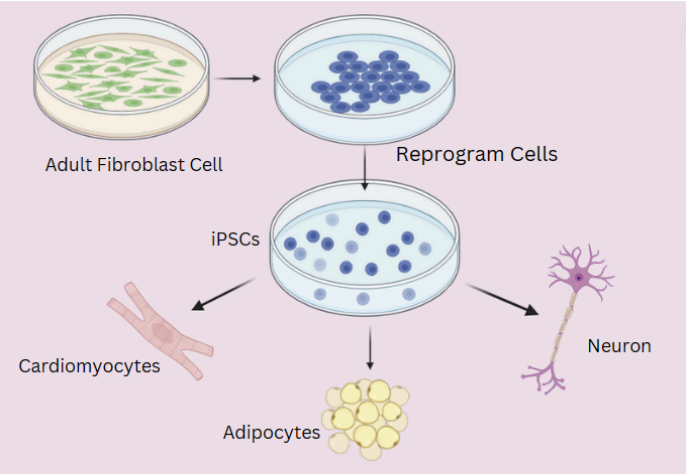

Figure 2: Schematic of Induced Pluripotent Stem Cells (iPSCs). Fibroblasts are reprogrammed into induced pluripotent stem cells, which can differentiate into diverse cell types like neurons, cardiomyocytes, and adipocytes. Created using biorender.

Section 4 - CAR T

Chimeric Antigen Receptor T (CAR T) cell therapy is a revolutionary cancer treatment that has been remarkably effective in treating certain types of blood cancers, namely B-cell leukemias and lymphomas (Sengsayadeth et al., 2021). CAR T-cell therapy re-engineers a patient's own T-cells, which are responsible for issuing the body's immune responses. Through the treatment, T-cells are genetically modified in a way that expresses a CAR on their surface, which enables CAR to recognize and target specific antigens (Gross et al., 1989). CAR T-cell therapy is also malleable. CAR T-cells can be engineered to target quite a few antigens, as they are both flexible and customizable in nature.

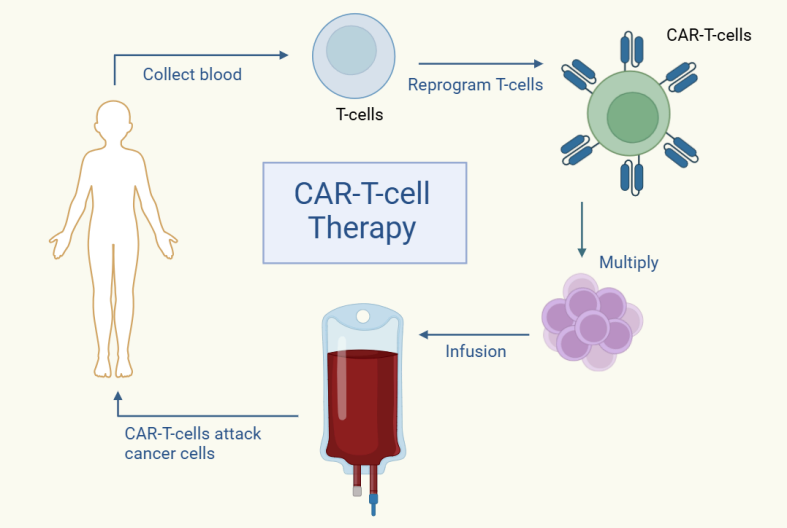

Figure 3: Schematic of CAR T-cell Therapy. CAR T-cell therapy transforms a patient's own T-cells into cancer-fighting agents through genetic engineering, cell expansion, and reinfusion. Created using biorender.

T-cells are a type of lymphocyte, a white blood cell that plays a central role in the body's immune response. There are two major types- CD4+ helper T-cells that coordinate the immune response when they detect an infection, and CD8+ cytotoxic T-cells that can directly kill infected or cancerous cells. Chimeric antigen receptor (CAR) T-cell therapy is a pioneering form of cancer immunotherapy that involves genetically engineering a patient's own T-cells to recognize and kill cancer cells (Sengsayadeth et al., 2021). An antigen-presenting cell (APC), most commonly a macrophage, recognizes foreign substances and presents their proteins to CD4+ helper cells. These helper cells then release interleukins, which are messenger chemicals that activate CD8+ cells.

The process of making CAR T-cells begins by collecting a patient's T-cells through leukapheresis and separating out the desired T-cell types (separating everything but white cells to get only T-cells). These T-cells are then activated and genetically modified by introducing a CAR gene construct. The CAR construct encodes an artificial receptor protein that binds to a specific antigen on the patient's cancer cells. This allows the engineered CAR T-cells to selectively recognize and target those cancer cells. Viral vectors, such as lentivirus and gamma retrovirus, are used to carry engineered DNA into a person's genome, specifically targeting T-cells. These vectors edit the T-cells but do not directly kill the cancer cells.

Once the CAR gene is integrated into the T-cells' genomes through reverse transcription, the engineered CAR T-cells can express the chimeric receptor on their surface. The receptor has an extracellular antigen-binding domain that attaches to the cancer cell, a transmembrane domain, and intracellular signaling domains that activate the cytotoxic machinery in the CAR T-cell. This allows the CAR T-cells to bind and kill cells displaying the targeted cancer antigen. Following expansion to increase numbers, the CAR T-cells are infused back into the patient to seek out and eradicate the cancer cells. The result of this is a treatment that is highly effective at getting rid of cancer cells, with response rates hitting up to 90% after 6 months in patients with certain types of B-cell leukemias and lymphomas (Maude et al., 2018).

Considering the effectiveness of CAR T-cell therapy on B-cell leukemias and lymphomas, researchers have set out to adapt the therapy for other cancer cells. The flexibility and customizability of CAR T-cell therapy make it a promising approach for treating various forms of cancer, beyond just blood cancer (Newick et al., 2017).

On the other hand, the treatment may cause toxicities within patients like cytokine release syndrome and neuroinflammation, both of which can be devastating if not fatal (Sterner & Sterner, 2021). Aside from the potential health risks the therapy poses, the therapy's novelty has established its high cost for prospective patients. CAR T-cell therapy requires specialized treatment centers, and the manufacturing of the cells is time-consuming and expensive. While we have been studying CAR T since the 1990s, we are unsure about the long term safety of CAR T-cell therapy. CAR T-cell therapy seems like a major step forward for helping those with blood cancers, but its side effects and high cost warrant much further research before the therapy is widely adopted.

Recently, CAR T-cell therapy trials have been rather successful. One such trial is when Stanford researchers engineered T-cells from 38 patients with large B-cell lymphoma, the cancer had shrunk in 68% of the patients and 53% had a complete response (Conger, 2024). However, this therapy remains unpromising for chronic lymphocytic leukemia (CLL) due to a 30% response rate (Mitra et al., 2023). There is a lot of variability in these results, and when this is coupled with the risks of CAR T-cell therapy, it may be useful to instead focus on the future prospects of CAR T-cell therapy and what it may mean for those afflicted with illnesses it may treat. Scientists have been working on CAR T-cell products that are not derived from the patient.

Researchers are exploring new approaches to CAR T-cell therapies, including using T-cells from healthy donors instead of patients, creating "off-the-shelf" CAR T-cell products (Li et al., 2020). This approach has the potential to provide readily available treatments, eliminating the need for patient-specific manufacturing and reducing treatment delays. They are utilizing gene-editing tools like CRISPR to induce donated T-cells to produce CARs, eliminating the need for viral vectors. Additionally, some trials are testing CAR therapies using natural killer cells (NK) rather than T-cells (Mitra et al., 2023). NK cells are a type of lymphocyte that recognize and eliminate infected, cancerous, and stressed cells. Researchers are also investigating techniques to manufacture CAR therapies inside the patient's body through nanotechnology and mRNA approaches, circumventing the need for external manufacturing.

Additionally, to increase effectiveness, researchers have been trying to produce CAR T-cells that target multiple antigens in order to mitigate the risk of treatment failure via antigen escape. CAR T-cell therapy may be a bit risky as of now, but this risk can be ameliorated by combining the therapy with other gene modification therapies such as CRISPR.

Section 5 - Integration

Combining the three powerful technologies of CRISPR gene editing, induced pluripotent stem cells, and CAR T-cell therapy could overcome some of the barriers for each of them individually. This approach has the potential to revolutionize personalized medicine, improving accessibility, efficacy, and safety for patients. In doing so, the convergence of these cutting-edge technologies paints a clearer picture of what the future of medicine may look like, with more effective, widely available, and precisely targeted cellular therapies on the horizon.

iPSCs and CRISPR

Utilizing iPSCs and CRISPR technology together can facilitate some important scientific breakthroughs. One application of stem cell technology is using iPSCs to create cellular models for drug screening and testing. By taking a sample like a skin or blood cell from a patient, scientists can reprogram those somatic cells back into a pluripotent stem cell state. These iPSCs can then be differentiated into specific cell types affected by the disease of interest, such as liver, heart, or neuronal cells. Potential drug compounds can be applied to these disease-relevant cell models to assess efficacy and toxicity before costly clinical trials. Additionally, CRISPR gene editing technology can be employed to introduce specific mutations into these iPSCs, allowing researchers to evaluate drug efficacy across various genetic backgrounds (De Masi et al., 2020). This process typically involves first testing the drug on unmodified cells, then progressively introducing common disease-associated mutations (De Masi et al., 2020). If the drug remains effective across these genetic variations, it suggests a higher likelihood of success in diverse patient populations. This variability underscores the potential for personalized medicine approaches, where iPSCs and CRISPR technologies could be used to develop tailored treatment plans based on an individual's unique genetic profile.

Another powerful use of stem cells, particularly iPSCs, is in the study of genetic disorders, such as Huntington's disease, caused by mutations. Many diseases arise from point mutations affecting a single base pair, while others involve frameshift mutations that shift the entire reading frame. By sequencing patient genomes, researchers can identify disease-associated mutations that cause loss or gain of protein function. Gene editing tools like CRISPR allow scientists to precisely correct these mutations in iPSC lines and model the impact on cellular behavior and disease pathology. The ability to generate isogenic cell lines that are genetically matched except for the disease-causing mutation enables better understanding of disease mechanisms and personalized drug screens (Ben Jehuda et al., 2018).

A third application combines iPSCs and CRISPR for *in vivo* gene therapy approaches. Here, patient-derived iPSCs are first gene-edited using CRISPR to correct a disease-causing mutation. The corrected iPSCs are then differentiated into the target cell type, such as hematopoietic stem cells for blood disorders or insulin-producing islet cells for diabetes. These genetically repaired cell products can potentially be transplanted back into the same patient they were derived from, circumventing immune rejection issues. While still an emerging field, this personalized cell therapy opens new avenues for treating genetic diseases and regenerating damaged tissues or organs.

iPSCs hold great promise for regenerative medicine, but they can potentially lead to cancer if not produced and handled properly. The cancer risk is associated with the activation of oncogenes, which are normally turned off in healthy cells, and the inactivation of tumor suppressor genes, which typically prevent uncontrolled cell growth. To mitigate this risk, researchers are exploring the use of gene editing techniques like CRISPR to turn off oncogene signaling in iPSCs. This approach could help create safer iPSC-derived therapies by reducing the chances of unintended tumor formation.

CAR T-cell therapy and CRISPR

Biologics are a class of medicines made from living organisms in a laboratory setting, representing a shift from traditional small-molecule drugs to therapeutic proteins and other biological molecules, such as CAR T-cell therapy (Morrow & Felcone, 2004). This therapy has the potential to be very effective, but it is not without the problems and risks that it may pose to

patients. CAR T-cell therapy can trigger a dangerous cytokine storm when T-cells are infused into the patient's body. This overproduction of cytokines can lead to severe inflammation, blood pressure drops, and kidney damage. To mitigate this risk, researchers are exploring the use of CRISPR gene editing to incorporate "suicide genes" into CAR T-cells, which can shut down the process if cytokine levels become excessive (Li et al., 2020). Additionally, CRISPR can enhance CAR T-cell efficacy by removing extraneous receptors on the T-cell surface that may interfere with the chimeric antigen receptor's ability to bind to cancer cells (Li et al., 2020). This precise editing ensures that the engineered T-cells have only the desired receptor, potentially improving their cancer-fighting capabilities while reducing off-target effects.

iPSCs and CAR T-cell therapy

Combining iPSC and chimeric antigen receptor technologies can provide doctors and scientists with a new source of T-cells, each with their own pre-determined antigen for cancer immunotherapy. One recent study involved researchers deriving T-cells from iPSCs. The researchers then genetically engineered these cells to express a CAR targeting the CD19 antigen, which can be found in the majority of leukemias and lymphomas (Themeli et al., 2013). The results proved to be positive, as the iPSC-derived CAR T-cells delayed tumor progression to a similar extent as normal CAR T-cell therapy, for B-cell leukemias and lymphomas. This treatment is not fully developed just yet, but it shows promise for the near future.

Section 6 - Conclusion

This paper has discussed the benefits and drawbacks of CRISPR, stem cell therapy, and CAR T-cell therapy. These treatments will revolutionize medicine, and have already begun to do so. Each of these treatments offers unique ways to modify human cells and genetic data to treat disease. CRISPR is used as a tool, allowing doctors to precisely edit genetic mutations. The differentiation and pluripotent nature of stem cells can facilitate regenerative and restorative treatments. And lastly, CAR T-cells may be a powerful tool for effectively battling certain types of cancer.

To enhance these treatments, and ameliorate their potential drawbacks, they may be combined to optimize their potential. CRISPR can be used as a tool to modify CAR T and stem cells, and by doing so they are made safer and more powerful. The future of medicine may very well be defined by the customization and combination of these treatments, as a patient’s cells and genes are edited to heal their ailment.

As discussed throughout the paper, these treatments unfortunately carry risks that must be prevented before widespread adoption. With that being said, the potential to help millions by curing diseases is too important to ignore. If pursued with these risks in mind, these treatments, and the combination of these treatments, may offer breathtaking possibilities to doctors. The door for a new era of modern medicine has been opened, and it is up to researchers to step through it.

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