It’s All in the Genes

What if the cure for sickle-cell sufferers was found in their own bodies?

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The current treatment for sickle-cell anemia is a bone marrow transplant that carries serious risks the body will reject donor cells. Only a small number of patients—usually kids with severe symptoms—undergo the procedure.

In this inherited blood disorder, unhealthy red blood cells are shaped like sickles (not like the healthy, round discs pictured at right). This limits their movement through vessels and causes pain and damage to limbs and organs.

But a new stem cell gene therapy is being studied by researchers at the University of California, Los Angeles. The scientists added an anti-sickling gene into patients’ blood-producing stem cells. Then, doctors transplanted these anti-sickling blood-producing stem cells back into the patients’ bone marrow to make red blood cells that don’t sickle. The disease affects about 100,00 Americans, many of them people of color.
