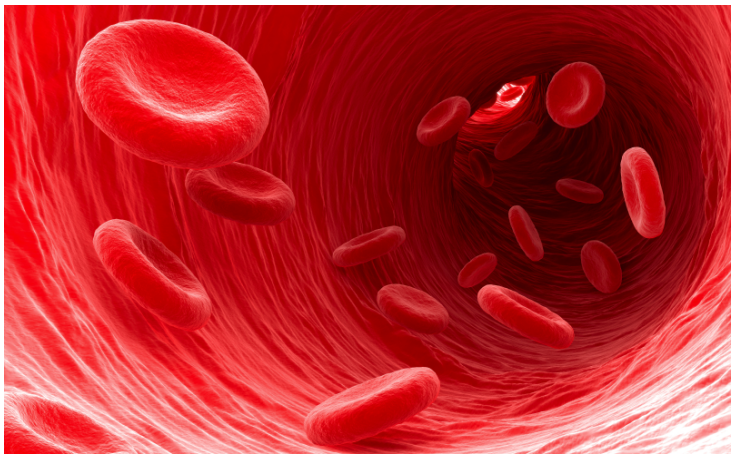
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ASIA EDITION

Editas Medicine announces pre-clinical data for treatment of Sickle Cell Diseases

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Editas Medicine, a genome editing company has announced results from experiments to demonstrate expanded CRISPR genome editing strategies in hematopoietic stem cells for the treatment of sickle cell disease and beta-thalassemia.

In these experiments, the Company demonstrated HBG1/2promoter-edited CD34+ cells robustly engrafted in mice without lineage skewing of red blood cell precursors.

In these experiments, NBSGW mice received an infusion of human CD34+ cells which had been edited either at the BCL11A erythroid enhancer (BCL11Ae) site or at the HBG1/2 promoter site. Analysis of bone marrow collected eight to 16 weeks post-infusion demonstrated that robust fetal hemoglobin induction was achieved when targeting HBG1/2 promoters.

Notably, editing HBG1/2 promoters upregulated fetal hemoglobin with superior repopulation of red blood cell precursors as compared to editing the BCL11Ae site. The red blood cell precursors from bone marrow edited at the BCL11Ae site had lower productive editing rates compared to other lineages and showed increased level of apoptosis, or programmed cell death, in erythroid culture compared to HBG1/2 promoter-edited cells.

Charles Albright, Chief Scientific Officer, Editas Medicine said, “We are encouraged by these preclinical results demonstrating cells edited at the HBG1/2 promoters repopulated all lineages of the blood system including, importantly, the red blood cell precursors. Editing at this site met our preclinical goals including robust, long-term induction of fetal hemoglobin and maintenance of normal hematopoietic stem/progenitor cell function. These findings further support our novel approach to developing a medicine for the potential treatment of sickle cell disease and beta-thalassemia. If these preclinical results translate to humans, we believe our editing approach for hemoglobinopathies may yield a safer and more effective medicine.”

Increased production of fetal hemoglobin can be beneficial to patients with sickle cell disease or beta-thalassemia. Editing at the HBG1/2 site is a differentiated approach for development of a human therapeutic for the treatment of sickle cell disease and beta-thalassemia as compared to other medicines currently under development that edit at the BCL11Ae site.