MULTIDISCIPLINARY CARE

Appropriate treatment of thalassaemia requires the cooperation and teamwork by different healthcare professionals. A patient must visit many medical specialists for the proper and systematic monitoring of various aspects of his health affected by the disease, including cardiologists, hepatologists, endocrinologists, and others.

Psychologists are often valuable members of the team, as patients have to tackle various challenges regarding the management of the disease and its therapy over different periods of their lifetime. Ideally, the members of this multidisciplinary team must often come together to discuss and cooperate on the best practices for the management of their patients.

KEY ISSUES IN THALASSAEMIA TREATMENT

One of the most important elements for the success of the management of thalassaemia is the patient's so-called ***adherence*** or ***commitment to therapy***. No transfusion and chelation scheme can be successful unless the patient follows it properly.

Advances in therapy over the last decades have transformed thalassaemia into a chronic disease with long-life expectancy for patients that receive appropriate care.

However, if left untreated or suboptimally treated, thalassaemia will lead to many complications, including death at a young age. Heart failure, liver cirrhosis, and diabetes are just some examples of the many serious complications that can appear in poorly treated patients.

Psychosocial support by the patient's family and community environment is often crucial for the achievement of proper adherence.

For more information you can visit
www.thalassaemia.org.cy

THALASSAEMIA INTERNATIONAL FEDERATION

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HOW DO YOU TREAT B-THALASSAEMIA?

A boy with β-thalassaemia major, the most severe form of the disease
Source: Sankalp India Foundation

THE ESSENTIALS OF DISEASE MANAGEMENT

Beta (β-) thalassaemia is an inherited blood disorder characterized by reduced levels of functional haemoglobin, a protein found in red blood cells, enabling them to carry and deliver oxygen throughout the body. Beta thalassaemia has three main forms – minor, intermedia and major, which indicate the severity of the disease.

Individuals with beta thalassemia minor, also known as carriers of thalassaemia or as having the thalassaemia trait, usually do not have any symptoms and are often unaware that they have the condition.

The symptoms of beta thalassaemia intermedia are widely variable and severity falls in the broad range between the two extremes of the major and minor forms. **Individuals with β-thalassaemia major have the most severe expression of the disorder.** They often require regular blood transfusions, iron chelation therapy and lifelong, ongoing medical care.

A young patient with thalassaemia getting a blood transfusion
Source: Al-Mustafa Welfare Society

BLOOD TRANSFUSION

The most important element in β-thalassaemia management is blood transfusions. Given that the patient's body cannot produce enough healthy red blood cells, the patient must receive blood donated by healthy individuals on a regular basis.

The frequency of blood transfusions and the amount of blood received each time, in other words *the transfusion scheme*, is determined by the treating physician. Blood transfusions are performed in hospitals or other healthcare settings (e.g. treatment centres/clinics), under the supervision of well-trained, specialized personnel (nurses and doctors).

Patients that receive regular blood transfusions throughout their lifetime and start transfusions during the first months of life have *transfusion-dependent thalassaemia (TDT)*, whereas patients that receive transfusions occasionally and usually at older ages have *non-transfusion dependent thalassaemia (NTDT)*. TDT patients usually receive 2 or more blood bags every 2-5 weeks. The frequency of transfusions for NTDT patients varies greatly.

IRON CHELATION

The second essential pillar of thalassaemia management is chelation therapy, whose purpose is to remove excess iron from the body.

Consequent to blood transfusions, iron included in red blood cells is gradually accumulated in the body. The more iron accumulated, the more toxic it becomes for many vital organs, such as the heart, the liver, the endocrine glands, and others.

Patients with β-thalassaemia require lifelong iron chelation therapy from early childhood usually on a daily basis.

Three different types of medicines are currently in use for the removal of iron, two of which are given orally and one via injection.

Deferoxamine is an iron chelator usually administered through subcutaneous injection. A small needle is placed in the patient's abdomen or elsewhere and the medicine is given through a syringe adjusted to a pump or through a small balloon pump over the period of several hours per day. ***Deferiprone and Deferasirox*** are both iron-chelating medicines administered to patients orally.

Either one of these medicines or a combination of them may be used.

The choice of chelators, the dose, and the frequency of administration is determined by the treating physician depending on different factors that have to do with the patient's needs, such as the frequency of transfusions, the amount of iron in the patient's body, the patient's lifestyle, as well as the availability and cost of each chelator in the country where the patient lives.

FAST FACTS ON THALASSAEMIA MANAGEMENT

Source: Global Thalassaemia Review, TIF publication (2021)

BLOOD INSUFFICIENCY IS EXPERIENCED BY MORE THAN 75% OF PATIENTS WITH THALASSAEMIA IN LOW-AND MIDDLE-INCOME COUNTRIES (LMICs).

APPROPRIATE IRON LOAD CONTROL IS REPORTED TO BE SUBOPTIMAL IN MORE THAN 80% OF PATIENTS WITH B-THALASSAEMIA IN LMICs.

LESS THAN 5% OF PATIENTS WITH B-THALASSAEMIA ACROSS THE WORLD HAVE ACCESS TO MULTIDISCIPLINARY CARE AND REFERENCE CENTRES.

IN MORE THAN 90% OF COUNTRIES WITH MEDIUM- AND HIGH-DISEASE PREVALENCE, PATIENTS HAVE TO RESORT TO OUT-OF-POCKET EXPENSES TO ACCESS NECESSARY HEALTHCARE.