NHS
The Leeds Teaching Hospitals
NHS Trust

Thalassaemia in children & young people

Information for patients & families

leeds children's hospital
caring about children

You have been referred to the Children's Haematology & Oncology Day Unit in the Children's Hospital at Leeds General Infirmary because you or your child has Thalassaemia. This booklet aims to give you some useful information about the unit and also some basic information about Thalassaemia.

What is Thalassaemia?

Thalassaemia is a broad term, which describes a group of disorders, which affect how well your body makes red blood cells. Red blood cells carry oxygen around the body. Oxygen gives us energy and enables all the other cells in the body to work properly. Children who are born with Thalassaemia cannot make enough healthy red blood cells, so without medical treatment most would get weaker and weaker and would fail to develop and grow normally. Children who have Thalassaemia will need medical treatment throughout their lives but the intensity of this treatment varies between each sub group and even between individuals with the same diagnosis.

Thalassaemia Major

This group of patient will need regular blood transfusions usually starting within the first year of life. Blood transfusions increase the amount of iron in the body so medication called iron chelation is needed to keep levels to a safe level.

Non Transfusion dependant Thalassaemia

Sometimes a child who has inherited Thalassaemia from both parents will have a form of Thalassaemia called non transfusion dependant Thalassaemia. These patients do not require regular blood transfusions, although many will eventually require them later in child hood or at some point in their life.

How will my child's life be affected by Thalassaemia?

It is very important to remember that, although Thalassaemia is a serious, lifelong medical disorder, with modern medical treatment it is manageable. Children who are born with Thalassaemia in the UK today should expect to reach their full educational potential, have careers, relationships and healthy children of their own. They should expect to live a normal or near normal lifespan. All these things are possible, provided that the treatment plan is followed closely.

(Please see the information racks located in the day unit for more in depth information about Thalassaemia, or speak to one of our doctors or Nurses if you have any specific questions. Ask for a copy of the "My Thal" DVD which through interviews with people with Thalassaemia, of varying ages and backgrounds, shows you can have Thalassaemia and live long, happy and fulfilled lives)

Information about the clinic

Clinic times

General Children's Haematology Clinic

Thursday Afternoon: 2:30-4pm

In nearly all cases your child will need to have bloods taken, the results will then be available when you see the doctor. For this reason **please be aware, the appointment time is for blood taking not the time you are due to see the doctor.** There is normally a 30 minute wait after having blood taken, before you are taken through to see the doctor.

Thalassaemia Annual Review Clinic

Tuesday afternoon: 1st appointment 1:30pm - last appointment 4:30pm

All children and young people will be offered an annual review appointment. During these sessions you will see the Consultant Doctor and also the Nurse.

These sessions aim to give you a chance to ask questions and to help with your understanding of Thalassaemia. As your child gets older we will start to look at developing their understanding and look towards them gaining independence and moving to adult services when they leave full time education.

Who will I see at Clinic?

Receptionist

Please report to reception on arrival. You will be given an appointment slip by the nurse or doctor to book your next appointment, please hand this into reception before leaving. You can also contact reception on **0113 392 7179** to make or rearrange an existing appointment.

Specialist Nurse

A nurse is always available to speak to if you have any questions or problems you want to discuss. You can also contact the Nurses on **0113 392 7379** or by email **benjamin.sykes@nhs.net**

Doctors

You will be seen by one of the Haematology Consultants (senior doctors specialising in blood disorders). **Or** one of the Haematology Specialist registrars (Experienced doctors specialising in disorders of the blood).

Thalassaemia Counsellors

Counsellors provide confidential care for children and adults affected by Thalassaemia.

Social Worker

Each child/young adult diagnosed with Thalassaemia is allocated a social worker to help access social services, benefits and immigration advice.

Nurses / Clinical support workers

You may be seen by a clinical support worker to check blood pressure, pulse and oxygen saturations. You will always be seen by a registered children's nurse before each blood transfusion.

Play Specialists

Play specialists use play as a distraction to help your child come to terms with medical procedures and their diagnosis.

Phlebotomists

Phlebotomists will take a blood sample prior to seeing the Doctor.

For psychology, interpreter or spiritual support

Please contact the Clinical Nurse Specialist.

How often do I need to come to clinic?

Babies who have Thalassaemia should be checked every month by their Thalassaemia doctor so the doctor can decide if and when to start blood transfusions.

When the decision has been made to start transfusions they are performed every 3-5 weeks depending on the need of the individual.

In addition to attending appointments on the day unit for blood transfusion, you will need to see the Doctor at least every three months. We will try to coordinate this visit with your transfusion appointment. These appointments are extremely important as they are essential for monitoring your child's health and identifying any potential problems early. We will also arrange an annual review appointment once a year.

Please note that we will always arrange to see your child if they are poorly, in pain, or you are concerned for whatever reason.

Please ring **0113 392 7379** between 8.30am - 6.00pm to speak to a nurse on the day unit **or** If it is a bank holiday or outside day unit hours ring **0113 392 7431** to speak to a nurse on ward L31.

You DO NOT need to go through Accident and Emergency (A&E)

What should I do if I can't come to clinic?

It is very important that you let us know if you cannot come to clinic. If you let us know that you cannot attend we can book someone else into your appointment time.

If you are unable to attend please call **0113 392 7179** between 9am-4:30pm

Treatment for Thalassaemia

Blood Transfusions

Our blood looks red because it is full of red blood cells. These cells are very important because they carry the oxygen from the air we breathe around our bodies. Oxygen gives us energy and enables all the other cells in the body to work properly. If our red blood cells are not working we get weaker and weaker and eventually will not be able to survive.

People who have Thalassaemia cannot make red blood cells which work properly, so usually need to be given blood transfusions at regular intervals. Transfused blood lasts in the body for about 28 days, so transfusions are usually given

every three or four weeks, although this may vary. In the week before a transfusion, the person with Thalassaemia can tire easily and may need more rest. After the transfusion the patient will usually feel much more energetic.

Doctors and nurses carefully check all Thalassaemia patients to make sure that they get transfusions often enough so that they have enough energy to live a normal life. As well as being important for giving energy, transfusions are essential in order for the child to grow and develop normally. Just as children need enough food and nourishment in order to grow, they also need enough oxygen in their bodies. If a child does not have blood often enough their blood levels drop and they become what we call anaemic. If a child is constantly "anaemic" their body will not have enough oxygen to develop correctly.

We make sure children are very carefully monitored and that they are transfused often enough to grow and develop normally. Transfusions can be started from a very young age. They are usually started at some time in the first year of life (this may vary). Babies who have Thalassaemia should be checked every month by their Thalassaemia doctor so the doctor can decide if and when to start transfusions.

What happens during transfusions?

In order to give the blood, a nurse or doctor will place a special needle called a cannula into a vein. Obviously this can be uncomfortable for the child but we can use a cream to numb the area. Our play specialists are trained how to calm and distract a child who is undergoing a medical procedure and can be of great support during your child's transfusions. Blood is then given through a drip into the cannula. A transfusion usually lasts between three and four hours.

When are blood transfusions given?

Blood transfusions are normally performed between the hours of 9am and 6pm Monday to Friday. We try, where possible to coordinate appointments to fit in with your needs, however during particularly busy times this may not be possible. In certain situations, for example during school exams, it may be possible to arrange a transfusion at the weekend on one of the inpatient wards or to bring a transfusion forward to an earlier date. Please ask a nurse if you think you are going to have difficulty attending an appointment.

Please see contact information at the end of this booklet.

Iron Chelation

Chelation treatment is the second essential step in treating Thalassaemia. When a person has a blood transfusion, along with the important red blood cells they also receive a lot of iron. We all need iron, but people who have regular blood transfusions have too much iron in their bodies because they cannot get rid of the extra iron which comes with the blood. If the extra iron is left in the system, it will settle in important organs of the body such as the heart and the liver where it causes damage. It can also affect other things such as growth and development. Almost all the complications which cause problems and illness for people with Thalassaemia are either caused or made worse by iron overload. It is just as important for children as adults because some of the damage done by iron overload can occur in childhood and is not reversible.

To stop damage to the organs, patients with Thalassaemia need special medicines to get the iron out of their systems.

This medication is called "chelation treatment". Chelation treatment is just as important as blood transfusions. It is the key to remaining as healthy as possible both in childhood and in future life. By ensuring that your child takes their chelation treatment properly, you are laying the foundation for his or her future. Chelation treatment is usually started once a child has received about 10 transfusions. Each child is carefully monitored so the doctor can be sure it is the right time to start chelation. There are different types of chelation treatment. Your doctor and nurse will discuss with you which chelation treatment is most suitable and you will receive a full explanation of which treatment has been chosen and why.

All medicines work better for some people than others and have side effects which some, but not all, may experience. You may therefore meet other people whose child's treatment is different from yours. If you have any questions about chelation treatment, do not hesitate to discuss them with your Thalassaemia doctor or nurse. As research progresses it is likely that more medicines which can be taken by mouth will become available and that doctors will use different combinations of medicines so that every patient has the treatment which suits him or her best.

Desferrioxamine (also known as Desferal®)

The treatment which has been around the longest is a medicine called Desferrioxamine (also known as Desferal®). This is very safe and very effective at removing iron from the body. However, because Desferrioxamine does not work if taken by mouth, it has to be given by a needle under the skin. It works best if given very slowly, so the needle is attached to a small pump which gradually pushes the Desferal® under the skin over about 8 hours. This must be done between 3-7

times per week. Some people find it most convenient to wear their pump while they are in bed at night; while others prefer to attach it during the daytime while they are working or going about their lives. A community nurse will teach you how to attach the pump and make sure it is working properly. A special cream can be used which numbs the area before putting the needle under the skin. As children get older, they are encouraged to learn how to attach the pump themselves.

Deferasirox (Exjade®) and Deferiprone (Ferriprox®)

Children over 6 years of age may be suitable for chelation tablets which can be taken by mouth. At the moment the medicines available which can be taken by mouth are called deferasirox (Exjade®) and deferiprone (Ferriprox®).

Please ask for information booklets regarding these medications

Stem Cell Transplant (also referred to as bone marrow transplant)

Stem cell transplants are the only cure for thalassaemia, but unfortunately they are not something that is suitable for everyone due to the significant risks involved.

What are stem cells?

Stem cells are produced in bone marrow (the spongy tissue found in the centre of some bones) and have the ability to develop into different types of blood cells.

For a stem cell transplant, stem cells from a healthy donor are given through a drip into a vein. These cells then start to produce healthy red blood cells to replace the cells affected by thalassaemia.

Will my child be able to have a stem cell transplant?

A stem cell transplant is an intensive treatment that carries a number of risks. Transplants carry a lesser risk if they are done using a brother or sister as the donor. If this is something that you want to consider we will take blood samples from each sibling to see if they are a "match" and can be used as donor. There is a 1 in 4 chance that a brother or sister will be a full match. Less frequently in families where parents are 1st cousins a mother or family could also be a full match to their child. If you want to know more about transfusion, please speak to the Doctor or Specialist Nurse at your next appointment.

How long does the treatment take?

A stem cell transplant requires an initial period of hospitalisation, in isolation, of around four to six weeks. This is followed by a period of immunosuppression requiring frequent specialised monitoring for approximately six months during which the patient cannot attend nursery or school, or use public transport and may face frequent readmissions to hospital to manage complications. This has significant implications for family life, including practical aspect of family arrangements, financial considerations, parents' employment, and effects on siblings.

Future pregnancies

If you're pregnant you should talk to your Doctor and midwife as soon as possible. You will then have the option of being fast-tracked for a diagnostic test to find out if your baby's affected by Thalassaemia.

Couples who have a child with thalassaemia may wish to discuss options for a future pregnancy that enables couples to avoid passing that condition on to their children. Options

include testing at 11/ 12 weeks of the pregnancy, collection of umbilical cord stem cells at delivery to assess if the cells could be a potential stem cell transplant match or Pre-implantation Genetic Diagnosis (PGD).

PGD helps potential parents prevent the birth of a child with a serious genetic condition. It involves the use of assisted reproductive technology (ART). Eggs are obtained from the mother and fertilised with sperm through in vitro fertilisation (IVF). Once fertilised, the embryos develop for 5-6 days and then a number of cells are removed from each embryo. The genetic material (DNA or chromosomes) within these cells of the embryo is then tested for the genetic or chromosomal abnormality. An unaffected embryo is then transferred to the mother's womb to implant. If successful, the procedure will result in pregnancy and the child should not be affected by the condition for which it was tested.

Admission to hospital

Sometimes it may be necessary to treat your child in hospital. Our in-patient unit consists of 3 wards that provide treatment and care for children and young people, who are being investigated or treated for cancer as well as Thalassaemia and other blood disorders.

Ward L31 - For children between 0-12 years old.

Ward L32 - Bone Marrow Transplant Unit

Ward L33 - Teenage unit

At times the above wards may be full, this will mean that your child may need to be cared for on one of the other wards within our unit, and sometimes on one of the other Children's

wards. At these times you will still be under our care and will be seen by one of the unit doctors.

Visitors are encouraged at any time up until 8pm

Who can stay?

Due to limited space only one person can stay with your child at night time. We do encourage parents or family members to stay if possible. We do understand that sometimes this is not possible. Please let staff know if no one will be staying with your child. It is not possible for anyone under the age of 16 years to stay with your child.

What do I need to bring to hospital?

If you think there is a possibility your child might be admitted to Hospital it is a good idea to bring:

- All your child's medication
- Change of clothes
- Towel
- Toothbrush/Toothpaste
- Soap / shower gel

Feedback

We appreciate your opinion, please let us know if there is something we can do to improve our service or fill in a comment slip and place in one of the suggestion boxes on the day unit.

Patient advice and Liaison Service (PALS)

The aim of PALS is to resolve any problem or concern quickly.

Tel: (0113) 206 6261 **- Available during normal working hours only.**

Tel: (0113) 206 7168 **- For queries outside of normal working hours, please leave a voicemail**

Fax: (0113) 206 6146

Email: patientexperience.leedsth@nhs.net

We do value concerns and complaints as a means of improving the quality of our service and staff within the Patient Experience Department will be happy to guide you through the process, or answer any questions you may have.

As a point of information, if you are solely seeking compensation we are unable to deal with such requests under the NHS complaints procedure.

Useful contacts

Children's Haematology and Oncology Day Unit

Level C, Clarendon wing, Leeds Children's Hospital at Leeds General Infirmary

Reception: 0113 392 7179 (To re-arrange, cancel or check appointments)

Nurses: 0113 392 7379 (For medical advice)

Ward L31

Level A, Clarendon Wing, LGI
Tel: 0113 392 7431

Ward L32

Level A, Clarendon Wing, LGI
Tel: 0113 392 7432

Ward L33

Level A, Clarendon Wing, LGI
Tel: 0113 392 7433

Clinical Nurse Specialist for Sickle cell and Thalassaemia

Ben Sykes
Tel: 0113 392 7179 or 0778 726 6096
Email: benjamin.sykes@nhs.net

Suzie Preston
Tel: 0777 522 8860
Email: suzanne.preston1@nhs.net

Social Worker
Tel: 0113 392 2504

NHS Choices (benefits and allowances)
www.nhs.uk/Planners/Yourhealth/Pages/Benefits.aspx

The UK National Screening Committee
www.screening.nhs.uk

UK Thalassaemia Society
www.ukts.org

Thalassaemia Patients and Friends
www.thalassemiapatientsandfriends.com

Thalassaemia International Federation
www.thalassaemia.org.cy

What did you think of your care?

Scan the QR code or visit **bit.ly/nhsleedsfft**

Your views matter

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Produced by: Medical Illustration Services • MID code: 20220304_007/BP

LN004303
Publication date 03/2022
Review date 03/2024