A Guide to Bone Marrow Transplantation for CGD

From us to you. Important considerations and our top tips for getting through a bone marrow transplant.

CGDSociety

Supporting families affected by chronic granulomatous disorder

About chronic granulomatous disorder

Chronic granulomatous disorder (CGD) prevents the immune system from fighting off certain infections.

People with CGD carry a faulty gene, which means that some white blood cells don't work properly. As a result, people affected by CGD tend to get frequent bacterial and fungal infections, and need to take daily medication to stay healthy. Even if they take this medication, problems can still arise. This could lead to serious illnesses and prolonged periods in hospital.

CGD affects around six to eleven in a million people, depending on the part of the world you live in.

Contents

01

Introduction

The prospect of a bone marrow transplant (BMT) can bring emotional stress for the patient and their family. By writing this booklet, the CGD Society aims to provide information about what a BMT is, what a BMT in children and adults with CGD entails, and things that you should think about when considering a BMT. Additionally, this booklet includes advice on what to do before, during and after a BMT.

Within this booklet, Part 1 aims to help you understand whether a BMT may be an option for yourself or your child. Part 1 explains what a BMT is, and provides information on things to think about when deciding whether a BMT is the right choice. Part 2 of this booklet provides you with advice on all practical matters to consider before undergoing a BMT. If you are considering a BMT for yourself or your child, this information aims to prepare you for the process of a BMT. We encourage you use this booklet as a helpful information tool, but to always discuss your thoughts and concerns on anything related to a BMT with your doctors. You can also contact the CGD Society to discuss points related to a BMT at **hello@cgdsociety.org**.

Part 1:

Considering whether a BMT is right for me or my child

02

What is a BMT and how can it treat CGD?

When a person has CGD, they have a faulty gene in all cells in their body. For someone with CGD, the main problem is that special white blood cells called ‘phagocytes’ (pronounced as fag-o-sites) are damaged because of this faulty gene. More specifically, in the phagocytes of someone with CGD, a protein called ‘NADPH oxidase’ does not work properly. NADPH oxidase helps phagocytes fight infections by killing bacteria and fungi. Therefore, when it does not work properly, the immune system cannot protect the body against bacteria and fungi like it should.

If a person with CGD can get healthy blood stem cells from somebody else, then these can produce phagocytes that work properly. The aim of a BMT is to give a person with CGD these healthy blood stem cells. It is called a BMT because the blood stem cells are located in the ‘bone marrow’ which can be found in people’s bones.

When a BMT is successful in someone with CGD, they will then have healthy blood stem cells which will make healthy phagocytes. This means that the immune system of someone with CGD will work properly to protect the body against bacteria and fungi, and fight off infections.

A BMT is also often called a HSCT ('haematopoietic stem cell transplant'). This is because researchers and medical professionals sometimes call blood stem cells 'haematopoietic stem cells'.

The process of a BMT involves many steps and can take a long time. **Figure 1** provides an overview of the key stages of a BMT.

Figure 1*. The key stages of BMT*

Currently, a BMT is the only curative treatment option with the potential to completely get rid of the symptoms of CGD. However, it is important to know that research into BMTs as a treatment for CGD is still ongoing. Results from ongoing trials are promising, but a BMT may not be medically possible for everyone. That is why you need to carefully consider whether a BMT is right for you. As part of this, it is important to make sure that you understand the benefits and risks of a BMT and to discuss these with your medical team.

03

Who can have a BMT?

There are two forms of CGD. These are known as ‘X-linked CGD’ and ‘autosomal recessive CGD’. A BMT can be considered for both types of CGD. Recent research has also found that a BMT could be considered for females who are X-linked CGD carriers and experience severe symptoms. However, BMTs in X-linked CGD carriers have only been studied in a very small number of patients. Therefore, we may not understand all the risks and benefits of BMTs in X-linked CGD carriers yet.

04

Considering a donor for the BMT

A BMT can only be an option if you have a suitable donor. This is because for a BMT you need stem cells from a healthy person, who does not have CGD.

To improve the chances of a BMT being successful, doctors want to make sure that your immune system will not attack the blood stem cells that are donated to you, and that the new healthy immune cells that develop from your transplanted stem cells will not attack you.

Doctors use a system called ‘human leukocyte antigen (HLA) typing’ to identify a suitable donor for someone that needs a BMT. This system looks at special genetic markers called ‘antigens’. The combination of these genetic markers (also referred to as HLA markers) differ between people. The immune system uses HLA markers to identify substances, such as fungi and bacteria, that do not belong in your body. If the immune system recognises any substances in your body that do not have the right combination of HLA markers, it will attack these to get rid of them.

The immune system may therefore also attack any cells from a donor that do not have the right combination of HLA markers.

Similarly, the ‘new’ immune cells from the donor’s stem cells might attack your cells when the combination of HLA markers is not right.

When the markers on the healthy cells from the donor match the markers on your cells, doctors will say that this is an ‘HLA match’. When you have a good HLA match, it is more likely that your body will not attack any new healthy cells and that any new healthy cells won’t attack you. This means that there is a greater chance that the BMT will be successful. Having a good HLA match can also lower the risk of complications after a BMT. Complications that may come with a BMT are described in more detail in the section called ‘Outcomes and risks of a BMT’.

If your HLA markers and the HLA markers of the donor are a perfect match, this is called an ‘identical HLA-matched donor’. The best chance of having an identical HLA-matched donor is through a brother or sister, or another close family member. These are called ‘matched family donors’. However, if you have CGD, your siblings or close family members could be X-linked CGD carriers. If they are an X-linked CGD carrier, they will not have healthy blood stem cells. Therefore, they are best not to be a donor.

If you do not have a relative who can be a donor, your medical team will search for a donor that is an HLA match. They will search for an HLA-matched donor in national or worldwide stem cell registries. If a suitable donor is found in a registry, this is called a ‘matched unrelated donor’ because this person is not related to you.

In the case that there are no matched donors, your doctors may consider a ‘mismatched donor’. A mismatched donor is a donor in whom one or more of the HLA markers are different from the HLA markers of the person who is receiving the BMT. Having a mismatched donor may change the risk of complications after a BMT.

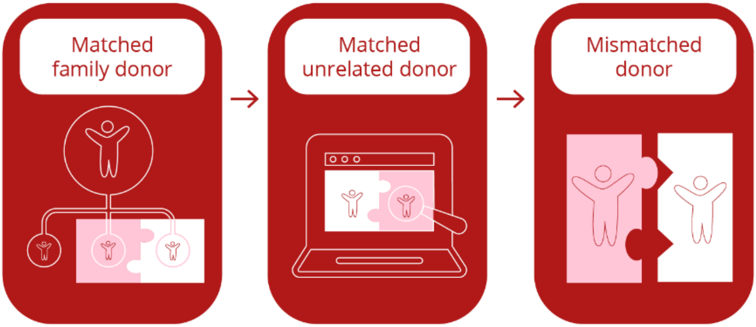

Figure 2. *The steps of searching for a BMT donor*

Matched unrelated donors are usually from the same ethnic group as the person who is receiving the BMT. This is because certain combinations of HLA markers are more common in certain ethnic groups. Depending on your ethnic background, it can be hard to find a matched unrelated donor. This is because bone marrow registries tend to have fewer potential donors from ethnic minorities.

If you are considering a BMT for your child with CGD, one of your other children may be a matched family donor if they do not have CGD and are not a carrier of CGD. When this is the case, there are several things to be aware of:

1. The child who is a donor may feel pressured, and feel frightened about the procedure and what lies ahead.
2. They may also carry an overwhelming sense of responsibility for their sibling’s well-being and survival.

Throughout the BMT process, your clinical nurse specialist should coordinate support for your child regarding their role as a donor. At the end of Part 1 of this booklet, we provide tips for how you can support a child who is a donor for their sibling.

The type of donor that you or your child will have, has an impact on how successful the BMT may be. The availability of a donor will be a big part of your decision to go ahead with a BMT and your medical team will help you decide what your best options are.

05

Conditioning therapy

As part of a BMT, you or your child will first have to go through 'conditioning therapy'. Conditioning therapy destroys the blood cells in the body of the person receiving a BMT. This is needed to make space for new healthy blood cells that will be made by the blood stem cells of the donor. Conditioning therapy also helps to make sure that the immune system of the person who has the BMT will not attack the blood stem cells from the donor. Conditioning therapy is often done using chemotherapy.

There are some side effects that may happen because of conditioning therapy. Side effects that happen quite often include:

- A loss of appetite
- Feeling and being sick
- Mouth ulcers
- Tiredness
- Rashes
- Hair loss

While these side effects usually only last a few weeks or months, there are also side effects that may last for a longer time. It is important to discuss the side effects and risks of conditioning therapy with your medical team.

06

Outcomes and risks of a BMT

The success of a BMT depends on many factors and is different for every person. While a BMT offers benefits, there are also risks that can come with having a BMT. Some people may experience minimal problems after a BMT, while others can get very ill after a BMT. The complications that may happen after a BMT sometimes require treatment, but can rarely be life-threatening.

Problems that may arise after a BMT

The problems that one person faces will differ from the problems that another person may face. Problems that may arise after a BMT include the following:

1. ‘Mixed chimerism’ – After a BMT, your ‘chimerism’ is measured regularly. Chimerism refers to how much of your bone marrow is from your donor. Your chimerism should be stable and as close to 100% as possible. After a BMT, it may be that your chimerism is consistently a lot lower than 100% or that it suddenly drops. This means that your bone marrow is a mix of blood stem cells from your donor and your original, unhealthy bone marrow. This is why it is known as ‘mixed chimerism’.

When your chimerism is low or it suddenly drops, you are at risk of ‘graft slippage’ or ‘graft failure’.

- ‘Graft slippage’ means that your chimerism is a lot lower than 100% and your body is not producing enough healthy blood cells. If you experience graft slippage, your medical team may decide to give you a ‘donor lymphocyte infusion’ (or DLI). This is an infusion of special cells from your donor called ‘T cells’. T cells are a type of white blood cell that help your immune system to better fight infections. This can push the chimerism back up to a higher level (closer to 100%).
- If you experience ‘graft failure’, this means that the donor cells don’t grow or develop properly. When you have graft failure, your medical team may decide to give you another BMT.

2. ‘Graft rejection’ – This happens when the immune system of the person who had the BMT attacks the donated healthy blood stem cells. They do this because the immune system thinks that the new healthy cells in the body are ‘bad cells’. Therefore, the immune system will try to get rid of the cells that they think are bad. They try to get rid of these cells in the same way that blood cells do with an infection. Doctors can monitor someone’s blood to see if they have graft rejection. Graft rejection can be treated in different ways.

3 ‘Graft-versus-host disease’ – This happens when the healthy blood stem cells from the donor make healthy white blood cells that attack the other cells in the body of the person who had the BMT. Graft-versus-host disease can happen shortly after a BMT, or even up to a few years after a BMT. The symptoms of graft-versus-host disease include an itchy rash, diarrhoea, shortness of breath and a dry and sensitive mouth.

The largest **study** that has been done on BMTs in people with CGD was published in 2020. This study presents how often people with CGD who have a BMT experience **graft rejection** or **graft-versus-host disease.** The study looked at 712 children and adults with CGD who had a BMT. Around **1 in 10** people experienced graft rejection or graft-versus-host disease. A more serious and long-lasting form of graft-versus-host disease is called ‘chronic graft-versus-host disease’. Chronic graft-versus-host disease was seen more often in **older people** with CGD who had a BMT than in young adults or children. People who received healthy blood stem cells from a **mismatched donor** experienced chronic-graft-versus-host disease more often compared with people who had a matched family donor.

4 Infections – There is a high risk of infections in the first weeks and months after someone has had a BMT. This is because the conditioning therapy, which has been described earlier in this booklet, makes the immune

system weak. Once you receive the stem cells from your donor, it will take some time before they produce healthy blood cells that can start fighting infections properly, even when everything goes well. These infections can be different from the ones you or your child may have had due to CGD, because different parts of the immune system are now weak.

- If you have graft-versus-host disease, you might need to stay on immunosuppressive drugs for a longer time meaning that the risk of infection will stay for a longer time.
- Additionally, if the new blood stem cells take a long time to start making immune cells (this is called ‘delayed engraftment’), the risk of infection will continue.
- Your doctors will ask you to take antibiotic and antiviral drugs to prevent infections, and will monitor you closely to see if any infection is present.

In addition to the problems described above, there are various potential long term effects of a BMT. Most of these effects tend to happen because of treatments that you receive before or after your BMT, such as the conditioning therapy. Long term effects may include:

1. Effects on fertility – Chemotherapy is the most common conditioning therapy and can cause permanent infertility. The effects that chemotherapy may have on fertility depends on the type and dose of chemotherapy, and the age of the person receiving a BMT. Your medical team can help determine the likelihood that chemotherapy may affect fertility.

2. Higher risk of developing skin cancer – Skin cancer may happen in later life for people who have had a BMT, because of the treatments they received before the BMT such as voriconazole. Therefore, it is important that someone who had a BMT wears high factor sun cream and a hat when out in the sun.

3. Weak bones (‘osteoporosis’) – This is when someone’s bones become thinner and less sturdy. In general, osteoporosis happens for many people as they get older, but it is more likely to happen after a BMT. It is also more likely to happen if someone takes ‘steroids’ (a type of treatment) for a long period of time to treat graft-versus-host disease or for any other reason.

4. Lung problems – Some people who had a BMT may experience lung problems. This can happen if someone

has chronic graft-versus-host disease or if someone had a lot of infections before or after a BMT. The main problems that people tend to experience are a dry cough that will not go away, breathlessness, wheezing and fevers.

5. Heart problems – Heart problems affect a lot of people, but the risk of developing a heart condition is slightly higher in people who had a BMT.

It is important to know that the risks, challenges and long-term outcomes of a BMT can be different for every person. It also differs from one person to another whether the risks of having a BMT outweigh the benefits. You should discuss all risks and benefits that come with a BMT with your doctor or medical team, to decide whether BMT is a good option for you or your child.

Factors that may affect the success of a BMT

There are general factors that can influence the risks of a BMT and how successful a BMT can be in someone with CGD. These factors are described in **Table 1**. You should discuss these factors with your doctor or medical team to see if they might impact how successful a BMT may be for you or your child.

Factor	Explanation
The health problems that someone is experiencing	If someone is experiencing problems such as inflammation (maybe due to inflammatory bowel disease) or has any other serious symptoms associated with CGD before having a BMT, they will likely experience more problems
How old someone is	A BMT is usually more successful in children or younger adults. This is because the number of risks that come with a BMT increase with age
The type of donor that is available	The risks and outcomes of a BMT depend on having a matched or mismatched donor.
If someone has access to a good transplant centre	Not all hospitals have a lot of experience with BMTs, especially in people with immune deficiencies such as CGD. When someone is considering a BMT, they should see if there are special 'transplant centres' nearby that have a lot of experience with performing BMTs, including BMTs in patients with immune deficiencies.

Table 1. *Factors that can influence the success of a BMT in someone with CGD*

07

Joining a clinical trial

Clinical trials help doctors understand how to treat a condition in the best way, and how to make a treatment such as BMT more successful. Clinical trials are an essential part of improving what doctors know about BMTs as a treatment for people with CGD. What doctors learn from clinical trials may benefit you or other people like you in the future.

Often, clinical trials look for volunteers who are people with a specific medical condition who are willing to test a treatment. There might be clinical trials that are looking for volunteers that have CGD and who are considering a BMT. These clinical trials might want to test aspects of your treatment before, during or after a BMT.

There are often strict criteria for who can join a clinical trial and who cannot. This may include criteria for how old you must be to join, or what type of CGD you have. This means that not everyone will be eligible to join a clinical trial if there is one.

If you do join a clinical trial, you can expect to have regular check-ups and tests (such as blood tests). This is to keep track of any effects the treatment may be having but also to ensure that the treatment is safe. Having regular check-ups also means that your doctor can identify any side-effects that might come up and that they can deal with these quickly.

While in the UK you can get a BMT via the NHS, if you or your child is considering a BMT you could look into joining a clinical trial. There may be clinical trials that want to learn more about how BMT can be made more successful in people with CGD. You can ask your medical team if they know of any clinical trials that you or your child could join.

If you want to learn more about what a clinical trial is, you can visit the **NHS webpage** about clinical trials, talk to your medical team or contact the CGD Society.

☎ **0800 987 8988** ✉ **hello@cgdsociety.org**

08

Alternative treatments to BMT

While a BMT might be the right treatment approach for one person, another person may benefit more from taking medicines to manage their CGD. For some people, taking daily antibiotics and antifungals works well to stop them from having infections and does not expose them to the serious risks that can be associated with a BMT. Therefore, it is important to discuss your personal situation with your doctor or medical team.

‘Gene therapy’ is a relatively new type of experimental treatment and researchers are still in the very early stages of investigating this treatment option.

In gene therapy, blood cells are taken from the person with CGD. In these blood cells, the faulty gene which causes CGD is replaced with a healthy version of the same gene. Once the blood cells contain the healthy gene, they can be put back into the person. When this happens, the person will have healthy blood cells that can fight infections as they should. Because the blood cells do not come from a donor, some risks that come with a BMT such as graft-versus-host disease are not an issue with gene therapy. However, gene therapy may come with other risks.

09

What will a BMT mean for me and my family?

A BMT can be a challenging process for the individual who is having the BMT and for their family. There are some practical matters which it would help to be aware of while you are considering the option of a BMT for yourself or your child.

Firstly, the extra costs associated with a BMT can be significant. In the United Kingdom, the NHS will cover the costs of the BMT itself. This includes all tests and examinations, conditioning therapy, the transplant itself and the hospital stay. However, you will have to pay for all the extra costs such as travel and preparing appropriate food. While there are grants and other sources of funding available, these are unlikely to cover all the extra costs that you may have.

1. We recommend visiting **Healthcare Travel Costs Scheme (HTCS) - NHS (www.nhs.uk)** to see how the NHS can help out with travel costs.

2. In the United Kingdom the charity Anthony Nolan may also be able to provide up to £250 in financial support through its grant programme. Visit the **Anthony Nolan Grant**

webpage to find out more, and email **patientinfo@anthonynolan.org** if you would like to apply for a grant. You may also be able to get help from The Family Fund via **www.familyfund.org.uk**.

3. The Sick Children’s Trust may be able to help provide ‘home from home’ accommodation for families with sick children in hospital. This needs to be referred by the hospital accommodation office. Visit **www.sickchildrenstrust.org** for more information.

4. The CGD Society may also be able to provide financial support through the Family Support Fund. You can find more information about this at **Family Support Fund - CGDSociety** or contact us at **hello@cgdsociety.org**.

A BMT also requires a large time commitment.

1. Ahead of having a BMT, you will have to visit your doctor a few times to do health tests and examinations. Closer to the day of your BMT, you will need to stay in hospital and have your conditioning therapy. This typically lasts around 1 week.

2. After you have your BMT, you will likely be in hospital to recover for at least 6 weeks. Usually, people who have a BMT will stay in hospital for approximately 10 weeks. This is because someone who has a BMT will need to be in a controlled environment like a hospital room. Being in this environment will help them to recover fully, and make sure

their new healthy blood stem cells can make new blood cells, without them being at risk of any infections.

3. After you leave the hospital, you will need to attend hospital appointments so that your medical team can see how you are doing. Initially, this can be as often as once a week. If everything goes well, the frequency of hospital visits will decrease. However, you should still expect to visit the hospital many times, at least during the first year.

If you want to know more about these and other practical matters, you will find more information and tips in Part 2 of this booklet.

Our tips for when you are considering a BMT for yourself or your child

1. Always feel able to ask your doctors questions about any aspect of the BMT.

“If a question comes into your head, it’s worth asking it.”

“Don’t go home with a doubt in your mind; you will sleep better for it.”

“Keep a note pad and write down questions as they occur to you, however silly they may seem. Having unanswered questions can lead to sleepless nights.”

2. Ask the doctors to be completely honest with you and to tell you the number of times they have performed the type of transplant under consideration and with what match of donor, the success rates and risks, the drugs that are used and any possible side effects the drugs might have.

"Doctors can only give you medical advice; they don't make the final decision. It is down to you to think about all the pros and cons and then to make the decision."

3. Don't let yourself get confused by technical medical terms. Always ask for a clear explanation in language that is easy to understand, no matter how many times this is required. Repeat the explanation back to the doctor so they can address any misunderstanding.

4. If you can't absorb all the information given to you verbally, ask for it to be written down. That way you can consider it in your own time.

5. If you are considering a BMT for your child and they are of an appropriate age, ask them how they feel about having a BMT.

"I want to do normal things."

"Sick of being different."

These are things Alex, aged 10, used to say. Alex wanted a BMT and he knew why.

> *"With teenagers and adolescents, it is important that they clearly understand everything, as ultimately the decision to go through a BMT is theirs. It is a difficult age to deal with something so enormous."*

1. Talk to other people or families who have gone through BMT, because they can be a source of support and knowledge. The CGD Society can help put you in touch with other people or families. Contact us at **hello@cgdsociety.org**.

2. Make sure you have a point of contact within the BMT team to keep you up to date regarding the plans for the BMT and to answer any questions.

3. Use the psychology support services available to you and accept all the support that the hospital offers you in helping you reach a decision about whether to proceed with BMT or not.

4. Get everyone on board with your decision. Family and friends will want to support you once they know what is going to happen. Really involve any brothers, sisters and grandparents.

> *"Making a decision to go for BMT is always extremely hard. However, once you've made the decision, try to stay as positive as you can. This can help you through the even harder times."*

Our tips for how to support your child who is a donor sibling

As previously mentioned in this booklet, a BMT may involve one of your children without CGD being a donor. We have summarised our tips for when one of your children may be a donor for their sibling.

1. Make sure the sibling donor knows what the donation procedure entails and check that they understand the information they are given.
2. The sibling donor may be frightened about donating. Offer constant reassurance and support through extra cuddles and attention throughout the BMT.
3. Encourage your donor child to discuss their feelings and concerns about their role.
4. The sibling donor might want to be actively involved in what happens throughout the BMT, so really keep them in the loop and consider their feelings throughout, in addition to caring for your child who is going through BMT. Being a donor creates an extra special bond between siblings, and the donor needs to feel included. However, it is also normal for sibling donors not to be interested.
5. Put in place good psychological support for your donor child before, during and after the BMT. Accept all the support that the hospital offers your donor child which can

include psychological support and the help of a play therapist. Having two children in hospital at the same time will be challenging. You may feel torn between them. If possible, arrange for your partner or another relative to help you with the care.

6 Donors often feel a huge sense of responsibility for how well the BMT goes. They might consider the setbacks and any problems associated with the BMT to be their fault. Make sure your child understands this is not the case.

7 Donor children should feel extremely proud of themselves. Let them know how proud of them you are and how brave they have been.

Part 2:

Preparation for a BMT and practical matters

10

Introduction

If you are considering a BMT for yourself for your child, there are practical matters that you need to consider. These include preparing for a BMT, going through the process and returning home after a BMT.

If you are an adult with CGD and you are considering to have a BMT, we recommend that you visit the **Anthony Nolan charity website** to learn more about what it means to have a BMT. The Anthony Nolan charity speaks about a ‘stem cell transplant’, which is the same as a BMT. Therefore, the information on the Anthony Nolan website may be very helpful to you. There is information provided on what you should do to prepare for the BMT, including how to prepare for your stay in hospital. The website also discusses various topics about recovering from a BMT. If you have questions that are not answered by the Anthony Nolan charity website, you can talk to your doctors or contact the CGD Society via **hello@cgdsociety.org**.

If you are a parent of a child with CGD and you are considering a BMT for your child, we invite you to read through the rest of this booklet. The CGD Society and parents of children who have had a BMT worked together to share advice and the things to think about before, during and after a BMT. Although parents had different experiences during their child's BMT, we found there were lots of common themes. We hope you find the information provided here helpful. If you have questions that are not answered by this booklet, you can talk to your doctors or contact the CGD Society via **hello@cgdsociety.org**.

11

Planning ahead for the BMT

Once the decision is made and you have the date for your child's BMT, you need to start planning ahead for admission to hospital. You can expect the stay in hospital to be six weeks minimum, but it may be a lot longer depending on how the BMT goes.

The time in hospital for the families involved in this project ranged from 44 days to 94 days, with an average stay of 10 weeks.

Getting your child in good shape for the BMT

It's important that your child is in good shape for the BMT. Often, people with CGD have low body fat and weight because of the underlying condition. During BMT your child may experience long periods of not wanting to eat or simply being unable to eat because of 'mucositis', a sore mouth or throat (caused by the chemotherapy), leading to further weight loss. This may potentially make the transplant and recovery period longer.

Our tips:

1. Talk with the dietitian to review your child's eating and drinking habits, and follow their advice. It is important that

your child eats a healthy diet, including five portions of fruit and vegetables a day.

> *"We were told to pile as much weight as possible on our baby Thomas before BMT, so we used high calorie milk with added cream and melted dairy cheese slices."*

2. For older children, high calorie drinks such as milkshakes are good, and letting them have treats such as cakes, crisps and sweets can help them gain as much weight as possible before a BMT.

3. Get your child used to drinking a lot (water, milk, squash, juice), because once in hospital having their BMT, they will need their kidneys to be working well to help flush out the toxic drugs used for the chemotherapy. During transplant, your medical team will advise you as to how much your child should drink. Their recommendations will depend on your child's body weight when they are transplanted. As a guide, it is about 1.7 litres per day for an 8 year old and around 2 litres for a 12 year old.

> *"I set a timer to go off hourly to encourage Jonathan to drink a cup of water (150ml). I felt it helped him establish a routine for drinking enough fluid during the BMT."*

3\. Don't let your child become over tired in the run up to the BMT, and be cautious about keeping your child free from infections.

“Sometimes this meant short breaks from school if Alex was tired, and keeping him away from other people with infections.”

Planning for taking time off work

It is likely that one of the main carers (usually mum or dad) will have to take time off work during the BMT.

Our tips:

1. Talk to your employer at least three months before the BMT.
2. Try to negotiate saving holiday for when your son or daughter has the transplant.

“It’s important that your employer knows and understands about the BMT and how it will affect your work. They and fellow co-workers can be strong supporters during BMT.”

“It can be helpful to negotiate with employers about carrying over holiday to save days lost.”

Thinking about your named carers

To limit the chances of infection during BMT, up to three people may be named as carers during BMT: for example, mother, father and one other. It's important to know you can't chop and change carers during the procedure. Only these named people will be allowed in the isolation room with your child.

Our tip:

1. Make sure the third carer or 'supporter' is really flexible with the time they can give, in case one of the main carers becomes ill. Their role is important as they are a fresh face and they bring in news from home.

Juggling family life

You may have other children or dependents at home who will also require support while you are away in hospital.

Our tips:

1. Start thinking about who will look after them while you are away. Grandparents, other relatives, and friends may be able to help out.

2. Don't forget that your other children may be just as anxious about what is happening as you are, so do keep them in the loop. They may also need the extra cuddle and reassurance.

3. Try and spend some time together doing something fun as a family unit before the BMT.

Accommodation during the BMT

Usually the BMT isolation room is fitted out to allow one extra person to stay overnight as a carer.

Our tips:

1. If you want another carer to stay overnight, enquire about the possibility of the hospital providing accommodation through the hospital's accommodation officer. Some hospitals may have rooms or flats that you can use. The key thing is to investigate what is available if you need it and to plan ahead.

2. If the donor is a brother or sister, then it may be possible to accommodate them in the same ward as your child having the BMT. Otherwise, it may be possible for them to stay in a patient hotel with a parent.

Planning for hair loss after chemotherapy

The chemotherapy used in BMT can inevitably lead to hair loss. Some people deal with hair loss as it happens during BMT, but some parents and children recommend working towards BMT with successively shorter haircuts so that losing hair isn't such a big thing when it happens.

"Tom worked towards his BMT with successively shorter haircuts. The day before going in for his BMT he had a close shave so that hair loss was not a big deal. This was to maximise hygiene – it's not good to have clumps of hair in the bed."

"Alex lost his hair. It was a bit traumatic and took ages to come out, about 2–3 weeks. It was like having pine needles everywhere. Eventually Alex decided we could shave it off and then he thought it was really cool."

"We didn't shave Jonathan's hair until the last minute. Do whatever you think is good for your kid or easier for you, but do tell them and explain fully in advance. It will help them to understand why."

"Sam, being a boy, had short hair anyway, but he wanted to keep it until the very end. I bought a soft bristle brush, gently brushed his hair every day, and quietly disposed of the hair in the bin after. When his hair really started to come out, he wanted it cut very short. I left it to his dad to use the clippers on the ward and to shave it short. It's so much better afterwards, and less itchy around their neck."

"We shaved Thomas's head as soon as his hair started coming

Getting yourself in shape

As a carer, you are going to need to be in good health.

Our tips:

1. If the transplant is scheduled to take place during the winter, consider having a flu jab to keep you well. You may be able to get this for free on the NHS because you and your child are in the 'at risk group'. If this is not possible, you can get the jab privately, in supermarkets and pharmacies.
2. Look after yourself by eating healthily.
3. If the stress starts to get to you, be kind to yourself. Take some time just for you and treat yourself a little.

Get your head around what is going to happen

We recommend getting your mindset ready. Remember you will not know how long the stay in hospital will be. It could be a short time, say, six weeks, or it could be longer owing to unforeseen events, such as an infection.

Our tips:

1. Don't plan too far ahead. Holidays may have to go 'on hold' for a while.
2. Talk of things that the family can do once you are all home together again, but don't put dates to them.

Work out a care schedule for the time in hospital

Everyone's situation is different. Sometimes the mother is the main carer, with the father becoming the carer at weekends, and the third carer providing company or cover. Other parents choose to alternate the care each day, and some stay with their child all the time.

Our tips:

1. Discuss with your partner and third carer what will work best, but remember you need to build in some flexibility just in case one of you becomes ill.
2. Remember there may be crucial times (e.g. the day of the transplant or if your child becomes seriously ill) when both parents will want to be close by. Try and plan for this contingency.
3. Keep the carer's schedule to a maximum of 3–4 days back-to-back and then a couple of days off, if possible. This will help to keep you grounded and to maintain

relationships with family at home and your friends.

"If both parents are present all the time it can take its toll on you, especially if you aren't used to spending so much time together."

4 Some hospitals may allow siblings to visit once restrictions are lowered, but they will need to be well. We advise checking with your hospital about this.

Telling your child's school or college

If your child attends school or college you will need to inform them about the plans for the BMT. You can expect your child to be out of mainstream education for at least six months.

Things you need to know:

The school is statutorily (this means, by law) obliged to make every effort to stay in touch with your child during any prolonged absence or long-term illness. The school must try to write, e-mail or video call with your child wherever possible and send regular communications to help them feel connected. They should also attempt to inform the child and parent of the work that is being missed and provide materials where possible. If you would like to read more about the obligations of the school and your local council to your child throughout their hospital stay, you can visit **www.gov.uk/illness-child-education**.

Your child will typically receive some tutoring in hospital if they are

16 years or younger. This is usually for one hour a day, and one-to-one. The hospital should collaborate with the school on this. It is important to know that some hospitals differ in their tutoring approach and may only provide tutoring to long-stay and recurring patients of all ages. Therefore, we recommend that you make yourself familiar with the approach of the hospital in which your child will stay.

“Don’t worry about your child catching up on schoolwork – they will.”

“Tom was not entitled to any schooling during his BMT as he was 17 and past the compulsory education age. This was extremely frustrating as he was due to take his AS levels at the end of his transplant. I think had he had the choice of schooling in hospital and the continuity of his education, he might not have left school so quickly after finally going back seven months post-transplant. I do think this is why he could not settle back in college and ended up leaving after three months.”

Our tips:

1. Call the school or college to let them know about your situation. Each school will probably be different and will tell you what they require in terms of paperwork, such as a letter from your doctor to explain the absence period.
2. It is worth speaking to the headteacher as well ahead of

the BMT. Remind them nicely of their duties to your child and ask them what strategies they will put in place for keeping in touch with and providing educational support for your child while they are away.

3. Ask the teachers to send a folder of the schoolwork that will be missed so that you and the hospital teacher know what needs covering.

> *"Don't necessarily rely on school to stay in touch and take the initiative. Take control yourself."*

4. Ask the form tutor to send pictures and letters from your child's class to the hospital. This can really cheer your child up, and pictures can be pinned up in your child's hospital room.

5. Find and contact your local education authority inclusion department to let them know. This is a good back up if the school does not respond to your child's needs.

6. If your child is older than 16 years of age, then consider planning, if you can afford, for private tuition in hospital.

Plan for communicating with the outside world during the stay in hospital

In our experience, staying in contact with friends and family during the BMT is vital to keep your spirits up and to maintain relationships.

Our tips:

1. Having a mobile phone is essential. Consider upgrading your contract so that it includes a larger bundle of free minutes, because you will be using your phone a lot. A mobile phone is also useful for sending e-mails and taking photos to share with others.

2. Make sure your phone has stored all the key phone numbers you need, such as your child's school, doctors and nurses.

3. Take a laptop or iPad into hospital. Ask the hospital how good (or bad) the internet connection is and how you access it.

4. Plan ahead and set up a video calling app. Ask others to do the same, so you can see and talk to others online.

5. You might consider doing a video diary or blog, so plan for that.

6. Letters and post from school, friends and family can be a highlight of the day once your child is in hospital.

7. Emphasise to others, in the lead up to the BMT, how much you would value them sending a letter. If they do send a letter, then ask them to double envelope it. This is because the outer envelope must be removed before the letter is allowed in the isolation room.

“A smartphone! I could email pictures and maintain contact with the outside world.”

“Think about having a mobile dongle if using lots of data. We set up Face Time with families and watched TV programmes and films online.”

“Keep up communication with other family members, such as grandparents. They will be very anxious, especially if they’re far away from you. Keep updating everyone on your child’s progress.”

Clothes, shoes, and lots of pyjamas

You will need to plan what clothes you are going to take into hospital for you and your child. Nappies are usually provided by the hospital for babies and young children.

We recommend asking what the temperature is likely to be on the ward during your stay. In our experience wards are usually fairly warm, regardless of the time of year, so think about this when planning what to pack for you and your child.

For your child, pack lots of loose, cool, lightweight, easy to wash, comfortable clothes that are easy to put on and take off. Select clothes that are loose around the chest area, so they don't press on any central line (a line that can give a person medicine, fluids, blood or nutrition) used for BMT. Think about buying at least one size too big.

Think about buying these well in advance of the transplant date, especially if the transplant is scheduled for the autumn or winter, when it is difficult to find lightweight cotton items.

If you need to buy new clothes, then buy the cheapest. They are going to be washed at 60°C to kill any bacteria and fungi, so buy inexpensive things and allow for shrinkage.

Overall, layering items of clothing is a good idea. If the temperature changes, you can add or take off layers.

Children are likely to have acute diarrhoea and vomiting as side effects of the chemotherapy, so take in lots of pyjamas, particularly pyjama bottoms.

Our recommendations for clothing are:

1. At least eight sets of lightweight cotton pyjamas
2. Five sets of socks and underwear

3. Three pairs of tracksuit bottoms or shorts
4. Three loose T-shirts
5. Slippers or flip-flops for wearing on the ward. These will also need to be washed at 60°C.

“Don’t plan on taking too many clothes into hospital for yourself – a week’s worth is enough at a time.”

“Take enough clothes so that you can wear a completely different set each day.”

“I bought clothes one size too big for Tom, so they were quite loose and comfy. But remember, your child will lose weight, so a variety of sizes is a good idea.”

“We bought a size 18 months too big for Thomas, who was 11 months old when he had his transplant, as we knew the clothes would shrink on washing. I’d recommend having lots of pairs of pyjamas on hand. The child may vomit and have diarrhoea for two or three weeks.”

“We used only vests and shorts for Jonathan during his BMT. Choose clothes that are as comfortable and as loose as possible, so it’s easy when medical things need to happen.”

Planning for your child’s room and entertainment

In our experience it’s good to make the isolation room as much like home as possible, within reason, and to think about how you will pass the time in hospital. Presents are allowed but the hospital will have a list of what is allowed on the ward and in the isolation room. For example, comics will have to be in sealed packets, and no old books are allowed. The hospital may have a large supply of DVDs and games, in which case you won’t have to take in lots of things.

> *“We filled our computer’s hard drive with Jonathan’s favourite cartoons and films.”*

Here are some additional things we think might help:

1. Displaying posters and photographs of family and friends can make you and your child feel more at ease. Make sure they are laminated or in a plastic cover, so they can be wiped down.

2. Some hospitals may allow you to bring in your child’s own duvet cover as long as it is washed at 60°C. It will have to be changed every few days. Ask the staff about the rules that apply in the hospital where your child will be receiving treatment.

3. For young children, you can arrange to bring in a small supply of their favourite toys. These too will have to be washed at 60°C.

4. Plan to take in a games console.
5. Consider taking in your own portable television.
6. For parents, buy a good, new book (although you may not have time to read it).

Our tips:

1. All toys, games and books need to be new or nearly new. Plastic toys, such as construction bricks, can be washed in hot soapy water and need to be dried thoroughly. Unvarnished wooden toys are not allowed because they carry a high risk of transferring infection.

2. You can't take newspapers into the isolation room but you can read them in the parents' room.DVDs and CDs can be brought into the hospital, but they will need to be wiped down.

3. Remember, the more things there are in the isolation room, the more the dust will gather. Plan to change things around after a couple of weeks, bringing in different items to keep everything fresh.

4. Don't forget to clean all items that have a hard surface with an alcohol-based wipe before taking them into the isolation room. This includes games consoles, a television, bottles, cans, and plastic toys.

12

Day of admission for the BMT

No matter how many times you have visited the ward and how much planning has been done, you and your child are going to feel anxious on the day of admission.

You will probably be bombarded with lots of do's and don'ts from the hospital. Here are our tips that may make a difference.

Bring some basic provisions for you and your child on admission

The hospital will have its own set of rules for what foods can be stored on the ward and what can be eaten. It should provide you with an important list of do's and don'ts. Stick to all the rules as there are valid reasons for them; the most important of which is to prevent infection.

When you first go into hospital it's good to have a few small provisions with you so you can have a drink or make a cup of tea until you find your feet in your new surroundings. The golden rule is to buy individual packets but also remember there might be limited space for storage. Remember, no raw meat, fish or eggs are allowed on the ward.

Some suggested provisions you might consider taking in are:

- Individual sachets of tea, coffee and sugar (plus your own mug)
- Individual cartons of fruit juice or cans of soft drink
- Milkshakes in sealed cartons
- Individual sachets of butter or margarine
- Small cartons of UHT milk
- Individually wrapped biscuits or cakes
- Tinned meat or fish
- Fresh bread

Toiletries and personal items

Opened bars of soap and face clothes are not normally permitted. Any toiletries, including shower gel and soap, should be new. Avoid strongly perfumed products, and buy alternatives such as Sanex. This is because during chemotherapy your child's sense of smell may be heightened and such products could add to any feelings of nausea they might have. In addition, your child's skin will be very sensitive following chemotherapy.

Our tips:

1. Buy disposable face wipes: they can be really handy.
2. Buy a new body puff or scrub for showering.

3. Shower gel tends to be more convenient than soap.

4. Take in your hairdryer, because you will have to wash your hair daily.

5. Toothbrushes are provided but take in your preferred toothpaste and a cocoa butter lip salve.

6. Invest in a good hand cream, because you will be washing your hands all the time. Your hands can get really sore, and broken skin can be an entry for infection.

7. As hand washing is so important in preventing the spread of infection, you will have to take off rings, bracelets and watches to wash your hands. It might be easier if you left these at home to avoid worry about them being lost or stolen.

8. Men can start to grow a beard instead of having to shave daily using disposable shavers.

9. For young children and babies, the hospital may let you bring in your child's usual feeding cup, bottle, teat, feeding spoons and bowls, but all equipment will need to be sterilised at the hospital.

> *"You will realise you need to wash your hands many times day and night, which makes them dry and sore. Use Diprobase cream after hand washing to help prevent them becoming sore."*

During your stay in hospital

Look after yourself as a carer

We think it is really important that you look after yourself, so that you stay in good health to care for your child or relative. If you become ill, you will not be allowed on the ward because you will be an infection risk to others. Remember anyone with signs of illness (e.g. a sore throat, cough, cold, runny nose, high temperature, diarrhoea, vomiting, an eye infection or a cold sore) will not be allowed on the BMT unit or to come into contact with your child.

Our tips:

1. A good night's sleep is essential. Some people found earplugs helpful in reducing the background noises of the hospital, especially if they were sleeping in the same room as their child. The noise from alarms on equipment and nurses popping in and out of the room during the night can be disturbing.

"Make sure you have a good breakfast – sometimes it may be the only meal you will get until much later in the day."

"The carer's bed in hospital was hard. A blow-up camping mattress as a topper can help, and these can be easily wiped down. Camping shops have lots of choice."

"The time flew by. There's lots to do, so time passes quickly."

2. Make sure you eat regularly and healthily, and drink plenty of fluids. You can tire of the hospital canteen pretty quickly, so microwaveable meals are essential. Ask where the nearest shops are.

“Try to eat little and often. Never make yourself go hungry. A lot of physical caring can easily make you hungry during the day. The busy days will make you easily forget to eat proper meals.”

3. Don’t stay in the same room for too long in a day. Make sure you leave the isolation room and hospital for at least a couple of hours each day. Make sure the person you are caring for understands the need for this. They may also want you to take a break to give them some time alone, as living together in isolation 24/7 can sometimes be difficult. Going for a walk or just doing something different can help. A good time to leave the hospital for a break is when the play specialist, teacher or volunteer visitor comes.

“Make sure you force yourself to leave and have a break.”

“Take time out on your own; even a 10-minute breather is good to get your head straight.”

“Remaining upbeat is easily said but is really needed. If possible, try and get a night away with friends. You really will be recharged and ready for the next round!”

"Some time alone is essential. A walk in the park is good – fresh air."

"I bought a walkie-talkie to use during Jonathan's BMT. When I had my meals in the sitting room, I used it to keep in contact with him. It gave me peace of mind and also made Jonathan feel that I was always nearby. You can even pass the walkie-talkie to the nurse if you need to go out of the hospital."

Coping with daily practical tasks

As well as just 'being there' caring and giving emotional support to your child, in our experience there are lots of practical responsibilities as well. These can drain you physically as well as mentally. The upside of this, though, is that these practical tasks keep you occupied and structure your day.

Here are some of the things parents had to do:

1. Strip the bed daily and remake it with clean sheets and blankets provided by the hospital, and again if the bed became wet or dirty.
2. Every day change the towels provided by the hospital.
3. Keep the isolation room clean and tidy.
4. Make sure clean clothes were available every day.
   You, as carer, and your child need to change your clothes in the morning and at night. You will be responsible for washing and drying the clothes used.

5. Shower and wash your hair every day (remember to take a hairdryer in).

6. Bathe your child (no flannels or sponges can be used and the hospital will provide disposable wash cloths) and cover them in moisturising cream, provided by the hospital, every day. This is to help keep the skin soft and supple, and to prevent drying so it's less prone to crack and be a site of infection.

7. Shop and cater for yourself.

"Prepare to be exhausted."

"Going to the laundry will be a big and endless job during BMT. I normally went at midnight, which is the off-peak time. It can be annoying if you go to the laundry during the day and have to wait or keep checking the availability of washing machines."

"I made a daily schedule to check what I had to do and what I had done every day, i.e. mouth washing, dusting, bathing."

"I asked our friends to help keep us topped up with what we needed: toiletries, clothes, fresh fruit, snacks, energy drinks and packed meals. This was especially important during the critical period. It let us concentrate on the caring and saved more time for rest."

Our tips:

1. Ask a friend or relative to take a few things home with them to wash. This will reduce the burden on you.

2. Be mindful of how much personal 'stuff' you take into hospital. The room is small so it gets easily cluttered. De-clutter from time to time and get others to take things home for you.

3. Enjoy the company of other parents when you are doing some of the housekeeping. The kitchen and hospital launderette can be good places to make friends.

> *"A community builds up in hospital as everyone in the ward is in the same boat. They become a sort of new family. You celebrate the happy moments together. A very supportive group built up for us all and has resulted in lasting friendships."*

4. Sticking to a routine helps structure the day and keeps things as normal as possible. For example, stick to the routine of pyjamas at night and getting up and dressed in clean day clothes every day.

5. Make the most of the quality time you get to spend with your child.

6. Make fun out of what you have to hand, e.g. blow up gloves, decorate the bathroom door, make hats.

Coping and being strong – the ups and downs of BMT

Our experience is that going through BMT is far from easy for your child or you. Many things are uncertain – how ill your child will be from the chemotherapy, whether the transplant will work, and whether there will be complications. There will be some days when your child will not want to communicate with you or anyone else because they feel so poorly. As a carer, this will all be on your shoulders, and it will take its toll.

Our tips:

1. Just look after yourself and your child. Trust the nurses and the BMT team, and let them get on with their work.

2. Take things one day at a time. Don't plan too far ahead.

> *"It would be difficult to cope with the immensity of it all if you were timing and chasing care all the time. I felt that I had to let go of the responsibility, and look to the next few days and not beyond. Otherwise you drive yourself mad."*

> *"Have faith in the doctors and nurses, but address with them any concerns or questions you may have."*

3. Build good relations with your care team. They will be a major source of support to you and your child.

4. If your child is in pain, it’s worth talking to the pain team because sometimes they may provide help that the nurses may be unaware of.

5. Remain 100% focused on your child and stay strong. Children are good at picking up your anxieties.

“Stay as positive as possible.”

6. Your child may inevitably feel pretty low some days. Get them to express their mood on the whiteboard in the room, so you and the nurses can tailor how you communicate with your child. Some days your child may be up for lively banter, and on others they may just want someone around but not necessarily to chat.

“Noah used this to describe his mood – a smiley face, grumpy, etc. This helped everyone understand how he was feeling, and helped the nurses communicate with him.”

7. Don’t set your expectations too high, especially concerning the timing of your return home.

8. Keep a note pad to hand and write down questions you want to ask the consultant prior to the ward round. Once the ward round has finished you may not see the

consultant again for another few days, and it's frustrating if you forget to ask things.

9 In our experience you may need help as you adjust to hospital life and separation from the rest of your family. Seek the help of the clinical psychologist, who should be available and visiting you from time to time. The clinical psychologist is there to offer you, your child and members of your family individual support and counselling while you consider BMT, then both during and after BMT.

10 Remember you can ask for your child's folder at any time. You can ask questions about the bloods, and staff will be reassuring.

> *"You become an expert on bloods very quickly – you start to speak the same language."*

11 Make the most of any alternative therapies that may be offered to help you de-stress, e.g. reflexology, back massage, Reiki healing. They are there for parents as well as the patient.

Special considerations if BMT is over Christmas or New Year

Some of our panel experienced BMT over the Christmas period. Hospitals put in a lot of effort to make Christmas extra special for children. Your child is likely to be well entertained and showered with presents.

Be aware that the bank holidays may affect access to your usual consultant, nurses, dietitian and psychologist. However, this should not affect your care.

Our tips:

1. If the BMT is scheduled to take place over the Christmas period it is best to check exactly what cover is being provided, to set your mind at ease. Talk it over with your medical team.

2. Over Christmas there will be no school, and this can be frustrating and boring for the child. This is when you need to ask the play specialists for help and use the volunteers who come in once a week to play with your child for an hour or so.

Practical care tips

This section lists some of the practical advice that we found helpful in caring for our children.

Our tips:

1. Bring in some tubi-grip stretch bandage: the bandage that is used to support joints during sport. This is great for keeping tubes and lines (such as the PEG and Hickman lines) secure so they don't dangle about. It's important to ensure that the lines are cared for in a way that will not put your child at risk of infection.

2 If your child is wearing nappies, make sure the ends of the Hickman line are not tucked into the nappy. The nurses will give you some elasticated material to make a vest, so that the ends of the line can be wrapped in soft gauze and tucked into the vest when it is not in use.

3 Follow the doctors' recommendations, especially concerning the precautions to take to help prevent and treat mucositis (where the mouth or gut becomes inflamed) through oral hygiene. This involves three 30-second treatments that entail washing out the child's mouth with a special sponge attached to a lollipop stick. It equates to 30 seconds of swabbing in the mouth with foam, followed by the use of mouthwash. Your child may not like it, but it's really worthwhile persevering as it helps reduce mucositis.

"It's being 'cruel to be kind' but it does help."

4 Tins of orange segments in juice (you will need to wash the can) and ice lollies are great for helping with mucositis.

5 Your child's bottom may become really sore due to the effects of the chemotherapy. The barrier cream 'Proshield' (which can be bought at a pharmacy) can be a really good treatment to help relieve a sore bottom.

6 For babies and young children, we recommend changing nappies every hour and use of a cream called 'Bepanthen'

(which can be bought from most supermarkets). This is because the chemotherapy results in faeces (poo) and urine that can irritate and cause severe peeling of the bottom. It is much worse than a nappy rash.

"This may seem excessive but it reduces your child's discomfort."

7 For babies and young children, some floor play is permitted, but you will have restricted space (a mat of 1 × 1.5m). Some days are 'line' heavy, so these may be most suited to play in the cot or bed. Make the most of days that are line-free. Don't forget, keeping your child infection-free means you have to adhere to strict guidelines concerning sterility of toys. If toys fall outside the sterile area, then they can't be used again until they've been re-sterilised.

"Some days your child will be up for play, others they won't."

Feeding your child in hospital

In our experience the use of restricted diets during BMT varied between hospitals. Here are the different approaches that were followed.

Until your child starts full isolation (after chemotherapy) they will be able to eat food from the regular hospital trolley. But once chemotherapy begins, your child's ability to fight infections will be reduced and they will be put on a BMT 'clean' diet. This is because many foods contain some small amounts of microorganisms, such as bacteria, that are usually harmless to a healthy immune system. The BMT diet restricts the use of some foods and sets guidelines for the preparation and cooking of other foods to reduce the number of microbes that your child encounters. Your child's sense of taste will also change, so that may add to their problems with eating.

At University College Hospital, London, which treats older children, takeaways are allowed pre-BMT and there are no 'clean' diet requirements. However, a stricter diet has to be followed after chemotherapy and during recovery, when your child's white blood cell levels, and hence their immunity, are low.

Our tips:

1. Always respect and follow the rules and guidelines of the BMT centre treating your child concerning what foods your child can eat and when.

2. Don't worry unduly about the 'clean' diet. It's really uncomplicated and easily manageable. Just read through the diet sheet that will list the foods allowed and foods to avoid. The foods allowed will form the basis of any snacks or meals you can prepare for your child. In general, tinned foods, such as fruit and puddings, meat (ham, corned beef, frankfurters) and fish (tuna, salmon), as well as canned vegetables, pasteurised milk and individual packets of food

and drink, are good staples. Remember to remove any uneaten hot food or opened food within your child's room after half an hour.

3. If your child is a baby, you may be able to keep breastfeeding – discuss this with your BMT consultant.

In our experience, at some point your child may go off their food and they may need to be fed by total parental nutrition (TPN), where liquid food is given via the veins (intravenously).

When your child goes off their food

Encouraging your child to eat and drink is vital, so their bone marrow can grow, but there may be times when your child is just not well enough to eat or drink. In these situations the doctors and nurses will discuss with you the use of a nasogastric tube (NGT). This is a tube that goes up your child's nose and into their stomach, and allows feeding support. We list below some of the advantages and disadvantages of NGT.

Advantages are:

1. It helps keep your child's appetite up and their stomach working for when they go home, and this aids recovery.
2. All oral medicines can go down the NGT.
3. It gives some freedom from an intravenous line; but an

intravenous line may still be necessary if your child needs antibiotic treatment.

Disadvantages are:

1. Sometimes your child will just resist and hate the idea of having a tube up their nose.
2. If your child has severe vomiting or diarrhoea, the NGT feeds may need to be stopped.

If the NGT is not suitable or has to be taken out, this is when TPN will be started through the Hickman line. TPN provides all the nutrients your child needs, but you should still encourage them to eat and drink what they can manage. This will help keep their stomach working normally.

> *"Thomas had TPN but we still tried to tempt him daily with his favourite food. He refused for ages and then one day he started to eat a small bit and then more each day."*

Consider having percutaneous endoscopic gastrostomy

In our experience some parents, following consultation with their medical team, decided to pursue percutaneous endoscopic gastrostomy (PEG) for their child. This involves a small operation to put a semi-permanent tube straight into a child's stomach.

Some advantages are:

1. All oral medicines and feeds can go through the PEG.
2. Your child might find they have a very sore mouth after chemotherapy, and this is when PEG is really useful.
3. PEG can be useful post-transplant if your child still requires lots of medicine and overnight feeds.

A disadvantage is:

1. Having a PEG involves a small operation to both put it in place and also to take it out when it is no longer needed, and as with all operations this carries some risks.

> *"Sam had a PEG, which was fantastic, as all his medications could be given through it."*

Taking medication in hospital

Our tip:

1. Sometimes crushing up tablets with a pestle and mortar and then mixing them up with fromage frais can help make them more palatable and easier to swallow.

Drinking

Your child will be expected to drink a lot to flush out the toxins from the chemotherapy, to stay well hydrated and to keep their organs working well. The amount of fluid taken in and the amount of fluid passed will be measured. Mineral water is not allowed. All water must be sterile or boiled, including water used to make up squash.

Our tips:

1. Cartons of juice, cans of drink and the juice from canned fruit are good ways to boost fluid intake as boiled and sterile water isn't very appetising.
2. Ice lollies made with fruit juice and boiled water are good too.
3. Boil up water in advance for the next day.

Other support for you and your child while in hospital

During BMT our children were provided with a range of support to pass the time and help them deal with any stress and anxiety they were having. Our experience was that the services below were hugely beneficial, and we would recommend that you take full advantage of them.

A play specialist – this is seen as an important diversion for your child when they are in hospital. It can give them an outlet for some of their feelings and emotions. The play specialist will want to know as much about your child as possible so they can tailor the play activities to suit. They will also work around you as to what time of day works best for you and your child.

> *"The childcare specialists will bend over backwards to help, so ask for things that your child would like, as they probably have them in their stock cupboard!"*

> *"Arrange the best time for the play worker and teaching team to spend time with your child. Try to avoid the same time as the ward round, so the kids can enjoy a relaxing time with the teacher or play worker."*

Physiotherapy – we found that sometimes being in bed over a long period of time can reduce your child's mobility and flexibility. This is nothing to be worried about and the hospital will have a physiotherapist and occupational therapist who will assess your child's mobility prior to transplant and work to maintain it during the transplant period.

Hospital clowns – there to make you laugh and brighten up your day.

Music therapy – this is musical play. It also helps to de-stress your child and provides a non-verbal means of expression and communication.

Volunteers – the hospital may have trained volunteers who will come in and give you a break from your child's bedside. Take advantage of their support to give yourself some 'me' time. They are also a fresh face for your child to see.

Social services – the hospital may have staff who can advise you on what benefits you may be entitled to during your hospital stay.

Schooling for your child in hospital

While in hospital your child will receive about an hour of schooling every day. The hospital school will operate during the same term times as regular schools though, so be prepared if the stay in hospital covers a school holiday period.

The teacher will visit you one day to arrange a daily time for your child's schooling. Our experience was that the teachers were really good and very skilled at tailoring the schoolwork to the child's level of wellness, and you will find that your child is well enough for this hour of school. The children tend to really look forward to the 'class', because it brings a fresh face and a mental break from being in hospital. The hospital teachers will try to speak to your child's class teacher to tie in with what is being covered at their school, and the work is done on iPads.

Our tip:

1. Actively seek the support of the play therapists during school holidays.

Waiting to come home

Our experience was that waiting to come home was hard; you do start to miss everyone enormously. We found that little setbacks can hold you back (e.g. blood counts not yet high enough and infections).

Our tips:

1. Lower your expectations about the timescale involved.
2. Be careful about mentioning going home to your child. As you can imagine, they can get really disappointed if it doesn't happen.

'Don't build up your hopes until you walk out the door.'

"It's so important that you or the nurses don't build up false hope of going home for your child."

Preparing for the return home

The wonderful day will come when the doctors and nurses will tell you that it's time to start planning for your return home. They will advise you on what actions to take at home, to reduce the risk of infection to your child.

Our tips:

1. Always follow the advice given by the doctors for recovery at home. The restrictions they advise are to protect your child from infection.

2. You have to make sure your house is clean, but you don't have to go overboard and start shampooing the carpets and washing the walls. Just make sure the house is well dusted and vacuumed, and continue to dust and vacuum weekly once your child is home. Using furniture polish helps pick up small pieces of dust.

3. Make sure uncarpeted floors are clean and curtains and bed linen are laundered, as these are common sources of infection in the home.

4. You should postpone any building work in your home until your child is fully recovered. This is to reduce the risk of infection from the fungus called Aspergillus.

5. Pets carry bacteria, viruses and fungi in their saliva, urine and faeces, so they can pose a health risk to your child during post-BMT recovery. If you have pets at home, then make sure your child's hands are washed thoroughly after touching them and that your child is not involved in clearing up any mess. It is important that dogs and cats do not sleep with or on your child's bed.

Discharge from hospital

"There were tears of joy as we were going home, but also tears of sadness at leaving what had been our home for the past six weeks. We had become so close to all the nurses and doctors … they had become a huge part of our lives during this time, and part of something life changing too. It was all very emotional."

"Discharge day was very exciting … we arranged for transport home with a family member, and while we were waiting packed our cases and bags so we were ready."

Our tips:

1. The day before discharge day, prepare boiled water for your journey home.

2. Get ready early on the day, because time runs away with you. You will need to clear:

   - The fridge and cupboard you have used and leave them in a fit state so that other parents can use them when their child is admitted onto the ward.

   - Your child's room. Keep a stock of spare carrier bags – they are useful for taking things to the car/taxi for your journey home.

3. The nurses will give you a month's supply of medicines that

your child will need to take at home. Make sure you go through all the drugs with the nurses so that you are confident that you know the doses and when and how to give them.

"It is worth taking some time to go over the chart of information you are given."

"We gave Sam his drugs as a run-through a couple of days before we left, so we knew what we were doing once home."

"Ensure that you feel confident to give medicine before discharge. I gave a lot of medicines, including intravenous medicines, to Jonathan. I always made sure I was competent to do any treatment at home and that I knew who to contact for help if needed."

4. Remember to take sick bowls, mints, boiled water and wipes in the car going home, as your child may feel sick during the journey.

5. Make sure you have the contact details and telephone numbers of the BMT team; that is, the BMT clinical nurse specialists and registrar, and their 9 to 5 service during the week. Also make sure you have the details of who to contact out of hours, at weekends and in an emergency.

13

Being at home – hurray!

“Coming home is just brilliant. Well adults can come and visit you. You can go out for a walk but you can’t interact with other children for six months. It’s the chance to live a normal life.”

“Although we were very happy to be home, we looked forward to our twice weekly visits to the transplant clinic. It did take some readjusting to get used to being home, as it seemed very quiet. In hospital there was always somebody in and out of the room, whether it was a nurse or a play specialist.”

Coping at home and support

Coming home and managing the caring yourself can be daunting. It can also be stressful and tiring, because lots of care and nursing is still needed. We found that we had to give a range of medication to our children at home, including:

1. Ciclosporin – to help control graft-versus-host disease.
2. Septrin – to prevent a lung infection called pneumocystis.

3. Aciclovir – to reduce the risk of viral infection.
4. Itraconazole and Voriconazole – to reduce the risk of fungal infection.
5. Penicillin – to reduce the risk of pneumococcal infection.
6. Sulfasalazine – used to treat inflammatory bowel disease.
7. Lansoprazole – used to reduce the acid made by the stomach, so allowing the stomach lining to heal better after BMT.
8. Prednisone – to help control graft-versus-host disease. Mycophenolate mofetil (MMF) – to help control graft-versus-host disease.
9. Immunoglobulin therapy – given intravenously, to provide extra protection against infection.

Keep in mind that the medications that your children receive may be different from this list, as every person is different.

Our tips:

1. Make a decision about how much of the care you want to take on at home. For example, you may not feel confident about giving your child intravenous medicine. It’s important that you take on what you are comfortable with and carefully balance being a parent and being a nurse for your child.

"It all felt very daunting … once home we were on our own! In hospital we felt safe as we had the reassurance of the nurses and doctors."

2. You can expect to receive support at home by community nursing teams for blood tests and dressing changes. This support will be organised by your BMT team and local hospital. If it doesn't happen, chase it up with your BMT team.

3. Sometimes intravenous medication can be given at your local hospital. Talk to your BMT team to see if they can arrange this for you.

"I had Healthcare at Home (HCH), who were fantastic for intravenous infusions. This was coordinated through Great Ormond Street Hospital, initially with my local hospital, then I liaised with HCH direct."

"Home care teams will support you if you want them to with intravenous infusions and line care."

4. Don't get overwhelmed. You are not on your own – your community team and BMT team are there to support you. They are at the end of the phone should you need them. You should feel comfortable about contacting them whenever you have any issues or worries.

5. Your BMT team should give you a month's supply of medication to be taken at home but after that you will be responsible for maintaining the stocks you need.

6. Before your monthly supply runs out it's vital you check in advance with your doctor's surgery its protocol for reordering drugs on repeat prescriptions. Our experience is that sometimes GPs may query the cost of an expensive drug such as voriconazole and won't prescribe it on repeat prescription. In this case, it may be possible to get it through your BMT centre.

> *"The main point is to check all drugs are available from your GP, as my BMT centre said some GPs won't prescribe voriconazole, and in that case we had to get it through them."*

> *"It is good to organise all medication in advance through the doctors so you have the stock when needed."*

7. Check with your surgery ahead of time how long it will take to get the prescriptions through the processes needed, as sometimes it can take four days from putting through your prescription to collecting your drugs, especially if they are unusual ones.

8. Ask for your prescriptions from the BMT team to go direct to a chemist rather than through your doctor. This saves time because when you are in town you can easily collect the medicines.

9 Consider using pharmacy delivery services. Lloyds Pharmacy and Boots will deliver NHS prescriptions straight to your door for free.

> *"I posted my prescription to my doctors, and asked them to forward it to Boots, the chemist, where I either collected the drugs or they delivered them. After 11 years of caring for my son, I found this method worked best for me."*

10 You still need support, so ask for help from friends and family. Our experience is that they are more than happy to help. Just simple things, such as collecting your meds, doing your ironing and collecting your other child from school, can make a difference.

> *"Delegate out big time, you need extra time with your child."*

> *"I couldn't cope with even part-time work when my child came home."*

Recovery at home

The doctors will tell you that the first six months of recovery after BMT are the most critical, and they will give you some key do’s and don’ts.

Our tips:

1. The diet for your child is likely to be significantly relaxed when you get home, but make sure you ask the dietitian what they recommend and ask if your child would benefit from having high-energy supplement drinks. To read more about the diet precautions for children that are returning home after a BMT, you can **visit this resource** made by Great Ormond Street Hospital.

2. Wash your child’s bed linen, towels and pyjamas at least weekly. They can be washed with other family laundry. Your BMT team will advise you as to any special precautions you may have to take for drying the clothes. Our experience is that some parents were told they could dry the washing outside, while others were told to tumble dry washing, as fungal spores could blow onto it. Check with your team!

3. Similarly, your child’s dishes and eating utensils can be washed in hot water with everyone else’s.

4. Make sure your child does not touch or handle any pets. If they do, then make sure they wash their hands thoroughly after contact.

5 It’s really important to reinforce the need for hand washing after using the toilet and before eating.

6 The great news is that you don’t have to stay indoors all the time:

- It’s just advised to stay away from crowded places, such as restaurants, shops, cinemas and parties, and to avoid crowded public transport.
- Going for walks in the local park and getting some fresh air, rather than going to town, is a good idea.
- Family and friends can visit, but not too many at once and not if they are ill. Remember, children at school have a greater chance of carrying infections because of their school environment.

> *“We were told to “screen” any school friends that came to visit. We had to look out for chicken pox, measles and the cold sore virus, herpes simplex. We were told to report any exposure to these to the doctor or BMT nurse immediately. Everyone, family and friends, understood when we told them they might pose a risk to recovery.”*

> *"I always asked any visitors to wash their hands, to avoid any awkward situations. This helped get people to understand any precautions we needed to take to avoid the spread of infections."*

7. You need to practise good hygiene and bath or shower your child every day. We recommend using a non-irritating mild soap or shampoo. Alpha Keri oil can be used in the water, and Diprobase or E45 cream on dry skin.

8. Good dental hygiene is also really important, so make sure teeth are brushed at least twice a day, using a soft toothbrush and toothpaste. Change the brush every three months. We were told to make sure the areas where the gums meet the upper and lower cheek are really brushed thoroughly.

9. If your child needs urgent dental work, this should be reported to your BMT team. They will help advise you on the way forward.

10. If your child has had graft-versus-host disease then you have to take special precautions and protect your child's skin from the sun. These include:

    - Covering the skin with clothing as much as possible.

    - Ensuring they wear a hat, especially until their hair grows back.

Plastering them in sunscreen. Doctors told us to use total sun block for the first year after a BMT.

11 It's a good idea to build up your child's muscles, because your child will have lost some muscle tone after their stay in hospital. Introduce some gradual exercise, such as walking. Our experience is that your child will tire easily and may complain of muscle aches and pains, but this will reduce over time.

12 Further on in the recovery period, ask your doctor about when you can relax or stop the various precautions they have advised you take at home. Your doctor is the best person to advise you, as they know how your child is recovering.

> *"It can seem like a long road to recovery, but with small steps things move along quite quickly."*

Dealing with the unexpected once home

Our experience is that during the recovery period you should expect some 'hiccups' in your child's recovery, and you may find that your child requires some further short stays in hospital. Some common problems that we had to deal with were infection of the central line (sometimes referred to as a Hickman line) that required intravenous antibiotics, shingles and sometimes graft-versus-host disease that needed specialised drugs such as Ciclosporin.

Our tips:

1. Watch out for signs or symptoms of infection and report them as soon as possible to your BMT nurse or doctor. These include:

    - A temperature that is greater than 38°C. It’s a good idea to have a thermometer at home and know how to use it. If your child has a high temperature you should speak to your doctor. Don’t be tempted to give them Calpol until you have spoken with a doctor.
    - This is because Calpol can mask a fever
      Any breathing problems, such as a runny nose, cough, sore throat or cold.
    - Stomach problems, such as diarrhoea, nausea, vomiting or cramps.
    - White patches (thrush) or sores in the mouth.

2. Look out for signs of graft-versus-host disease and report them as soon as possible to your BMT nurse or doctor. Things to watch out for are:

    - Any new rash over the body, palms of the hands or soles of the feet. The rash may or may not be itchy.

- Yellowing (jaundice) in the white of the eyes or skin.
- Watery diarrhoea.
- Loss of appetite with weight loss.

3 Also common post-BMT is shingles. This is caused by the reactivation of the chicken pox virus, herpes zoster. Our experience was that shingles can develop quite slowly, starting with a sore back, and then a rash that develops into raised pimple blisters. Our advice is to contact your local GP or local hospital. They will give you the appropriate anti-viral drugs (the Aciclovir family of drugs) to treat the shingles.

> *"The nurse told me I could use a cold sore treatment cream on the spots to treat Sam's shingles. He found it soothing and it calmed things down."*

> *"Applying calamine on the spots can also be soothing for shingles."*

4 Don't panic or be too alarmed if your child needs to be re-admitted to hospital. We found that often treatments can be dealt with by the local hospital. In our experience the hospital involved with your child's BMT will maintain links and give advice to your local hospital.

“Remember though, the “curve-ball” can come at any time and you may need to go back into hospital if there is a line infection, or some other problem, that can’t be treated at home.”

“Alex has had four hospital stays of three or four days each post-BMT. He had a fever that might have been because of a line infection, and some reaction to medicines post-transplant. He is now doing really well and is back at school.”

“Set up a good relationship with the local hospital. Always remember the name and try to talk to the exact doctor or nurse who knows your kid very well.”

“If your child is to have intravenous treatment at the local hospital, recognise that different hospitals follow similar but different procedures.”

“React quickly, especially if there is a line infection. Sometimes it can be crucial. Call the local hospital before arriving, so they can plan a room.”

“Remember, after the BMT you will be full of knowledge about how to care for your kids, and this knowledge may be better than that of most of the local nurses or doctors. Be prepared to challenge them if you think your kids are not treated properly, especially post-BMT if a line infection occurs.”

Outpatient clinics post-BMT

The doctors will want to carefully monitor your child's progress after a BMT so, initially after discharge, you will need to return to the hospital every week for check-ups. The frequency of these visits will decrease over time as your child recovers and moves on from the transplant.

Our tips:

1. Plan for these clinic visits, because travelling on crowded public transport is not recommended. You could ask your friends and family if they could give you a lift, or sometimes the hospital might be able to offer help with transport.

2. Use the time with the doctors to cover any questions or concerns you may have. Keep a list of questions at home and ask them at the clinic.

> *"Outpatient visits – we are helped incredibly because the hospital arranges transport. I don't have to worry about it. I drive but worry about parking, and without hospital transport I would have to try to line people up. We are collected 2–3 hours before an appointment."*

Schooling at home

Your child is entitled to home schooling while they recover from BMT until the point when your consultant says it is OK for your child to return to regular school. The hospital school will speak to you when you are approaching discharge, and they will connect with your local school and home education service. You will be allocated a suitable teacher, who should be skilled in children who cannot go to school, and they will come to your home. In our experience this took some time to set up but worked well once arranged. Your child can expect to receive a statutory minimum of five hours' schooling a week and they will be given homework. The home tutor will keep the lines of communication with the school open.

Our tips:

1. If you don't hear from your home education service, then chase them.

2. There may be some subjects that your local education authority won't cover (e.g. French). For these subjects you could consider paying for a private tutor. Costs are around £30/hour at GCSE level.

3. Stay in contact with the school or college and keep them informed of your child's progress. You can do this by telephoning the headteacher/principal every two weeks.

> *"The one-to-one tuition provided at home is very good and we found that our son covered as much work in five hours of one-to-one as he would have in a whole school week in a class of 30! He continued to progress and is not at all behind with his schooling."*

Returning to school or college

The wonderful day has come when your child's immune system is strong enough to allow them to return to school or college. However, it can be a daunting experience for your child.

Our tips:

1. Don't expect them to attend full time immediately. Consider doing just mornings perhaps every other day, then, just mornings. Introduce them to full school days gradually, because they can become extremely tired.

2. They may feel a bit displaced and emotional, so it's worth seeing if the school/college can provide some extra help during lessons for a few weeks and some emotional support.

3. It might be a good idea to get in touch with the psychological support services at the hospital to see what they advise for your child returning to school and whether they can help to brief the teachers and the wider school on what to expect.

> *"It is a huge thing for your child to return to school after so much time off and try to fit back in. Seek support! We didn't think to and it proved to be a rocky ride for a couple of months."*

Vaccinations after BMT

About a year after the BMT, your doctor will check if your child has immunity to common diseases. Our experience is that the doctors will recommend your child be revaccinated against conditions such as measles and tetanus.

Your child and life without CGD

It can take up to two years for your child's immune system to be fully functioning, but don't be surprised if once the BMT is over your child starts to feel well and wants to spread their wings. This is absolutely normal after living with CGD and the restrictions it has placed on what they can and can't do. For parents, it's our experience that it's quite difficult to let go of the old concerns and anxieties that have been a major part of your life, sometimes for so long. Some parents refer to it as a new reset position and find it hard to adjust.

Our tips:

1. Give your child space and time to readjust. It's likely they will not want to be fussed over in the same way as before.

2. Ask your child to tell you when they think you are being too protective about their health and what activities they now want to do. Address it together and work out a pact on how to deal with these situations that works for you all.

3. Ground yourself in the new reality and share with them the new things your child can do without you worrying.

> *"Tom learned to drive and passed his test second time. It gave him so much independence: something that he hadn't had."*

> *"Once cured and the lines were out and the wounds were healed, my child went on to do lots of things they could not do before, such as cleaning out the pets' cages, joining eco clubs using compost and mulch, going to play on playgrounds where mulch is on the ground, going on biology field trips and visiting old places where there is a lot of dust."*

4. Consider having some psychological support to help you deal with any lingering anxieties or behaviour that has become engrained.

We wish you well and every success for you and your child and your journey through BMT.

Acknowledgements

We are grateful to the XLP-Research Trust, who inspired us to develop this booklet; to the Children's BMT Unit, Great North Children's Hospital, Newcastle, who shared their pre-admission information with us; and to all the parents who helped us compile this information.

Costello Medical Consulting has provided Scientific and Creative support for the 2023 update of this material on a pro-bono basis.

This booklet was developed through interviews with parents, conducted by Dr Susan Walsh.

Contributors

Claire Bonner, mother of Max, who had a BMT on 29 June 2012.

Gill Gregory, mother of Joe, who had a BMT on 11 February 2010.

Louise Hannard, mother of Alex, who had a BMT on 22 November 2011.

Ning He, father of Jonathan, who had a BMT in January 2013.

Jackie Irvine, mother of Tom, who had a BMT on 15 April 2011.

Kevin McGuiness, father of Thomas, who had a BMT in April 2012.

Caroline Somers, mother of Noah, who had a BMT on 27 January 2012.

Angela Stockwell, mother of Sam, who had a BMT on 16 December 2011.

14

Our lives post-BMT

These are a few stories from people who have had a BMT to treat CGD. These stories show how, when successful, a BMT can improve someone's life. But it is still important to remember that while these are all success stories, there are also stories of people who experienced problems with BMT.

Max's story

"To go ahead and have the BMT was the best decision ever. After the BMT I grew taller, became stronger and felt so much better. It was great to feel well, normal and not need to return to the hospital all the time; it was like a huge burden had been lifted.

Now I'm at university in Nottingham studying property finance and investment. I'm also interested in photography. I go to the gym and like working out and eating healthily – I make a lot of smoothies! I love partying with my friends. I'm really enjoying student life."

Tom's story, as told by him and his mother

Tom says: "My BMT has been life changing as I am now pain free, I can eat what I like, I have put on weight and only have to take one medication each day. The first couple of years following BMT I was making up for lost time and enjoying life and all the things I was unable to do when I had CGD.

Following BMT I was fortunate to holiday with friends in Thailand – something I would never have contemplated before. I wasn't interested in settling into a career straight away, I just wanted to enjoy and experience life, so worked in bars, but I have now completed my Apprenticeship and am enjoying my job as a recruitment resourcer.

I spend much of my weekends with my son, Freddie, who is three years old; he is great. I know how fortunate I was to receive a BMT. For the first time in my life I can make plans for the future, as now I have one."

Tom's mother says: "Tom is living life to the full. In the months immediately after his BMT he focused on learning to drive and passed his driving test. Since then, he has completed his Apprenticeship and is now working for a global recruitment company as a recruitment resourcer in the construction and engineering sectors. He left home for a year, renting a flat, but is now back home to save some pennies. He enjoys time with his stepson and has a great network of friends who he loves to socialise with.

Sam's story, as told by his mother

"Sam is doing really well. He's at university in Norwich studying politics and is loving the whole experience, so we are delighted.

The hospital visits are reduced to seeing specialists at Guy's and St Thomas' Hospital in London. We have a few skin issues owing to the high levels of Voriconazole, which has resulted in some pre-cancerous cells. But we have a check-up every three months and the hospital staff have been fantastic. Any skin tags or blemishes that look suspect are removed by local anaesthetic immediately, so the treatment is extremely efficient. Voriconazole saved Sam's life, so the skin issues are a small price to pay in some regards when you look at the big picture."

……

Joe's story, as told by him and his mother

Joe says: "I'm very grateful to have been given the opportunity to lead a normal life and not worry about the impact of CGD. I feel as if I owe my life to my donor and I will always be thankful for what they did."

Joe's mother says: "Since his BMT in March 2010 Joe has been leading a very normal life. He has achieved excellent grades in ten GCSEs and is currently in his final year studying A levels in biology, chemistry and economics. He is looking forward to going to university in September."

Jonathan's story, as told by his father

"Jonathan was born with X-linked CGD, severe visual impairment and hearing loss. He had a lot of treatments and procedures to manage the recurring infections and inflammation that he experienced since birth. His condition was kept stable with routine treatments until he was five years old, then he developed pancreatitis in 2012.

He had to be prevented from eating and drinking for eight months. Jonathan was lucky because a donor was found quickly. He had his BMT in January 2013 at Great Ormond Street Hospital and was discharged after 63 days as an in- patient.

His cheerful and loving personality had an impact on all the staff and patients around him. In 2013, post-BMT, his bravery was recognised and highly praised when he received a WellChild Inspirational Child Award, which is his biggest achievement to date. He met Prince Harry at the awards ceremony. Jonathan was also on TV, in newspapers and magazines and became a very "famous" young star. It was a fantastic experience, which not only increased Jonathan's confidence but also boosted the morale of the whole family.

Jonathan is now ten years old, and it is four years since his BMT. He had a very long recovery period after the BMT owing to graft-versus-host disease. He is getting stronger and healthier and hopefully the steroid he is on will be stopped soon.

BMT has made a real difference for Jonathan and our family. Not only has it rescued his life and cured CGD but it has also helped him to lead a healthier and happier life. He now can attend school full time and try out lots of new challenges and activities. He likes cooking, telling jokes and all kinds of sports.

The BMT has also taught him to care for other people who need help, as we received massive support and encouragement from others before, during and after the BMT. He has been very keen to become involved in fundraising for different charities.

Nothing could stop Jonathan from discovering the world now. The future is brighter and we are all looking forward to seeing his new achievements."

15

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 **https://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 **The CGD Society - Membership Registration** to become a member.

If we can be of any help, please contact us:

☎ **0800 987 8988** ✉ **hello@cgdsociety.org**

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

16

My notes

My notes

My notes

CGDSociety
Supporting families affected by chronic granulomatous disorder

Jeffrey Modell Foundation
Curing PI. Worldwide.

This leaflet was made possible by a grant provided by the Jeffrey Modell Foundation WIN Program.