Gene therapy to cure sickle cell anemia in final stages of development

Scientists are finalizing plans to use gene therapy to treat one of the world's most widespread inherited diseases – sickle cell anemia. The technique could begin trials next year, say researchers.

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[I]n some individuals, fetal hemoglobin production[, which protects the body from the disease] is not turned off at birth.
"Those individuals are supplied with fetal hemoglobin throughout their lives and for those who also inherit sickle cell anemia this protects them against the disease by making a substance that can carry oxygen round the bodies," said [Prof Stuart Orkin of Harvard Medical School].

…

"We can now use gene-editing technologies to cut out that little enhancer so that the BCL11A gene stops shutting down fetal hemoglobin production and allow children with sickle cell disease to start making it in their blood," added Orkin. "Essentially, we will take bone marrow – where blood cells are made – from a patient, gene-edit it so that those cells produce enhanced levels of fetal hemoglobin, and return them to that patient."

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