

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

It is important for people who undergo a BMT for CGD 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 CGD. 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 CGD. It is therefore useful to know which other centre or centres your BMT service works with.

Need more information on BMT?

The CGD Society website has a wealth of information about BMT. Visit www.cgdsociety.org/bonemarrowtransplantation for films about the BMT process, patient stories, the BMT expert perspective and the latest news about BMT research.

About the CGD Society

The Chronic Granulomatous Disorder Society (CGD Society) is the leading global charity dedicated to promoting an understanding of CGD and providing support to affected individuals and their families.

Our website www.cgdsociety.org provides medical information and practical advice on living with CGD. It is free to become a member of the CGD Society. Please go to www.cgdsociety.org/register.

If we can be of any help, please contact us at hello@cgdsociety.org or on 0800 987 8988, where you can leave a message.

Our charity is reliant on voluntary donations. To make a donation, please go to www.cgdsociety.org/donate.



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Key questions to ask when considering a BMT

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Establishing at which centre you or your child will have a bone marrow transplant (BMT; also known as 'hematopoietic stem cell transplant' or HSCT) is a major decision. To help you research which centres have the necessary experience in BMT for CGD, we have put together a list of questions that we recommend you ask before considering a BMT at a particular centre.

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 CGD?

As with any condition, people who undergo BMT for CGD will have their own unique requirements. Even though treatment and procedures for all transplants are fundamentally the same, patients with CGD 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 have these patients with CGD had before or going into BMT?

It is extremely important to understand the impact of BMT on people with CGD. It has been recognised, and indeed recommended, that wherever possible BMT should be undertaken when a patient is well. However, people with CGD often go into transplant with chronic infection (usually with fungus) 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 experience of BMT for CGD is important.

4. What are 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.

11-month-old Thomas received bone marrow from an unrelated donor to treat his X-linked CGD in April 2012.



5. What type of donors did the patients have? Were they really good matches or did some of them 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 CGD, will have demonstrated success with a variety of types of donor.

For a well person with CGD 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.

6. What short- and long-term complications have you seen among CGD 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 CGD transplants.