Advancement in Thalassemia Treatment: A View from Gene Therapy

Arulmani Thiyagarajan*

Department of Public Health, SRM University, Chennai, Tamilnadu, India

Editorial

alassemia is a group of genetic disorders resulting in the synthesis of little or no α-chains or β-globin chains which leads to haemoglobin deficiency and blood-related complications. e clinical signs may range from early thrombotic events to numerous complications such as pulmonary hypertension, infections, endocrine dysfunction and sometimes leg ulcers [1]. e clinical signs displayed are based on the degree of alpha to non-alpha globin chain imbalance, genetic and environmental factors. Regular blood transfusion is required for beta-thalassemia major patients to survive [2]. is can cause iron overload which might lead to severe problems like liver diseases, splenomegaly, transfusion-related acute lung injury, metabolic and coagulation abnormalities, red cell all immunization and delayed haemolysis, iron overload, Immune System Effects [3]. Even with blood transfusion, only 50-65% of patients live beyond the age of 35 years in high-income countries as per existing literature [4-6].

Among beta-thalassemia patients, only a few have an option of curative treatment of allogeneic transplantation of hematopoietic stem and progenitor cells [7,8]. Gene therapy in focusing autogenic hematopoietic stem cell transplantation, which is currently on Phase-3 clinical trial would answer many unsolved questions and particularly the permanent cure to the thalassemia patients in coming years.