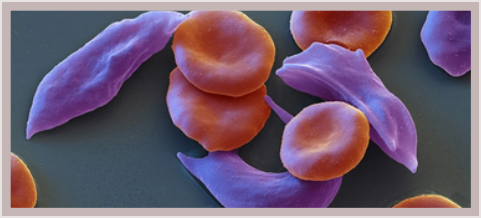
UK First to Approve CRISPR Treatment for Diseases



Britain's medical regulators have approved the world's first gene therapy treatment for sickle cell disease, which could help thousands of individuals. On Thursday, November 16, 2023, the Medicines and Healthcare Regulatory Agency approved Casgevy, which uses the gene editing tool CRISPR that had won a Nobel prize back in 2020. Up until now, extremely arduous procedures that come with harsh side effects, such as bone marrow transplants, have been recognized as the only long-lasting treatment against the diseases. Casgevy was produced by the companies Vertex Pharmaceuticals Ltd. and CRISPR Therapeutics and was approved to treat sickle cell disease and thalassemia patients above the age of 12. Both diseases are caused by gene mutations that carry hemoglobin, which is the protein in red blood cells that carry oxygen. Casgevy works by targeting and editing the gene in bone marrow stem cells, allowing the patient's body to make proper hemoglobin. Patients will first receive chemotherapy before doctors abstract stem cells from their bone marrow, which is then altered in a laboratory with genetic editing techniques. The gene-editing tool is an RNA molecule which guides enzymes to reach the target genes. An instance is that once the Cas9 enzyme reaches the gene known as BCL11A, it cuts both DNA strands to prevent the gene suppressing the formation of hemoglobin in fetuses. The cells are then administered and infused back into the patient for a permanent treatment. After some time, symptoms are often eased with increased oxygen flowing to the patient's tissues.

Endorsing this treatment for sickle cell disease was based on a trial of 29 individuals, 28 of whom reported no severe pain concerns for at least a year following treatment. With thalassemia, 39 of 43 patients required no red blood cell transfusions for at least a year after treatment. The trials are still ongoing, but participants reported experiencing some symptoms such as fever, nausea, and an increased risk of infection without any severe issues.

An official price of the treatment has not yet been established, but experts from the Institute for Clinical and Economic Review estimates that prices around $2 million would be the most cost-effective. Casgevy is currently still in the process of being reviewed by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency who are expected to make a decision by early of December this year. Even if the treatment were to be approved globally, the high cost will likely limit who can benefit from it, making the therapy inaccessible to low- and middle-income populations.

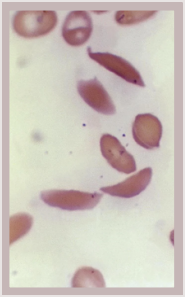
Background Information

Sickle cell disease and thalassemia are both caused by mistakes in the genes which carry hemoglobin, the protein in red blood cells which carry oxygen.

People who suffer from sickle cell disease, common in people with African or Caribbean backgrounds, have a mutation in which cells become crescent shaped like a sickle (hence the name) which can block blood flow and cause extreme pain, damage to the organs, strokes and other severe issues.

Thalassemia, which predominantly people of South Asian, Southeast Asian and Middle Eastern descent, is an inherited blood disorder where the human body does not make enough of hemoglobin. The mutation can cause symptoms like fatigue, shortness of breath and extreme anemia, meaning patients usually require blood transfusions every few weeks with injections and treatment for the rest of their life.

Casgevy uses the gene editing tool of CRISPR, also known as Cas, stands for "Clustered Regularly Interspaced Short Palindromic Repeats". In 2012, it was demonstrated that RNAs could be coded to guide a Cas nucleus to DNA sequences, meaning that DNA could be cut from a specific site to disrupt a targeted gene. It is an extremely powerful tool that also has the potential to insert a new DNA sequence to correct a mutation. It also has a few drawbacks, such as the difficulty to administer into mature cells in large numbers, it not being 100% efficient and the possibility of inaccuracy which may pose severe consequences. There are also ethical concerns, since edits in the human genes not only affects the individual but also their offspring. With this, CRISPR could theoretically be used to enhance desirable traits instead of curing diseases.



Glossary

CRISPR - Technology that researchers and scientists utilize to selectively modify living organisms' DNA, which is adapted from naturally occurring gene-editing processes in bacteria.
Cas9 - A CRISPR associated protein that plays a crucial role in bacterial immunological response against DNA viruses.
BCL11A - A diagnostic indicator and target in human diseases which codes for a C2H2 type zinc-finger protein, that can bind to DNA
Thalassemia - Inherited blood condition caused by the body's inability to produce enough hemoglobin.
Palindromic - Referring to or encompassing of a double-stranded DNA sequence in which the nucleotide sequence is comparable on either side but runs in opposite directions.
Hemoglobin - Protein found in red blood cells that transports oxygen.
Arduous - Involving or requires demanding efforts; difficult and rigorous;

*References on seperate page**