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Short Commentary

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Desired Sickle Cell Gene Therapy Promise

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Short Commentary

Sickle cell disease is the most important heritable monogenic disease. More than 275000 infants are born with the disease every year [1]. This syndrome was first identified in 1956 due to a single amino acid substitution in β^A-globin ((Glu6Val) stemming from a single base replacement (A → T) in the first exon of the human β^A globin gene. This genetic defect is referred to as the missense mutation, which causes polymerization of hemoglobin S [2]. Patients with sickle cell disease have extremely painful Vaso-occlusive attacks, which contribute to irreversible organ injury, poor quality of life, and declined life expectancy [3].

Blood exchange transfusion is functional for both acute and chronic complications of sickle cell disease. The patient's red blood cells are removed and displaced by unaffected exogenous red cells. This exchange prevents the elimination of sickle cells involved in new Vaso-occlusive events, reduces hemolytic complications, and provides extra oxygen-carrying capacity [4]. The vast majority of red cell transfusions for sickle cell disease are helpful but may be associated with bleeding or swift red cell destruction as well as hemoglobin concentration and blood viscosity are expected to increase. Moreover, Cytotoxic medications have been used to increase fetal hemoglobin levels in some patients as a modifying therapy approved for sickle cell disease. Until recently, allogenic hemopoietic stem cell transplantation provides the only curative option for a patient with progressive sickle cell disease, but only 18% of patients have access to match a sibling donor [1]. Gene therapies today can provide a long-term and potentially curative remedy for sickle cell disease [5]. Pieces of evidence of the efficacy of gene therapy were reported earlier in sickle cell disease [1,2,4].

A recent publication suggests that the mutation causing sickle cell disease can be directly corrected in hemopoietic stem cells using CRISPR (clustered regularly interspaced short palindromic repeats)-Cas9 system [2]. Broadly speaking, four major types of gene therapy that are obtainable for the treatment of sickle cell disease. These comprise gene addition therapy, gene editing, gene silencing, and gene correction therapy. Each type of therapy differs in how it triggers the replacement of hemoglobin S with non-sickling hemoglobin [5].

Interestingly, this method makes it possible to purify the modified cells by co-expression of cell surface marker, and it is not difficult afterward to anticipate that this strategy will soon be used in clinical trials [3]. Direct correction of the gene in situ has several utilities because it permits native regular mechanisms to be used and dodge the possibility of insertional mutagenesis and altered endogenous gene expression via lentiviral integration in nearby genomic sites [5]. Ultimately, this letter indicates that the non-sickling hemoglobin is the target protein in each therapy and will have to be assessed using a variety of new ways and terms.

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