CRISPR therapy demonstrates ‘real curative potential’ for sickle cell disease, beta thalassemia

The first two patients to receive a CRISPR-based treatment for the inherited blood disorders sickle cell disease and beta thalassemia have benefited from the experimental therapy and experienced only temporary and treatable side effects, the companies developing the treatment announced on [November 19].

The two patients, enrolled in a pair of ongoing clinical trials, have been free from blood transfusions and disease symptoms for a relatively short time, but the encouraging data offer hope that genome editing might one day offer a safe, durable cure for both blood diseases.

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Since her treatment in July, the sickle cell patient, Victoria Gray, has not suffered any vaso-occlusive crises, in which sickled blood cells basically get stuck in blood vessels, causing severe pain and organ damage.

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After the CRISPR therapy, Gray’s total hemoglobin reached normal levels; of which 47% was fetal hemoglobin. It’s thought that a fetal hemoglobin level of 25%-30% is sufficient to “cure” a patient with sickle cell disease.

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“These results are remarkable because they represent the first clinical evidence that CRISPR-Cas9 has real curative potential. From a scientific and medical validation standpoint, the data are important,” Vertex chief executive Jeff Leiden told STAT.

Read full, original post: First CRISPR treatment for blood diseases shows early benefits in two patients