CRISPR corrects mutant gene for incurable blood disorder

The genome-editing method involving CRISPR and Cas9 has been called into duty for a wide variety of jobs, from cutting integrated HIV out of the human genome to turning off genes in primates. In a new development published in *Genome Research*, researchers have used CRISPR/Cas9 in human cell lines to rewrite a mutant gene that causes a blood disorder called ?-thalassemia.

“It is an incremental step forward to use genome editing to correct disease-causing mutations,” said Paul Schmidt from Children’s Hospital Boston and Harvard Medical School who did not participate in the study. Still, he added, there are a number of hurdles that “need to be overcome before it can be used in the clinic.”

?-thalassemia is caused by a mutation in the *HBB* gene, resulting in a severe hemoglobin deficiency. It’s estimated to affect one in 100,000 people globally, including one in 10,000 in Europe. Patients require transfusions that can overload them with iron, a complication that also requires treatment. Some researchers are working to develop a gene therapy for the problematic gene, but so far there is no cure.

Read the full, original story: CRISPR corrects blood disorder gene