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BETA THALASSEMIA: AN INHERITED BLOOD DISORDER AND ITS TREATMENT APPROACHES

Sabita Upreti[*1],
Mrs. Mrudula Giri[2] ,
Arjan Aryal [3]

[1]M Pharm 1st year, Pharmacology Depatment, Krupanidhi College of Pharmacy, Bengaluru

[2]Associate professor, Department of pharmacology and pharmacy practice, Krupanidhi College of Pharmacy,Bengaluru

[3] Pharm.D 5th year, Department of Pharmacy Practice, Krupanidhi College of Pharmacy, Bengaluru

Beta-thalassemia is a genetic haematological disorders characterized by abnormalities in the synthesis of the beta chains of hemoglobin resulting in variable phenotypes ranging from severe anemia to clinically asymptomatic individuals.

The prevalence of this disorder is estimated to be 1 in /100,000 of total world population. It is most common in people of African, Mediterranean and Southeast Asian descent. Major complications associated with Beta thalassemia are hemolytic anemia, poor body growth and skeletal abnormalities during infancy as a result regular lifelong blood transfusion is required. The minor complication is the carrier of genetic trait for beta thalassemia and do not experience major health problem except mild anaemia.

Diagnosis of thalassemia is based on hematologic and molecular genetic testing. Differential diagnosis is usually upfront but may include genetic sideroblastic anemias, congenital dyserythropoietic anemias, and other conditions with high levels of Haemoglobin - F (such as juvenile myelomonocytic leukemia and aplastic anemia). Regular RBC transfusions, iron chelation ,management of complications of iron overdose are some treatment approaches for thalassemia major.

While, Bone marrow transplantation is the definite cure currently available. Individuals with thalassemia intermedia may require splenectomy, folic acid supplementation, treatment of extramedullary erythropoietic masses and leg ulcers, prevention and therapy of thromboembolic events.

Prognosis for individuals with beta-thalassemia has improved substantially in the last 20 years. However, cardiac disease remains the main cause of death in patients with iron overload. Induced pluripotent stem cells, prenatal diagnosis, oral sodium phenyl butyrate therapy,drugs such as *Deferoxamine , Deferasirox* (ICL670), *Deferiprone* which are clinical trials are new approaches to treat beta thalassemia.

Keywords: Beta thalassemia, bone marrow transplant, induced pluripotent stem cells.