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US approves sickle cell breakthrough with gene editing therapy

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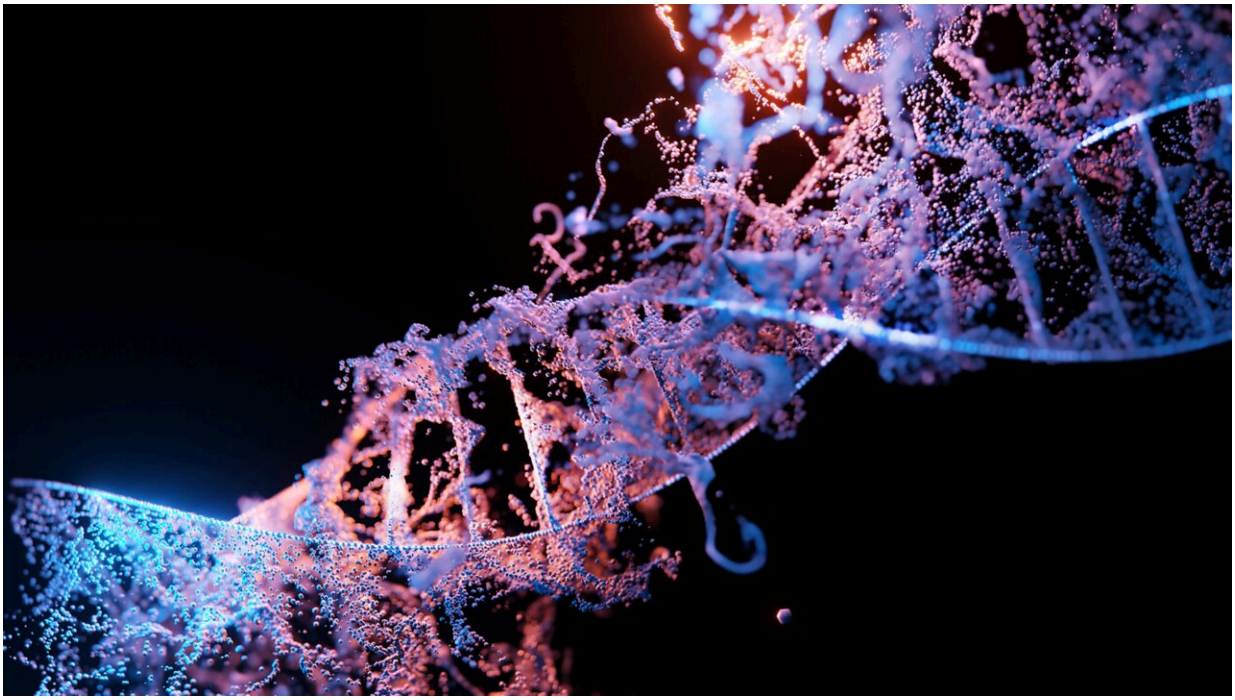

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The United States on Friday approved a breakthrough therapy that uses revolutionary gene editing tool CRISPR to treat the debilitating blood disorder sickle cell disease.

More than 100,000 Americans, most of them Black, suffer from sickle cell disease, a painful, life-threatening illness that medical science has

struggled to address.

US Food and Drug Administration (FDA) regulators approved two gene therapies, including one called Casgevy that uses the Nobel Prize-winning technology CRISPR.

"These treatments represent a major advancement in the field of gene therapy for patients with sickle cell disease," said Peter Marks, director of the FDA Center for Biologics Evaluation and Research.

"The potential these products have to transform the lives of patients living with sickle cell disease is enormous."

CRISPR, a gene editing technique whose founders won a Nobel Prize in 2020, can change the DNA of animals, plants and microorganisms with extreme precision.

Hailed for its breathtaking potential, the technology has revolutionized the study of molecular life, already contributing to experimental cancer treatments and drought-resistant crops.

"This approval means that for the first time, approximately 16,000 patients with SCD may be eligible for a durable one-time therapy that offers the potential of a functional cure for their disease," Casgevy maker Vertex said in a statement.

The second approved treatment, called Lyfgenia, uses a harmless virus for genetic modification.

Red blood cells normally move easily through blood vessels, but in sickle cell disease, they become crescent—or "sickle"-shaped—blocking blood flow and leading to strokes, eye problems, severe pain and even death.

Until now, the only cure for sickle cell disease has been a bone marrow transplant.

US President Joe Biden said Friday's announcement "represents the power of medical innovation to improve Americans' lives."

"My Administration will continue our efforts to accelerate the development of cures for rare diseases and support the medical research and innovation that achieved this breakthrough," he said in a statement.

Successful trials

Britain's drugs regulator approved the gene therapy that uses CRISPR last month, but Friday's authorization was a first for the United States.

Under the treatment, patients' blood stem cells are modified by genome editing using CRISPR technology—then transplanted back into the patient.

The modified blood stem cells increase production of fetal hemoglobin (HbF), which helps oxygen delivery and prevents the "sickling" of red blood cells.

During a clinical trial, 29 out of 31 patients did not experience a "pain crisis" for at least a year over a follow-up period of two years.

Among the side effects were mouth sores, nausea, abdominal pain and vomiting.

"Gene therapy holds the promise of delivering more targeted and effective treatments, especially for individuals with rare diseases where the current treatment options are limited," said Nicole Verdun, director of the FDA's Office of Therapeutic Products.

Follow-up studies will take place over 15 years, she said, with further monitoring planned for the rest of patients' lives.

Scientists are also developing methods for repairing genes in large organs, including the brain, potentially paving the way to treatment for devastating diseases such as muscular dystrophy and Huntington's disease.

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