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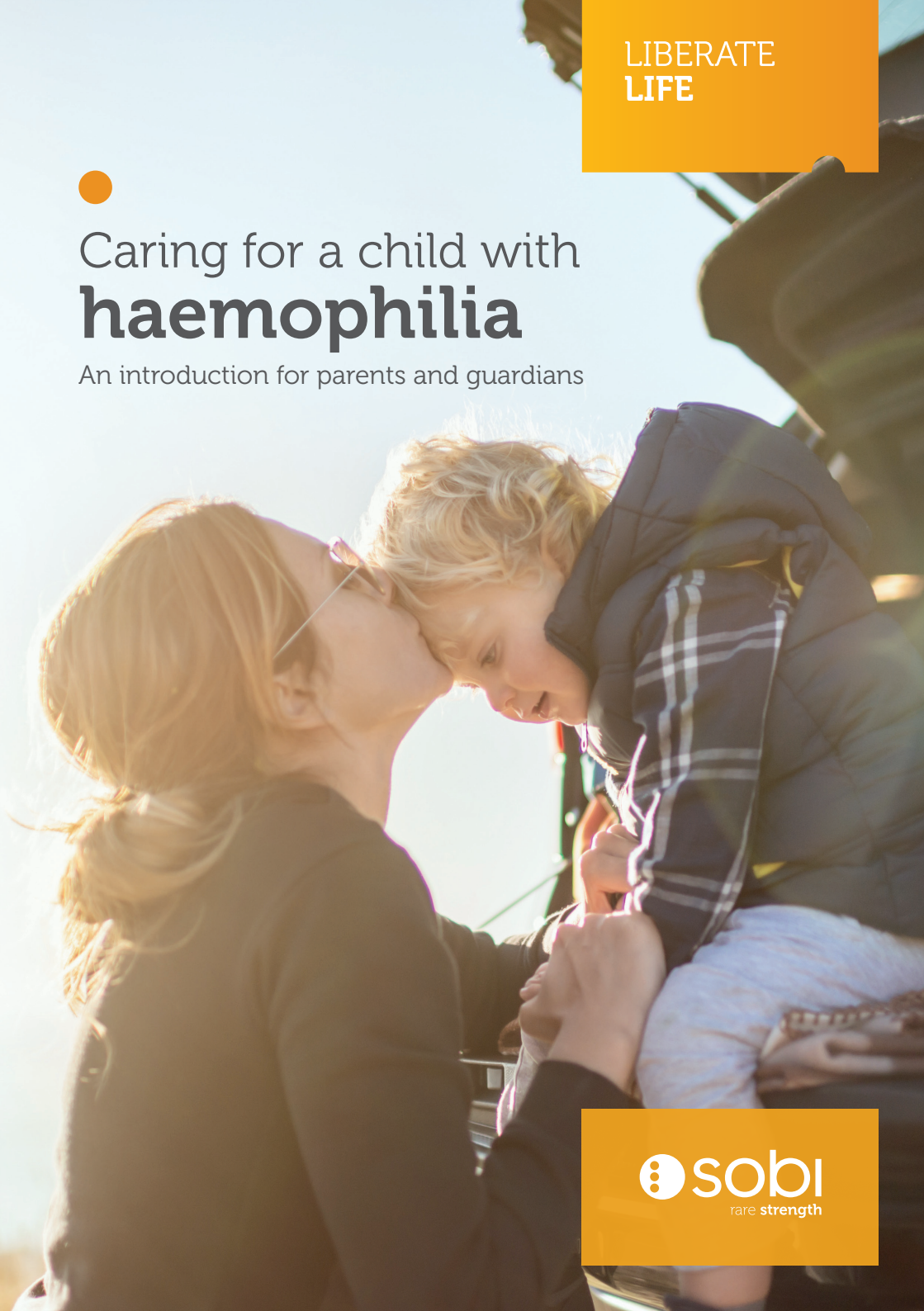
LIFE

Caring for a child with **haemophilia**

An introduction for parents and guardians



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rare **strength**

Contents

This booklet is only intended for people with haemophilia A or B whose haemophilia has not been complicated by the development of inhibitors.

A life like any other in almost every way

Discovering that your child has a chronic illness can come as a shock. Even the word 'chronic' can seem daunting at first. But, just because an illness is life-long, it doesn't mean that the future is going to be unmanageable. When it comes to haemophilia, there are treatments available that will enable your child to live a full, healthy and active life, with many of the same opportunities as any other child.

Life comes first

It's easy to jump to conclusions about what living with a chronic bleeding disorder will be like. Initially you might think it will have a huge impact on daily life, but, as many parents of children with haemophilia have discovered, the condition is manageable. Appropriate treatment can help protect your child from bleeding episodes and help them live a full and active life.

Help for you and your child

Today, haemophilia can be successfully managed with treatment and the help of an integrated team that specialises in bleeding disorders. At first, you will have a lot of contact with your child's doctor, nurse and care team at the hospital or clinic where they are receiving treatment. In time, this is usually reduced to around two follow-up consultations a year.

What is haemophilia?

Haemophilia is a type of bleeding disorder in which the blood does not clot properly. It is a rare disease that is usually inherited and is more common in males than females. With appropriate treatment, children with haemophilia can participate in most everyday activities, but will usually need to be treated regularly to reduce the risk of bleeding.

Blood takes a long time to clot

Because the blood of people with haemophilia cannot clot normally, they bleed for longer than normal. This not only applies to visible bleeding such as cuts and nosebleeds, but also to internal bleeding, for example in the joints and muscles. Haemophilia is due to a deficiency in the body of a specific protein needed for blood clotting, called a 'clotting factor'. Fortunately, the missing clotting factor can be injected into the body to restore the ability to form blood clots.

Injections of the missing clotting factor that are given to stop bleeding when it is already occurring are called 'on-demand' treatments. Injections that are given regularly to protect against bleeds before they start are called 'prophylactic' treatments.

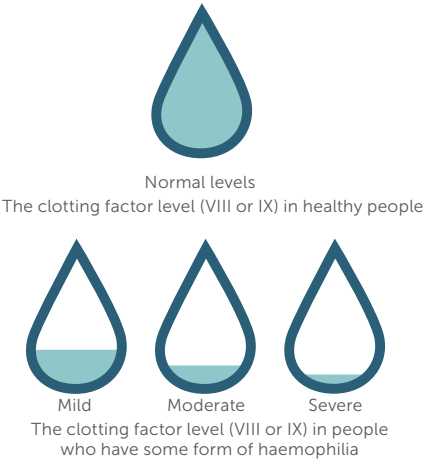
Types of haemophilia

There are two types of haemophilia: haemophilia A and haemophilia B. In haemophilia A there is a lack of clotting factor VIII (eight) and in haemophilia B there is a lack of clotting factor IX (nine). Haemophilia A affects around 1 in 5,000 male births, while haemophilia B is less common and only affects around 1 in 25,000 male births. Haemophilia A and B are usually inherited conditions and both types occur mainly in males.

Levels of severity

Haemophilia is divided into three levels of severity: severe, moderate and mild. The level of severity usually depends on the amount of clotting factor present in the blood.

- Severe haemophilia: the level of clotting factor in the blood is less than 1% of the normal level
- Moderate haemophilia: the level of clotting factor in the blood is between 1–5% of the normal level
- Mild haemophilia: the level of clotting factor in the blood is between 5–40% of the normal level



Normal levels
The clotting factor level (VIII or IX) in healthy people

The clotting factor level (VIII or IX) in people who have some form of haemophilia

People with severe haemophilia can bleed frequently and often for no apparent reason. This is known as spontaneous bleeding and occurs most commonly in the joints or muscles. People with moderate haemophilia usually bleed less frequently than those with severe haemophilia, and do not usually bleed spontaneously. They may bleed for a long time after injury, surgery or dental procedures. People with mild haemophilia usually only have prolonged bleeding after serious injury or surgery and may never bleed spontaneously.

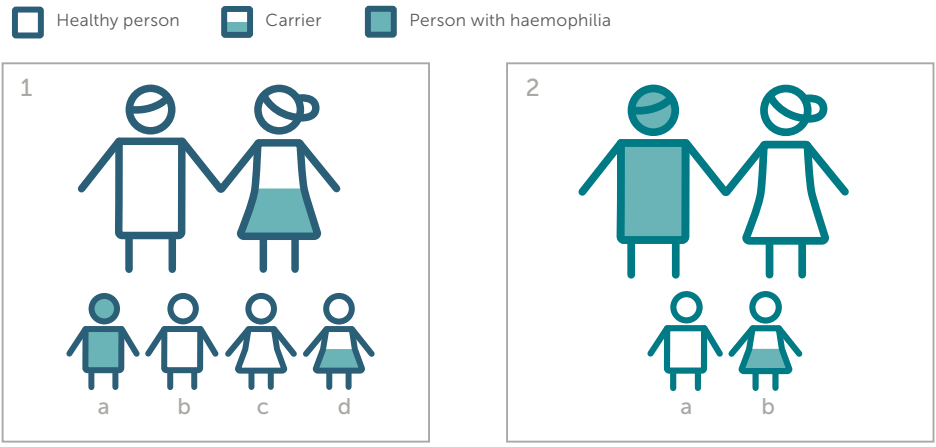
What causes **haemophilia?**

Haemophilia is usually caused by inheritance of a damaged or ‘mutated’ gene from the parents. People with haemophilia can’t produce enough clotting factor because of this damaged gene. In some cases, haemophilia can occur without a family history due to a change in the person’s own genes.

From mother to son

Haemophilia is caused by a damaged gene on the X chromosome. Men only have one X chromosome while women have two. This means that men who inherit the damaged gene always develop haemophilia. If a woman inherits the damaged gene she will become a 'carrier'. Female carriers of haemophilia are not usually affected by the disease, although some do have mild symptoms, but they can pass the damaged gene on to their children.

When a mother is a carrier and the father does not have haemophilia, there is a 50% chance that each son will have haemophilia and a 50% chance that each daughter will be a carrier (see figure 1). When a father has haemophilia and the mother is not a carrier, no sons will inherit haemophilia but all daughters will be carriers of the damaged gene (see figure 2).



A healthy father and a carrier mother will have either:
a) a boy with haemophilia, b) a healthy boy, c) a healthy girl, d) a girl who is a carrier

A father with haemophilia and a healthy mother will have either:
a) a healthy boy, b) a girl who is a carrier

In extremely rare cases it is possible for a daughter to inherit haemophilia. This happens when the father has haemophilia and the mother is a carrier so the daughter inherits the affected X chromosome from both parents. If a parent has the disease or is a carrier, genetic testing can determine the risk of a child developing haemophilia during pregnancy.

When there's no apparent family history

Some children are born with haemophilia despite the fact that neither of their parents are carriers or have the disease. These cases are usually caused by a new gene mutation occurring. Around 30% of people with haemophilia A or B have this sporadic form.

What happens when you bleed?

Normally, the body reacts to bleeding by triggering a series of events that help the blood to clot. Proteins called clotting factors interact with each other in a predetermined sequence to stop the bleeding — much like falling dominoes.

The missing 'domino'

People with haemophilia lack an essential clotting factor, so when they start to bleed a disruption occurs in the clotting process. In haemophilia A there is a lack of factor VIII (eight) and in haemophilia B there is a lack of factor IX (nine). By administering medication containing the missing 'domino', the clot formation process can be restored.

Types of bleeding

Bleeding in people with haemophilia can happen anywhere in the body, including beneath the skin. This 'hidden' bleeding usually occurs in the joints and muscles, typically affecting the ankles, elbows and knees. In severe haemophilia, bleeding in the joints often occurs for no apparent reason. It often appears around the age of one, when the child starts to walk and move around.

Signs of bleeding in the joints are the child feeling pain and not wanting to walk. You should also be especially alert to any stiffness, warmth and swelling. Muscle bleeding is usually more difficult to see; the muscles are located so far beneath the skin that bruising is not visible. Common signs are that the child is experiencing pain and showing a reluctance to move around.

Bleeding inside the skull is rare but very serious and you should call an ambulance if you suspect it. Signs include a severe headache, stiff neck, vomiting and confusion. If your child receives a severe blow to the head, you should always contact your child's doctor, nurse or member of the care team at the hospital or clinic where they are receiving treatment.

If you suspect internal bleeding it is always best to seek advice from a healthcare professional.

How is **haemophilia managed?**

Severe haemophilia is usually managed by replacing the deficient clotting factor to try to prevent bleeds and to allow the person with haemophilia to lead a full and normal life. With appropriate management, your child can participate in many of the same activities as many other children.

Injecting the missing clotting factor

Treatment for haemophilia is given by an injection into a vein to administer the clotting factor that is completely or partially missing from the blood. The frequency of the injections depends on the type of haemophilia, the severity and the type of treatment and may be given preventively (prophylaxis) or as needed (on-demand).

People on prophylactic treatment have regular injections to replace the clotting factor in their blood to help prevent a bleed. Prophylaxis is recommended for most people with severe haemophilia as it decreases the number of bleeding episodes and may protect against joint disease and help improve quality of life.

Types of clotting factor

Replacement clotting factors can be taken from donated human blood (in which case they are called 'plasma-derived') or made in a laboratory using special cells (in which case they are called 'recombinant' products).

Development of antibodies

For most people, treatment works extremely well. However, for some, their bodies react as if the clotting factor was a foreign substance. Their immune system then develops antibodies known as inhibitors, which means that the injected clotting factor may not work as well or work at all. The antibodies may disappear spontaneously after a period of regular treatment, or a higher dose of the injection may be needed for a period of time. In some people, the antibodies remain and their bleeding must be controlled using a different treatment.

Research and progress

Over the past ten years important progress has been made in haemophilia research. These advances can help provide children with haemophilia with the freedom to lead more active lives and look forward to a bright future.

Living with **haemophilia**

Modern treatment helps people with haemophilia to live their lives to the full and participate in a range of activities. This is not just about physical activities such as playing sports, although this is important. It also means being able to carry out everyday activities, like walking to school, without the worry of a bleeding episode.

As your child gets older, try to encourage them to be physically active. Strong muscles are needed to support the joints and provide better balance and bodily control. Remember that active children, especially those who play sports, need to take their medication regularly to ensure they are protected.

With appropriate treatment it is possible that your child will not have to prioritise their health over their opportunities. It is possible, for example, for a child with haemophilia to travel or study full time and experience the opportunities that life has to offer.

Build yourself a support network

When you live with a child who suffers from haemophilia it is important that you make time for yourself. Caring for a child with haemophilia can be challenging so it's important to develop a network of friends and family who understand your child's situation and can be there to help support your family when needed. All parents need some breathing space, especially parents of children who require a lot of care and attention. Therefore, it is a good idea to inform family (including older siblings), friends and your child's nursery school and childminder of your child's condition as soon as possible.

Helpful tips for managing **haemophilia**

As a parent of a child with haemophilia you will need to take a number of things into consideration as your child grows up. Although you may be worried initially, haemophilia can be managed and your child can look forward to living a long and fulfilling life.

Create a safe environment at home

When your child begins to stand up and take their first steps it is natural for them to fall over occasionally. Learning to walk is a key stage in every child's development and should be encouraged as much as possible, but it is important to be aware of how injuries could happen and how to minimise them.

- You may wish to install safety gates, childproof locks and radiator covers and attach corner guards to any sharp edges
- When furnishing your home, consider soft upholstered chairs and tables with rounded corners, and secure or remove any items that could be tripped over or pulled down
- Elbows, feet and knees are usually the areas that are most prone to bleeding — it could be a good idea to sew knee and elbow pads into clothing to protect vulnerable joints

Inform healthcare professionals

- When you contact your dentist, family doctor or other members of the healthcare profession it is important to inform them that your child has haemophilia
- Your child should have a 'bleeding disorder alert card' with their diagnosis, blood type, current medication and the telephone numbers of the clinic or hospital, contact person and on-call service unit. Always keep this card in an easily accessible place to be able to give it to healthcare professionals, especially dentists or in emergency situations

Ask for help and support

- Do not hesitate to contact doctors and nurses at the clinic or hospital if you have concerns or questions. They will be happy to share their knowledge and experience
- In addition to the healthcare sector, there are patient associations that can provide further information about haemophilia as well as offering support, such as The Haemophilia Society www.haemophilia.org.uk
- Create a support network around your family that can provide relief when needed so you can recharge and come back with renewed energy

Maintain good health and hygiene

- Have a clear routine regarding your child's haemophilia treatment right from the start
- Good oral hygiene is important to keep teeth and gums healthy, making it less likely for the gums to bleed or for your child to need dental surgery
- Avoid intramuscular injections as they can cause muscle bleeds. Vaccinations normally given in the muscle should instead be given under the skin (subcutaneously)
- Use paracetamol for pain relief. The use of aspirin, ibuprofen, diclofenac and other non-steroidal anti-inflammatory drugs (NSAIDs) can be harmful for people with bleeding disorders because they can interfere with blood clotting

Stay positive and encourage your child to lead a full and active life

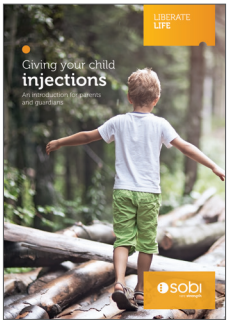
- Physical activity is particularly important for people with haemophilia to help strengthen muscles and support joints; just be aware that a child who engages in sporting activities may need to take their medication more often
- Remember that with proper management, haemophilia will not control every aspect of your child's life. Their ability to participate in certain activities and opportunities will not be limited by their condition
- Don't forget to enjoy all the wonderful things that having a child entails

Thank you to our contributors

Sobi would like to express our warmest thanks to senior physician Susanna Ranta and nurses Evangelia Vlachou and Eva Strand from Barnkoagulationsmottagningen (the Children's Coagulation Clinic), Karolinska University Hospital, Solna, Sweden. These healthcare professionals all have extensive experience in haemophilia and have kindly helped us to fact-check the contents of this brochure.
Their insights and advice, together with FBIS (Förbundet blödarsjuka i Sverige — the Swedish haemophilia patient association), have been invaluable in the development of this brochure.

NP-8280 September 2019

Discovering that your child has haemophilia may seem daunting, but modern treatments are available that will enable your child to live a full and active life. To support you and ease any concerns that you may have now and as your child gets older, you may also find the companion booklet helpful:



A step-by-step guide to help you get the hang of giving your child injections.

Endorsed by:

THE HAEMOPHILIA SOCIETY

Registered Charity No. 288260
Charity registered in Scotland No. SC039732

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