

# Haematopoietic stem cell (bone marrow) transplant for sickle cell disease: information for patients

**This leaflet gives information to help answer any questions that you and your potential donors may have about bone marrow transplants for sickle cell disease. If you have any further questions or concerns, please speak to one of the nurses or doctors caring for you.**

## How is blood made?

Blood is a mixture of fluid, proteins and cells. All of these have an important role in providing energy and nutrients to the cells of your body, as well as clearing away waste products through the kidney and liver. There are three main types of cells in blood:

- white cells, which help to fight infection and repair damage
- platelets, which are bits of cells that help to stop bleeding
- red cells, which transport oxygen from the lungs to all areas of the body and then carry carbon dioxide back to the lungs for us to breathe out.

All three cell types are made from a parent cell, which is found in the bone marrow (the fatty bit in the middle of a bone). This is known as the stem cell.

## What is sickle cell disease?

Sickle cell disease is a blood condition caused by mutated genes inherited from each of your parents. Blood is made from stem cells in the bone marrow. The stem cells in people who carry the sickle cell genes produce red blood cells that can become sickle-shaped when there is low oxygen. When red cells have become sickle cells, they are destroyed by the body. This results in fewer red cells, and causes anaemia. These sickle cells are also the cause of the crises suffered by patients with sickle cell disease. For example, painful crises are sometimes caused when the sickled red cells block the blood supply to different areas of the body.

## How does a stem cell transplant work?

The aim of a stem cell transplant is to replace a patient's stem cells with those from their donor so that the patient's bone marrow starts to make the same healthy blood cells as the donor's. For example, if your donor is a carrier for sickle cell disease (has one sickle gene and one normal gene), having a transplant will mean that you will make blood that has a small percentage of sickle cells, but just like them you will not develop crises. Alternatively, if your donor has no sickle genes, you will also make blood that has no sickle cells at all.

## Preparation for transplant

### Finding a donor

The first step in this process is to find a donor among your brothers or sisters. A donor must have the same mother and father as you and must not have sickle cell disease. They may either be a carrier of sickle cell disease or may not have inherited the sickle mutation at all. Both you and your potential donor will be tested by taking a blood sample to see if you are a match. Each brother or sister has a 25% chance of being a full match for you, a 50% chance of being a half match and a 25% chance of not being a match at all. It is entirely up to each sibling if they wish to be tested or not. You must accept their response, and should not try to persuade them to help if they do not feel able to. You must also accept that it is their right to decide how to proceed when the results of the blood tests are known. This means that they may be a match, but choose not to become a donor for you. You will be given time to discuss this in detail with the medical and psychology teams before asking your siblings if they wish to be tested.

### What is a match?

Our immune system uses a group of proteins to tell the difference between us and invading germs and viruses. It is these proteins, which we inherit from our parents, which we use to match you and your donor. You may also hear the term 'HLA system' used to describe these proteins. You and your donor are usually matched using five pairs of proteins – half of each pair from each parent. This is why we only test your brothers and sisters who are from the same mother and father as you. If you match these five proteins, you will be considered to be a full HLA match.

Half match (haplo-identical) stem cell transplants are being investigated, and successful transplants have been reported. We are likely to consider this method in the future, but it is not yet available. Each of your parents (and some of your brothers and sisters) will be a half match to you.

### Once you have a donor

Once it is confirmed that you have a donor who is a match and willing to donate stem cells to you, you will meet the transplant doctors at their clinic. During this appointment, the transplant process will be explained to you in detail. You will need to have a number of investigations first to make sure you are healthy enough to tolerate the procedure. When all of your health checks are completed, you will be scheduled for your transplant admission.

At the start of your transplant admission, you will receive chemotherapy treatment (conditioning therapy) for several days to clear space in your bone marrow. At the end of this process, you will receive your donor stem cells. This is a similar procedure to a blood transfusion – please see our leaflet, **Having a haematopoietic stem cell (bone marrow) transplant for sickle cell disease** for more details. You will remain in hospital for 4-6 weeks, until your new bone marrow and immune system are producing just enough white blood cells to reduce your risk of catching an infection. You are then usually able to go home, but will be monitored very closely at least twice a week in the transplant clinic.

## Are there any potential problems with bone marrow transplant?

Although bone marrow transplant may cure your sickle cell disease, there are a number of potential side effects. Not everyone experiences side effects, but it is important that you have some understanding of the possible problems.

You may experience side effects from the chemotherapy, which can include nausea (feeling sick), low blood pressure, fever and/or vomiting (being sick). You may also find that your new immune system finds it more difficult to fight infections, causing you to get ill more often.

One particular complication of transplant is graft versus host disease (GVHD). GVHD happens when the donor cells recognise your body as being different to the body they came from. This can result in the donor immune system reacting against you. Symptoms of this include rash, diarrhoea or abnormal liver tests. GVHD can occur early after transplant (called acute GVHD) or several months after (called chronic GVHD). GVHD can last for a short period of time or may be a long-term effect of the transplant, and in a few patients, it can affect the way they live and their quality of life. You will have a detailed discussion with the transplant and sickle doctors about all of the possible side effects before you have a transplant – this is so that you can decide whether transplant is the right treatment option for you.

## What is a successful transplant?

We know that the transplant has been successful when:

- we see that your bone marrow is making the same blood as your donor
- you no longer experience sickle cell crises
- you do not experience any significant side effects from the transplant, such as GVHD.

All patients who have a transplant will need to take at least one type of medication, and will continue to be followed up by the transplant team, at least once a year, for the rest of their lives.

### Contact us

If you have any questions, please speak to a doctor or nurse caring for you. Some useful numbers are below.

Consultant haematologists, **t:** 020 7188 2741 (secretary)  
Specialist registrar – **bleep** 0248  
SHO – **bleep** 2283

Advanced nurse practitioner  
**t:** 020 7188 2710, **bleep:** 1843, **mobile:** 07770 683947

Clinical nurse specialists  
**t:** 020 7188 2710, **bleep:** 2256, **mobile:** 07770 678851/07920 711266

Psychologists:  
Consultant health psychologist – **t:** 020 7188 2725 or 020 7188 2718  
Haematology clinic – **t:** 020 7188 2743/2724  
Haematology day unit – **t:** 020 7188 2745/2710

For more information leaflets on conditions, procedures, treatments and services offered at our hospitals, please visit **w:** [www.guysandstthomas.nhs.uk/leaflets](http://www.guysandstthomas.nhs.uk/leaflets)

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# Having a haematopoietic stem cell (bone marrow) transplant for sickle cell disease

**This leaflet is for patients who are having a haematopoietic stem cell transplant. It explains what will happen just before, during and after the procedure on the day of your transplant, and offers advice on how to care for yourself once you are back at home. If you have any further questions or concerns, please speak to a nurse or doctor caring for you.**

## Before you come into hospital for your transplant

You may have an exchange blood transfusion before your admission to reduce the risk of any sickle cell related problems when you come into hospital. An exchange blood transfusion is where your own blood is removed from your body and replaced with a transfusion of plasma or donor blood. If you are taking hydroxycarbamide, we may increase the dose of this in the six months leading up to your transplant, to maximise your response to treatment and your blood levels. Hydroxycarbamide will be stopped in the week before your admission – your doctor will give you the exact instructions.

You will have a number of hospital appointments before your transplant. These are to:

- discuss the transplant process
- discuss the risks involved
- assess the function of vital organs, such as your kidneys and lungs.

The psychology team will carry out a cognitive (looking at your memory and concentration) assessment, and a brain scan will also be done in the month before your transplant.

One to two weeks before your transplant, you will be given an appointment in the Radiology Department. A long, thin tube (a Hickman<sup>®</sup> line) will be inserted into your chest under a local anaesthetic. The line is inserted so that you can receive all of your medicines and blood products through it, and so that your blood samples can be taken without the use of needles. You will be awake while the line is inserted, but you will not feel any pain.

In the weeks before your transplant, you will be invited to the hospital for a morning session, so that you can learn more about what usually happens on the wards. You will meet dietitians, physiotherapists, ward nurses and the pre-transplant nurses, who will talk to you about what will happen during your hospital stay. The session offers an opportunity for you to ask questions, visit the ward to see what the rooms are like, and meet other patients who are having similar types of stem cell transplant.

During this process you will be allocated a dedicated transplant nurse (in addition to your normal sickle nurse) who will coordinate all of your appointments and answer any questions you have about the transplant.

## While you are in hospital having the transplant

You will be admitted to King's College Hospital the day before you are due to start the chemotherapy treatment. On your first morning in hospital, you will discuss the transplant procedure again with nurses and doctors from the transplant coordination team. They will confirm that you understand the procedure, answer any questions, and confirm that you are happy to go ahead before the start of the chemotherapy treatment.

You will be given a copy of your treatment plan so that you know what to expect each day. For the first five days you will be given chemotherapy via the Hickman® line. On your sixth day, you will be transported to and from Guy's Hospital to receive a dose of radiotherapy. Once you are back at King's College Hospital, you will begin taking medication that will help to protect your transplant, and prevent you from developing GVHD.

The stem cells are given to you on your eighth day in hospital, although the day the stem cells are infused is usually called day zero, so that the days following your transplant can be measured from here. This is important so that the transplant team can plan any assessments you need after the transplant.

It is normal to experience the following side effects from the chemotherapy treatment:

- nausea (feeling sick)
- low blood pressure
- fever and/or vomiting (being sick)
- suppression of your immune system.

Any nausea and vomiting will be well managed with medication.

While your immune system is suppressed, you will be more likely to pick up infections. To reduce this risk, you will be given mouth washes and preventive antibiotics every day, and you will be closely monitored for signs of infection.

The ward transplant team will review you every day, and the sickle team will review you at least twice a week (or more often if needed). The psychology team will see you at least once a week, but can come more often if necessary.

After you have received the stem cells, we will keep a close eye on:

- any signs of infection, such as a fever
- your temperature, blood pressure and oxygen levels (at least six times every day)
- your blood test results daily, as you may require transfusions of red blood cells or platelets during your time in hospital
- the levels of the drug you will be given to protect your new stem cells.

The length of the hospital stay after the transplant is different for each person, but you should prepare to be in hospital for at least four weeks. During your time in hospital, you will be mostly kept in isolation (in a room on your own) in a side room. You will be able to have visitors during normal visiting hours, but you are restricted to two people at a time to reduce the risk of infection. We advise that children younger than 12 only visit under very special circumstances.

Your diet during the period when your immune system is suppressed is very important. You must not eat:

- any food that is brought from home and requires reheating
- any uncooked food
- foods with live bacteria in or on them, such as cheeses, yoghurts, salads.

A full list will be given to you by your dietitian before your transplant.

## Once you are at home

You can only go home when your new stem cells are making enough white cells to protect you from infection. It will be many weeks before your new immune system is able to cope as well with infections as you do presently.

Coughs and colds are common, but where possible, try to avoid direct contact with anyone who is displaying symptoms of an illness until their symptoms have fully disappeared.

### If you feel unwell

If you become unwell with a temperature or diarrhoea and/or vomiting, please contact the post-transplant team:

- Post-bone marrow transplant nurse, **t:** 020 3299 4385
- Post-bone marrow transplant doctor, **t:** 020 3299 4694

You will probably be advised to come to the day unit/supportive therapy area for an assessment. If you are very unwell, you may be admitted to hospital.

Out of hours, please call the King's College Hospital switchboard, **t:** 020 3299 9000, and ask for the haematology registrar on call.

If you become suddenly unwell, for example, if you collapse or suffer a significant injury, call an ambulance and make sure they know that you have had a bone marrow transplant.

If you are exposed to any childhood infections, such as chicken pox, mumps or measles, or are concerned about any other infections, inform your transplant doctors at King's College Hospital so that appropriate action can be taken.

## Coming in for hospital appointments

Following your transplant, you will be seen in the transplant clinic at King's College Hospital at least once a week. This is so that we can check your blood count and drug levels, monitor you for infections, and look for any signs of GVHD. Your sickle doctor will attend your transplant clinic review at least once a month.

Four weeks after receiving the stem cells, you will have a bone marrow test to assess how well the cells have embedded into your bone marrow. If the cells have embedded well, your appointments will become less frequent, and you will be seen every other week in the outpatient clinic. Three months after your transplant, the frequency of your appointments will reduce again, to monthly visits at first.

Six months after your transplant, we will carry out a number of investigations, including:

- a lung function check
- an echo (ultrasound scan of your heart)
- a brain scan.

These results are compared to your test results before the transplant, so that we can monitor your improvement.

One year after your transplant, you will return to being seen in your normal sickle clinic. The frequency of your reviews will be agreed between you and your sickle team, but you will be reviewed at least twice a year to begin with. Even after you return to the sickle clinic, the transplant team would like to see you for long-term follow-up in the late effects clinic. The timing of these appointments will be agreed between your sickle and transplant doctors.

About one year after transplant, your doctors will start to gradually wean you off your protective drug. You will need to continue on this medication, however, if you have any signs of GVHD. At your first end-of-year review, you will be given an immunisation schedule to complete via your GP practice. It is important that you are vaccinated as outlined in the schedule and that you do not receive any live vaccines (such as yellow fever) until two years after transplant. This will be especially important if you start travelling abroad.

## Returning to work or school

Going back to work or your studies following a bone marrow transplant can be both an exciting and anxious period. Most people will have a phased return, starting off on a part-time basis and easing back into full-time work or studies. You may find that you become more tired as you adjust to the routine of work/school, and it may take some time for you to adapt. Everybody is different, and some people find it easier than others. Your sickle team can help you to discuss your needs with your employer or tutors. Continued support will be available from the sickle psychology team, based at Guy's Hospital.

### Contact us

If you have any questions, please speak to a doctor or nurse caring for you. Some useful numbers are below.

Consultant haematologists, **t:** 020 7188 2741 (secretary)  
Specialist registrar – **bleep** 0248  
SHO – **bleep** 2283

Advanced nurse practitioner  
**t:** 020 7188 2710, **bleep:** 1843, **mobile:** 07770 683947

Clinical nurse specialists  
**t:** 020 7188 2710, **bleep:** 2256, **mobile:** 07770 678851/07920 711266

Psychologists:

Consultant health psychologist – **t:** 020 7188 2725 or 020 7188 2718

Haematology clinic – **t:** 020 7188 2743/2724

Haematology day unit – **t:** 020 7188 2745/2710

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## Pharmacy Medicines Helpline

If you have any questions or concerns about your medicines, please speak to the staff caring for you or call our helpline.

**t:** 020 7188 8748, Monday to Friday, 9am-5pm

## Your comments and concerns

For advice, support or to raise a concern, contact our Patient Advice and Liaison Service (PALS). To make a complaint, contact the complaints department.

**t:** 020 7188 8801 (PALS)      **e:** [pals@gstt.nhs.uk](mailto:pals@gstt.nhs.uk)

**t:** 020 7188 3514 (complaints)      **e:** [complaints2@gstt.nhs.uk](mailto:complaints2@gstt.nhs.uk)

## Language and accessible support services

If you need an interpreter or information about your care in a different language or format, please get in touch.

**t:** 020 7188 8815      **e:** [languagesupport@gstt.nhs.uk](mailto:languagesupport@gstt.nhs.uk)

## NHS 111

Offers medical help and advice from fully trained advisers supported by experienced nurses and paramedics. Available over the phone 24 hours a day.

**t:** 111      **w:** [111.nhs.uk](http://111.nhs.uk)

## NHS website

Online information and guidance on all aspects of health and healthcare, to help you take control of your health and wellbeing.

**w:** [www.nhs.uk](http://www.nhs.uk)

## Get involved and have your say: become a member of the Trust

Members of Guy's and St Thomas' NHS Foundation Trust contribute to the organisation on a voluntary basis. We count on them for feedback, local knowledge and support. Membership is free and it is up to you how much you get involved. To find out more, please get in touch.

**t:** 0800 731 0319      **e:** [members@gstt.nhs.uk](mailto:members@gstt.nhs.uk)      **w:** [www.guysandstthomas.nhs.uk/membership](http://www.guysandstthomas.nhs.uk/membership)

### Was this leaflet useful?

We want to make sure the information you receive is helpful to you. If you have any comments about this leaflet, we would be happy to hear from you, fill in our simple online form, **w:** [www.guysandstthomas.nhs.uk/leaflets](http://www.guysandstthomas.nhs.uk/leaflets), or **e:** [patientinformationteam@gstt.nhs.uk](mailto:patientinformationteam@gstt.nhs.uk)

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# Haematopoietic stem cell (bone marrow) transplant for sickle cell disease: information for donors

**This leaflet offers information for potential stem cell donors. It explains what is involved in testing to see whether you are a match, as well as what happens if you choose to donate stem cells to your sibling. If you have any further questions or concerns, please speak to a nurse or doctor caring for you.**

## Why are donors needed?

Your brother or sister has sickle cell disease – a blood condition that can be cured by a bone marrow transplant. The stem cells that create all the cells in your sibling's blood have the sickle mutation, which causes the red cells in particular to be destroyed by the body. This results in a reduction in the total number of red cells, and leads to anaemia and painful crises. If a sibling with matching blood proteins agrees to be a donor, we can replace your sibling's stem cells with ones that do not make sickle blood, and their condition can be cured.

## Before being tested

Blood testing is the first step towards finding out whether you are a match for your sibling. It is important that any questions you have about what being a donor involves are answered before you have the blood test. If you are able to, you should attend a clinic appointment with (or without) your sibling before committing to the test. You should also contact the sickle team to discuss any questions or concerns that you may have (contact details at the end of this leaflet).

It is important that you only proceed to be tested if you are doing so freely, without feeling pressured into it. If you have any concerns about being pressured into donating stem cells, please speak to the sickle team.

## What does testing involve for you?

Your sibling will ask if you are happy and willing to have your blood tested to see if you can be a stem cell donor for them. Once you have had all your questions answered and agreed to take the test, you will either receive a pack in the post which you can take to your GP to have the blood tests taken and posted back to us, or you can choose to come up to our clinic at the hospital, where we can take the blood sample. About 15mls (three tablespoons) of blood is taken for the test.

The results usually take 2-3 weeks to be processed and will be sent out directly to you in the post. We will not share your results with anyone unless you (or your parents, if you are under 16 years of age) expressly give us permission to do so. We usually ask that you tell your sibling the results of your blood test, but if you would prefer that we do it on your behalf, we are happy to do so.

## The results and how you proceed

### **If you are not a match:**

There is only a one in four (25%) chance that you will be a match for your sibling, so most siblings are not matches. If you are not a match, it is not possible for you to donate stem cells to your sibling.

### **If you are a match but either do not wish to, or are unsure if you wish to be a donor:**

It is your right to proceed as you wish with your test results. If you do not want to be a donor and would like us to support you to tell to your sibling about this, please contact the sickle and psychology teams (numbers at the end of this leaflet). If you have any questions or would like more detail on what donation involves, before sharing your results with your sibling, please contact the sickle cell team.

### **If you are a match and happy to be considered as a donor:**

If you are a match and would like to go ahead with the donation, please inform your sibling. You will then receive an appointment for a consultation with the sickle doctor and the transplant doctors. You will also be seen by the psychology team – this may be at the same consultation, but could be at a separate appointment.

Nothing much happens until:

- your sibling is fully committed to having a transplant
- you are fully committed to being their donor.

You will then have formal meetings with the transplant coordination team at King's College Hospital, and a detailed explanation of the process of being a donor will be given. You will also require a health check to assess your fitness to move forward with the donation.

### **If both you and another sibling are a match**

If more than one sibling is found to be a match for the sibling with sickle cell disease, then other factors, such as blood group and age, may be used to decide which donor is most appropriate for stem cell transplant.

## What exactly happens when I donate the stem cells?

Normally, there is no operation involved in the stem cell collection procedure. You will be given a stem cell growth hormone injection once a day for four days before the stem cell collection, which is carried out on the fifth day. The process of collecting the stem cells from your bloodstream is known as peripheral blood stem cell harvesting (PBSCH) and is done using a cell separator (apheresis) machine. You will have a needle inserted into a vein in each arm. Blood will be taken from you, passed through the apheresis machine and returned to you. The whole process takes 3-4 hours and a plaster is applied where the needles have been in each arm once it is completed. You are able to go home and return to normal activities on the same day. We will be happy to discuss the process and any questions you have at any point and you will also be given more information about this process closer to the time of the transplant. Most collections are done over one day, but very occasionally you may require a fifth injection and need to return on the sixth day for a further collection.

## What happens after donation?

Once the stem cells have been collected, your part in the transplant process is usually complete. Very rarely the stem cells may fail to grow in your sibling's bone marrow, and in this case you may be called back for a second donation.

If you wish to see the psychology team during your sibling's time in hospital, please contact them on the numbers at the end of this leaflet. The sickle team will also be happy to discuss any questions you may have.

### Contact us

If you have any questions, please speak to a doctor or nurse caring for you. Some useful numbers are below.

Consultant haematologists, **t:** 020 7188 2741 (secretary)  
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Psychologists:  
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