This leaflet contains important information for β-thalassaemia patients on hematopoietic stem cell transplantation (HSCT), more commonly known as bone marrow transplantation (BMT), a term to be used throughout this leaflet. Patients and families can use this leaflet as a guide for further relevant discussions with their treating physicians.

The content of this educational leaflet (Number 1 in the series), has been adapted from the electronic educational platform of TIF - TIF’s Thal e-Course1 for patients and parents - with the support of members of TIF’s advisory medical and scientific panel. It has been reviewed and edited by thalassaemia patients from TIF’s expert patient advisory panel and Professor Pietro Sodani2, who has provided the data included herein.

**Disease definition**

β-thalassaemia or thalassaemia major is a genetic disorder of the blood caused by the inheritance3 of a defective (or mutated) β-globin gene. As a result, the body can only produce little or no normal adult haemoglobin (Hb), the substance inside the red blood cells (RBCs) that, on account of the iron that it contains, is responsible for carrying oxygen throughout the body to support normal bodily function and maintain human life.

The Pathophysiology of β-Thalassaemia

β-thalassaemia is caused by reduced or absent synthesis of the beta-globin chains of the adult haemoglobin (HbA) tetramer, which is made up of two alpha- and two beta-globin chains (α2β2). When beta-globin chains (β) are absent, alpha-globin chains (α) and their degradation or breakdown products precipitate (deposited in an insoluble form), causing ineffective erythropoiesis4 and haemolysis, which lead to severe anaemia. In turn, anaemia stimulates erythropoietin synthesis, resulting in intense proliferation5 of the bone marrow, skeletal deformities, and a variety of growth and metabolic abnormalities.

The patient can only survive through extensive multidisciplinary support including mainly lifelong blood transfusions of donated Red Blood Cells (RBCs), containing healthy Hb. Furthermore, as transfused RBCs complete their lifecycle in the blood and break down (a normal biological process of the body), they release Hb and the iron contained in it. Thus, these patients need lifelong iron chelation therapy to treat (remove) this excess and toxic iron which causes, if left unaddressed, severe damage to vital organs of the body.

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1 The Thal e-Course is offered and created by the Thalassaemia International Federation (TIF) free of charge, following registration at http://academy.thalassaemia.org.cy/.
2 Dr Pietro Sodani, Cure Thalassaemia co-founder & Scientific coordinator.
3 When both parents are carriers of the thalassaemia trait, they have 25% chance in every pregnancy of having a child with thalassaemia, an inheritance mode referred to in science as autosomal recessive Mendelian inheritance. To avoid having an affected child, couples can get tested or screened to investigate whether they are carriers of thalassaemia trait before they decide to have a family.
4 Erythropoiesis is production of red blood cells; ineffective erythropoiesis indicates that despite active erythropoiesis there is a lack of effective production of mature red cells.
5 Bone marrow cells divide repeatedly, producing large numbers of red cell precursors.
About Thalassaemia, Treatment and Cure

Thalassaemia major is described as a multi-organ disease (i.e. one in which many organs of the body may be affected) with many and complex national, public health and social repercussions and, as such, addressing effectively its management requires well structured, multidisciplinary approaches in the context of national programmes within the healthcare systems.

The patient as described previously suffers from severe anaemia and can only survive through lifelong blood transfusions of donated RBCs, containing healthy normal haemoglobin (Hb). As transfused RBCs finish their lifecycle in the blood, they break down releasing their Hb and the iron which is contained in it. Iron is the element that is responsible for binding oxygen and transferring it from the lungs through the blood across the body. Thus, in addition to blood transfusions, thalassaemia patients essentially need another treatment intervention referred to as iron chelation therapy in order to treat (remove) this excess iron which is very toxic. The iron, if left in the body, will cause serious damage to vital organs such as the heart, liver and endocrine systems leading to many and serious medical complications and early death.

In low- and middle-income countries sadly, a big number of children with β-thalassemia die each year due to the lack of adequate and/or safe blood transfusion treatment and suboptimal or no chelation therapy. Furthermore, the aging of this population leads to increasing development of new chronic complications, such as heart disease, liver disease, and endocrine disorders, leading to additional morbidity and premature mortality.

To date, the only available approach that has been used for decades now to cure β-thalassaemia is allogeneic bone marrow transplantation (BMT) or allogeneic haematopoietic stem cell transplantation (HSCT) although considerable and intensive genetic-based research has given in more recent years the global healthcare professional and the patient communities immense optimism and hope of having in the near or very near future another totally curative approach. For the purposes of this leaflet, references will only be made to the bone marrow transplantation approach.

Bone Marrow Transplantation ... all about it.

More than 30 years have passed, since the first allogeneic haemopoietic stem cell transplantations (HSCT) were performed in patients with thalassaemia. After pioneering experiences in the 80s and early 90s, allogeneic transplantation remains the only widely available curative option for thalassaemia major. In recent years, the indications for HSCT have been expanded, and it is now increasingly applied worldwide.

Survival rates for 1493 Bone Marrow transplant in Thalassemia During the Period 2000-2014

<table>
<thead>
<tr>
<th>Patients</th>
<th>Events</th>
<th>Probability</th>
</tr>
</thead>
<tbody>
<tr>
<td>A. Overall Survival (2 years)</td>
<td>1493</td>
<td>154</td>
</tr>
<tr>
<td>B. Thalassaemia-Free Survival (2 years)</td>
<td>1493</td>
<td>253</td>
</tr>
</tbody>
</table>


HSCT is more popularly known as BMT since the BM is the main source of stem cells of transplantation. Bone marrow transplantation (BMT) is a medical procedure during which stem cells (a special young form of blood cells) are transferred from a healthy individual (i.e., the donor) into the blood-producing tissue in the bone marrow of the individual who has a blood disease such as β-thalassaemia (i.e., the recipient).

Stem cells are starter cells which like almost all other parts of the blood, are produced in the marrow, that is, the soft blood-forming tissue within some large bones of the human body. Stem cells progressively develop into highly specialised cells. Red blood cells (RBCs) are one type of such highly specialised cell. These contain Hb, develop from the maturation of stem cells and have the specialised ability to carry oxygen across the body organs and tissues which is required for their growth and development.

BMT is also referred to as allogeneic HSCT because it uses stem cells from an “allo” (Greek for “other”) donor. Gene therapy, on the other hand, a currently experimental approach to thalassaemia cure, uses stem cells from the patient him/herself (“auto” Greek for “self”) and is thus called autologous HSCT.
Bone marrow transplantation (BMT) is to date the only well-established curative treatment for thalassaemia and shows excellent long-term results.

Outcomes, however, depend on the patient’s pretransplant, clinical condition and the expertise of the centre performing the BMT procedure.

**Classification of risks**
A risk stratification system based on (1) hepatomegaly (i.e. large liver) greater than 2 cm, (2) liver fibrosis (liver cell damage), and (3) irregular chelation history categorizes patients with thalassaemia into 3 risk groups:

1. Class 1, with none of these factors;
2. Class 2 including 1 or 2 of these factors; and
3. Class 3 with all the 3 above factors.

Transplantation outcome worsens with increasing risk category, with the best outcomes achieved in risk Class 1 or 2 (as described above) and in patients transplanted with an HLA-fully-matched sibling donor. Modified protocols however developed in more recent years have the potential to make allogeneic BMT accessible to all class 3 younger patients with results equal or near to those of class 1 or class 2 patients with thalassemia.

**More about Bone Marrow Transplantation**

**Requirements for a successful Bone Marrow Transplant**

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healthy liver and (3) have low body iron content (minimal excess iron). Older patients with pre-existing conditions run more risks of developing complications following BMT, even when having HLA fully-matched sibling donor.

**The donor**

**Donor-related:** For a successful transplantation i.e., with minimal or absent unwanted reactions, the donor’s stem cells must have identical or nearly the same HLA characteristics as the stem cells of the recipient. Only siblings (brothers or sisters of the patient) can have exactly or almost exactly the same HLA characteristics and therefore be fully-matched donors to the patient. BMT can also be performed with less compatible (matched) donors, which can be HLA-matched but unrelated (non-family) donors, or parent donors (known as haploidentical donors). In these cases, the chances of a successful BMT are considerably reduced and the patient is thus at increased risk of developing complications after the transplant.

The doctor must carefully assess the risks and benefits of transplantation according to the clinical condition of each individual patient and the expertise of the transplantation centre. The doctor must then fully share all the risks and benefits with the patient (or parents if the patient is very young) in order to allow the patient (or parents) to make a fully informed choice about going ahead with the transplant.

**Donor compatibility and transplantation success rates**

To check HLA compatibility, blood or, more recently, saliva is used to compare the cells of the donor with those of the patient.

**Fully matched sibling donors:** Siblings, i.e., the brothers and sisters, are more likely to share the same HLA characteristics and are therefore the best donors of bone marrow for a patient. However, less than 30% of the patients are able to find a fully-matched sibling stem cell donor. The chances may decrease or increase depending on the size of the family. The chances increase also in societies with high prevalence of customary cousin marriage (consanguinity).

**Matched unrelated donors:** It is, of course, possible for recipients to find a suitable donor in the general population, by searching in international database platforms of volunteer donors, such as the Matched Unrelated Donors (MUDs) platforms. However, the difficulties of trying to find a match from unrelated (non-family) donors are considerably greater, while, on the other hand, the existence of even very small differences between the donor and the recipient can cause unwanted reactions, thus increasing the risks for the patient. More recent data strongly suggest that improvements in donor selection and transplantation preparation have improved the safety of unrelated donor BMT for thalassemia treatment. This appears to be a viable treatment option for selected patients when there is no suitable sibling donor.

**Haploidentical donor:** Haematopoietic cell transplantation (HCT) from an HLA-matched related or unrelated donor remains the only curative therapy for haemoglobinopathies to date. However, less than 30% of patients have an HLA-matched sibling donor, and in a recent study from the US National Marrow Donor Program, <52% of non-Europeans found a matched unrelated donor. Furthermore, the lack of donor registries and cost of recruiting unrelated donors makes this approach unaffordable for developing countries, where many patients with haemoglobinopathies reside.

A haploidentical related donor is often available and represents an alternate source of stem cells.

More specifically, the mother or father of the patient can become stem cell donors for their child and they are referred to as haploidentical donors because they only have 50% HLA match with the patient child. The 50% mismatch, however, can cause severe and dangerous complications to patients after the transplant. Research is ongoing and already some protocols have been developed by experts in the field aiming to reduce the severity of these adverse reactions, and increase the chances of patients using a parent’s stem cells with lesser or, if possible, no side effects.

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7 HLA characteristics or Human Leukocyte Antigens are proteins found on the surface of almost all the cells of the body. They are used by the immune system to recognize and thus not attack the individual's own cells, and to differentiate them from foreign cells such as bacteria.
Compatibility testing

Once the initial blood tests have shown that the donor and recipient’s HLA are compatible, the doctors perform a test in the laboratory, known as cross-matching, to make sure that no reaction is produced when the donor’s cells are added to the recipient’s blood. If no reaction is produced, the transplantation procedure can proceed. In very rare cases, the recipient’s blood might reject the blood of HLA-compatible donors during the cross-matching test, in which case the doctor decides not to continue the transplantation procedure.

Stem Cell Donation

Stem cells are usually harvested from the pelvic bone (iliac crest) of the healthy donor (bone marrow donation). Nonetheless, stem cells can also be drawn from the donor’s bloodstream directly using a needle (peripheral donation), which is the least invasive technique currently available. In special occasions, and if specialised staff and procedures are available, stem cells are harvested from cord blood through the umbilical cord of a newborn baby, in which case the quantity of stem cells obtained may present challenges to the success of the transplant.

Myeloablation

This procedure prepares (or conditions) the patient to receive the donor’s stem cells. Before the transplant takes place, the patient undergoes a period of treatment that destroys the patient’s own bone marrow cells in order to make room for and create the environment to host the donor’s cells, a process known as myeloablation. This process is carried out using mainly chemotherapy (drugs that destroy bone marrow cells) or sometimes irradiation (this is no longer recommended in non-malignant disorders such as thalassaemia).

Transplantation

The donor’s stem cells are transfused into the recipient’s bloodstream in a similar way to a regular blood transfusion. Once in the recipient’s blood, the donor’s stem cells travel to find their way to the bone marrow, which is located in the large bones of the body, and start producing normal, healthy blood cells, including red cells. This process takes about 2-3 weeks.

Cure

If the transplantation is successful, the healthy donor’s stem cells, with a normal β-globin gene, take over the recipient’s bone marrow activity and continue to produce healthy red cells for the rest of patient’s life. This means that the patient is cured of β-thalassaemia.

<table>
<thead>
<tr>
<th>Donor Type</th>
<th>Overall Survival Rate(%)* (2 years post-BMT)</th>
<th>Thalassaemia-Free Survival Rate(%)* (2 years post-BMT)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Matched Sibling Donor (n=1061)</td>
<td>91 ± 0.01</td>
<td>83 ± 0.01</td>
</tr>
<tr>
<td>Matched Related Donor (n=127)</td>
<td>88 ± 0.04</td>
<td>78 ± 0.05</td>
</tr>
<tr>
<td>Mismatched (n=57)</td>
<td>68 ± 0.11</td>
<td>68 ± 0.11</td>
</tr>
<tr>
<td>Unrelated Donor (n=210)</td>
<td>77 ± 0.03</td>
<td>77 ± 0.03</td>
</tr>
</tbody>
</table>

NB: The success rate and outcomes of BMT always depend on the experience of the centres, the condition of the patient before the transplant, as well as other factors.

Therefore, the results are not the same for every centre even within the same country. The above percentages indicating the success rate of BMT procedures used to treat β-thalassaemia are taken from research performed, mainly in Europe in well experienced centres, albeit through the years a number of expert centres with very successful outcomes published in the literature have developed in other regions of the world.

Bone Marrow Transplantation procedure

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Risks and adverse events associated with Bone Marrow Transplantation

- **Infections and bleeding:** They are caused during the process of myeloablation, because of the toxic effects of chemotherapy or irradiation. Considerable research is ongoing to use “milder” chemotherapy treatment to reduce to the maximum possible level the myeloablation treatment-related side effects.

- **Graft versus Host Disease (GvHD):** The donated stem cells attack the patient’s own cells because they consider them as foreign. GvHD can be acute (which is often lethal) or chronic, and severely affects the patient’s quality of life.

- **Graft rejection:** The patient’s body rejects and attacks the donated cells. The chances of GvHD or graft rejection are increased when the donors are not fully HLA-matched sibling donors. To prevent GvHD and graft rejection, patients need to take medication (immunosuppression) for a long time after the transplantation to keep suppressing their immune system and prevent their body from reacting to or completely rejecting the donated cells. However, immunosuppression is not always successful in doing so, and transplanted patients may also in addition develop other unwanted effects (due to immunosuppression) e.g., infections.

- **Inadequate dose of stem cells from the donor** (usually when stem cells are harvested through the umbilical cord): This may lead to either failure of the transplant or the production of a mixed population of red cells, some from the donor and some red cells that are still thalassaemic (this is called a chimera).

- **Reduced fertility:** Occurs as a result of the myeloablation process that takes place before the transplantation, during which chemotherapy is used to completely or partially destroy the patient’s bone marrow. The chemotherapy medications are very toxic and can damage the function of the gonads i.e., the testes in males and the ovaries in females. Male sperm and female ova (eggs) may be destroyed either temporarily or permanently. Factors that affect fertility are also: (1) the age of the patient at the time of the transplantation (children below the age of 12 have a greater chance of infertility), and (2) the conditioning regimen (i.e. the drugs used in myeloablation).

FERTILITY... more information

According to existing data, approximately 40% of transplanted patients (males and females) may enter or pass through puberty\(^9\) normally, despite the fact that most of them may present clinical evidence of gonadal dysfunction\(^10\); that is, low levels of testosterone in males and oestrogen in females. This means that both transplanted males and females may show signs of normal puberty, including the development of secondary sexual characteristics such as adult breasts, testicles, facial hair, and female menstruation. In some patients with thalassaemia, the insufficient levels of gonadotropin\(^11\) which is responsible for the absence of pubertal development is probably caused by the iron overload that patients suffered before they had bone marrow transplantation - particularly seen in older patients. This is added to the effect of chemotherapy on gonadal development.

**Most affected group:** Females who have passed through puberty, experiencing menstruation (post-menarcheal females), seem to be an extremely sensitive group to the harmful effects of the transplantation process since nearly 100% of them are reported to exhibit secondary amenorrhea (i.e. absence of menstruation) and elevated gonadotropin levels after BMT. For those transplanted below the age of 12, pregnancies are rare.

Overall, there are limited data available in the literature about the late effects of BMT on the pubertal development and future fertility, which calls for more research into this field.

However, fertility constitutes one of the very important issues that need to be thoroughly discussed between the treating doctors and the patients very early in time and before any decisions are made or channeled.

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\(^9\) Puberty is the period during which adolescents reach sexual maturity and become capable of reproduction. Girls experience puberty between the ages of 8 and 13, while boys experience puberty between the ages of 10 and 15. Some individuals may go through puberty earlier or later. During this time, the body undergoes various changes (e.g., development of secondary sexual characteristics such as adult breasts, testicles, facial hair and female menstruation).

\(^10\) Gonadal dysfunction is the damage to the function of the gonads, that is, the testes in males and ovaries in females. The gonads are responsible for producing the hormones that assist in the development of the reproductive organs of the individual and the promotion of secondary sexual characteristics.

\(^11\) Gonadotropin is the hormone that is produced by the pituitary gland and which stimulates the ovaries or the testes.
After the BMT, the transplanted patient needs to:

- Have careful and expert follow up by haematologists (and according to international protocols) since GvHD may appear after the patient is discharged from the hospital (which is a major cause of death in patients particularly living in developing countries).
- Continue iron chelation treatment to dispose of the toxic iron that was accumulated in their body before the transplant. Iron chelation can be done by removing blood through phlebotomy (venipuncture) or through iron chelation regimens, depending on the level of ferritin in the patient’s blood. The treating physician assesses the patient and decides which option is best for him or her.
- Continue to have comprehensive and reliable organ monitoring (according to international guidelines) to prevent disease complications. For example, heart or endocrine disease that developed before BMT should still be treated adequately after the transplantation.
- In any case, the patient should continue to be very carefully monitored throughout his/her lifetime.

Recommendations

1. Transplanted patients planning a pregnancy should remember that their genes remain affected by β-thalassaemia, and therefore, the disease can still be passed on to their children.

2. Patients are advised to discuss all options and concerns they have about fertility and the potential negative effects of BMT with their treating physician before the initiation of the procedure.

3. Patients at puberty and older patients, as well as their families, should know about the fertility risks involved in BMT well before the transplant in order to make an informed decision whether to go ahead with the transplant or not.

4. Patients at puberty and older patients (as well as their families) should be fully informed by their treating physicians about currently available fertility preservation options prior to the initiation of BMT therapy.

Fertility preservation options may include saving sperm or ova in deep freeze before the transplant for later use. There are also other fertility preservation options for both female and male patients prior to or at puberty. These options are still considered experimental but patients should nevertheless discuss them with their treating physicians.
Frequently Asked Questions

5 FAQs written by Dr. Pietro Sodani, Cure Thalassemia co-founder & Scientific coordinator Bone Marrow Transplantation (BMT) for Thalassemia (Sodani, 2019)
http://www.curethalassemia.org/faq

General questions

1. **Is there a cure for thalassaemia major?**

Yes, to date and until new curative methods (which are on the way) are fully and officially licensed (authorized) by the European Medicines Agency (EMA) and the Food and Drug Administration (FDA), bone marrow transplantation (BMT) is the only method of definitive cure for thalassaemia, which when successful can result in no more need of blood transfusions (thalassaemia free).

2. **My child has beta thalassaemia major, can he be cured?**

Your child may be cured (no more transfusions) with bone marrow transplant (BMT). The best potential donors for a BMT are brothers and sisters and the BMT experts need to know their age and review, with the involvement of treating specialists, their clinical status before initiation of discussions and any planning.

3. **My baby doesn’t have a matching sibling. What can we do to use BMT and cure him/her from thalassaemia?**

These are other options that you have:
- a) you can find a donor in a bone marrow donor bank. It usually takes time (3-6 months) and has a cost. There are centres and experts that can help you to seek for the donor
- b) sometimes the mother is a compatible donor
- c) even if the mother is not fully compatible, she can be used as donor

For options b) and c), we suggest for both parents (sometimes the father can be used as donor) take a simple blood test called HLA typing to see how it matches the patient’s HLA.

4. **I’ve given you all the information about my son, who has thalassaemia major. Do you suggest doing the BMT?**

From an ethical point of view we can’t tell you what to do. The decision is yours (parents) and in cases of adults, of the patient himself/herself, after receiving and understanding all relevant information regarding the whole process from a RELIABLE SOURCE. The role of the medical community is to provide you (or your parents) with all the facts and the risks, as documented in international published literature, but the final decision must be taken by you (or your parents) taking into consideration all parameters including the country you (or the parents) live in, the health care system, the cost, the experience of the Centre and the risk/benefit, based on your (or your child’s) age, clinical state and the quality of care you (or your child) has been receiving throughout your life.

5. **Why should I choose BMT to cure my thalassaemia?**

BMT medical experts say that there are many good reasons. Read them on relevant websites such as http://www.curethalassemia.org/faq/ which includes 115 FAQ written by Dr. Pietro Sodani, Cure Thalassemia co-founder & scientific coordinator.

At the same time, you should keep being updated on all new information on any medical/drug advancement and progress in the area of treatment and cure of thalassaemia that may indeed be appropriate for your case. You may need all this information before taking any decisions. Please consult with your treating doctor or the medical advisors of Thalassaemia International Federation (TIF) for the best and most suitable option for you or your child.

TIF’s website is regularly updated and constitutes a most reliable source of information.
Useful sites:


World Marrow Donor Association. Available at [https://www.wmda.info/](https://www.wmda.info/)

National Bone Marrow Donor Program’s Be the Match. Available at [https://bethematch.org/](https://bethematch.org/)

Asia-Pacific Blood and Marrow Transplantation Group. Available at [https://www.apbmt.org/about](https://www.apbmt.org/about)


EuroBloodNet [https://www.eurobloodnet.eu/](https://www.eurobloodnet.eu/)

For any questions and/or feedback please contact us

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Website: [http://thalassaemia.org.cy](http://thalassaemia.org.cy)

Your Opinion Matters to Us!!

Educational leaflets

Other educational leaflets available:

Educational Leaflet 2: Gene Therapy in β-thalassaemia
Educational Leaflet 3: Clinical trials in β-thalassaemia
Educational Leaflet 4: Iron chelation and monitoring or iron load in β-thalassaemia
Educational Leaflet 5: Liver Disease in β-thalassaemia
Educational Leaflet 6: MRI testing in β-thalassaemia
Educational Leaflet 7: Effective Organ Monitoring in β-thalassaemia
Educational Leaflet 8: Blood safety in thalassaemia and other haemoglobin disorders
Educational Leaflet 9: Prevention of Thalassaemia and other haemoglobin disorders
Educational Leaflet 10: New drugs and their impact on the pathophysiology and management of thalassaemia

International Thalassaemia Day 2014 Photo Contest, 1st Place
TIF’s Educational Programme (in brief)

The Thalassaemia International Federation (TIF) has developed an internationally-recognised educational programme, with the objective of providing lifelong education opportunities for healthcare professionals, patients and their families, and raise awareness amongst policy makers and the community at large. These include:

1. Conferences and Workshops (national, regional, international)
2. Fellowships and Preceptorships
   i. Renzo Galanello Fellowship Programme
   ii. TIF Preceptorships
3. Electronic and Mobile Learning
   i. TIF e-Academy
      - Thal e-course for patients/parents
      - e-courses for health care professionals
   ii. ThaliMe app
   iii. TIF Digital Library

HOW TO PARTICIPATE: https://thalassaemia.org.cy/education/

TIF Publications

HOW TO ORDER https://thalassaemia.org.cy/publications/tif-publications/
A few words about Thalassaemia International Federation (TIF):

TIF
Thalassaemia International Federation is an NGO founded in 1986 by a small number of patients and families representing National Thalassaemia Associations in Cyprus, Greece, UK, USA, and Italy, countries in which these diseases have been recognised as an important matter for public health and where the first programmes for prevention and management have been implemented.

MISSION
To improve the survival and quality of life of patients with thalassaemia through the promotion and support of: education, advocacy and capacity building of patients’ and their families’ awareness and education programmes for the community collaboration with national, regional and international health authorities aiming to (a) prioritise thalassaemia on national, regional and International health agendas; (b) develop and implement national disease specific programmes for its effective control, prevention and holistic care, and research programmes and studies focused on the final, total cure (c) establish equal access of every patient with thalassaemia to high quality health and social care services provided through truly patient-centred healthcare systems.

VISION
Establishment of equal access of every patient with thalassaemia to high quality health and social care services provided through truly patient-centred healthcare systems.

The content of this Educational Leaflet for the Patient entitled ‘Bone Marrow Transplantation (BMT) in β-thalassaemia' represents the views of the author only and is his/her sole responsibility; it cannot be considered to reflect the views of the European Commission and/ or the Consumers, Health, Agriculture and Food Executive Agency or any other body of the European Union. The European Commission and the Agency do not accept any responsibility for use that may be made of the information it contains.
This Educational Leaflet for the Patient entitled ‘Bone Marrow Transplantation (BMT) in β-thalassaemia received funding under an operating grant from the European Union’s Health Programme (2014-2020).