WHAT IS BETA-THALASSEMIA?

Beta-thalassemia is a rare, inherited blood disorder caused by a genetic defect in hemoglobin, which resides in red blood cells (RBCs) and carries oxygen throughout the body. This defect leads to the production of fewer and less healthy RBCs and can lead to severe anemia and other serious symptoms.[1,2]

Types[2,3,4]

Beta-thalassemia is often divided into 2 categories

MAJOR
Symptoms appear within the first two years of life

INTERMEDIA
Typically presents in pre-adolescence

Additionally, some people may carry the gene for thalassemia, but be asymptomatic. This is known as trait or minor.

Symptoms[2,3]

Major and Intermedia may present with common symptoms, such as:

- Anemia
- Bone and muscle abnormalities
- Growth deficiencies
- Abnormalities and complications of the spleen, liver and heart
- Hepatic and endocrine complications

Symptoms are often dependent on treatment and disease severity.

Diagnosis[6]

- **BLOOD TESTS INCLUDING A COMPLETE BLOOD COUNT (CBC)**
- **SPECIAL HEMOGLOBIN TESTS**
- **GENETIC TESTS**

Patient Population[4,5]

WORLDWIDE

1.5%
(80-90 MILLION)
are **CARRIERS** of the genetic mutation that causes beta-thalassemia

BETA-THALASSEMIA AFFECTS MEN AND WOMEN EQUALLY

Beta-thalassemia is most common in countries around the Mediterranean Sea, the Middle East, Transcaucasia, the Indian subcontinent and the Far East, but has a growing prevalence worldwide

Treatments[7]

STEM CELL TRANSPLANT
Most common for patients **<16 years of age** and/or those with an appropriate match

Supportive Care[7]

- **BLOOD TRANSFUSIONS**
- **Fe↓ IRON CHELATION THERAPY**
- **B9 FOLIC ACID SUPPLEMENTATION**

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