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Alternative Gene Therapy

The article I am writing about, explains how Bluebird Bio Inc. is developing an alternative gene therapy method that will be an alternative for patients that undergo frequent blood transfusions. The therapy is designed to replace an infected gene in the red blood cells of patients0 with just one blood transfusion. This may reduce the number of annual blood transfusions required for a patient. This may also reduce the cost of drugs required for a patient to take due to the high amount of blood transfusions. Bluebird is developing a plan that will allow the patient to pay for the therapy over a five-year installment plan. Pharmaceutical companies and health insurance providers are seriously considering this approach because of the rising cost of drug treatment and visits to the emergency room.

Beta thalassemia is a prime example of a genetic disease. Beta thalassemia is one of the most common genetic disorders in the world. This disease can cause severe anemia, fatigue, and organ damage. Beta thalassemia is caused by a malfunction in a gene that makes beta globin. This is a protein used by red blood cells to carry oxygen throughout the body. Normal treatment is blood transfusions throughout the life of a patient. However, using this method causes a buildup of iron, which can cause organ damage.

Recent studies have also shown that gene therapy for Beta thalassemia treatment can reduce the number of annual blood transfusions. In a study printed by the New England Journal of Medicine, twelve of thirteen patients stopped receiving red cell transfusions after 26 months of therapy. Three of the patients were completely discontinued from blood transfusion treatments. Very little adverse effects were seen. The article also explains how gene therapy is a safer and more effective method compared to allogeneic hematopoietic-cell transplantation. Hematopoietic-cell transplantation is basically a bone marrow transplant. Because hematopoietic-cell transplantation has risks such as graft-versus-host disease, this method is usually reserved for young children with a sibling donor that is HLA-identical.

Beta thalassemia is just one example of what gene therapy can help treat. The Washington Post article also mentions how Novartis AG is developing gene-therapy for a muscle disorder known as spinal muscular atrophy. Spinal muscular atrophy is a deadly genetic disease that causes atrophy of the skeletal muscles and is often times fatal. The main disadvantage for gene therapy is the cost of the drugs. Although most companies promise a cure, the drugs could cost patients up to one million dollars.

In conclusion, this article is less about the disease Beta thalassemia and more about promoting the cost and effectiveness of gene therapy techniques. Although I was skeptical at first, after researching and finding out how effective the treatment was for Beta thalassemia that gene therapy techniques are going to become more common. This article also tries to promote how gene therapy techniques are branching out and looking to treat, if not cure other diseases that were once thought incurable. It is not only important that we find a cure for these disorders, but also make them affordable to all patients with these ailments.

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