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FDA approves exagamglogene autotemcel to treat beta-thalassemia

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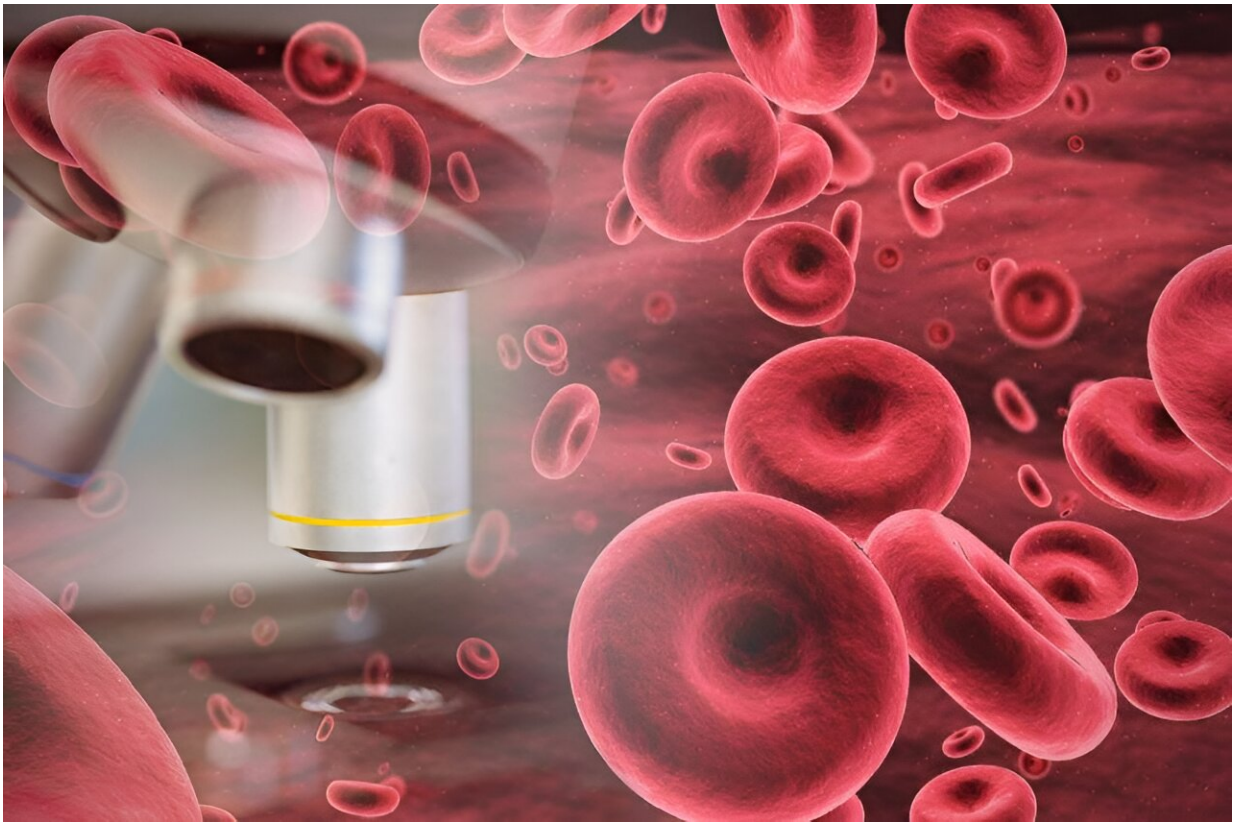

After approving Casgevy (exagamglogene autotemcel) in December to treat sickle cell disease, the U.S. Food and Drug Administration announced Tuesday that the therapy has now been approved to treat

patients older than 12 years with transfusion-dependent beta-thalassemia.

Casgevy is the first CRISPR-based medicine, where gene editing is used to develop the treatment, to be approved for use in the United States. The one-time dose permanently changes DNA in a patient's blood cells, but experts note the relief will not come cheap. The treatment list price is $2.2 million for its use in both sickle cell disease and beta-thalassemia, *CNN* reported.

"Today's approval is an important step in the advancement of an additional treatment option for individuals with beta-thalassemia," Nicole Verdun, M.D., director of the Office of Therapeutic Products within the FDA Center for Biologics Evaluation and Research, said in an agency news release. "The approval of a cell-based gene therapy for this condition using CRISPR/Cas9 technology reflects FDA's continued commitment to supporting safe and effective treatments that leverage the most promising and cutting-edge medical technologies."

The latest FDA decision was expected, but it comes about two months earlier than the agency's deadline for acting, *CNN* reported.

To make Casgevy, a person's stem cells are removed and modified using a gene-editing technique called CRISPR/Cas9. The altered cells are then transplanted back into the patient's body, where they multiply and increase the production of hemoglobin, which eases symptoms.

The most common side effects with Casgevy were mouth sores, febrile neutropenia, and decreased appetite, according to the FDA.

autotemcel-beta-thalassemia.html