STEM CELL-BASED THERAPEUTIC APPROACHES FOR THALASSEMIA: A SYSTEMATIC REVIEW

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Thalassemia is a hereditary hematologic disorder characterized by defective hemoglobin synthesis, resulting in chronic anemia and rekated systemic complications,which are often managed through lifelong blood transfusions and iron chelation therapies. Recent advances in cell-based therapeutic strategies, particularly hematopoietic stem cell (HCS) gene therapy, have demonstrated substantial potential in addressing the underlying genetic defects and improving erythropoiesis. This systematic review evaluates preclinical and clinical studies, from PubMed, ScienceDirect, SCOPUS, focusing on the efficacy of HSC gene therapy and other immunomodulatory cellular approaches in thalassemia models. Studies involving thalassemic mice indicated that HCS-based gene therapy significantly enhanced β-globin expression and restored normal red blood cell phenotypes,suggesting functional hematologic improvements.Complementary strategies involving regulatory T cells (Tregs) and engineered immune cells, including CAR-T and NK cells, offer additional promise for immune modulation, transplant tolerance,and the reduction of therapy-related complications. Despite these advances, challenges including limited cell availability, complex ex vivo culture conditions, immune rejection, and scalability remain. Innovations in genome editing,engineered TCR/CAR technology and CRISPR/Cas-edited iPSC-derived cells may further improve specificity, stability, and efficacy. Collectively, cell-based therapies offer a transformative approach for thalassemia by correcting the underlying genetic defect and modulating immune responses, with the potential to reduce dependence on conventional transfusions and enhance quality of life. Further clinical studies are required to establish long-term safety, feasibility and therapeutic efficiency in human patients.

Keywords : Thalassemia,Hematopoietic Stem Cell,Gene Therapy,Regulatory T Cells,Cell-Based Therapy