

# **CRISPR Solution to Cure Acute Myeloid Leukemia**

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## **Abstract**

Leukemia is a type of cancer that affects the blood and bone marrow, the spongy tissue inside bones where blood cells are produced. In leukemia, there is an overproduction of abnormal white blood cells, which are produced in the bone marrow and accumulate in the blood, disrupting normal blood function. The abnormal white blood cells, called leukemic cells, are unable to perform their usual function of fighting infections and protecting the body from diseases, and instead, they crowd out healthy white blood cells, red blood cells, and platelets, leading to anemia, infections, and bleeding.

There are several types of leukemia, including acute lymphoblastic leukemia (ALL), acute myeloid leukemia (AML), chronic lymphocytic leukemia (CLL), and chronic myeloid leukemia (CML). ALL and AML affect immature white blood cells that have not fully developed, while CLL and CML affect mature white blood cells that have developed more fully. ALL is more common in children, while AML is more common in adults.

Leukemia can be acute or chronic, depending on how quickly the disease develops and progresses. In acute leukemia, the abnormal white blood cells multiply rapidly, and the disease progresses quickly, sometimes over a matter of weeks or months. Acute leukemia requires immediate treatment because the uncontrolled growth of leukemic cells can quickly lead to life-threatening complications. In chronic leukemia, the disease develops slowly, sometimes over many years, and the abnormal white blood cells accumulate gradually. Chronic leukemia may not cause symptoms for a long time, and it may be discovered incidentally during a routine blood test.

Acute leukemia is identified by the rapid proliferation of immature, abnormal white blood cells in the bone marrow and blood. In contrast, chronic leukemia is identified by the accumulation of mature, abnormal white blood cells in the blood and bone marrow.

The exact cause of leukemia is unknown, but several factors may increase the risk of developing the disease. These factors include exposure to radiation, certain chemicals, and viruses such as the human T-cell leukemia virus (HTLV-1) and the Epstein-Barr virus (EBV). Genetic factors may also play a role in the development of leukemia, as some types of the disease are more common in families.

The symptoms of leukemia can vary depending on the type of leukemia and the stage of the disease. Common symptoms include fatigue, weakness, fever, night sweats, weight loss, and frequent infections. Other symptoms may include bone pain, swollen lymph nodes, and easy bruising or bleeding.

Diagnosis of leukemia usually involves a physical examination, blood tests, and a bone marrow biopsy. In a bone marrow biopsy, a small sample of bone marrow is removed and examined under a microscope for abnormal cells. Additional tests, such as imaging studies, may also be performed to determine the extent of the disease.

Treatment for leukemia depends on the type of leukemia, the stage of the disease, and the age and overall health of the patient. Treatment options may include chemotherapy, radiation therapy, stem cell transplantation, or targeted therapy. Chemotherapy involves the use of drugs to kill cancer cells, while radiation therapy uses high-energy radiation to destroy cancer cells. Stem cell transplantation involves replacing damaged bone marrow with healthy stem cells from a donor. Targeted therapy involves the use of drugs that target specific proteins or genes that are involved in the growth and spread of cancer cells.

Acute leukemia is generally more difficult to treat than chronic leukemia, and the prognosis for advanced stages of the disease is generally poorer. However, advances in the treatment of leukemia have led to improved survival rates in recent years. In conclusion, leukemia is a type of cancer that affects the blood and bone marrow. It is characterized by the uncontrolled growth of abnormal white blood cells, which interfere with the normal functioning of the immune system. The exact cause of leukemia is unknown, but several factors may increase the risk of developing the disease. Treatment for leukemia depends on the type of leukemia, the stage of the disease, and the age and overall health of the patient. While leukemia can be a serious and life-threatening disease, advances in treatment have led to improved

survival rates in recent years. Early detection and prompt treatment are important for improving outcomes in patients with leukemia. Your chance of getting leukemia is strongly related to your age. Around 1.9 million people get cancer each year and roughly 680,000 people die of cancer each year. What can cause cancer? There are several factors that can contribute to the development of leukemia, including exposure to chemicals, radiation, and viruses. Here's how each one can increase the chance of getting cancer.

Exposure to certain chemicals can cause cancer because they alter the cell's DNA. Any chemical that raises the chance of getting cancer is called a Carcinogen. DNA is made up of four base pairs: Adenine (C<sub>5</sub>H<sub>5</sub>N<sub>5</sub>), Thymine (C<sub>5</sub>H<sub>6</sub>N<sub>2</sub>O<sub>2</sub>), Cytosine (C<sub>4</sub>H<sub>5</sub>N<sub>3</sub>O), and Guanine (C<sub>5</sub>H<sub>5</sub>N<sub>5</sub>O). These base pairs are held together by hydrogen bonds that allow them to hold together the sugar and phosphates. One of the ways that carcinogens alter your DNA is through Carcinogen Adducts. Carcinogen adduction is when a carcinogen, such as benzene (C<sub>6</sub>H<sub>6</sub>), forms a covalent bond with the oxygen or nitrogen parts of DNA during cell division. During cell division, the cell cuts the DNA in half. This is when the benzene (C<sub>6</sub>H<sub>6</sub>) will form a covalent bond with one of the four base pairs and can generate H<sub>2</sub>O<sub>2</sub> which can lead to apoptosis or AML. Benzene is found in plastics, synthetic fibers, rubber, and other materials. It is also found in gasoline, cigarette smoke, and some cleaning products.

What is CRISPR?

CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) is a revolutionary gene-editing technology that has transformed the field of genetic engineering. It was discovered in the late 1980s in the immune systems of bacteria that were fighting off viruses such as bacteriophages. Bacteriophages inject their DNA into the bacteria to hijack the bacteria so that the bacteriophage can make more of itself. But scientists discovered that the bacteria's immune system could edit the phage's

DNA and render it useless.

The CRISPR system consists of two main components: the Cas9 enzyme and a guide RNA (gRNA). The Cas9 enzyme acts like a pair of molecular scissors, cutting the DNA at a specific location within the genome. The gRNA serves as a sort of GPS, directing the Cas9 enzyme to the precise location within the DNA that needs to be edited.

The way CRISPR works is that the guide RNA is designed to recognize a specific sequence of DNA within the genome. Once it binds to that sequence, the Cas9 enzyme is activated and cuts the DNA at that specific location. This creates a double-stranded break in the DNA, which triggers the cell's natural repair mechanisms to fix the break. Depending on the type of repair mechanism used, the cell can either repair the DNA with an error-prone process called non-homologous end joining (NHEJ), or it can repair the DNA using a more precise process called homology-directed repair (HDR). Using CRISPR, scientists can introduce specific changes into the genome of an organism, such as deleting or adding genes, changing the expression of genes, or correcting genetic mutations that cause disease. This technology has the potential to revolutionize medicine by allowing researchers to create targeted therapies for a variety of genetic disorders.

Despite its many potential benefits, CRISPR is not without its risks and ethical concerns. There is the possibility of off-target effects, where the Cas9 enzyme cuts DNA at unintended locations, potentially causing unintended consequences. In conclusion, CRISPR is a powerful gene-editing technology that has the potential to revolutionize medicine, agriculture, and many other fields. Its ability to precisely and efficiently edit the genome has opened up new possibilities for targeted therapies and genetic engineering. However, its use also raises important ethical and safety concerns that must be carefully considered and addressed.

Solution.

In CRISPR gene editing, a CRISPR solution refers to the combination of the Cas9 enzyme and a guide RNA (gRNA) that is used to target a specific sequence of DNA for editing. The CRISPR solution is delivered into cells, where it can introduce changes to the genome, such as deleting or adding genes, altering the expression of genes, or correcting genetic mutations that cause disease. The CRISPR solution is typically created by combining the Cas9 enzyme with a custom-designed guide RNA that is complementary to the DNA sequence to be targeted. The guide RNA is designed using computational tools to ensure specificity and avoid off-target effects. One of the challenges in developing a CRISPR solution is ensuring that the guide RNA is specific enough to target the desired DNA sequence without binding to other, unintended sequences in the genome. To address this challenge, researchers use computational tools to predict potential off-target sites and experimental methods to assess the specificity of the CRISPR system.

Validation

So, what is the proof that any of this works? Well, several things determine whether a gRNA sequence would be

effective. One of those factors is called on target score. An on-target score in CRISPR refers to a measure of the accuracy of the CRISPR-Cas9 system in targeting the desired DNA sequence for editing. When using CRISPR to edit an organism's genome, it is important to ensure that the Cas9 enzyme and guide RNA are only targeting the intended location within the genome, and not accidentally cutting or modifying other parts of the DNA. The on-target score is usually calculated by examining the sequence of the DNA that was targeted by the gRNA and determining how many potential off-target sites exist within the genome that could also be affected by the CRISPR-Cas9 system. A higher on-target score indicates that the system is more specific in targeting the desired DNA sequence, while a lower score indicates a higher risk of off-target effects. An off-target score refers to the potential for the Cas9 enzyme to cut DNA at unintended locations in the genome. The guide RNA used in CRISPR is designed to recognize a specific sequence of DNA, but there is always the possibility that it may bind to similar or related sequences elsewhere in the genome, leading to off-target effects.

Off-target effects can have unintended consequences, such as disrupting normal gene function or causing genetic mutations that lead to disease. To minimize the risk of off-target effects, researchers use computational tools to predict potential off-target sites and experimental methods to assess the specificity of the CRISPR system.

The off-target score is a measure of the likelihood that the CRISPR system will cut DNA at unintended locations. A lower off-target score indicates a higher degree of specificity, which is important for ensuring the safety and efficacy of CRISPR-based therapies and genetic engineering. The sequence I present has a high on-target and off-target score, meaning it will be effective on the gene but it will cause very little to no damage to the surrounding genes. In conclusion, it is effective while posing minimal risk to the patient.