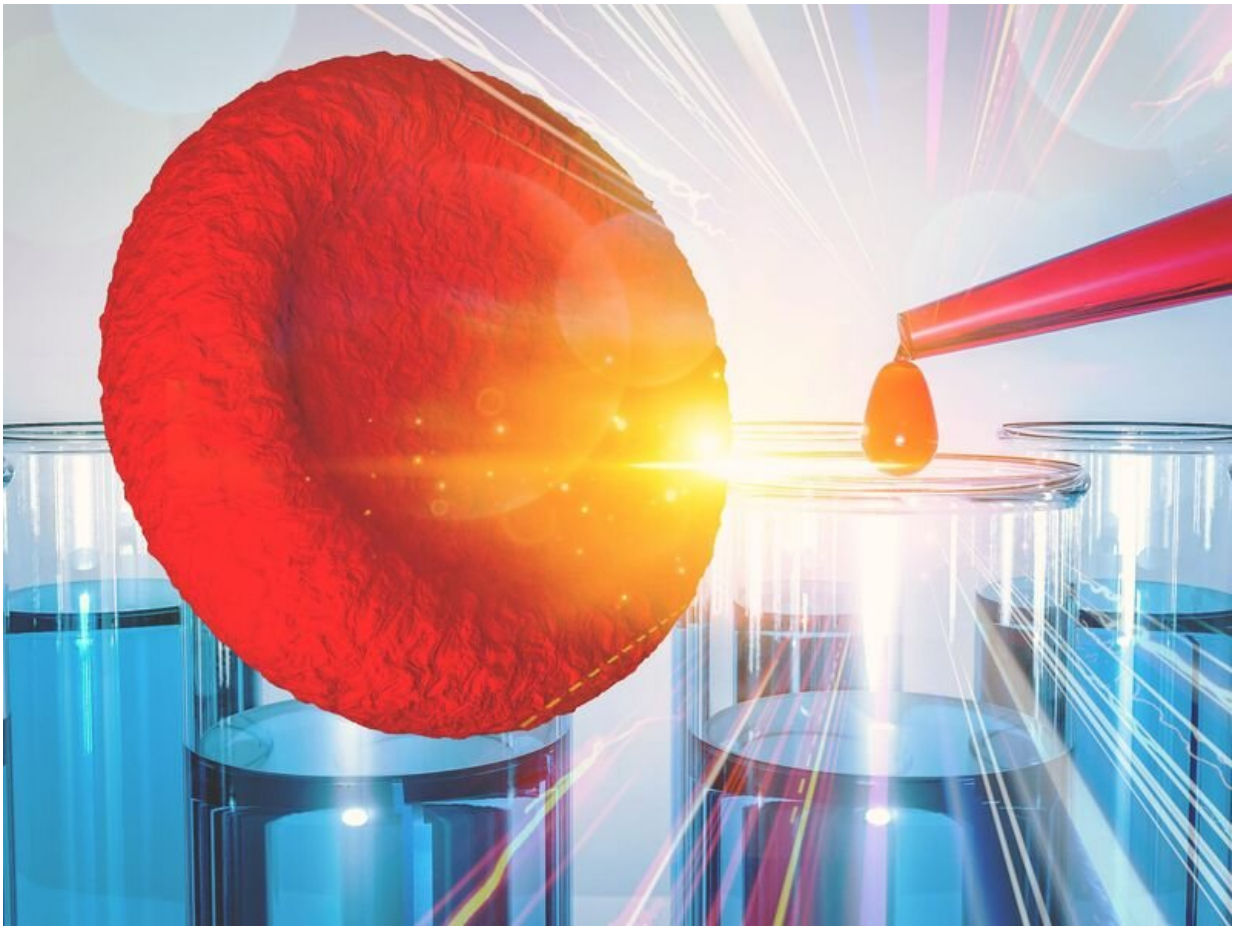
FDA approves first cell-based gene therapy for beta-thalassemia

August 23 2022



The U.S. Food and Drug Administration has approved Zynteglo

(betibeglogene autotemcel), the first cell-based gene therapy for the treatment of adult and pediatric patients with beta-thalassemia who require regular red blood cell transfusions.

Zynteglo is a one-time, single-dose gene therapy product. Each dose of Zynteglo is customized and created using the patient's own bone marrow stem cells, which are genetically modified to produce functional beta-globin. The application was granted a rare pediatric disease voucher, as well as priority review, fast-track, breakthrough therapy, and orphan designations.

The approval was based on two multicenter clinical studies. Of 41 patients receiving Zynteglo, 89 percent achieved transfusion independence, defined as maintaining a predetermined level of hemoglobin without needing any red blood cell transfusions for at least 12 months. The most common adverse reactions seen with Zynteglo included reduced platelet and other blood cell levels, mucositis, febrile neutropenia, vomiting, fever, alopecia, nosebleed, abdominal pain, musculoskeletal pain, cough, headache, diarrhea, rash, constipation, nausea, decreased appetite, pigmentation disorder, and itch.

Given the potential risk for blood cancer associated with this treatment, patients receiving Zynteglo should have their blood monitored for at least 15 years for evidence of cancer. The FDA says patients should also be monitored for hypersensitivity reactions during Zynteglo administration and for thrombocytopenia and bleeding.

More information: FDA Approval

Citation: FDA approves first cell-based gene therapy for beta-thalassemia (2022, August 23)