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Therapeutic Application of Crisper CAS 9 for Sickle Cell Anemia : Current Progress and Future Prospects

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Abstract: *Sickle cell disease (SCD) is a congenital blood illness caused by faulty haemoglobinsynthesis, resulting in sickle-shaped red blood cells. This condition is caused by a single point mutation in the beta globin gene. SCD is primarily caused by a mutation in the gene that produces haemoglobin, the protein that transports oxygen in red blood cells. SCD patients may have chronic pain, exhaustion, anemia, stroke, organ damage, and increased infection risk. SCD treatment options focus on symptom management and preventing complications. Treatment options include supportive care, pharmacological therapies, hematopoietic stem cell transplantation, gene therapy, and gene editing. Gene editing can accurately fix inherited blood diseases like SCD.SCD focuses on symptom management and preventing complications. This covers supportive care, pharmacological therapies, hematopoietic stem cell transplantation, gene therapy, and gene editing. Gene editing is a promising treatment for genetic blood disorders like SCD. It can remove harmful variations, alleviate symptoms, and even cure the condition altogether. The CRISPR-Cas9 gene editing method is utilized to cure SCD. Sickle Cell Anemia SCA is a severe genetic disorder involving point mutations in the HBB gene encoding haemoglobin beta, which leads to abnormal haemoglobin and subsequent red blood cell sickling. Recently developed CRISPR-Cas9 gene editing has opened up newavenues for its targeted therapies. In the case of SCA, this CRISPR-Cas9 edits the HBB at precise locations. On the fronts of clinical success, two such next-generation treatments that are at the top of the race include Casegvy and Lyfgenia by CRISPR Therapeutics. These gene therapies are known to reprogram hematopoietic stem cells so that the production of healthy haemoglobin can be produced, hence potentially able to cure SCA and demonstrate the transformative power of CRISPR in precision medicine*

Keywords: Sickle Cell Disease (SCD); HBB gene; CRISPR-Cas9; gene editing; haemoglobin; hematopoietic stem cell therapy; Casgevy; Lyfgenia; precision medicine; genetic therapy

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