UNDERSTANDING SICKLE CELL DISEASE AND OFFERING YOUR CHILD THE RIGHT CARE

COMPANION GUIDE

FOR FAMILIES

Votre gouvernement

Québec

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AUTHORS

Dr. Sharon Abish
Julie Brouillard, MSN
Marie Gale, BSN
John Kayser, BSN
Debbie Pealow, MSW
Elissa Remmer, MSN
Josefina Revuelta, BSN
Christina Rosmus, MSN
Anelise Santo, MSN

UPDATED GUIDE

Dr. Sharon Abish
Dr. Janie Charlebois
Dr. Valérie Larouche
Dr. Yves Pastore
Dr. Nancy Robitaille
Dr. Anne-Marie Vincent
Vanessa Di Schiavi, PNP, MSc (A)
Nathalie Fournier, BSN
Virginie Lamy, pediatric nurse practitioner
Pamela Bou Malhab, MSSS

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ÉDITION

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The masculine gender is used without discrimination in order to simplify the text.

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FOREWORD

Dear parents,

Learning that your child has sickle cell disease is a distressing experience. First of all, you should know that most children will not develop all the complications described in this Guide. With proper care, most children will enjoy a good quality of life: they will go to school, be able to take part in extracurricular activities, reach their full potential and lead successful lives.

Other parents are faced with the same situation as you, and a care team is there to support you. Even so, you likely have many questions in mind:

"How will this disease change my child's life today and in the future?"

"What are the symptoms of this disease, what do they mean and what should I do when they appear?"

"Will my child be able to lead a normal life?"

These are some of the questions prompting many parents to want to know more about sickle cell disease and how to offer their child the best care possible.

Based on the experience of care teams in the field and scientific evidence, and in light of parents' reports of their personal experiences, this Guide is designed to be useful, practical and easy to read.

It contains valuable information on sickle cell disease and answers to many of your questions. We hope that it will be of great help to you.

The hematology care team of the Québec centres for sickle cell disease.

HOW TO USE THIS GUIDE

Many parents feel overwhelmed by the sheer amount of information they receive about sickle cell disease. They are often afraid of forgetting important aspects about their child's health.

This is perfectly normal. To help you read and understand all this information, this Guide is divided into sections drawing together information sheets (infosheets) on the topic in question.

This Guide is available for information purposes only. It must not be used as substitute for the advice you will receive from your health professional. Since each patient is unique, treatment options will differ from patient to patient. Talk to a member of your care team if you have any doubts or concerns.

SECTION 1

Family Services

Hematology team

The hematology team is composed of several health professionals who will work with you to make it easier for you and your child to adapt to your new life situation.

Québec has five centres specializing in diagnosing and treating sickle cell disease and other serious hemoglobin disorders in children:

- Centre hospitalier universitaire de Québec – Université Laval
- Centre hospitalier universitaire de Sherbrooke – Hôpital Fleurimont
- McGill University Health Centre – Montreal Children's Hospital
- Centre hospitalier universitaire Sainte-Justine
- Hôpital Maisonneuve-Rosemont

The services of the following specialists may not all be offered in all these centres.

Hematology

When you visit the hospital, you will meet many doctors. They are there to help you and your child and to answer your questions. A hematologist is a doctor specializing in blood disorders such as sickle cell disease. In addition to your regular hematology appointments, you are strongly encouraged to see your child's pediatrician or family doctor for medical care that does not require a hematologist's expertise.

Licensed practical nurse

A licensed practical nurse will meet with your child at each visit to the clinic. This nurse will take your child's vital signs, measure and weigh your child and may give vaccines, or help supervise your child's treatments. They work in close collaboration with the nurse clinician and will notify them or the doctor of any change in your child's health.

Specialized nurse practitioner

A specialized nurse practitioner monitors your child throughout their care, both at the clinic and during hospitalizations. These nurses perform a range of activities, such as helping complete questionnaires, performing physical examinations, prescribing medications, and keeping track of laboratory test and imaging results. They will also participate in adjusting your child's treatment plan in collaboration with the care team. Holding a Master of Science in Nursing (MSN), they perform medical and nursing activities.

Nurse clinician

The nurse clinician coordinates a patient's overall care (blood tests, vaccines and medications, transfusions). You can also talk to the nurse clinician when you have questions about your child's health. They will meet with you at the time of diagnosis and will offer you very useful information. Working together with the other members of the care team, they will help you better understand your child's disease and better manage some of the symptoms. Lastly, if your child needs to see a doctor, the nurse clinician will facilitate your visit to the clinic or to emergency.

Social worker

The social worker helps meet your needs related to issues such as transportation, financial questions, psychological support, academic problems or crisis intervention. They evaluate each situation and help you find the right resources.

Office staff

Medical secretaries will take your calls when you need to reach a member of the care team. They can help you by responding to your concerns and referring you to the right health professional. They will also coordinate your appointments at the clinic. Notify them as soon as possible if you change your phone number or address.

Pain clinics

Pain management can be challenging for children with sickle cell disease. Acute and chronic pain management teams can help you ensure the best possible treatment to relieve your child's pain.

Genetic counsellor

You could make use of genetic counselling services if you intend to have more children. Genetic counsellors can help you evaluate your probability of having other children with sickle cell disease. They can also offer you advice on family planning and prenatal diagnostic tests. You are encouraged to talk about this to your doctor or nurse.

Child life specialists

Child life specialists help with the development of infants, children and teenagers who are hospitalized by offering play or education programs that resemble normal life experiences. Their aim is to reduce as much as possible the psychological impact of hospitalization. These specialists offer children opportunities to play and to develop their self-confidence. They also encourage learning, self-expression, interaction with the other children, socialization and family participation.

Association d'anémie falciforme du Québec

The Association d'anémie falciforme du Québec offers many services to support families, such as family respite activities as well as emergency childcare services. Other services such as massage therapy, active listening and patient navigation are also offered. This Association is a valuable source of information and sharing for young people, their parents and adults with this disease. It offers discussion groups and literature on the disease, screening and carrier status. For more information, visit the Association's website www.anemie-falciforme.org/.

INFOSHEET 1: **CONTACT INFORMATION FOR MY HEMATOLOGY TEAM**

MCGILL UNIVERSITY HEALTH CENTRE – MONTREAL CHILDREN'S HOSPITAL

- Doctor: _____
- Medical secretaries: _____
- Specialized nurse practitioner: _____
- Nurse clinician: _____
- Licensed practical nurse: _____
- Administrative agent: _____
- Child life specialist: _____

How to reach us:

Monday to Friday, from 8 a.m. to 4 p.m.: 514 412-4400, ext. 22428.

Evenings, nights and weekends: 514 412-4400, ext. 53333, and ask for the hematologist on call.

CENTRE HOSPITALIER UNIVERSITAIRE DE SHERBROOKE – HÔPITAL FLEURIMONT

- Doctor: _____
- Nurse: _____
- Social worker: _____
- Administrative agent: _____

How to reach us:

Monday to Friday, from 8 a.m. to 4 p.m.: 819 346-1110, ext. 15129.

Evenings, nights and weekends: 819 346-1110, ext. 0, and ask to speak to the hematologist on call.

CENTRE HOSPITALIER UNIVERSITAIRE DE QUÉBEC – UNIVERSITÉ LAVAL

- Doctor: _____
- Nurse navigator: _____
- Administrative agent: _____
- Social worker: _____

How to reach us:

Monday to Friday, from 8 a.m. to 4 p.m.: 418 525-4444, ext. 40118 or ext. 40105.

To book an appointment with the hematology clinic: 418 525-4444, ext. 40107.

Evenings, nights and weekends: 418 525-4444, and ask to speak to the hematologist on call or go to emergency.

CENTRE HOSPITALIER UNIVERSITAIRE SAINTE-JUSTINE

- ○ Doctor: _____
- ○ Specialized nurse practitioner: _____
- ○ Nurse navigator: _____
- ○ Social worker: _____
- ○ Psychologist: _____

How to reach us:

Monday to Friday, from 8 a.m. to 4 p.m.: 514 345-4931, ext. 2712
(Please leave the nurses a voicemail message if the situation is not urgent).
Clinic phone number: 514 345-4830.
Evenings, nights, weekends and emergencies: 514 345-4931, ext. 2111 or ext. 4881.
You can also contact the hematologist on call at 514 345-4788 (hospital reception desk) or go to emergency.

HÔPITAL MAISONNEUVE-ROSEMONT

- ○ Doctor: _____
- ○ Nurse: _____
- ○ Medical secretaries: _____
- ○ Social worker: _____

How to reach us:

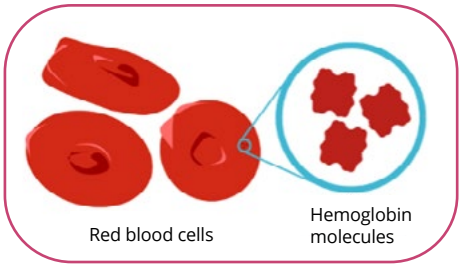
Monday to Friday, from 8 a.m. to 4 p.m.: 514 374-7940 (pediatric outpatient clinic)
Evenings, nights and weekends: **go to emergency**.

SECTION 2

Understanding Sickle Cell Disease

INFOSHEET 2: **UNDERSTANDING SICKLE CELL DISEASE**

What is sickle cell disease?



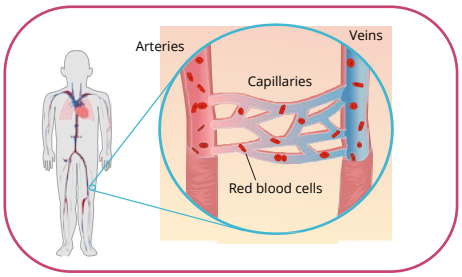
Sickle cell is a chronic disease. Also called "sickle cell anemia," sickle cell disease is a blood disorder affecting the hemoglobin (a protein that carries oxygen) in your child's red blood cells. Often diagnosed in early childhood, it is an inherited genetic disease, which means that your child is born with this disease. It is not contagious, so you cannot catch it like a cold. It is one of the most common genetic diseases.

Treatments and medications can relieve some symptoms and possibly prevent some complications. To better understand this disease, you must know how blood normally flows in the body.

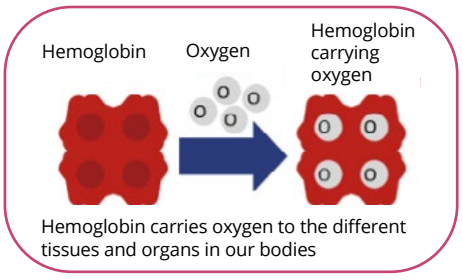
How does blood normally flow in the body?

Blood flows through blood vessels by way of a complex system of arteries, veins and capillaries.

- **Arteries** carry blood from the heart to the rest of the body (such as toward the brain, eyes, liver and muscles).
- **Veins** return blood from different parts of the body back towards the heart.
- **Capillaries** connect the arteries to the veins. A capillary is about 1000 times smaller than a vein or an artery.

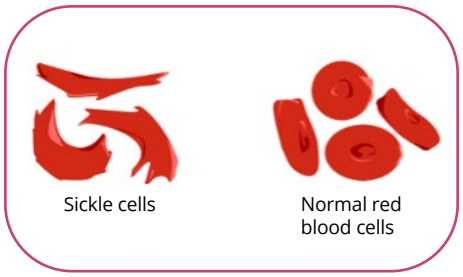


Blood is composed mainly of microscopic cells called "red blood cells." Normal red blood cells are disk-shaped (round) and flexible. Their shape and flexibility allow them to flow freely in the arteries and veins and to travel through the very narrow passages of the capillaries.



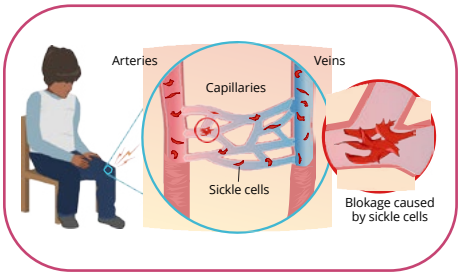
Each red blood cell is full of small hemoglobin molecules. One of the important roles of hemoglobin is to carry oxygen to the different parts of the body. This role is important because our body depends on oxygen to function properly. Oxygen is like gas for a car: if there isn't enough, the car will not run properly.

How does sickle cell disease affect blood flow?



Many of the red blood cells of children with sickle cell disease are not round. They are called "sickle cells" because they are instead shaped in the form of a crescent, like the letter C. These blood cells are stiffer and more fragile than normal red blood cells. As mentioned previously, normal red blood cells can travel through the very narrow passages of the capillaries because they are round and flexible. Sickle cells cannot pass as easily and can therefore get stuck in the capillaries.

Sickle cells are stiff and not very flexible. They can therefore cause blockages in blood vessels.



The blockage of blood vessels by sickle cells can cause a lack of blood and oxygen in some parts of the body, leading to different symptoms. This in fact is what causes most "sickle cell crises." Pain is the most common symptom in these crises. It can vary in intensity (from mild to severe), can be felt in different parts of the body, and can last from a few hours to a few days.

Many factors can increase the risk for sickle cell crises

A few of them include fever, dehydration, exposure to cold, sudden weather changes, strenuous physical exertion, and stress. However, most crises occur for no particular reason. They are unpredictable and can appear gradually or suddenly.

Why is the hemoglobin level of children with sickle cell disease generally low?

Sickle cells have shorter life spans than normal red blood cells. They are destroyed more quickly by the body because of their unusual shape and increased fragility. While normal red blood cells live 120 days on average, sickle cells live only around 20 days.

Normally, the body constantly makes new red blood cells. In the case of sickle cell disease, the body does not make red blood cells as quickly as it destroys them. As a result, children with sickle cell disease have fewer red blood cells in their blood than other children. Having fewer red blood cells also means less hemoglobin, and therefore less oxygen.

A low hemoglobin level can cause specific symptoms. You may notice that:

- your child is more tired than usual.
- your child's eyes have turned yellowish when they are not usually that colour.

If you notice either of these symptoms, call your care team. It may be necessary to check your child's hemoglobin.

To help you keep track of your child's hemoglobin level, we have included, at the end of this Guide, an Infosheet called "Healthcare appointments and blood tests." Bring this sheet to your hematology clinic appointments and write down the hemoglobin level that the nurse tells you.

Your child's hemoglobin level must be measured at each hematology clinic appointment through a blood test. A hemoglobin level that is lower than usual may require more frequent medical visits to ensure that your child receives good care. Sometimes, a blood transfusion may also be necessary.

What causes sickle cell disease?

Sickle cell disease is common around the world. In Québec, from 5 to 6 children in 10,000 births are estimated to have sickle cell disease at the time of neonatal screening. In 2022, around 750 children aged from 0 to 18 years were receiving care for this disease in specialized pediatric centres. Any one can have sickle cell disease. However, it is more common in children with ancestry from Africa, the Middle East, some parts of southern India, the Mediterranean, South America and the Caribbean.

Why does my child have sickle cell disease?

How were the sickle cell genes passed on to my child?

At the time of conception, each child receives two hemoglobin genes, one from the mother and one from the father. Genes are found in all the cells of the body, and genes determine everything about us, such as the colour of our eyes, our skin, our hair, and our type of hemoglobin.

Sickle hemoglobin is generally known as "HbS." A child with sickle cell disease has received a sickle hemoglobin gene (HbS) from one of their parents, along with an abnormal hemoglobin gene (such as hemoglobin C [HbC]) from the other parent.

What type of sickle cell disease does my child have?

The type of sickle cell disease that your child has is determined through a blood test. This test identifies the different types of hemoglobin and measures the percentage of continuous HbS in their red blood cells.

The three types of sickle cell disease are characterized by the presence of the hemoglobin S gene (HbS). Hemoglobin S is responsible for the stiffness and elongated crescent shape of the red blood cells.

Hemoglobin SS disease or HbSS

- This is the most common type.
- Children with this type of sickle cell disease received a hemoglobin S gene from each of their parents (HbS + HbS = HbSS).
- In these children's red blood cells, 100% of the hemoglobin is type S.

Hemoglobin SC disease or HbSC

- Children with this type of sickle cell disease received a hemoglobin S gene from one of their parents and a hemoglobin C gene from the other (HbS + HbC = HbSC).
- Hemoglobin C (HbC) is another type of abnormal hemoglobin.

Hemoglobin S–beta-thalassemia disease (hemoglobin SβThal or HbSβThal)

- Children with this type of sickle cell disease received a hemoglobin S gene from one of their parents and a beta-thalassemia gene from the other (HbS + HβThal =HbSβThal).
- Beta thalassemia is another form of abnormal hemoglobin.

The severity of the disease can vary from person to person. It is nevertheless recognized that people with the SC type generally tend to be less symptomatic than people with the SS type.

Check off your child's type of sickle cell disease

- ○ Hemoglobin SS disease (HbSS)
- ○ Hemoglobin SC Disease (HbSC)
- ○ Hemoglobin S beta thalassemia (HbSβThal)
  - ○ Hemoglobin SB0 (beta zero) thalassemia (HbS beta0)
  - ○ Hemoglobin SB+ (beta plus) thalassemia (HbS beta+)

Sickle cell carriers

A child receiving a hemoglobin S gene (HbS) from one of their parents and a normal hemoglobin gene (HbA) from the other parent will be a "carrier" of the sickle cell gene. They are also said to have "sickle cell trait." Carriers are not sick and do not have a greater risk of health problems than the other children. However, they can pass the sickle cell gene on to their children.

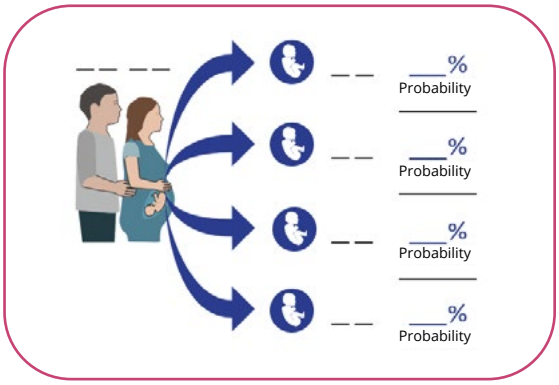
No link has been demonstrated between the fact of being a carrier of the hemoglobin S gene (HbS) and the possibility of having health problems. Some very rare cases of health problems have been reported among carriers placed in extreme conditions (deep sea diving, low oxygen atmosphere, high-altitude mountain climbing, strenuous exertion and severe dehydration).

If you know that you are a carrier of the gene for sickle hemoglobin, talk to your doctor to find out the probabilities of having children with sickle cell disease.

Are you planning to have another child?

If you are pregnant or planning to have more children, each of the designated centres in Québec can offer you genetic counselling services. Genetic counsellors are experts in inherited diseases. They will help you evaluate your probability of having other children with sickle cell disease. They can also offer family planning and prenatal screening options.

In case of a new pregnancy, collecting blood from your future baby's umbilical cord, after birth, can also be considered because in some cases it could help treat your child living with sickle cell disease. A stem cell transplant is in fact a treatment worth discussing for a person with sickle cell disease and can be performed using the umbilical cord blood from a brother or sister who is an HLA match (HLA type is similar to the blood group in the immune system). You are encouraged to discuss these options with your doctor or nurse. Tell your care team if you are pregnant or if you intend to have more children.



A member of the care team will help you complete this diagram so that you can better understand the probabilities of having a child with sickle cell disease. These probabilities are the same for each pregnancy.

INFOSHEET 3: **RECOGNIZING THE SYMPTOMS TO WATCH FOR**

Sickle cell disease is very unpredictable, it is difficult to identify the affected people who will develop more symptoms than others.

Children with sickle cell disease generally begin to experience symptoms in the first year of life, usually after six months of age. Symptoms and complications can differ from child to child and vary in intensity (from mild to severe). It is important to understand that there is no treatment that suits all children with sickle cell disease.

Treatment decisions will depend on your child's condition and symptoms.

When does my child need medical care?

Sickle cell is a disease that can have serious consequences if certain symptoms are left untreated. It is therefore very important to know how to recognize these symptoms in your child as soon as they appear so that your child receives the necessary treatment for them. You can contact a member of your care team at any time if you are concerned about your child's health. Infections and pain are the most commonly seen health problems in children with sickle cell disease. It is important to recognize the warning signs in order to take rapid action.

Symptoms to watch for include:

- fever;
- intense or prolonged pain;
- swollen belly (enlarged spleen);
- difficulty breathing;
- **painful** persistent erection (priapism);
- severe headache;
- stiff neck (difficulty bending or nodding the head);
- convulsions;
- paralysis, weakness or numbness in the arms or legs;
- vision problems;
- severe stomach ache;
- irregular gait (limping);
- fatigue or new paleness (which may indicate a drop in hemoglobin).

When these symptoms appear, you must quickly see a doctor. Call your care team, when possible, and take your child to the hematology clinic or to emergency. Remember to tell the emergency team that your child has sickle cell disease

Fever

Children have a fever when:

- their rectal temperature (in the anus) is 38.5 °C (101 °F) or higher;
- their oral temperature (in the mouth) is 38.0 °C (100.4 °F) or higher;
- their axillary temperature (in the armpit) is 38.0 °C (100.4 °F) or higher.

It is best to take their temperature rectally or orally. These methods provide more accurate readings in your child.

Any episode of fever in your child with sickle cell disease must be taken seriously. Go immediately to the hospital. Do not delay!

Pain

When your child's pain is not relieved with rest, good hydration, pain-relief medication such as acetaminophen (Tylenol™ or Tempra™), ibuprofen (Advil™ or Motrin™), or morphine (as prescribed by your doctor), or when the pain is in a high-risk location (headache, chest pain), go to the hospital for an assessment.

Difficulty breathing

The following symptoms may indicate that your child is having difficulty breathing:

- breathing rapidly;
- shortness of breath;
- laboured breathing (increased effort to breathe);
- persistent cough;
- chest pain.

Swollen stomach (enlarged spleen)

Your child's belly is swollen and hard to the touch. This may mean that blood cells are trapped in the spleen, making it swell. Your doctor will teach you how to feel for your child's spleen.

Painful erection

Your son has a persistent **painful** erection (pain in the erect penis). If this lasts more than an hour, go immediately to emergency.

Other symptoms

Below are other symptoms that may appear and that require you to see a doctor:

- severe headache not relieved by acetaminophen (Tylenol™ or Tempra™) or ibuprofen (Advil™ or Motrin™);
- stiff neck;
- paralysis, weakness or numbness in the arms or legs;
- **de novo** vision problem (that is, it has recently appeared);
- severe stomach ache.

SECTION 3

Knowing the possible complications

INFOSHEET 4: **FEVER**

Children with sickle cell disease have a greater risk of severe, fulminant (rapidly escalating) infections because their spleens were damaged by sickle cells at a very young age. If your child has a fever, they may have an infection. Fever is often the first sign of infection and **must always be taken seriously in children with sickle cell disease**.

The spleen is an organ responsible for filtering blood and expelling bacteria from it. The spleen of children with sickle cell disease cannot perform this function properly. That is why bacteria can remain in the blood and even cause an infection called "septicemia." This type of infection is extremely serious, fulminant (worsens very quickly), and can even be fatal. Effective ways of protecting your child against infections include proper vaccination and antibiotic prophylaxis with penicillin or another antibiotic taken daily as prescribed by a doctor. It is of critical importance to get medical care quickly if your child develops a fever.

Children who have a fever may show the following symptoms:

- they become hot to the touch (forehead and neck);
- they have red or rosy cheeks;
- they have shivers;
- they have a headache;
- they have body aches;
- they are irritable.

It is important to properly take your child's temperature so that you can inform your nurse or doctor. If you do not have a thermometer at home, you should get one.

Choosing a thermometer

Digital thermometers are recommended because they are easy to read. They give an accurate reading within 30 seconds to 2 minutes. Carefully read the instructions that come with your digital thermometer to find out how it works.

Glass thermometers containing mercury are not recommended.

Oral (mouth) temperature

It is recommended to take an oral temperature only if your child is old enough to follow instructions and able to cooperate (which usually occurs at the age of 5 or 6 years). Do not give your child a hot or cold beverage 15 to 30 minutes before taking their oral temperature and follow these instructions:

- Make sure that your child does not have any food, candies or chewing gum in their mouth;
- Ask your child to sit down and keep still while you take their temperature;
- Ask your child not to talk, not to bite down on the thermometer and to keep their lips closed while the thermometer is in their mouth;
- Keep the thermometer under your child's tongue until you hear a beep;
- Stay with your child while taking their temperature;
- Remove the thermometer and read the number appearing on it.

Your child has a fever if they have an oral (mouth) temperature of 38.0 °C (100.4 °F) or higher.

Rectal temperature (in the anus, for children younger than 5 years)

If you choose this method to check your child's temperature, make sure to follow these instructions:

- Place your child on your knees or on a firm surface such as a changing table;
- Apply a lubricant jelly or petroleum jelly, such as Vaseline, on the tip of the thermometer;
- Gently insert the thermometer about half an inch to an inch (1 to 2 cm) into your child's rectum ;
- Hold your child still;
- Keep the thermometer in place until it beeps;
- Remove the thermometer and read the number;
- Clean the thermometer after each use.

If you use several thermometers at home, clearly identify the rectal thermometer. This will prevent you from placing it in your child's mouth, and will also limit the risks of infection.

Your child has a fever if they have a rectal temperature of 38.5 °C (101 °F) or higher.

Armpit (axillary) temperature (not recommended because less accurate)

If you choose this method to check your child's temperature, make sure to follow these instructions:

- Remove your child's top to make it easier to get to their armpit;
- Lift your child's arm and place the tip of the thermometer deep in the centre of their armpit;
- Lower your child's arm and hug it tightly against their chest to keep the thermometer in place;
- Keep the thermometer in place until it beeps;
- Stay with your child during this time;
- Remove the thermometer and read the number;
- If your child is very young and not being cooperative, sit them on your lap and hold them while taking their temperature.

Your child has a fever if they have an axillary (armpit) temperature of 38.0 °C (100.4 °F) or higher.

Tympanic temperature (in the ear, not recommended because less accurate)

Ear thermometers are not very reliable for detecting fever. Readings are always lower than a child's actual temperature. If you choose this method to check your child's temperature and the reading is 37 °C or higher, you must retake their temperature using another method. In such case, rectal temperature is indicated for children under 5 years of age, who are too young for an oral thermometer. Temperature can be taken under the tongue in children able to cooperate (5-6 years and older).

Forehead temperature (not recommended because less accurate)

Taking your child's forehead temperature is not reliable enough, and that is why it is not recommended. A temperature of 37 °C could indicate that your child has a fever. However, you should always check their temperature using one of the methods described above (oral or rectal temperature).

Don't give acetaminophen (such as Tempra™ or Tylenol™) or ibuprofen (such as Advil™ or Motrin™) if your child has a low-grade fever (which means they do not yet have fever, their temperature is lower than those presented above). Giving this medication could mask a spike in fever.

Remember: Fever in a child with sickle cell disease should never be ignored! Call your care team, when possible, and take your child to the hematology clinic or to emergency. Do not delay!

INFOSHEET 5: PAIN

Pain is another common symptom in children with sickle cell disease. Pain crises occur when sickle cells clog blood vessels, therefore depriving some parts of the body of blood and oxygen.

Signs of pain in your child

As your child gets older, they will be able to better express, locate and possibly describe their pain. In younger children, you can recognize some signs of pain by observing behaviour such as:

- they cry inconsolably;
- they press down on the area that hurts;
- they refuse to use an arm or a leg;
- they refuse to walk;
- the painful area is swollen.

Possible areas of pain during a sickle cell crisis

Possible areas of pain are the following:

- back;
- sides;
- arms;
- stomach;
- hands;
- arms or legs.

In young children (usually under two years of age), a sickle cell crisis could cause swelling in the hands or feet, fingers or toes. This is called "dactylitis." This condition can last from a few days to a week and may be accompanied by fever.

Pain management

The best way to relieve your child's pain is to treat it as soon as it appears. Pain location and intensity, as well as the frequency of pain crises, can vary. Pain is generally treated at home, but in some cases, hospitalization may be necessary.

Pain relief may sometimes require the regular administration of acetaminophen (Tempra™ or Tylenol™) and ibuprofen (Advil™ or Motrin™), (every four or six hours, depending on the medication given or as recommended by a health professional) for a few days, until the pain goes away.

No pain (prevention)	First signs of pain (treatment)	While waiting for medications to take effect	If the pain does not go away within one hour
• Encourage your child to drink plenty of fluids. This is an effective way of preventing pain. Good hydration improves the flow of blood in your child's blood vessels.	• Take your child to a comfortable place where they can sit or lie down.	• Distract your child with a calm activity. For example, read a book to them or play their favourite relaxation music.	• Stronger medication may be necessary. If this is not your child's first pain crisis and you were prescribed morphine or hydromorphone, give your child a dose.
• Dress your child in comfortable weather-appropriate clothes, and make sure to dress them well if it's cold outside.	• Give your child acetaminophen (Tylenol™) or ibuprofen (Advil™), as indicated on the packaging or as recommended by your doctor.	• You can give your child a warm bath or place a warm dry therapeutic compress (such as a Magic Bag™) on the painful area.	• If this is your child's first pain crisis or if morphine or hydromorphone do not relieve the pain in less than one hour, contact your care team for advice.
• Encourage your child to be physically active while respecting their exercise tolerance. Get them to take breaks between activities.	• Encourage your child to drink more fluids than usual.	• Give your child a gentle massage.	• You may need to go to the hematology clinic or to emergency for treatment.
			• While waiting to see the nurse or doctor, continue distracting your child with a calm activity.

When to go to emergency when my child is in pain?

You should **go to emergency** if your child has one of the following symptoms:

- They have a fever in addition to pain;
- Their pain is not relieved by the medications prescribed by the medical team;
- Their pain comes back quickly (in less than three hours) after receiving the medications prescribed by the medical team;
- They have severe headaches;
- They have chest pain, especially if accompanied by difficulty breathing, or if they complain that they are feeling short of breath or choking.

If your child often has pain crises, the hematology team and pain management specialists can help you find the best way to relieve their pain.

Also consider talking to your medical team if:

- Their pain has lasted for several days or is only partially relieved;
- The pain is affecting their shoulder or hip (possibility of a complication known as “osteonecrosis”).

INFOSHEET 6: **STROKE (CEREBROVASCULAR ACCIDENT)**

Why be concerned about strokes in patients with sickle cell disease?

A stroke occurs when part of the brain is deprived of blood and oxygen. Stroke is a sudden and serious complication of sickle cell disease and is a medical emergency.

Around 11% of people with sickle cell disease will have a stroke episode before the age of 20 years without treatment or a screening test such as transcranial Doppler ultrasound. However, this risk is higher in the first 10 years of life, more particularly between the ages of 2 and 10 years. Stroke risk increases to 24% by the age of 45 years. A child with sickle cell disease therefore has a greater risk of having a stroke compared with a child who does not have sickle cell disease. Children with sickle cell disease who receive an abnormal result on a Doppler ultrasound have an even greater risk of having a stroke. A transcranial Doppler ultrasound exam is therefore a very important part of your child's healthcare.

Here are some of the symptoms associated with stroke:

- loss of consciousness (your child faints);
- weakness in the arms or legs;
- difficulty speaking;
- abnormal gait;
- vision problems;
- inability to move the legs or arms, or an entire side of the body;
- convulsions (epileptic seizures):
    - fainting;
    - body stiffness;
    - saliva or foam coming from the mouth;
    - sweating, tremors, rapid limb movements.

If you think your child is showing symptoms of a stroke, call 911. You must immediately take them to emergency.

Transcranial Doppler ultrasound

Your doctor can prescribe a specialized test, which is very useful for preventing stroke. This test, called "transcranial Doppler ultrasound," helps detect narowed blood vessels in the brain. It is a very important test. **Don't miss your appointment for this test**. Whether or not this test is prescribed will depend on the type of sickle cell disease your child has, your child's age and the doctor's clinical assessment. Talk to your doctor or nurses if you have questions about this test.

What is a transcranial Doppler ultrasound?

Doppler ultrasound uses sound waves to check the condition of blood vessels in the brain. The blood vessels that have been damaged by sickle cells are often narrower, and the blood flowing through them produces a sound. This information allows the doctor to identify the children who are at greater risk of having a stroke in the future.

What happens during a transcranial Doppler ultrasound?

The test is performed in the radiology department of some specialized centres. A medical secretary will call you to book an appointment. It is not usually very long (about half an hour), except for young children. Your child will not be put to sleep but will need to lie down or sit quietly during the test. The test is not painful, and you can stay with your child throughout the procedure.

What happens after the test?

The Doppler ultrasound results will be sent to the doctor treating your child's sickle cell disease. If the results are normal, no treatment will be necessary. This test should be repeated at least once a year. If the results are abnormal or suspicious, your next doctor's appointment will be moved up.

What happens if the results show that my child is at risk of having a stroke in the future?

If two consecutive tests show abnormal results, the care team will meet with you to discuss the possibility of getting a treatment involving regular blood transfusions to reduce your child's probability of having a stroke. Studies have actually shown that giving blood transfusions at different intervals, according to the transfusion modalities for children with abnormal results on transcranial Doppler ultrasound, reduces the probability of future stroke by 90%. This test is therefore very important.

Make sure that your child does not miss their appointment.

INFOSHEET 7: **OTHER POSSIBLE HEALTH PROBLEMS**

Children with sickle cell disease are likely to have different health problems that may sometimes become serious. Being able to recognize these potential problems will help you get prompt and effective medical care.

Below are a few additional health problems that children with sickle cell disease could potentially experience:

- acute chest syndrome (blood flow in the lungs is blocked by sickle cells);
- splenic sequestration (enlarged spleen);
- priapism (painful persistent erection);
- aplastic anemia (severe anemia caused by a viral infection);
- avascular necrosis (a bone disorder causing pain and decreased movement);
- retinopathy (damage to the retina of the eye);
- pulmonary hypertension or cardiac dysfunction;
- nephropathy (kidney failure);
- asthma and sleep apnea.

Acute chest syndrome

Acute chest syndrome is a serious problem that requires immediate treatment. It is a common cause of hospitalization in children with sickle cell disease, which can be considered a medical emergency. Acute chest syndrome occurs when sickle cells block blood flow in the lungs. A lung infection (pneumonia) can also trigger acute chest syndrome. Symptoms can vary in intensity (from mild to severe) and can progress rapidly. In all cases, hospitalization is required because your child's condition can deteriorate quickly.

Here are some of the symptoms associated with acute chest syndrome:

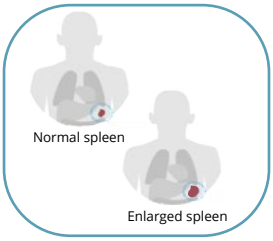
- chest pain;
- abnormally rapid breathing or trouble breathing;
- cough;
- stomach pain;
- fever.

If your child has one of these symptoms, you must absolutely see a doctor. Call your care team, when possible, and take your child to emergency or to the hematology clinic. Do not delay!

Splenic sequestration (enlarged spleen)

The spleen is an organ located on the left side of the stomach. Splenic sequestration is a serious complication that occurs when part of the blood gets blocked in the spleen, enlarging it beyond its normal size, which can lead to rapid drop in hemoglobin. When this occurs, a blood transfusion may be necessary, even urgent.

The spleen of children with sickle cell disease is damaged at a very young age by sickle cells. The blood vessels that carry blood to the spleen also become damaged over time. That is why sickle cells can easily remain blocked in vessels or in the spleen. When this occurs, the spleen can become enlarged and palpable (capable of being felt with the hand). This is called "splenic sequestration".



Some children's spleen is constantly swollen or tender. In this case, the hemoglobin in the blood is often stable, and the situation is not as serious. In all cases, any increase in the size of the spleen must be closely monitored. Your doctor will teach you how to feel for your child's spleen; practise this regularly so that you know its normal size.

Here are some symptoms associated with splenic sequestration:

- irritability;
- unusual weakness or a fainting spell (abnormal sleepiness);
- the lips and mucous membranes inside the mouth become very pale;
- the heart beats faster than usual;
- swollen belly;
- pain on the left side of the belly.

If your child has one of these symptoms, you must absolutely see a doctor. Call your care team, when possible, and take your child to emergency or to the hematology clinic. Do not delay!

Aplastic anemia

Aplastic anemia develops when the body completely stops making new red blood cells. This condition is often caused by a virus known as "parvovirus B19" (which initially causes erythema *infectiosum*, also called "fifth disease"). When this occurs, hemoglobin can drop to a concerning level within only a few days. This condition may therefore require one or more blood transfusions until the body re-starts to make new red blood cells.

Here are some symptoms associated with aplastic anemia:

- overall body weakness;
- lethargy (feeling sleepy and drained of energy);
- paleness (for example, the lips and tongue are greyish or bluish);
- dizziness or fainting;
- headache.

If your child has one of these symptoms, you must absolutely see a doctor. Call your care team, when possible, and take your child to emergency or to the hematology clinic. Do not delay!

Priapism (prolonged painful erection)

Priapism is a serious health problem that can occur in boys with sickle cell disease. Unlike a physiological (normal) erection, a priapism crisis is an unwanted persistent erection, which is often very **painful** and can last from a few minutes to several hours. This erection is caused by sickle cells blocking the blood vessels in the penis, preventing proper blood flow. This condition, when lasting more than a few hours, can sometimes lead to impotence if left untreated. Priapism episodes can happen during sleep or during regular daily activities and can start at any time of life. Medication can relieve the pain, though a procedure performed by a urologist could be recommended. If you child complains of painful erections or if you suspect such a condition, talk to your doctor or nurse.

If the erection lasts more than an hour, go to the hospital without delay!

Avascular necrosis

Avascular necrosis occurs when sickle cells damage bones and joints. The hips and shoulders are the areas most often affected. This damage causes pain and can even prevent your child from walking normally. If your child complains of hip pain, or develops an abnormal gait (a limp), talk to your doctor as soon as possible. It is important to diagnose and treat avascular necrosis as soon as the first symptoms appear.

Retinopathy

Sickle cell disease can lead to complications in the eyes and impair vision. Retinopathy occurs when sickle cells damage the blood vessels in the retina of the eye. Only an ophthalmologist (eye specialist) can diagnose this disorder. Damage that is detected early can be treated. It is therefore generally recommended for children with sickle cell disease to have an eye exam performed by an ophthalmologist no later than age 7 years, then yearly, in addition to regular eye tests by an optometrist.

Pulmonary hypertension or cardiac injury (heart damage)

The flow of red blood cells between the heart and lungs can also be disrupted by deformed red blood cells or by the condition of the blood vessels. Moreover, the heart must compensate for the effects of sickle cell disease by sometimes beating faster and harder. Your doctor will occasionally want to check your child's heart health through ultrasound, a quick and painless method to see how the blood is flowing inside the heart and toward the blood vessels in the lungs.

Nephropathy

Nephropathy refers to kidney failure. A person with sickle cell disease has a greater risk of developing various kidney diseases. These complications are nevertheless more rare in children, but they may appear in adulthood. To screen for kidney problems, your doctor will occasionally order urine tests designed to detect any possible kidney damage in your child.

Asthma

Asthma is a common childhood problem. It causes breathing problems and sometimes reduces the amount of oxygen available in the body. This raises the risk of complications for children who also have sickle cell disease by increasing pain crises, for example.

Symptoms of asthma are:

- difficulty breathing;
- feeling of tightness in the chest;
- cough upon exertion, exposure to cold or when laughing hard;
- cough during the night;
- wheezing.

If your child has these symptoms, talk to your doctor.

Sleep apnea

If your child snores often or loudly at night, this could mean that they have sleep apnea. In some children, snoring comes with pauses in breathing, which reduce the oxygen available in the body. If you notice one of these symptoms, talk to your doctor. Nighttime oxygen levels can be recorded to make a diagnosis. If your child has this problem, you should know that there are options to solve it. In some cases, seeing an ENT (ear, nose and throat) specialist could be indicated.

SECTION 4

How to help your child stay well

INFOSHEET 8: **PREVENTION**

Here are some recommendations to make sure that your child stays healthy and to prevent complications.

Hydration

Making sure that your child stays hydrated could reduce the risk of sickle cells blocking blood flow in small blood vessels. Children with sickle cell disease must drink more than other children. Their urine should ideally be as clear as water or pale yellow. Offer fluids to your child whenever they feel thirsty, especially when:

- they have a fever;
- they are in pain;
- they are very active;
- it is hot outside;
- you are travelling.

Growth and development

Most children with sickle cell disease develop normally as infants. However, during their remaining childhood, some children grow less quickly and can be smaller and thinner than other children of the same age. Puberty can also be delayed. Yet, this situation is usually temporary. You can therefore reassure your teenager that they will eventually catch up to the others.

More and more child living with sickle cell disease are also overweight, even obese. So, it is important to encourage your child to adopt healthy eating habits and to be physically active to prevent possible complications related of overweight.

Know which symptoms to watch for and what to do if you notice them

Being well informed of the particular features of sickle cell disease will give you the confidence to offer your child the right care. Quickly recognizing symptoms is a key aspect in the treatment of the disease and can help decrease the risks for complications. While it is not possible to prevent all health problems, the earlier symptoms are detected, the easier they will be to treat. See Infosheets 3 to 7 in this Guide.

Adopt a healthy lifestyle

Adopting a healthy lifestyle is important for all children, especially those living with sickle cell disease.

As time goes by, your child will learn what fosters their well-being and what causes them problems. Encourage them to take part in activities that are normal for their age when possible. A healthy lifestyle during childhood will allow them to make better choices in the future.

People with sickle cell disease may have decreased exercise tolerance. It has nonetheless been demonstrated that physical activity offers many benefits. It is important for your child to be able to take part in physical education classes at school: participating in sports should be encouraged, according to your child's tolerance level.

Your child can also take part in extracurricular sports activities if they wish. Make sure to tell the activity leaders about your child's health and talk about this to your medical team.

Follow this advice:

- Adopt a **balanced diet:** there is no need to offer your child iron supplements. A healthy varied diet should suffice. In the case of iron deficiency, the care team will make sure to prescribe some iron supplements. A balanced diet contains food from the three food groups (fruits and vegetables, proteins, cereals) as recommended in Canada's Food Guide. This is the best way to get all the vitamins and minerals necessary for your child's health.
- Make sure that your child **drinks fluids regularly**. Get them to choose water over other types of beverages to avoid too much sugar intake.
- Encourage your child to take part in regular **physical or sports activities**, and limit screen time.
- Make sure that your child gets **enough sleep and rest**. The recommended amount of sleep (including naps) for children aged from 4 to 12 months is 12 to 16 hours a day. Between the ages of 1 and 2 years, 11 to 14 hours of sleep a day are recommended. Children aged 3 to 5 years, need 10 to 13 hours of sleep a day, while children aged 6 to 12 years need from 9 to 12 hours of sleep a day.
- Maintain your child's body temperature by **dressing them in weather-appropriate clothing**, so they stay comfortable.

Visit your doctor regularly

Regular medical visits are important. It is essential to regularly talk to your doctor or nurse to make sure that your child is growing and developing well and to ensure appropriate screening and treatment if complications were to appear. This will also help create a solid bond with the care team, contributing to good medical care for your child's health and to your participating in a close partnership. **At each of your visits to the clinic, make sure to get all the prescriptions your child will need until their next appointment.** If you have to miss an appointment, contact the care team so they can schedule another one.

Self-care

Taking care of a child with sickle cell disease can be difficult. Parents sometimes tend to concentrate only on the child with the disease and forget their own needs. It is important for you to find the time to rest so that you can stay healthy and continue to take care of your child as best as you can. If possible, consider having someone take care of your child for a few hours so that you can do something that will help you recharge your batteries. This can do a lot of good for some parents. Your child might benefit from this respite as much as you.

Some parents find it useful to advise their employers that they may occasionally have to take time off work. Sickle cell is an unpredictable disease. When a crisis occurs, you may be obliged to miss work.

If you travel

It is possible to travel with a child living with sickle cell disease, but there are precautions to take. Before leaving, it is best to find out if your child can receive suitable medical care in case of a problem. Make sure that your child is properly vaccinated for your destination and that they have received a preventive treatment against malaria or any other infectious disease endemic in the destination country. If necessary, your care team can refer you to a travel health clinic.

Notify your care team about your travel plans so that they can give you a "travel letter." In addition, travel only in a pressurized airplane and bring the medications you need to treat your child's disease. Also follow these helpful tips:

- Offer your child plenty of fluids;
- Keep an eye on your child at high altitude;
- Make sure that your child is dressed in weather-appropriate clothing so that they are neither too hot nor too cold;
- Do not hesitate to see a doctor during your trip if your child is not feeling well, and mention that they have sickle cell disease.

Make sure that your child has all the recommended vaccines

Proper vaccination is extremely important for your child's health because children with sickle cell disease are more susceptible to infections. Make sure that your child receives all the vaccines recommended for all children, along with any other vaccines suggested by your hematology team. Have on hand your child's vaccination booklet when you go to your clinic appointments. Also, make sure that every member of your family gets a flu shot every year.

INFOSHEET 9: **VACCINATION**

According to the regular immunization schedule, all children born in Québec receive vaccines at the age of 2 months, 4 months, 1 year, 18 months, and before starting school (4-6 years). Other regular vaccines are also given during their school years (Elementary 4 and Secondary III). Along with the regular vaccines, children with sickle cell disease must receive a few additional vaccines, given that they are predisposed to certain bacterial infections. Fortunately, the vaccines protecting them against these infections are very effective and safe, and they are extremely important! A single vaccine, such as those presented below, often protects children against several diseases because they have been combined to reduce the number of injections given to your child.

Bring your vaccination booklet to your medical appointments. The nurse and doctor will want to make sure that your child is well protected.

Regular vaccines

- **DtaP**: protects against diphtheria, tetanus, and pertussis (whooping cough). Diphtheria can lead to breathing problems and sometimes serious heart problems. Thanks to vaccination, this disease has virtually disappeared from Québec, but it still exists in other countries. Pertussis causes a cough that can last up to two months and can sometimes cause infants to stop breathing. Tetanus causes violent muscle spasms that can seriously affect the heart muscles and breathing.
- **HB**: protects against hepatitis B, which is caused by a virus that affects the liver, potentially leading to liver failure.
- **IPV**: protects against polio, an infectious disease caused by a virus, which can lead to muscle paralysis.
- **Hib**: protects against Haemophilus influenzae type b. Before the introduction of vaccines, this bacterium was the leading cause of meningitis and severe respiratory tract infections in children under five years of age.
- **Pneu-C**: protects against pneumonia, blood infection and meningitis, which are caused by pneumococcus. Poor spleen function related to sickle cell disease makes this bacterium highly dangerous and that is why it is necessary to take special precautions if your child has sickle cell disease (see below for the additions to the regular vaccines).
- **RV** : protects against rotavirus. This vaccine is given by mouth and protects against rotavirus gastroenteritis, which causes diarrhea and dehydration.
- **MMR-Var**: protects against measles, mumps, rubella and chickenpox (varicella). Measles can lead to permanent brain damage. It still exists in countries where the vaccine against this disease is not available. Pregnant women can pass measles on to their fetus, sometimes causing fetal brain damage, and even death. Mumps can lead to meningitis or deafness. Lastly, chickenpox can cause complications such as pneumonia, secondary infection of the skin or bone, or brain injury.
- **Men-C-C**: protects against meningococcus, a bacterium responsible for meningitis and very serious blood infections. The poor function of the spleen caused by sickle cell disease makes this bacterium even more dangerous. Special precautions are therefore necessary if your child has sickle cell disease (see below for the additions or changes to the regular vaccines).

- **HAHB**: protects against hepatitis A and hepatitis B, which affect the liver. Hepatitis A can be contracted by ingesting contaminated water or food, and it is very common in many countries. Hepatitis B is spread through contact with blood and during sexual relations.
- **VPH** : protects against several strains of the HPV virus, responsible for cancers of the cervix, vulva, anus, penis and oropharyngeal cavity (throat).

Vaccines added to the regular schedule

People living with sickle cell disease are entitled to other vaccines specific to their condition (which are not available free of charge to healthy people):

- **Pneu-C**: as described above, this vaccine protects against pneumonia, blood infection and pneumococcal meningitis. The poor function of the spleen caused by sickle cell disease makes this bacterium very dangerous. Your hematologist will make sure that your child receives the most complete formulation of this vaccine, that is, the one that protects against the most strains possible.
- **Men-C-ACWY**: protects against four types of meningococci, a bacterium responsible for meningitis and a very serious blood infection. Poor spleen function caused by sickle cell disease makes this bacterium even more dangerous. This vaccine replaces the Men-C-C vaccine described above, in children with sickle cell disease, because it offers more complete protection. This vaccine requires booster shots every three to five years, depending on your child's age.
- **Men-B**: protects against group B meningococcal disease. The number of doses varies with the age of your child.
- **Influenza (seasonal flu)**: it is strongly recommended that children with sickle cell disease get a flu shot every year. This virus, which causes a heavy fever, is often responsible for hospitalizations and pneumonia (or acute chest syndrome), particularly in people with sickle cell disease.
- **COVID-19**: It is strongly recommended that children with sickle cell disease get vaccinated against COVID-19. Vaccination is in fact the best protection against COVID-19 and its complications. Talk to your medical team to find out how many doses are recommended for your child.

Your team may recommend other vaccines, depending on the appearance of new infections or advancements in medical knowledge.

If you have any questions concerning vaccination, do not hesitate to talk to your nurse and doctor.

INFOSHEET 10: **RECOMMENDED MEDICATIONS**

MEDICATION	DESCRIPTION	DOSAGE	BENEFITS	SIDE EFFECTS TO WATCH FOR
Penicillin or amoxicillin	**Penicillin and other antibiotics** (such as amoxicillin) are effective for preventing dangerous infections in children with sickle cell disease. Your child will need to take a preventive antibiotic as soon as they receive their diagnosis and must continue to take it until the age of 5 or 6 years.	Twice a day, morning and evening, every day, until the age of 5 or 6 years. Do not stop giving this medication even if your child feels well and keep giving it until your doctor tells you to stop. In some cases, your doctor may recommend continuing or re-starting a preventive antibiotic after the age of 5 or 6 years.	Fewer serious infections.	• Diarrhea • Bitter taste in the mouth • Nausea • Vomiting • Rashes • Allergic reactions **These side effects are rare.**
Folic acid	**Folic acid** is a vitamin that is essential for the production of red blood cells. A balanced diet, along with a folic acid supplement taken as prescribed by a doctor, will help your child's body make new red blood cells.	As prescribed.	Helps your child's bone marrow make new red blood cells.	Generally, well tolerated.
Hydroxyurea	**Hydroxyurea** is the first medication approved for the treatment of sickle cell disease by the US Food and Drug Administration (FDA). It has major benefits for your child's health. Prescribing hydroxyurea is considered a standard of care in the management of children with sickle cell disease.	As prescribed.	• Fewer sickle cell crises • Fewer hospitalizations • Fewer blood transfusions • Increases the level of fetal hemoglobin, reduces the severity of anemia • Helps prevent acute chest syndrome	• Loss of appetite • Diarrhea • Nausea • Vomiting • Rashes • Darkened nails

Hydroxyurea

How does hydroxyurea help?

Hydroxyurea improves the quality of the blood through its beneficial effect on red blood cells by preventing them from becoming deformed and making them more solid. Hydroxyurea may also have an effect on the other blood cells and even blood vessels, especially by reducing the inflammation often associated with sickle cell disease.

Is hydroxyurea a safe medication?

Hydroxyurea is safe and effective. The role of hydroxyurea has been evaluated since the 1980s in many scientific studies that have assessed not only its efficacy but its safety. The health benefits that hydroxyurea offers to people with sickle cell disease significantly outweigh the side effects that could occur. Moreover, the risks of not treating sickle cell anemia are enormous and far greater than the risks of side effects that might be associated with hydroxyurea treatment.

As with any other medication, hydroxyurea may cause side effects. These are mild, however. One of the most common is the decrease in the number of white blood cells and platelets (which is partially wanted, because it helps decrease inflammation, which is often associated with sickle cell disease). For this reason, routine blood tests will allow your hematologist to monitor the effect of the treatment and adjust the dosage if necessary. Hydroxyurea can sometimes also cause mild nausea and blackening of the fingernails and toenails. The other side effects reported are quite rare.

How is hydroxyurea given?

Hydroxyurea is given by mouth every day. For infants, pharmacists can prepare it as a suspension. For older children, it is offered in the form of gel caps. These can be opened, and the medication sprinkled on a spoon containing food, such as applesauce or yogurt.

What are the health benefits of hydroxyurea for my child?

Hydroxyurea has multiple benefits for children with sickle cell disease. It has been shown, in particular, that hydroxyurea decreases the number of pain crises, the risk of hospitalization, acute chest syndrome episodes, emergency blood transfusions, and several chronic complications associated with sickle cell disease. Hydroxyurea also has positive effects on the blood vessels in the brain, heart and kidneys.

What type of sickle cell disease is hydroxyurea prescribed for?

As mentioned previously, the use of hydroxyurea has a major role to play in the primary prevention of complications from sickle cell disease. Because it reduces the complications of the disease, all children with the SS or S–beta-thalassemia (S/β-Thal) type of sickle cell disease should start taking hydroxyurea in early childhood (usually between 9 and 12 months). This medication is also sometimes indicated for the other types of sickle cell disease.

There are several brochures on hydroxyurea, including "Hydroxyurea for Pediatric SCD Patients" at:
https://www.canhaem.org/wp-content/uploads/2018/09/can-haem-brochure-pediatrique-HU.pdf

To find out more about this medication, talk to your doctor or nurse.

INFOSHEET 11: **TREATMENTS**

Blood transfusions

In some cases, people with sickle cell disease may need to have blood transfusions. These transfusions could be recommended in the following situations:

- in emergency, to treat severe sickle cell complications, such as splenic sequestration or acute chest syndrome;
- as a regular treatment prescribed under a "transfusion program." These blood transfusions are used to treat or prevent chronic complications of sickle cell disease, such as strokes. The transfusion program can be done either through simple transfusions given at regular intervals or exchange transfusions, which can be done manually (manual exchange transfusion) or by automated red cell exchange (a procedure called "erythrocytapheresis");
- if your child needs an operation, your hematology team may prescribe a blood transfusion before the surgical procedure to avoid some complications related to sickle cell disease potentially occurring after the operation. That is why it is important to notify the care team when your child must have a surgical procedure.

Blood transfusions can be given in different ways. In general, a simple transfusion involves injecting a solution intravenously through a catheter for a period of around three hours. Some sickle cell complications sometimes require the need to quickly decrease the number of sickle cells. In such cases, the doctor will prescribe an exchange transfusion in which part of your child's blood will be withdrawn and replaced with a blood transfusion. Your medical team will explain in detail how these treatments will be given if they prove necessary for your child.

Before proceeding with any transfusion, your doctor will explain why your child needs it, its associated benefits and risks, and will present other options if they exist. Except in an emergency situation, you will be asked to sign a consent form indicating that you agree to the transfusion. If your child is 14 years of age or older, your child must sign the transfusion consent form. If transfusions are not acceptable to you or your child for any given reason, it is best to talk to your doctor in advance to be able to arrange a treatment plan if a transfusion proves indicated.

Blood transfusions are well tolerated by most patients, but they may cause side effects, as is the case with any other medical treatment. Take the time to talk about this to your doctor and do not hesitate to ask your questions.

Pain relief medications

Several medications can be used to quickly relieve pain. Always keep acetaminophen (Tylenol™ or Tempra™) or ibuprofen (Advil™ ou Motrin™) in your medicine cabinet. Stronger medications, such as morphine or hydromorphone, may become necessary to relieve your child's pain. Keep these medications in a secure place and give them only as recommended by your doctor. Remember that pain is easier to manage if treated early. If you must use morphine or hydromorphone to relieve your child's pain, also continue giving acetaminophen or ibuprofen regularly. These medications work together to provide more effective relief.

Stem cell transplants

A stem cell transplant is currently the only possible treatment to cure sickle cell disease. However, it is not intended for all children who have the disease. For more information on stem cell transplants, talk to your doctor or nurse.

Gene therapy

Gene therapy is a method that involves replacing a defective gene with a functional gene within sick cells, with a view to treating a disease. This treatment is still experimental for several genetic diseases. Research studies are underway to allow it to be used in the treatment of people with sickle cell disease.

PARTICIPATION IN RESEARCH FOR A CHILD LIVING WITH SICKLE CELL DISEASE

Although we have known about sickle cell disease for a long time and several treatments are available, there is still room for progress toward improving the care for this disease.

Your child may be invited to take part in research studies. These studies can deal with various issues. You might, for example, be asked to:

- allow certain information to be collected about your child. Some studies aim to continue expanding our knowledge about the disease and to gain a better understanding of certain complications. Collecting certain information will help improve our knowledge about the disease and its complications, as well as treatments.
- to take part in novel approaches or novel protocols. To improve your child's care, you may be invited to participate in studies aimed at improving medical practices. This participation could then contribute to changing certain care protocols, for example.
- to participate in evaluating new treatments: over the past few years, research teams have been attempting to discover and apply better treatments for sickle cell disease. Your care team may invite you to participate in a research protocol aimed at knowing more about treatment safety and efficacy.

Your participation in research is important to help improve our knowledge and develop better treatments. **You are under no obligation to participate in a research protocol**. However, you are encouraged to talk to your care team in order to better understand your involvement in the research being done. If you are considering participating in a research protocol, care teams can also inform you about the risks and benefits of the study.

SECTION 5

Tools to assist medical care

INFOSHEET 12: CHILD DEVELOPMENT AND DENTAL HEALTH

Was your child breastfed? Yes ◯ No ◯

If so, for how long? _____

Did your child receive infant formula? Yes ◯ No ◯

If so, which brand or brands were used (in case it was necessary to change it)? _____

_____

When did your child start to eat solid food? _____

Which foods were introduced first? _____

How old was your child when they started to do the following things:

Say their first words: _____ months

Say words other than "dada" and "mama": _____ months

Make short sentences: _____ months

Clap their hands: _____ months

Sit up on their own: _____ months

Walk on their own:_____ months

Run and jump on two feet: _____ months

Scribble with a pencil: _____ months

Use their index finger and thumb to pick up objects: _____ months

Point to things with their fingers: _____ months

Drink from a cup:_____ months

Use a spoon : _____ months

Dental health

When did you start brushing your child's teeth? _____

When did your child visit a dentist the first time? _____
(The first visit to a dentist is now recommended to take place at the age of 12 months.)

Write down your child's different dentist appointments and the treatments received:

Date	Reason for the visit	Treatment received

INFOSHEET 13: **MEDICAL HISTORY AND TREATMENT RECEIVED**

Food allergies:

Food name	Age when the allergy first occurred	Reaction	Treatment received

Drug allergies:

Drug name	Age when the allergy first occurred	Reaction	Treatment received

Write down all your close relatives' serious diseases:

Relationship with your child	Health problem

Write down all the injuries and serious illnesses that led your child to be hospitalized, the name of the hospital, and the treatments received:

Date	Illness or injury	Treatment or medication received	Name of the hospital

INFOSHEET 14: **HEALTHCARE APPOINTMENTS AND BLOOD TESTS**

Write down your child's appointments with other health professionals such as a speech therapist, psychologist, ophthalmologist, social worker, and physiotherapist:

Date	Reason for the visit	Professional's name	Profession

Write down all your child's blood test results:

Date	Leucocytes (white blood cells)	Hemoglobin	Platelets	Neutrophils	Hb F %	Hb S %	Hb C %

SECTION 6

Resources

INFOSHEET 15: **RESOURCES AND WEBSITES**

Association d'anémie falciforme du Canada : https://sicklecelldiseasecanada.com/

Association d'anémie falciforme du Québec : www.anemie-falciforme.org

Center for Disease Control and Prevention. (n. d.). *Sickle Cell Disease (SCD*). www.cdc.gov/ncbddd/sicklecell

Health Canada. (2019). Canada's Food Guide. Healthy eating recommendations. https://food-guide.canada.ca/en/

National Institutes of Health. (2022). *Sickle Cell Disease*. https://www.nhlbi.nih.gov/health-topics/education-and-awareness/sickle-cell

Sickle Cell Information Center. (2016). http://scinfo.org/

St. Jude children's research hospital. (2022). *Sickle cell disease treatment*. www.stjude.org/sicklecell (anglais seulement)

The Canadian Hæmoglobinopathy Association. (2022). *Resource document library*. https://www.canhaem.org/healthcare-professionals/

Do not hesitate to ask your care team for any additional information that could help you.

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