Can gene therapy offer a cure for sickle-cell disease?

[I]n November, six months after [21-year-old Manny] Johnson became the first patient to receive an experimental therapy aimed at curing his [sickle cell] disease, the port that had become part of him — requiring special approval to play sports, used when he was excused from school for a day or two every month for treatments — was removed. Johnson hasn't needed a transfusion or had any symptoms since May.
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In the case of sickle cell, a one-letter typo buried in the 3 billion letters of DNA that spell out the genetic instruction manual for a human being causes people's red blood cells to form a crescent-moon shape. Those misshapen blood cells can get stuck in blood vessels and cause inflammation, infections and organ damage.

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[Hematologist Erica] Esrick and colleagues focused on trying to boost levels of a normally functioning fetal form of hemoglobin that is typically shut off after birth. They removed blood stem cells from Johnson and altered them in the laboratory, using a virus to insert a molecule that flips a genetic switch to turn fetal hemoglobin back on. They gave Johnson a form of chemotherapy and then reinfused him with the altered cells.
At six months out, they found no sickle cells in his blood, and he has not needed any more transfusions. They plan to give the therapy to the next patient in February.

Read full, original post: Gene therapies could transform the treatment of sickle cell disease