Sickle cell disease targeted with CRISPR therapy as ‘the only potential cure’

[F]or the first time, doctors in the United States used the gene editing tool CRISPR to attempt to remedy a genetic disease in a living person. Victoria Gray, a 41-year-old woman from Mississippi was born with sickle cell disease, an often painful and debilitating condition caused by a genetic mutation that alters the shape of red blood cells. As of now, only one treatment for the condition exists—a donor transplant that works for just 10 percent of patients—but doctors think editing cells extracted from Gray’s own bone marrow could restore proper red blood cell formation. If successful, it could prove to be the treatment 90 percent of sickle cell patients have been waiting for.

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[R]esearchers will have to watch Gray and other patients for at least 15 years, [hematologist Vivien] Sheehan says, possibly longer. It could take that long to understand if fetal hemoglobin is a long-term solution and to see if there are any unintentional effects from using CRISPR.

It’s too early to draw conclusions but researchers are still eager, because for the vast majority of people with sickle cell disease, [hematologist Vivien] Sheehan says, “this is the only potential cure.”

Read full, original post: Doctors altered a person’s genes with CRISPR for the first time in the U.S. Here’s what could be next.