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

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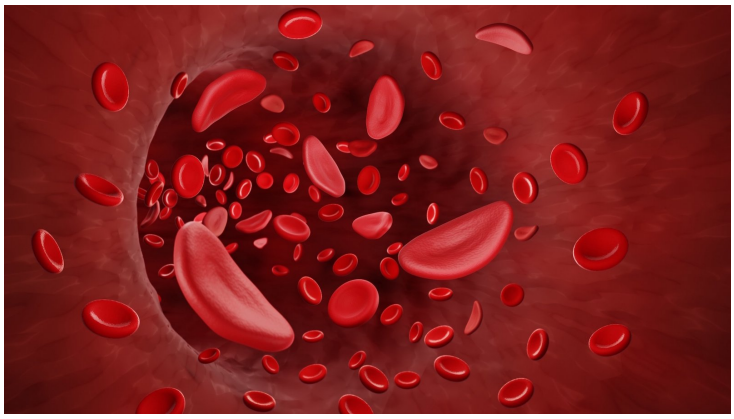
Sickle cell disease is a group of inherited blood disorders affecting approximately 100,000 people in the US



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.