Transfusion Management of Beta (β) Thalassemia: Initiating Regular Transfusions

A Thalassemia Resource for Healthcare Providers

Key Facts About β Thalassemia

- β thalassemia is caused by β-globin gene variants that reduce the production of adult hemoglobin (HbA) which may cause anemia.
- Chronic anemia can have serious consequences for people with β thalassemia.
  - » Children: reduced activity, growth problems and delayed puberty, hepatosplenomegaly, osteopenia, cognitive impairment
  - » Adults: fatigue, decline in capacity to work or perform usual activities, cognitive impairment, osteopenia, fractures, hypersplenism, reduced quality of life

Why People with β Thalassemia Need Transfusions

The primary management of severe anemia in β thalassemia is regular red cell transfusions. These transfusions provide the following benefits:

- Improve oxygen transport: HbA is reduced or absent with a variable increase in fetal hemoglobin (HbF). The predominance of HbF with its high oxygen affinity makes transporting oxygen less efficient. Compared with other anemias, people with thalassemia can be symptomatic at a higher hemoglobin level.
- Control ineffective erythropoiesis: Ineffective erythropoiesis, a distinctive and principal feature of thalassemia, causes bone marrow expansion, elevated basal metabolism, extra-medullary hematopoietic masses, skeletal deformities of face and skull, fragile bones, and increased absorption of dietary iron. The hemoglobin threshold to suppress ineffective erythropoiesis may be higher than the level needed to alleviate symptoms of anemia.

Centers for Disease Control and Prevention
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Initiating Regular Transfusions for People with β Thalassemia

The decision to initiate transfusions attempts to balance consequences from anemia and ineffective erythropoiesis against complications of chronic transfusion therapy. The Thalassemia Western Consortium recommends regular transfusions if either of the following conditions are met:

1. Hemoglobin <7 g/dL on 2 occasions at least 2 weeks apart
    a. β thalassemia major: <7 g/dL on 2 occasions, with or without severe symptoms
    b. HbE β thalassemia: <7 g/dL on 2 occasions AND one or more severe symptoms
2. Hemoglobin ≥7 g/dL, with one or more severe symptoms

The severe symptoms are:

1. Growth delay:
    a. Infants (<2 years): failure to gain weight for 3 months without another etiology
    b. Children: height velocity <3 cm/year
2. Delayed onset of puberty: >12 years in females, >13 years in males, with endocrine evaluation
3. Skeletal facial changes: subjective, longitudinal photo record may be helpful
4. Splenomegaly: spleen size >6 cm or enlargement >1 cm/year after 2 years of age
5. Extra-medullary hematopoiesis: symptomatic or moderate-severe
6. Cerebrovascular: overt stroke, silent infarcts, arterial stenosis, moyamoya disease
7. Venous thromboembolism
8. Pulmonary hypertension
9. Osteoporotic fracture
10. Poor quality of life in adults: chronic fatigue, decline in capacity to work or perform usual duties

Reference: Lal A, Wong T, Keel S, Pagano M, Chung J, Kamdar A, et al. The transfusion management of beta thalassemia in the United States. Transfusion. 2021; 1–13. https://onlinelibrary.wiley.com/doi/10.1111/trf.16640

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These materials are not part of the CDC guidelines and recommendations.The original evidence-based report and recommendations were developed in 2021 by a multi-disciplinary committee, the Thalassemia Western Consortium, consisting of members from 10 hematology centers that was partially funded under a prior cooperative agreement CDC-RFA-DD14-1406. While the Consortium was supported with federal funding, their work on clinical recommendations for transfusion practices was independent of CDC, and the contents of the resulting published report are solely the responsibility of the authors and do not necessarily represent the official views of, nor an endorsement by, CDC or the Department of Health and Human Services.