Cure for sickle cell diseases inches closer with launch of gene therapy trial

For 65 years, scientists have known the cause of sickle cell disease but have been unable to cure it without a bone marrow transplant. By fixing the underlying genetic problem… new research buoys hopes for thousands of suffering people — and opens up the possibility of treating other simple inherited disorders.

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The study, which will take four years, plans to enroll its first patients this fall. It will start with six adult patients between the ages of 18 and 35 who are very sick.

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While trials at other universities have successfully added extra copies of the healthy gene, or knocked out the bad gene, the new trial is a gene “knock-in.” It is very targeted — replacing the defective gene with a repaired version.

In the lab, not every bad blood cell must be repaired. The team is currently correcting about 20% to 25% of the genes. The goal is to boost efficiency and correct at least 40% of the blood stem cells.

“It will hopefully reconstitute a blood system that no longer makes a significant number of sickle red blood cells,” said [researcher Mark] Walters. “The sickle mutation is gone and the regular healthy hemoglobin is made in its stead.”

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