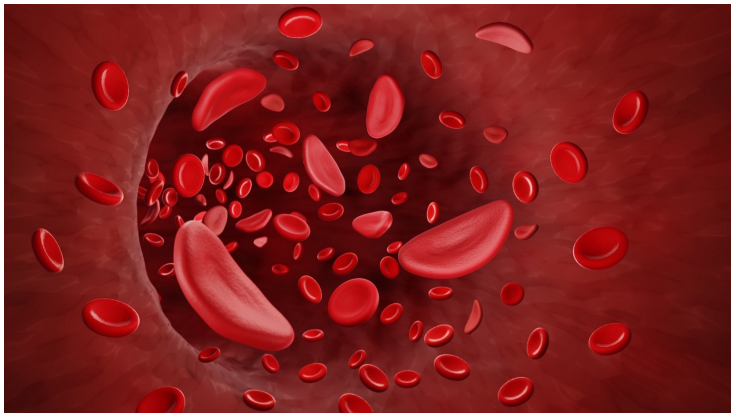
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# US FDA approves first gene therapies to treat patients with sickle cell disease

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**Sickle cell disease is a group of inherited blood disorders affecting approximately 100,000 people in the US**



The US Food and Drug Administration (FDA) has approved two milestone treatments, Casgevy (Vertex Pharmaceuticals Inc.) and Lyfgenia (Bluebird Bio Inc.), representing the first cell-based gene therapies for the treatment of sickle cell disease (SCD) in patients 12 years and older.

Additionally, one of these therapies, Casgevy, is the first FDA-approved treatment to utilise a type of novel genome editing technology, signaling an innovative advancement in the field of gene therapy.

Casgevy, a cell-based gene therapy, is approved for the treatment of sickle cell disease in patients 12 years of age and older with recurrent vaso-occlusive crises. Casgevy is the first FDA-approved therapy utilising CRISPR/Cas9, a type of genome editing technology. Patients' hematopoietic (blood) stem cells are modified by genome editing using CRISPR/Cas9 technology.

On the other hand, Lyfgenia is a cell-based gene therapy. Lyfgenia uses a lentiviral vector (gene delivery vehicle) for genetic modification and is approved for the treatment of patients 12 years of age and older with sickle cell disease and a history of vaso-occlusive events. With Lyfgenia, the patient's blood stem cells are genetically modified to produce $HbA^{T87Q}$, a gene-therapy derived hemoglobin that functions similarly to hemoglobin A, which is the normal adult hemoglobin produced in persons not affected by sickle cell disease. Red blood cells containing $HbA^{T87Q}$ have a lower risk of sickling and occluding blood flow. These modified stem cells are then delivered to the patient.

Patients who received Casgevy or Lyfgenia will be followed in a long-term study to evaluate each product's safety and effectiveness.