Sickle cell cure now seen as possible as scientists remove faulty gene with CRISPR

Researchers have found a way to repair the faulty gene that causes sickle cell disease, which they suggest is a significant step forward in the search for a viable gene therapy.

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[U]sing the CRISPR gene-editing tool, they corrected the gene in stem cells from diseased patients and showed they could make red blood cells capable of making functioning hemoglobin. They also transplanted the stem cells into mice and found them thriving in their bone marrow months later.

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Matthew H. Porteus, associate professor of pediatrics at Stanford University, CA,…has been trying ways to target sickle cell genes with gene-editing technology for several years.

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Prof. Porteus says that the hematopoietic stem cells have the ability to travel from the bloodstream into the bone marrow, where they then “set up shop and start making other blood cells.”

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A gene therapy for sickle cell disease would not have to replace all of a patient’s sickle cells Prof. Porteus explains. You just need a sufficient amount of normal cells. Patients whose sickle cells are below 30 percent show no symptoms of disease.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: Gene therapy for sickle cell disease steps closer