100,000 Americans, mostly Black, are victims of sickle cell anemia. CRISPR is on the cusp of treating it

Sickle cell anemia [is] a genetic abnormality that is the scourge of approximately 100,000 Americans, primarily Black, who are afflicted with it.

Sickle cell disease (SCD) is an inherited disorder marked by abnormal hemoglobin, the protein that delivers oxygen to the cells of the body. Normal red blood cells are disc-shaped and flexible enough to move smoothly through the blood vessels. In SCD, red blood cells become crescent or “sickle” shaped due to a genetic mutation in the patient’s hemoglobin.

Currently, a bone marrow transplant is the only treatment for some patients with SCD, but a new, potentially revolutionary approach might soon be available. The FDA is nearing the end of its evaluation of Exa-cel, a CRISPR-based gene therapy approach to reversing the genetic defect. The treatment involves gene editing of the patient’s blood-forming stem cells to induce them to produce high levels of fetal hemoglobin (HbF, or hemoglobin F) in red blood cells. HbF is the form of the oxygen-carrying hemoglobin that is naturally present during fetal development, but the body switches to the adult form of hemoglobin after birth. The increased production of HbF by Exa-cel reduces painful and debilitating sickle crises for patients with SCD.

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