BMT

Bone marrow transplantation for immunodeficiency

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IMMUNO DEFICIENCY UK

Supporting families affected by primary and secondary immunodeficiency

About this leaflet

Bone marrow transplant (BMT; also known as ‘haematopoietic stem cell transplant’ or HSCT) is increasingly being used as a curative option for many immunodeficiency conditions. This leaflet will help you understand more about BMT and lists questions we recommend you ask before considering BMT at a particular centre.

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About BMT

In most cases, BMT can offer a long-term cure for many primary immunodeficiency conditions and some disorders that cause secondary immunodeficiency. Successful outcomes are possible for children, young people and adults.

BMT aims to replace the faulty immune system with an immune system from a healthy donor. Stem cells, from which all the cells of the immune system develop (Figure 1), can be obtained from healthy bone marrow or, in some cases, umbilical cord blood or donor blood. The stem cells from a suitable, healthy donor are given by transfusion into a vein of the person affected by immunodeficiency. These donor stem cells find their way from the bloodstream to the bone marrow of the recipient, where they start to produce healthy blood cells.

Over the last few years there has been significant progress in BMT techniques, and recent survival rates are extremely good for many conditions. However, BMT does involve a number of risks, and complications can arise afterwards. Some of these complications are temporary, others can be life-threatening. The risks are different for each patient depending on their health, the complications associated with having an immunodeficiency and the specific condition that is being treated. You will have the opportunity to discuss the risks in detail with an immunologist and transplant consultant on several occasions to fully understand how or if BMT could benefit you.

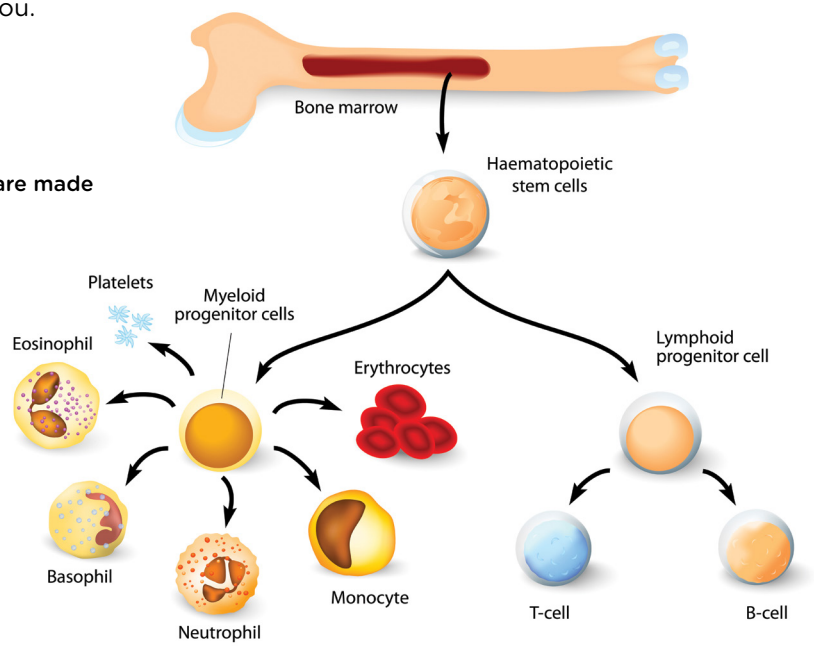

Figure 1.
How blood cells are made

Key questions to ask when considering a BMT

1. How many transplants has the unit undertaken?

It is important to know this information so that you can feel confident that the unit is able to support all aspects of care associated with the BMT. Centres that undertake a large number of transplants will be able to provide good-quality data that can inform your decision making.

2. How many of these transplants were for patients with an immunodeficiency?

As with any condition, people who undergo BMT for immunodeficiency will have their own unique requirements and considerations. Even though treatment and procedures for all transplants are fundamentally the same, patients with primary immunodeficiency are managed slightly differently from patients who have a blood cancer. It is important that the unit knows this and is familiar with the differences.

3. What type of infections and problems did these patients with immunodeficiency have before or going into BMT?

It is vital to understand the impact of BMT on people with immunodeficiency. It has been recognised, and indeed recommended, that wherever possible BMT should be undertaken when a patient is well. However, some people with immunodeficiency often go into transplant with chronic infection or with chronic inflammation, such as colitis. Management of these symptoms before, during and after BMT is key in ensuring good outcomes. This is one reason why knowing a centre's experience of BMT for different types of primary and secondary immunodeficiency is important.

4. Does the centre have a team of infection specialists to care for patients with immunodeficiency-associated infections?

It is important that the centre has infection specialists who are experienced in recognising and treating the unusual or atypical infections that can occur in immunodeficiency.

5. What have been the outcomes for the patients you have transplanted?

This may be a difficult question to hear the answer to because the team will discuss survival rates and complications with you. However, this is really important, as it will tell you how well the service can manage complications and also how good its results are.

6. What type of donors did the patients have? Were they really good matches or did some patients have matches that were less than 10/10?

Matching a patient to the right donor is important in ensuring the best outcome in BMT. The larger centres, which have a lot of experience transplanting patients with immunodeficiency, will have demonstrated success with a variety of types of donor.

For a well person with an immunodeficiency who has had no infections and no inflammatory problems and who has a fully matched sibling donor, a BMT would be considered a 'low risk' procedure. People who have had complications and have a donor who isn't as well matched will need the support of a team that is experienced in undertaking BMT in this patient cohort.

7. Are there clinical immunologists on site, experienced in immunodeficiency and the management/ complications in adult immunodeficiency patients undergoing BMT?

Having this expertise on site means that any problems during the BMT can be addressed quickly using expert knowledge and understanding of the immunodeficiency condition being treated.

8. What short- and long-term complications have you seen among immunodeficiency patients who have had a BMT?

As with any type of treatment, complications or side effects can occur. In BMT, complications can happen for many reasons, as there are several stages involved in the procedure. What is important is early recognition of complications and early intervention, and this is helped by the experience the BMT team has with dealing with these situations. It is therefore important to know what specific experience the centre has in dealing with the complications of transplants for immunodeficiency.

9. Do you work in partnership with other BMT centres and consult with other experts in the management of people with immunodeficiency? How is this done?

It is important for people who undergo a BMT for immunodeficiency to know that many centres across Europe, and indeed the world, share their experience both in medical journals and at international conferences. They also work together, discussing their knowledge and expertise. Such collaboration helps inform the medical profession on the best way to undertake BMT for people with immunodeficiency. This network of professionals is dedicated to ensuring that care, where possible, is supported by research and evidence, which is particularly important for rare conditions such as primary and secondary immunodeficiency. It is therefore useful to know which other centre or centres your BMT service works with.

Need more information on BMT?

The Immunodeficiency UK website offers lots of information about BMT, including frequently asked questions – **www.immunodeficiencyuk.org**.

The NHS England Clinical Commissioning Policy for Allogeneic Haematopoietic Stem Cell Transplant for Primary Immunodeficiencies (all ages) can be found at **https://bit.ly/3reoytz**.

The charity Anthony Nolan has information on BMT for patients and families and information on how to join their bone marrow donor registry – **www.anthonynolan.org**.

The charity Blood Cancer UK also has a useful booklet on the transplant process. The booklet can be found at **https://bit.ly/3r7EcXr**.

Glossary of useful terms

basophil a type of white blood cell that is involved in allergic responses

B-cell a type of white blood cell (lymphocyte) that produces antibodies

bone marrow soft, spongy tissue located in the hollow centres of most bones; it contains developing blood cells and cells of the immune system

bone marrow transplantation (BMT) the transfer of bone marrow, obtained by aspiration usually from the hip bones, from a donor – either related or unrelated – to a recipient. The donor bone marrow replaces the recipient's bone marrow, giving the recipient a new immune system and curing the immunodeficiency (see also *haematopoietic stem cell transplantation*)

donor an individual who donates bone marrow or stem cells for transplantation. Donors may be family members or unrelated, but they need to be well matched with the potential recipient by tissue-typing

eosinophil a type of white blood cell involved in the defence of parasitic infections and development of allergic reactions

erythrocyte a red blood cell

haematopoietic stem cells cells from which all blood cells and immune cells are derived

haematopoietic stem cell transplantation (HSCT) the transfer of bone marrow (obtained by a medical procedure) or stem cells (obtained from blood or stored umbilical cord blood) from a donor – either related or unrelated – to a recipient. Haematopoietic means blood-forming. The donor cells are given by intravenous infusion and make their way to the recipient's bone marrow to provide a new immune system, curing the immunodeficiency

haploidentical transplant a BMT using healthy, blood-forming cells from a half-matched donor. The donor is typically a family member and can be the recipient's parent, sibling or child

lymphocytes small white blood cells, normally present in the blood and in lymphoid tissue, that carry out specialised functions of the immune system. There are two major forms of lymphocytes, B-cells and T-cells, which have distinct but related functions in generating an immune response and are responsible for immunological 'memory'

monocyte a type of white blood cell that acts as a scavenger and is capable of destroying invading bacteria or other foreign material. These cells develop into macrophages in tissues

myeloid progenitor cells cells that are the precursors of red blood cells, platelets, eosinophils, basophils, neutrophils and monocytes

neutrophil a type of granulocyte found in the blood and tissues that can take in and destroy microorganisms

platelet a blood cell that works to prevent bleeding in the body by producing blood clots

T-cells (or T-lymphocytes) specialised white blood cells that develop in the thymus, an organ in the chest. They are responsible, in part, for carrying out the immune response

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About Immunodeficiency UK

Immunodeficiency UK is a national organisation supporting individuals and families affected by primary and secondary immunodeficiency.

We are the UK national member of IPOPI, an association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for PID patients worldwide.

Our website has useful information on a range of conditions and topics, and explains the work we do to ensure the voice of patients with primary and secondary immunodeficiency is heard. If we can be of any help, please email us or call on the number above, where you can leave a message.

Support us by becoming a member of Immunodeficiency UK. It's free and easy to do via our website. Members get monthly bulletins.

Immunodeficiency UK is reliant on voluntary donations. To make a donation, please go to **www.immunodeficiencyuk.org/donate**

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