

# The sickle cell anemia biology essay

[Science](#), [Biology](#)



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\n[/toc]\n \nSickle cell anemia is a genetic blood disorder affecting the protein hemoglobin, specifically the beta-globin gene. The beta-globin gene makes up part of the hemoglobin. The sickling occurs because of a mutation in the hemoglobin gene. Sickle hemoglobin causes the cells to develop a sickle shape, because when oxygen is released from the sickle hemoglobin, it sticks together and forms long rods, which damage and change the shape of the red blood cell. D: DropboxGrade 10MYP Coordinated Science A IGCSE (G10)Genetic diseases OWEPicsSickelcells2. jpg(What Is Sickle Cell Anemia?)Their lifespan is very short compared to normal round red blood cells, which leads anemia, a low number of red blood cells. A normal red blood cell lives for about 120 days in circulation, whereas a sickle cell lives for only 10 to 20 days. The red blood cells are stiff and not flexible so it may block the blood flow by getting stuck in the blood vessels causing pain (called crisis) to the affected person. Some more symptoms could be swollen hands and feet, frequent infections, delayed growth and vision problems. Sickle cell anemia is caused by inherited genes, where people with the disease are born with two abnormal genes of the sickle cell, one from each parent. However, if the person is born with one normal gene and one sickle

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cell gene, will have a condition called sickle cell trait, meaning that he won't have the disease but may have the chance to pass it on to his children (Sickle cell disease).(Sickle Cells Symptoms)D: DropboxGrade 10MYP Coordinated Science A IGCSE (G10)Genetic diseases OWEPicssickle\_cell\_disease. gif(Sickle Cells - Inherited)

## Scientific Solution

Gene therapy is being developed in order to achieve a better cure for sickle cell anemia.(Gene Therapy - Vector Inject)Gene therapy, through viral method, allows compensation for abnormal genes, by inserting genetic material into cells. It may be able to place a normal copy of the gene to restore function if mutated causes a vital protein to be faulty. It works by inserting a carrier called a vector which is genetically engineered to bring the normal gene. Some viruses are used as vectors because they can infect cells, allowing delivery of the new gene. The viruses are modified so no disease is caused when used (Pg. 99 Jones, Phill). Some types of viruses used are retroviruses and adenoviruses. Retroviruses can integrate into chromosomes of host cells. Adenoviruses presents their DNA into the nucleus of the cell, but is not integrated into a chromosome. The vector can be injected or given through an intravenous therapy (infusion of liquid substances directly into a vein) directly into a specific tissue in the body, or a sample of the patient's cells can be removed and exposed to the vector under laboratory conditions. " Infected" cells containing the vector are then returned to the patient. In case of success, the new gene will make a functioning protein (How Does Gene Therapy Work?). The diagrams represent the process of the gene therapy; the extraction of the cells from

patient and the vector being inserted into the cells. D: DropboxGrade 10MYP Coordinated Science A IGCSE (G10)Genetic diseases OWEpicsl\_gene.therapy-ms. gifD: DropboxGrade 10MYP Coordinated Science A IGCSE (G10)Genetic diseases OWEpicsimage. jpgGene therapy has successfully cured mice with the sickle cell disease. St. Jude Department of Hematology, have done some experiments on mice and has observed that the treated mice showed no anemia, their organ function was basically normal, and little could be detected of the disease (Gene Therapy Corrects Sickle Cell Disease In Laboratory Study).(Gene Therapy Using Adenovirus Vector)

## **Aspect 2**

### **Benefits**

There are many options to reduce the symptoms by the use of life-long intake of medications, like antibiotics, pain-relieving medications and hydroxyurea (reduces frequency of painful crisis), blood transfusions to increase red blood cells count and many more. However, this does not get rid of the problem; it only eases the symptoms on the patient. Bone marrow transplant (BMT) offers a potential cure for the disease, nevertheless, it is hard to find donors, and the procedure has severe risks, including death. Gene therapy has fewer risks, no donor is really needed, and is less intensive than BMT where no chemotherapy is needed. Thus, the use of gene therapy allows the diminish of the disease, bringing in new gene which are normal, sickle cell disease free, allowing development of a normal hemoglobin. Unlike BMT, gene therapy can also prevent the defective genes from being inherited by offspring, if it targets the reproductive cells of the carriers, which on a bigger scale could obliterate it completely; this is called germline

gene therapy. (Pros and Cons). According to Sciencedaily. com, " Although the scientists caution that applying the gene therapy to humans presents significant technical obstacles, they believe that the new therapy will become an important treatment for the disease."

## **Detriments**

As a result of gene therapy being in its early stages of development, the understanding and knowledge of the safety of the treatment is undetermined. It is mostly based on theory than facts; however research and practice can be further improved. In addition to that, it is not 100% guaranteed that the " infected" gene with the normal gene will end up at the wanted place, which might end in causing even more harm to the genetic structure (Pros and Cons). If foreign cells are used in the therapy the immune system will attack it, reducing the effectiveness of the therapy. As well as the immune system responding to already seen " invaders" will make it difficult to repeat the therapy. When a multi-gene disorder is present, such as heart disease, high blood pressure, Alzheimer, and diabetes, caused by the combined effects of variations in many genes, makes it very difficult to treat using gene therapy. When integrating the DNA into the wrong place in the genome (the haploid set of chromosomes), such as a tumor suppressor gene, it could induce a tumor. Death has occurred to patients put in trial of gene therapy, when DNA has been integrated in the wrong place (Gene Therapy Issues).

### Aspect 3

For human beings is it acceptable to manipulate human gene? It seems like that kind of action is like playing " God", by changing the original aspects of a human, which leads to control your faith/future. It is also playing with nature which is something dangerous, where we don't have the rights nor the knowledge and skills to do. However, when you consider that gene therapy will allow saving lives of humans, decrease number of sick people and inherited diseases, and it provides an opportunity for the potential and current diseased people to live a normal life (Moral and Ethical Issues in Gene Therapy). Focusing on a specific example, people will have the choice to use gene therapy on their embryos incase during pregnancy tests show presence of genetic disease. A positive impact would be that there will be fewer infants born with genetic disease. On the other hand, people will have the opportunity to manipulate new born characteristics, based on criteria that are morally wrong, for example imagine that people would decide that only blonde children should be born. This would mean that any child with black will be an outcast; this is unacceptable. Hence, ethical rules should be created as a foundation of using gene therapy (What Are the Ethical Issues Surrounding Gene Therapy?). As the gene therapy evolves from the scientific perspective it must be accompanied by strong ethical regulations, to make sure that there is an international agreements on those rules. Throughout the years a lot of money has been invested into the development and research of gene therapy, thus, when it will be available for the public it will most likely be very expensive. According to the Santa Clara University (SCU), the cost per individual might be as high as \$100, 000. Each year, National

Institutes of Health provides around \$200 million of support for gene therapy. Hence, not everybody will be able to afford the therapy (Access to Gene Therapy). On the other hand, less will be spent on the drugs required for the disease, letting the economy to save more money, which can be used for better purposes, such as investing in developing the economy and improving our life (The Economic Impact of the Genomic Revolution's Failure). Around 1.1\$ billion annually is the medical care cost in total for people who suffer from sickle cell anemia in the US (Sickle Cell Disease Healthcare Costs High in U. S.). As time goes by, the cost of the therapy as usual will become cheaper, therefore, more people will be capable to afford the therapy. Hence, less people will be sick and the dependency ratio will decrease, granting benefits to the economy and improving the lives of everyone (The Economics of Gene Therapy and of Pharmacogenetics). Around 120, 000 infants are born with sickle cell anemia every year worldwide; therefore, having many of them cured, and reducing the number of them, will benefit the economy (Sickle Cell Disease - Future). Is it really fair having the limitation of people not being capable of getting the treatment just because it is a financial burden on them, is it morally right?