

SWEDISH GUIDELINES FOR:

**THE CARE AND  
TREATMENT OF  
HAEMOPHILIACS**

PUBLISHED 2003



“Swedish Guidelines for the Care and Treatment of Haemophiliacs” contains a description of the disorder and its treatment and supplements our current knowledge about haemophilia. The guidelines are aimed at hospital staff, those with administrative responsibility within the health sector, health-care and hospital politicians, social insurance employees and other professional groups, that in various ways may be involved in decisions which affect the haemophiliac patient’s life and health as well as being aimed at the haemophiliacs themselves and their relatives.

“Swedish Guidelines for the Care and Treatment of Haemophiliacs” is also available in a condensed version. The guidelines are supplemented by a series of information sheets “Facts and advice” which deal with different aspects of the disorder and are aimed at everyone who wants to gain more knowledge about haemophilia.

The document is an updated version of “National guidelines for the care and treatment of haemophiliacs”, published 1997.

The haemophilia centres in Malmö, Gothenburg and Stockholm.

The Swedish Haemophilia Society.

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## Swedish guidelines for the care and treatment of haemophilia

Swedish guidelines for haemophilia refer to the hereditary haemophilia disorders haemophilia A and haemophilia B. Separate guidelines are being compiled for von Willebrand disease.

The guidelines have been compiled by representatives for Sweden's three haemophilia centres, in co-operation with the Swedish Haemophilia Society in order to give all haemophiliacs identical, knowledge-based and efficient care.

The guidelines are revised continuously and establish the conclusion that has been reached between the representatives for haemophilia care and the patient organisation.

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Haemophilia is caused by a congenital deficiency of/or complete absence of the functioning coagulation factor VIII (haemophilia A) or coagulation factor IX (haemophilia B). Haemophilia A and B are gender related and hereditary and affect almost exclusively boys. There are also other types of haemophilia.

Haemophilia is characterized by a tendency to have lengthy bleedings. The bleedings can appear to occur spontaneously or after minimal trauma. It is typical that bleeding occurs in the joints and muscles. Untreated bleedings cause gradual degeneration of the body's motility apparatus.

Sometimes bleeding in the mucous membrane happens. Life threatening bleedings can occur after a slight trauma to the skull or internal organs. Such complications can happen in the case of all types of haemophilia.

Within a few years, haemophilia in its untreated form leads to serious damage in the form of deformed joints with severe limitation of movement, contractures, chronic pain and muscle atrophy. The disabling joint bleeds can be prevented or treated with the coagulation factor concentrate that the haemophilia patient lacks, the so-called factor concentrate.

About 30 per cent of those who have a severe form of haemophilia develop antibodies (inhibitors), which reduce the effect of the treatment. During the period 1950 – 1980 treatment took place with plasma-based factor concentrate that was not virus inactivated. This led to complications in the form of transfusion infection with hiv, hepatitis A, B and C. Allergic reactions were commoner than with modern products.

Haemophilia A and B are rare and in Sweden there are about 800 individuals who need specialised treatment. Today care is divided between the three haemophilia centres: Karolinska Hospital in Stockholm, Sahlgrenska University Hospital in Gothenburg and Malmö University Hospital in Malmö. Centralisation makes it possible to maintain knowledge about haemophilia on

a high level with an international exchange of experience. It also facilitates research into the disorder, co-ordination between different specialities at the university hospitals, quality control, as well as the regular follow-up of care and treatment, which makes health care more cost-effective. Health care should be pursued, according to recommendations from the World Health Organization (WHO) and the World Federation of Haemophilia (WHF), from the overall viewpoint of the haemophiliac with a team of specially-trained staff that can offer the best possible solutions for the haemophiliac's problems.

At the haemophilia centre the haemophiliac is taught about his disorder and learns how to take responsibility for his own treatment. Continuous repeated further education is therefore needed. The disorder is relatively unknown among hospital staff. The haemophilia centres must therefore inform them about haemophilia and about the care and work that is performed there so that the correct measures are taken in, for example, an acute situation. It is important that the disorder is diagnosed early in life in order to avoid fatal complications.

So that care of haemophiliacs, even on a local level, can take place according to an overall view, co-operation between the haemophilia centres, the patient's local hospital, the regional authorities and the municipal authorities must be improved. Communication in different ways between the haemophilia centres and those involved on the local level, as well as the individual care schemes that the patients are provided with, must make it easier for the haemophiliac to gain access to qualified medical care and satisfactory support irrespective of residential area.

## The aim of care

**The care of haemophiliacs must have as its aim the highest possible quality of life for all haemophiliacs.**

- By preventing bleedings, disablement and other complications of the disorder and treatment
- By having as its starting point, an overall view of the patient and the patient's relatives

**This presupposes:**

- Prophylactic treatment from an early age
- Treatment at home
- Centralised haemophilia care
- A chain of care that functions: the haemophilia centre – the patient's local hospital unit – the patient
- Education and information campaigns
- National and international co-operation around research and development
- A continuous evaluation and quality control of the care process

# Definition of haemophilia

## HAEMOPHILIA A AND B

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Haemophilia A and haemophilia B are hereditary and transmitted recessively. The disorder affects almost exclusively men, while women are the carriers. However, in about half of the new cases of the disorder there is no known person with haemophilia in the family. Haemophilia A means that there is a deficiency or absence of functioning coagulation factor VIII and haemophilia B means that there is a deficiency or absence of functioning coagulation factor IX. Haemophilia A is five times more common than haemophilia B. About one in 10 000 men suffer from haemophilia. There are about 800 individuals who have been diagnosed as having haemophilia in Sweden today. The degree of seriousness of the disorder is dependent on the concentration of coagulation factor in the blood.

### The varying degrees of severity of haemophilia

Severe form ..... less than 1% \*  
Moderate form ..... 1 to less than 5%  
Mild form ..... 5 to 40%  
The normal population has 50-150% of the respective factor  
\* 100% = 1.00 kIU/L

## OTHER FORMS OF HAEMOPHILIA

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Von Willebrand disease is autosomal (i.e. inherited independent of sex chromosome). Men and women, therefore, run the same risk of getting the disease. Von Willebrand disease means there is a deficiency of, or defective, von Willebrand factor. The serious form of von Willebrand disease is present in only about 50 individuals in Sweden.

Isolated cases of other forms of haemophilia occur, caused by a hereditary deficiency of or total absence of other functioning coagulation factors such as: factor XI, XIII, VII, V, II or fibrinogen (factor I). At present there are about 100 individuals who are registered as having a deficiency of

other coagulation factor and about ten individuals registered with a total absence of other coagulation factor.

Other forms of haemophilia are inherited autosomally and consequently affect both sexes equally. These forms occur in about the same degrees of severity as haemophilia A and B.

Hereditary thrombocytic functional disorders also occur, of which Glanzmann's thrombasthenia disease and Bernard-Soulier's syndrome are well defined but very unusual.

## **SYMPTOMS**

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Severe and moderate forms of haemophilia are chiefly characterised by bleedings in the joints and muscles, occurring either apparently spontaneously or after slight trauma. "Late bleedings" are typical, that is to say, a bleeding appears to stop and then starts again later. The bleedings are very painful and can cause, if untreated, lasting changes in the joint capsule, joint cartilage and bone. Repeated bleedings can, after several years, bring about increasing disability, stiffness and a chronic pain condition, as well as result in weakness in the muscles. In elderly haemophiliacs all the various grades of severity can be seen, from individual damaged joints with relatively maintained motility to wheelchair dependence.

**THE MILD FORM OF HAEMOPHILIA** does not usually lead to joint or muscle bleedings without external trauma. The risk is minimal for lasting disability. The definition mild as well as the lack of spontaneous bleeding problems can mean, however, that the seriousness of mild haemophilia can be underestimated both by the haemophiliac himself and health care. This is why a readiness to deal with the severe bleedings, which can arise even after minor traumas and operations, is sometimes lacking. Trauma to the skull or stomach, as indeed all sorts of operations, can lead to bleeding with fatal outcome even in individuals with mild haemophilia if treatment is not given. Mild haemophilia can be diagnosed at all ages unlike more severe forms of haemophilia, which are usually diagnosed at an early age.

## **The organisation of health care**

Haemophilia is an unusual disorder with severe symptoms and complications. The complicated treatment has even meant serious side-effects. An important prerequisite for optimal care is a health-care organisation with highly specialised haemophilia centres. So that all haemophiliacs, irrespective of where they live, have access to the same care quality, a follow-up of treatment and support from the patient's local medical facilities is required.

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The value of centralising haemophilia care in Sweden was observed already in the 1950's. Experiences, even from other countries, as well as recommendations from WHO and WFH mean that this care structure must apply.

## **HAEMOPHILIA CENTRES**

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At Sweden's three haemophilia centres there are doctors with specialist qualifications both in internal medicine or paediatrics as well as in coagulation disorders and with experience of haemophilia medical care. In addition there are specially-trained staff: a nurse, physiotherapist, orthopaedic surgeon, dentist, general surgeon, gynaecologist, geneticist, counsellor and a psychologist (the combination varies somewhat between the different centres). Co-operation is also possible with a doctor specialising in infectious diseases, a pain specialist and doctors with other specialities when necessary. In this team, medical, psychological, pedagogical, social and technical contributions are combined according to the haemophiliac's needs. The competence profile varies between the three centres, which is why close collaboration between them is important. There is, for example, a formal co-operation agreement between Gothenburg and Malmö.

## **LABORATORY RESOURCES**

Every haemophilia centre has a special laboratory attached to it that has possibilities to diagnose different types of haemophilia and antibody development. By performing acute analyses of the factor level in the blood, treatment in connection with surgical operations can be carefully monitored. Furthermore, certain laboratories perform genetic testing to verify the diagnosis, to confirm possible carrier state in women and for foetal diagnostics.

## **REGULAR CHECK-UPS**

Individuals with haemophilia are monitored regularly at a haemophilia centre to evaluate the effects of the treatment. The check-ups comprise a medical examination, an assessment of the status of the joints, hepatitis and hiv infection as well as the presence of antibodies to factor concentrate, a decision on continued dosage, the need for pain relief, physiotherapy and orthopaedic surgery and the need for dental care. When necessary, genetic advice is also given. It is possible to meet a counsellor.

The check-ups are performed every six months or every other year depending on the severity of the disorder and possible complications. Each check-up is followed by a written result including treatment recommendations, which are sent to the patient's local doctor and to the haemophiliac.

### **AROUND THE CLOCK SERVICE**

It is essential that haemophiliacs and their relatives can get around the clock advice from a haemophilia doctor so that acute bleeding conditions can be correctly dealt with. In this situation the patient or the local hospital must contact the haemophilia centre.

Many doctors and other health-care staff seldom come in contact with haemophilia and general knowledge about the disorder is therefore insufficient. The around the clock service at the haemophilia centre is also used for consultations about patients with other bleeding conditions/coagulation disorders.

#### **A CHECK-LIST FOR CHECK-UPS AT THE HAEMOPHILIA CENTRE**

- √ Treatment (medication, dosage, etc)
- √ Bleeding and days of absence
- √ Joint status
- √ Blood pressure
- √ Laboratory tests including virus testing
- √ Vaccination
- √ Pain and pain relief
- √ Family situation and occupation
- √ Dental care
- √ Psychosocial support
- √ Physiotherapy
- √ Smoking/alcohol consumption
- √ Other medication, prescriptions
- √ Information to the local health-care facilities
- √ Other items (patient organisation, etc)

### **EDUCATION AND INFORMATION**

The haemophilia centres provide education and information for health-care staff as well as for the haemophiliac and his/her relatives. Individuals with severe or moderate haemophilia need

continuous education about prophylactic treatment, about dosages in different types of bleedings and about possible complications.

Individuals with mild haemophilia have not had the same frequent contact with a haemophilia centre and therefore have not had the same amount of education. Since even mild haemophilia means a risk of life threatening bleeding after a trauma, there is also a need for information and education for individuals with mild haemophilia.

## **MONITORING**

Individual care planning assumes that the patient is monitored both at his local hospital unit and at a haemophilia centre. The monitoring of care at a haemophilia centre ensures the implementation of the measures decided upon and contributes to the maintenance of a good standard of care.

A patient register with easily available data is an additional foundation stone for an efficient and quality assured care and facilitates general evaluations of epidemiology, health-care needs and treatment policy.

## **ECONOMY**

Treatment at the haemophilia centres is cost-effective for society. Developmental work is performed here in order to make the treatment more effective and to reduce the need for factor concentrate by means of new methods of administration and pharmacokinetic calculations.

Newer and safer factor concentrates as well as alternative forms of treatment can in the future lead to better treatment at a lower cost and with fewer side-effects for haemophiliacs. A prerequisite for this is that care should not be spread out between a lot of small hospital units with not enough haemophilia patients and low competence among their staff.

## **RESEARCH**

Haemophilia research has had an internationally prominent position for a long time. Haemophilia centres carry out haemophilia studies that are mostly international. International co-operation on haemophilia is important for development, not least against the background that the disorder is unusual. Co-operation takes place with other haemophilia centres, the World Federation of Haemophilia (WFH), the World Health Organisation (WHO) and the International Society of Thrombosis and Haemostasis (ISTH), among others. This type of co-operation is necessary in order to maintain the best possible standard of care.

## CARE IN THE PATIENT'S HOME AREA

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In the case of bleedings, individuals with haemophilia must be taken care of first. It is important that the around the clock coagulation service is consulted. A store of the factor concentrate that may be needed must be kept either in the haemophiliac's home or at the hospital unit where the haemophiliac most probably will be treated.

Haemophiliacs who cannot manage the injection technique must get fast help with intravenous injections in the case of an acute bleeding.

After a bleeding the haemophiliac may need physiotherapy, without having to wait a long time, in order to regain lost joint or muscle function. Many also need regular help with training to counteract or compensate damage done to joints. Curative and psychological support may be needed during critical periods in the haemophiliac's life. Haemophiliacs and their relatives should also be given the opportunity to participate in education aimed at rehabilitation and self-care, camping activities and rehabilitation stays, etc.

All individuals with haemophilia A and B, including those with mild haemophilia, must receive a specialist care referral for consultation at a haemophilia centre. The amount of contact is to be decided individually.
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## Diagnostics

Patients with pronounced bleeding symptoms, where haemophilia or other bleeding disorders can be suspected, must be examined at a haemophilia centre where there are clinically active coagulation specialists who have experience of haemophilia.

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In the case of **SEVERE AND MODERATE HAEMOPHILIA** the bleeding symptoms are so pronounced that the diagnosis is often made early. The most usual début symptoms are bruises, sometimes with a lumpy appearance, bleeding from the mouth after trauma or bleedings after injections, blood sampling or other surgical operations. Brain haemorrhage after birth can happen with relatively slight birth complications, but is proportionately rare. On the other hand, the occurrence of cephalhaematoma, for example, is usual in the neonatal period (almost 20% in a Swedish study). Joint and muscle bleedings usually make their début when the child begins walking. It has happened that parents have been suspected of child abuse because the child's bruises are in unusual places.

**MILD HAEMOPHILIA** can remain undetected into adult age and make its *début* with life-threatening bleedings after surgical operations, tooth extraction or other trauma. Large-scale muscle haematoma can take arise even after moderate trauma. The term “mild” can therefore be misleading and lull both the haemophiliac and hospital staff into a sense of false security. Even individuals with mild haemophilia should be monitored at a haemophilia centre.

When haemophilia is suspected, the first blood sample is taken at the patient’s local hospital and is sent to a haemophilia centre for examination. If the child has haemophilia, the child and its parents are remitted to a haemophilia centre for a confirmatory examination. The diagnosis of haemophilia means a severe, mental ordeal especially for parents who have not had haemophilia in their family before. They must therefore receive support from a counsellor or a psychologist in order to cope with the crisis reaction which the diagnosis gives rise to. The parents of the newly diagnosed child are also offered contact with another family with a haemophiliac child.

When the diagnosis haemophilia is made, an investigation of relatives, registration of the family and the patient is provided with a bleeding risk card.

If necessary, a home visit by the counsellor and the nurse from the haemophilia centre to follow up, can be arranged.

## **Carrier and foetal diagnostics**

Women who belong to families with haemophilia should be offered carrier diagnostics. If the woman is a carrier she should receive more extensive information and genetic advice as well as information on whether the factor VIII/IX-content involves a risk of bleeding.

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Guidelines for carriers and foetal diagnostics (prenatal diagnostics) have been compiled jointly by a group of experts for WFH and WHO. A complete investigation demands knowledge and access to the genealogical tree, coagulation analyses, immunological methods, as well as indirect and direct gene diagnostics, the latter based on charted mutation. These methods are, however, marred by sources of error that have to be taken into consideration.

This work is highly specialised and must therefore be centralised at a haemophilia centre.

In many cases, access to blood samples from a sick member of the family and, depending on the degree of the relationship, a varied number of individuals within the family. In Sweden 50% of the families with only one member with haemophilia, are so-called sporadic families with one spontaneous mutation for haemophilia. These families with only one known case of haemophilia are especially difficult to investigate.

## ADVICE

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Many of the molecular biological gene analyses are time-consuming and it is important that the diagnostics of the carrier take place before pregnancy begins. Diagnostics should be preceded by genetic advice that should be given by an individual with the capacity to communicate sensitively and a sound ability to imagine other people's situation. The advice ought to be as objective as possible and based on knowledge of haemophilia, genetics, prenatal diagnostics as well as molecular biology. In genetic advice, the individual's freedom of choice must be respected. The advice is aimed at giving the parents-to-be enough information for them to be able to make an independent decision as to possible parenthood, as well as giving them support during the entire process. The investigation and the information must therefore take place at a haemophilia centre. The ethics connected to prenatal diagnostics of haemophilia are particularly difficult because nowadays the disorder can be treated successfully with regular prophylactic treatment. Some women in families with haemophilia want to have prenatal diagnostics even if they have no plans to end the pregnancy. In individual cases, it can be of great value to find out that the foetus is healthy, alternatively to be able to prepare for the baby being a boy with haemophilia. In these situations, the same broad indications apply as for other disorders where prenatal diagnostics are offered. Diagnostics before implantation in the womb, that is to say in conjunction with artificial insemination, is today performed in the case of some severe illnesses and in some countries even for haemophilia. As abortions, sometimes repeated abortions, when the foetus again and again proves to have haemophilia, are psychologically burdensome for the mother, the possibility ought to be developed even in Sweden.

## Treatment

The aim of the treatment of haemophiliacs is to counteract and prevent bleedings and the reduction of the body's motility capacity and other complications of the disorder. During the past thirty years, the treatment of haemophiliacs has undergone a revolutionary development. Preventive treatment, so-called "prophylaxis", with factor concentrate has proved to be the key to a life without the motility apparatus being destroyed. Thoroughly purified and virus inactivated and recombinant preparations have meant less and less risk of side-effects lately. Progress within gene therapy research provides new hope for haemophiliacs but can only primarily be seen as a cure for the disorder in a longer time perspective.

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If one excludes the complications (hiv and hepatitis C with severe liver damage) which have befallen haemophiliacs, the treatment of bleedings has meant that the average length of life for haemophiliacs in Sweden has more than tripled since the 1950's. It is now insignificantly less than for a non-haemophiliac.

Sweden was the country that first began regular prophylactic treatment in severe forms of haemophilia. The aim was to eliminate the disabling joint bleedings, which otherwise occur 20-30 times a year. Several studies have shown that the general condition and joint status of severely ill haemophiliacs in Sweden is considerably better than in most other countries. The haemophiliacs' social life has improved substantially.

Prophylactic treatment has greatly reduced the risks of severe bleedings in the head and the abdomen after trauma and has reduced the number of days spent in hospital. In those who have been treated from a very early age, prophylaxis will even eliminate the need for expensive, reconstructive, orthopaedic surgery.

## FORMS OF TREATMENT

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- **PROPHYLACTIC TREATMENT** entails intravenous injections of factor concentrate 2-3 times a week. Children with the severe form of haemophilia and in some cases, even children with the moderate form, already begin treatment at 1-3 years old. The treatment is often lifelong and it requires a lot of support and help initially, in order to work.

The aim of the treatment is to continually maintain the factor level in the blood on a level that prevents the patient from bleeding. The effect of the factor concentrate and the half-life varies between individuals. Even ageing affects the pharmacokinetics. This demands regular pharmacokinetic studies in order to optimise treatment. Generally, it is sufficient with single blood sample tests in connection with routine check-ups.

Prophylactic treatment can be further improved when a better technique for the continuous administration of factor concentrate has been developed.

- In the mild or moderate form of haemophilia it is normally sufficient with so-called **TREATMENT ON DEMAND**. The haemophiliac injects himself with factor concentrate at the level needed to stop the bleeding. (See the facts information, page 16). The effect of the treatment is dependent on how fast it is begun after a bleeding has started, a few hours delay can considerably worsen the result. Usually, the treatment is repeated a couple of times during the next 24 hours. The haemophiliac must have sound knowledge of the disorder, have access to the preparation and have learnt how to treat himself at home.

In the mild form of haemophilia A there is an **ALTERNATIVE FORM OF TREATMENT**.

Desmopressin (DDAVP), registered as Octostim® is a synthetically produced, hormone-like medicine with several blood-clotting effects. It stimulates a fast and powerful increase in the concentration of factor VIII and von Willebrand factor. Octostim® is not only registered as an injection substance but also in the form of a high dose nasal spray which is suitable for home treatment. The effect of desmopressin varies from individual to individual but functions in the same way in the same individual from one time to another. This is why a test dose is often given at the time of diagnosis in order to evaluate how the patient responds to desmopressin.

From experience it is known that patients with a basic level of factor VIII under 15% do not as a rule gain a sufficient enough increase in factor VIII to give protection in connection with larger surgical operations or larger bleedings. Desmopressin can as a rule, therefore, only be used for the mildest haemophilia A patients in such situations. For those patients who respond satisfactorily to desmopressin, it is preferable to factor concentrate, by reason of cost.

Tranexamsyra (Cyklokapron®) is used as an addition to treatment in many different bleedings, above all from mucous membranes. It delays premature disintegration of the fragile coagula in the haemophiliacs, but almost never has sufficient effect when taken alone.

- There is also factor concentrate for **the treatment of severe von Willebrand disease and certain other unusual forms of lack of factor**.

## **TREATMENT PREPARATIONS**

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Until the end of the 1950's, the treatment of haemophilia bleedings was limited to painkillers, rest and if necessary, a blood transfusion. In 1957 factor VIII concentrate from blood plasma began to be produced on a small scale and the prophylactic treatment of haemophilia could begin. It was first in 1969, that haemophilia B patients gained access to a factor IX concentrate.

The first generations of factor concentrate were impure, that is to say, they contained, in addition to the coagulation factor in question, a large amount of plasma proteins and even virus. The patients were afflicted with allergic reactions and transfusion infections but above all, various forms of hepatitis and hiv.

Today, preparations are used that have undergone different forms of purification stages so that almost only the desired coagulation factor is in the end product. In order to reduce the risk of transfusion infection to the highest possible degree, the plasma donors are selected and tested and the factor concentrate is treated with different combinations of wet heat, steam heat, chemical methods and other forms of high-grade purification.

Recombinant-produced factor VIII and factor IX concentrate for haemophilia A and haemophilia B, respectively, is registered and nowadays dominates factor VIII usage. The new factor concentrates have, however, potential risks. This is why it is important that treatment is monitored carefully in order to discover possible complications, above all, when it concerns the development of antibodies.

The transition from plasma-based factor VIII concentrate to recombinant preparations is taking place gradually. All children are nowadays recommended treatment with recombinant preparations. Plasma-based factor VIII preparations are still used mainly because there has been a lack of recombinant products. The effect and the safety of recombinant in respect to modern plasma-based preparations can be said to be equivalent. Recombinant-produced factor VIII concentrate leads to the development of antibodies to the same degree as plasma-based preparations. At the same time, safety has improved with the plasma-based preparations through more effective purification and virus inactivation.

All factor concentrates must be given by intravenous injection, which places special demands on psychological preparation and technique when treating the youngest children. Sometimes, it is necessary to insert central vein catheters in the form of Port-A-Cath. As every delay in giving the medicine means a risk of an increased bleeding and complications in the form of joint and muscle damage, instruction in home treatment is necessary. Experience has shown that parents can learn to give the injections even to children aged 1-2 years old. The children themselves can be trained at school age to take over the task. This presupposes help from a nurse who knows a great deal about the disorder.

## Treatment with factor concentrate

### **More severe forms of haemophilia**

- Regular prophylactic treatment with factor concentrate intravenously every other day or every third day. The treatment is begun at 1-3 years old and is in most cases lifelong.
- The haemophiliac/relative learns home treatment early.
- In the case of acute symptoms treatment is given with factor concentrate. If the patient has prophylactic treatment, an extra dosage is given in an acute bleeding. The haemophiliac/relative contacts the around the clock doctor at the haemophilia centre. If necessary, the haemophilia centre contacts doctors at the patient's local hospital.
- Indications for emergency or planned surgery and orthopaedic measures are the same as for non-haemophiliacs.
- Inhibitors to factor concentrate are treated as soon as they appear.

### **Milder forms of haemophilia**

- Mild haemophilia A is usually treated with Octostim®. Other forms, i.e. moderate haemophilia A and B as well as mild haemophilia B, with factor concentrate.

## Guidelines for treatment with factor concentrate

The units of factor concentrate that should be given are calculated with the aid of: the level that ought to be reached in plasma (kIU/L) and weight (kg)

- In mild joint or muscle bleeding the factor level is increased to 30-40% of the normal factor percentage (0.3-0.4 kIU/L)
- In larger or severe bleeding the factor level is increased to 60-80% of the normal percentage (0.6-0.8 kIU/L)
- In major surgical operations or life threatening bleeding the factor level is increased to 100% of normal factor percentage (1.0 kIU/L)

The recovery of intravenously given factor VIII is about 100%. The recovery of factor IX is, because of its larger distribution volume, only 50-60%, lower for recombinant than for plasma-based factor IX.

The half-time for factor VIII concentrate is normally 10-15 hours, somewhat shorter for children and in the case of acute, larger bleedings. The half-time for factor IX concentrate is longer, 20-30 hours. This means that in acute, larger bleedings, factor VIII concentrate is usually given three times a day (24 hrs) and factor IX concentrate twice a day (24 hrs).

**GENERAL RULE:** One unit of factor VIII concentrate per kilo of body weight increases the percentage of factor VIII in the blood by 2% (0.02 kIU/L). In an individual with a severe form of haemophilia A and 0% factor VIII in the blood, 50 units per kilo is calculated to increase the concentration to 100%. Consequently, an individual weighing 70 kilos, in the event of severe bleeding is given 3 500 units of factor VIII.

For factor IX concentrate the dosage is increased to correspond to the lower recovery. An individual weighing 70 kilos with haemophilia B, is initially given 5000 units of factor IX in the event of a severe bleeding.

## TREATMENT AT THE PATIENT'S LOCAL HOSPITAL

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Contact with the around the clock emergency service at the patient's haemophilia centre must be established immediately in the event of trauma or bleeding. Normally, the haemophiliac himself, or a relative, telephones the around the clock emergency service at the haemophilia centre, which then decides what steps should be taken. If an emergency examination is needed, the haemophilia centre then contacts the patient's local doctor. This is done to reduce any delay in the treatment. Often the patient treats himself at home with factor concentrate and then goes to his local hospital. The haemophiliac must bring his own factor concentrate with him to the hospital. Factor concentrate is also usually deposited at the patient's local hospital by the hospital pharmacies at Karolinska Hospital, Sahlgrenska University Hospital and Malmö University

Hospital so that it is readily available if the patient's own supply is finished. A store of factor concentrate is also kept at the CW Scheele pharmacy in Stockholm.

Factor concentrate is also available for individuals with milder forms of haemophilia, who do not usually have home treatment. Individuals with a mild form of haemophilia can in connection with e.g. trauma to the skull or abdomen develop a life threatening bleeding. The dosage of factor concentrate and how long the treatment is to last is decided from case to case, depending on the nature of the bleeding and the degree of severity of the haemophilia. Trauma should not be underestimated.

All surgical operations on haemophiliacs must take place in consultation with a haemophilia specialist. Larger operations must be performed at a haemophilia centre.

This is motivated partly by the need for proximity to a coagulation laboratory that can perform emergency factor analyses to guide the treatment, partly by the clinical experience of treatment and complications that is necessary, so that the risk from surgical operations will not be higher for haemophiliacs than for other patients.

Patients with haemophilia who have suffered trauma or bleeding, and are unable to inject themselves with factor concentrate, must be taken care of quickly. As long as the factor concentrate has not been given, the bleeding will continue.

## **Complications of treatment**

### **Hepatitis**

Factor concentrate made from blood plasma from many blood donors was used earlier to treat haemophilia. It meant a risk of infection with various sorts of blood borne viruses. Today, many different hepatitis viruses are known. Hepatitis B and C have been identified as the most usual causes of acute and chronic hepatitis in haemophilia. Cases of hepatitis A have previously been discovered among haemophiliacs abroad. In order to have sound knowledge of the possible risk of blood borne, infectious matter the haemophilia doctor must be very familiar with the way factor concentrate is produced and purified and must follow international development continuously. Of those haemophiliacs who previously received non-virus activated factor concentrate, at least 80% have been infected by hepatitis C and to a large extent, even by hepatitis B. Only a few of them are still carriers of the hepatitis B virus, with chronic infection. From the beginning of the 1980's, all haemophiliacs, who were not yet infected, were vaccinated against hepatitis B if they

were being treated with plasma-based concentrate. Hepatitis C means a chronic liver inflammation and thereby an increased risk of developing cirrhosis of the liver and liver cancer. Treatment with interferon in order to eliminate infection has had a lasting effect in only 10-15% of haemophiliacs. Combined treatment with interferon and ribavirin has had a lasting effect in about 40%. At present treatment is in progress with PEG-interferon and ribavirin that has had a slightly higher effect. The treatment is marred by side-effects. The most common is high temperature and muscle pain. The side-effects are often improved during treatment and often tolerated by the patient. There is no vaccine against hepatitis C. The factor concentrates used today are considered safe in regard to hepatitis C.

Outbreaks of hepatitis A have been described abroad in individuals with haemophilia who have been treated with factor concentrate that has been virus inactivated only by chemical means (so-called solvent-detergent), effective for inactivating hepatitis B and C, as well as hiv, but not for hepatitis A. Hepatitis A does not lead to chronic inflammation of the liver but can sometimes, especially if the individual has had other hepatitis infections previously, lead to a life threatening form of hepatitis. The vaccination of haemophiliacs against hepatitis A has therefore been performed at Sweden's haemophilia centres. Even if hepatitis infection via factor concentrate today is negligible, vaccination is recommended, because the risk of infection exists via foodstuffs in certain countries.

Via Sweden's three haemophilia centres hepatitis infection has been reported to the county medical officers of communicable disease control. Furthermore, the hepatitis-infected individual has been informed about current infectious disease control regulations and his obligation to inform dentists and hospital staff if there is a risk for contact with blood. The hepatitis-infected individual has also received help in notifying medical insurance.

The special needs for advice and support, which the hepatitis-infected individual requires, are met within the haemophilia centre's sphere of activity.

## **Hiv-AIDS**

When the first tests for hiv (human immunodeficiency virus) became available in 1984, an extensive examination among haemophiliacs was undertaken in Sweden. It showed that about a hundred individuals were infected with hiv. They had received non-heat-treated factor VIII concentrate made of foreign plasma and were infected mainly in 1980-82. Two thirds of these hiv-infected individuals had died by the turn of the year 2002/2003.

Since the turn of the year 1985/86 when four haemophilia B patients were infected by a Swedish factor IX concentrate, no new cases have occurred. The factor concentrates used today are deemed

to be completely safe as far as hiv is concerned. A few sexual partners contracted the infection but no other family members, however. The hiv-infected haemophiliacs were taken care of at Sweden's three haemophilia centres right from the start. Contacts were developed with infection clinics. Nowadays, all involved have, in principal, contact with infection doctors. The infection doctor regulates the medical treatment of hiv. Advanced forms of treatment mean that the illness can now be actively checked.

The hiv-positive haemophiliacs are monitored at the haemophilia centre, but check-ups are somewhat more frequent than for other haemophiliacs. No extra resources have been given to haemophilia care by reason of the hiv-catastrophe. The principle has been that those affected are to be taken care of within the existing organisation, i.e. at a haemophilia centre and at a local hospital unit.

## PSYCHOSOCIAL CONTRIBUTIONS

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Central funding has made possible the active work of counsellors, nurses and, where appropriate, psychologists. Individuals, who were infected with hiv by blood products within the medical service, have the right to receive free medical treatment, dental care, medicine and an increased allowance for close relatives since 1993.

The support activities for hiv-positive haemophiliacs have entailed conversations in groups or individually, home visits, socially supportive activities and psychosocial support. The support efforts must be continuously improved and they take place in co-operation between the medical services, those involved and their relatives. Support must be very individualised.

### **Inhibitors**

Antibodies (so-called blockers or inhibitors), which neutralise factor VIII or factor IX, develop in about a third of haemophiliacs with severe haemophilia. To a certain extent the risk can be foreseen, guided by knowing which mutation the patient has in his factor VIII/IX gene. In a small number of patients the antibodies disappear spontaneously. As a rule, the antibodies appear already within the first ten treatment sessions and most of those affected are children who begin the prophylactic treatment. When antibodies develop, treatment with factor concentrate does not give the desired effect. Bleedings occur despite regular prophylaxis and administering extra factor concentrate does not stop an acute bleeding.

Antibodies to factor concentrate in haemophilia A patients do not, in themselves, give any general symptoms. In individuals with haemophilia B, allergic reactions can occur when administering

factor IX concentrate, for example nettle rash, asthma-like problems, fall in blood pressure, kidney damage and shock. Probably, all types of concentrate, including genetically-produced ones, cause these complications.

With a high titre of antibodies, treatment with factor VIII or IX is made completely impossible. The patient has a tendency to be affected by severe, difficult to treat bleedings. With a low titre of antibodies it can be enough with an increase in the amount of factor concentrate supplied.

## **THE ELIMINATION OF ANTIBODIES**

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The final goal of the treatment of haemophiliacs with inhibitors is to induce immune tolerance as soon as possible, so that treatment with the missing factor can be effective again. Early tolerance treatment seems to give greater possibilities of achieving a successful result.

In Sweden there is a consensus that this treatment ought to be given, even if it is expensive and demanding.

The results are good in cases of inhibitors of low or moderate titres. Patients with inhibitors of very high titres are, however, difficult to treat.

The treatment principle involves administering large amounts of factor VIII or factor IX over a long period. Treatment is sometimes combined with cell-growth suppressors (cytostatics) and/or medication affecting the immune response to shorten the treatment. As those affected are often young children, a problem of with injecting in the veins may occur, which is why the implantation of a so-called Porth-A-Cath may be necessary.

## **THE TREATMENT OF BLEEDINGS**

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Before immune tolerance is induced, a readiness for how acute bleedings should be handled is needed. If the antibody titre is low, treatment with high dosage factor concentrate, at least temporarily, can cause blood coagulation. If the antibody titre is high special concentrates containing activated coagulation factors (so-called "by passing agents, e.g. Feiba, NovoSeven) can be used. The effects are often difficult to foresee. The concentrates are very expensive.

Another method is to temporarily remove the antibodies by letting the plasma pass through a filter which binds gamma globulin to it and thereby the coagulation inhibitor antibody (protein A-absorption).

This can be utilised in connection with bleedings or prior to essential surgical operations. The treatment is complicated and is not used in young children. The effect wears off from within a few days to a week.

The problem with inhibitors will continue in the foreseeable future and is perhaps the most important question to deal with. Treatment is expensive and complicated and therefore ought to be scientifically aimed, so that medical development is encouraged.

## Orthopaedic care

The most typical symptom in haemophilia is joint bleedings. They can occur both as a result of injury and spontaneously. The hip, knee and elbow joints are especially affected.

In the acute stage, a joint bleeding is very painful and causes reduced joint function with impaired mobility and strength as a result. In the long term, changes develop which can render the joint completely useless.

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Repeated joint bleedings lead to chronic damage involving cartilage, joint capsules, the surrounding skeletal structure and muscles. In haemophiliacs who, during their childhood and adolescence, have had many joint bleedings and where modern therapy and prophylaxis have been begun too late, changes already exist involving varying degrees of severity in several large joints. These haemophiliacs need orthopaedic monitoring and sometimes even orthopaedic correction. Treatment by surgical operation can be a possibility for haemophiliacs with severe joint pains, a reduction in motility or incorrect posture. Treatment is carried out by an orthopaedist with experience of haemophiliacs at a haemophilia centre.

The operations are performed during reinforced factor protection. Consideration is given to the patient's bleeding situation and the nature of the operation. The giving of extra factor takes place with successively reduced dosages during the healing process. Before an operation, a treatment plan is drawn up in consultation between the orthopaedic surgeon and the haemophilia specialist. Care takes place in departments with special experience of haemophilia. This is also true for patients with hiv infection.

## CHECK-UPS

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- **CHILDREN WITH HAEMOPHILIA** are monitored regularly by a paediatrician who examines their joints and, when necessary, consults an orthopaedist. Haemophiliac children do not as a rule have any joint problems if they have been given prophylactic treatment from an early age and have not developed any inhibitors to the coagulation factor.

- **ADULT HAEMOPHILIACS** have varying degrees of joint problems depending on the degree of severity of the haemophilia and if prophylactic treatment has been given and when it was begun. Adult haemophiliacs with chronic joint changes are therefore monitored regularly by an orthopaedist connected to the haemophilia centre. The patient's joint status is examined and registered. When necessary, a supplementary X-ray examination is performed. In moderate cases, the patient is given general, orthopaedic advice e.g. on footwear and wrist/ankle protection. Various types of motility training and treatment can be had with the help of a physiotherapist (see below).

## **TREATMENT**

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- **SURGICAL OPERATIONS** that may be warranted can be of the classical orthopaedic type, e.g. synovectomies, osteotomies, and arthrodesis. Even orthopaedic operations of the arthrosis type and joint plastic surgery operations on the elbow, shoulder, knee and hip joint are performed.
- **POST-OPERATIVE TREATMENT.** Post-operative training takes place in consultation with a physiotherapist after an individual training programme has been set up. Co-operation also takes place with the physiotherapist at the home area.

## **Physiotherapy**

Good physical capacity has great importance for the haemophiliac's general condition and well-being. Physical training strengthens the muscles and reduces the risk of recurring bleedings. Training also contributes to maintaining motility in the joints and suppleness in the muscles. After a bleeding, physiotherapy is needed to replace lost functions.

## **CHILDREN**

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Prophylactic treatment with factor concentrate has meant that children growing up nowadays can, generally speaking, live a normal life and participate in the activities at their day nursery and in school. The exception is those children who have developed antibodies to factor concentrate. The work of a children's physiotherapist is primarily preventive.

By teaching children to train strength, stability and co-ordination, damage to joints and muscles can be avoided or reduced. Young children are stimulated naturally through play. Older children can be trained more goal orientated.

## ADULTS

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Those haemophiliacs who have not had access to prophylaxis during their childhood and adolescence have problems with their joints and muscles. General deterioration, inflammation, contractures with incorrect posture, as well as new bleedings, result in pain and decreased motility. The pain triggers off a weakening of the muscles around the affected joint, which, together with reduced motility in the joints, leads to the thin, atrophic muscles which belong to the picture of severe haemophilia without adequate treatment.

### THE PHYSIOTHERAPIST

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- **GIVES INFORMATION** on how joints and muscles function. This knowledge is important because parents are the first people who must be observant if the child has a joint bleeding.
- **JUDGES MOTILITY AND MUSCLE STRENGTH.**
- **SUGGESTS SUITABLE SPARE-TIME AND SPORTS ACTIVITIES** and gives encouragement for regular, physical training.
- **TESTS AND GIVES TRAINING IN AIDS** such as joint protection, sturdy footwear, helmets (for children) and gives advice on housing adaptation (sometimes this is done by a therapist connected to the haemophilia centre).
- **IMPLEMENTS A TRAINING PROGRAMME AFTER BLEEDING.** Lost motility and muscle strength must be regained with careful movements to a pain-free extent and without provoking a new bleeding.
- **TRAINS PATIENTS POST-OPERATIVELY AFTER ORTHOPAEDIC OPERATIONS**
- **WORKS WITH METHODS THAT EASE PAIN**, e.g. TENS, pool training, massage, heat-cold, relaxation, relief and the correction of incorrect posture.
- **SUPPORTS AND INFORMS COLLEAGUES IN THE HAEMOPHILIACS' HOME AREA** on how treatment should be continued.

### Pain care

Among many adult haemophiliacs, pain is an obvious reality because of the destructive changes in overloaded joints. Children become exposed early on to needles in the form of sampling and treatment.

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Prophylaxis with factor concentrate and the training of the joints and muscles reduces the risk of bleedings and are therefore important preventive measures against pain. In the event of a bleeding, adequate treatment with factor concentrate, rest, cold etc, are measures that reduce the acute pain.

## **PAIN TREATMENT**

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- In order to be able to offer the patient the correct treatment, the **PAIN SITUATION MUST BE ANALYSED** taking into account both the disorder and the patient's life situation. The treatment can be directed towards the basic disorder (e.g. joint plastic surgery) or towards the symptoms.
- In the case of chronic pain (longer than 3-6 months), a **MULTIDISCIPLINARY METHOD OF TREATMENT** is the most fruitful. Measures to rehabilitate the patient with the efforts of counsellors, psychologists, haemophilia nurses, orthopaedists and physiotherapists play an important role in the treatment.
- **ORTHOPAEDIC MEASURES** are always considered when pain is a primary symptom of joint damage. Current treatment alternatives are cortisone injections, synovectomy (the removal of the synovial membrane in the joint), joint prosthesis etc. During the waiting period before the operation, pain must also be relieved. The overloading of other joints can cause even more pain. Insertions, orthosis, the heightening of shoe heels, etc, are possible aids for the alleviation of pain and the improvement of function.
- **THE PHYSIOTHERAPIST** can provide increased bodily awareness and knowledge about pain and through treatment, decrease pain.
- **TRANSCUTANEOUS NERVE STIMULATION (TENS)** sometimes has a good effect and has no known negative side-effects. Surgical operations with the insertion of electrodes for nerve stimulation are not used for haemophiliacs. Even blockades and other operations have a limited use because of the bleeding risk.

A long drawn out pain condition can be made permanent, even if the underlying cause has been removed operatively for instance. Sensitivity to pain often increases in long drawn out pain conditions. The interplay with the individual's mental and social situation can lead to disabling, chronic pain. In these situations special knowledge about pain and its treatment is often necessary and the work of the pain clinics can be very valuable

- The use of **ANALGESICS AND/OR INFLAMMATION ALLEVIATING MEDICINE** is often necessary. Paracetamol forms the basic treatment and can, if necessary, be combined with extropropoxifen or codeine, however, these can be addictive

In the case of changes in the joints there are often inflammations, whose pain component can be alleviated with anti-inflammatory medicine. So-called COX-inhibitors do not affect the thrombocytic function.

Certain medicines for depression and epilepsy can have an increased effect on the analgesic treatment and can be tested in lengthy pain. In the case of severe pain, morphine and similar analgesics will need to be employed. The side-effects and the addiction risk, however, require clear limitations, especially in the case of chronic pain.

## **CHILDREN AND PAIN**

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The realisation that children have the same right as adults to pain relief has taken a long time to be accepted. Even today, there is a tendency to underestimate children's needs for pain relief because their ability to express pain differs from adults'. The treatment of children requires sound knowledge of children and the methods that exist to reduce pain, as well as a psychological power of insight both from parents and staff. Before painful injections it is usual nowadays to use EMLA cream to anaesthetise the child's skin.

### **Dental care**

Regular and prophylactic dental care is important for the early detection and prevention of dental damage and disruptions to the development of the teeth and thereby, avoid future complicated operations. Surgical operations in the mouth of haemophiliacs demand experience and special routines.

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Oral surgical operations are performed at a maxilla facial surgical unit connected to a haemophilia centre. This co-operation is a prerequisite for efficient and safe treatment. Haemophiliacs should also be invited to regular check-ups of their dental status connected to a haemophilia centre.

Patients with inhibitors, hiv or hepatitis infections create special demands on dental care.

The haemophilia centre provides information for local dental care staff on what can and should be done in each individual case. Some haemophiliacs have all their dental care at the hospital where the haemophilia centre is situated.

Prophylactic treatment with factor concentrate or other blood-clotting preparations is always necessary, even when visiting the dental hygienist.

## **Gynaecology and obstetrics**

Female carriers of haemophilia with low factor values often have great problems with menorrhagia - excessive menstrual bleedings. They can also suffer severe abdominal bleedings at ovulation.

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Cyklokapron® is the first choice of medicine for these women, possibly in combination with Octostim®. If this is not effective, contraceptive pills of the combination type are prescribed.

Women with an increased tendency to bleed should be investigated before a planned pregnancy when prenatal diagnostics with chorion biopsy or an amniotic fluid test can be necessary

The pregnancy, birth and its aftermath must be taken care of in collaboration with a haemophilia centre. An individual plan of action must be drawn up which includes treatment with

Cyklokapron®, Octostim® and factor VIII/IX, according to the diagnosis and degree of severity.

Epidural anaesthesia should not be given if the factor levels are decreased.

In the case of a suspected or confirmed bleeding disorder in the foetus, the birth is handled according to ordinary procedures. Delivery using instruments should be avoided.

## **Support and self-care**

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Haemophiliacs require an attitude founded on a long ranging, overall view. This means that support and rehabilitation must take place in co-operation with a variety of professions and sections of the care chain, in order to provide the haemophiliac with the best possible capacity to function normally.

Regular training and information efforts are necessary so that the haemophiliac will be able to take care of himself.

### **PSYCHOSOCIAL SUPPORT**

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The haemophiliac's need for psychological support can arise in serious situations, in complications of the disorder or in the battle with pain. Life crises in connection with ageing,

divorce, death and unemployment can be extra pressing when a person is living with a chronic disorder. It is well known that psychological stress can increase the tendency to bleed. Psychological support and the taking care of families with children should be provided when the diagnosis is new, in situations of acute illness or during episodes of pressure in the family life. Parents need to discuss and receive support in their role as parents. Haemophiliac adolescents can require help sometimes to tackle serious thoughts about their future and building a family. Even the relatives need support sometimes, to enable them to be a positive support for the haemophiliac. Not least, those haemophiliacs who have been affected by antibodies, hiv or hepatitis, are in need of qualified psychological support.

## **COUNSELLING**

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The connection between social situation and ill health has been confirmed. The counsellor at the haemophilia centre is part of the team surrounding the haemophiliac and his family and fulfils the practical, psychological and social needs that can arise as a result of the illness.

The counsellor has a co-ordinating function between the haemophilia centre, other caregivers and the authorities. Through supportive talks and therapeutic conversations, for shorter or longer periods, the counsellor helps the haemophiliac to cope with a difficult situation. The counsellor also participates in the planning of rehabilitation and provides support for the haemophiliac's relatives. At the haemophilia centre, the counsellor can take part, by way of conversations, individually, or in groups, in supporting the work of other members of staff.

For families with a haemophiliac child, early and regular contact with a counsellor during the first year, as well as in acute or pressing situations, is needed. Haemophiliacs with inhibitors and their relatives have a special need for counselling help. It is also important to pay attention to the older, quieter haemophiliacs' situation. Patient questionnaires show that many live with disabilities and under the influence of pain, a number with a meagre economy because of a disability pension. Many haemophiliacs have one or several more severe diagnoses, such as hiv or hepatitis, on top of their bleeding disorder. The possibility to receive counselling support must even be made available in the local, home area.

## **FINANCIAL SUPPORT**

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Some of the most usual forms of financial support available for haemophiliacs are dealt with here. Within the social insurance system there are even more forms of support forms that may be

appropriate for haemophiliacs. For further information, see Facts and Advice no. 13 - "Community support for haemophiliacs".

- **CARE ALLOWANCE.** Children with a severe/moderate form of haemophilia need increased care and supervision, which can involve extra costs. As considerable improvements have been made within haemophilia care, care allowance will probably decrease in due course. However, haemophiliacs with inhibitors may be in need of maximum care allowance for a long time.
- **OTHER ALLOWANCES.** Haemophiliacs with pronounced disabilities have the same need as others with movement disabilities. One allowance, which may be necessary for adults, is a car allowance and handicap benefit. Assessment is made on an individual basis. A medical certificate should be provided by doctors at the haemophilia reception. The home must sometimes be adapted.
- **DAY NURSERY AND SCHOOL.** During the first years at day nursery, children with haemophilia need extra support and supervision. Extra staff resources for the nursery group can meet this need. For children with inhibitors, this need may persist even during the first years at school and these patients may require a more personal assistant.

## **TRAINING IN SELF-CARE**

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The responsibility for caring for the individual's illness and looking after the individual's health and welfare, rests to a large part on the haemophiliacs themselves and their relatives.

The following, among other things, lie within this sphere of responsibility:

- To regularly monitor their disorder at the haemophilia centre
- To be well-informed
- To prevent bleedings by, for example, home care, physical training, protection, choice of profession and lifestyle
- To treat bleedings when necessary
- To keep in contact with the haemophilia centre and the local care unit
- To see to it that there are extra supplies of factor concentrate at home

Haemophiliacs must therefore be very familiar with many aspects of their illness. In order to ensure this, the haemophilia centre and the patient organisation must provide regular information and training. It is therefore important that the haemophiliacs and their relatives are provided with the opportunity to participate in courses and conferences aimed at rehabilitation and training and education about the illness and self-care.

Topics in the self-care training programme for haemophiliacs are among other things:

- **PROPHYLACTIC CARE AT HOME.** An important part of the responsibility for everyday treatment has been transferred to the haemophiliac. Home treatment requires that the haemophiliac follows the medical prescription, has mastered the injection technique, keeps a record of the treatment in the treatment record sheet and reports deviations from the agreed treatment. Parents must be informed about how to be able to support their child when the treatment is introduced.

As soon as possible, the child should be allowed to take part in the treatment and gradually learn how to take responsibility himself. At 9-12-years old most children are sufficiently interested and mature to learn the intravenous treatment technique. Patients and parents should know how the factor level gradually decreases after the dose is given and when there is a need for extra factor concentrate. A sufficient supply of factor concentrate must be kept at home.

- **STEPS TO TAKE IN SEVERE BLEEDINGS.** All haemophiliacs must be so well informed that they can decide when to contact a haemophilia centre in acute bleedings and trauma.

- **KNOWLEDGE OF JOINTS AND THE NEED FOR PHYSICAL TRAINING.** In order to detect and treat early bleedings, the patient and the parents should have sound knowledge of joint and muscle function, be able to recognise early signals of joint or muscle bleedings, be able to maintain good muscle strength through adequate training and be aware of which activities mean too great a risk for trauma.

- **PROTECTION.** Parents must see to it that the child with haemophilia does not risk bleedings unnecessarily. Protection, in addition to helmets in the toddler stage, should be discussed with the staff concerned at the haemophilia centre the patient belongs to.

- **STRESS COPING.** The tendency to bleed can possibly increase with stress. Therefore, psychological well-being is important. Planning, self-knowledge and knowledge of different relaxation methods can generally be important instruments in coping with stressful situations.

- **ALCOHOL.** Many haemophiliacs have got a chronic infection/inflammation from hepatitis C in the liver, via blood products. This means an increased sensitivity in the liver towards, among other things, alcohol, which should therefore be consumed moderately. Even the combination of alcohol and the pain relievers, that many haemophiliacs must use, should be avoided.

- **INTRAMUSCULAR INJECTIONS** must not be given to haemophiliacs because of the great risk for ecchymosis in the muscle tissue. Haemophiliacs must be aware of this risk in their contacts with the health-care services. They should also always inform the health services about their illness in order to avoid risky and unnecessary medical procedures.

- **INCREASED BLOOD PRESSURE,** when not treated, can cause brain haemorrhage. It is especially important that haemophiliacs follow the medical prescription about blood pressure treatment in order to decrease this risk.

- Individuals with hiv or hepatitis must be aware of, among other things, **THE INFECTIOUS DISEASE CONTROL REGULATIONS**, the using of protection in sexual intercourse, their duty to inform hospital staff and dentists about the infection risk in treatment, the packaging of blood-soiled material, the changing of wound dressings, the risk of lending toilet articles, the piercing of ears and tatooining, etc.
- **WHEN TRAVELLING** haemophiliacs should take factor concentrate with them in sufficient amounts to be able to begin treatment in a severe bleeding, as well as other blood-clotting preparations and pain-relieving medicine. When travelling abroad the patient is advised to get hold of, via the haemophilia centre, addresses to hospitals where haemophiliacs are treated. The patient must have a certificate verifying the possession of medicine and injection needles, from the haemophilia centre to show to passport control authorities, Injection needles must, however, not be packed in hand luggage when travelling by plane.
- **A BLEEDING RISK CARD** is provided by the haemophilia centre at diagnosis. It must be renewed regularly. The card contains information on the type of haemophilia, recommended treatment and information on telephone numbers to the haemophilia centre. It must also be shown when visiting the doctor or dentist.
- **INFORMATION** to those around them is a responsibility, which, to a great extent, rests on the haemophiliacs themselves. Parents of children with haemophilia have a responsibility to inform the nursery school and school about the special conditions, which apply to the child.

## Glossary

**AIDS:** Acquired immune deficiency syndrome; actually the final stage of hiv infection

**ARTHRODESIS:** Arthroplasty

**ARTHROSCOPY:** Minimally-invasive examination of the interior of a joint with an arthroscope (keyhole surgery)

**AUTOSOMAL:** Inherited with the bodily chromosone, affects both sexes similarly

**COMPREHENSIVE CARE:** All care that can be considered to be part of the basic disorder, undertaken at an expert centre

**DESMOPRESSIN:** A synthetically-produced, hormone-like medicine with several blood-clotting effects. Marketed as Octostim® in the form of a nasal spray and injection

**DIRECT AND INDIRECT GENE DIAGNOSTICS:** A method of analysis either to indicate the flaw on the exact spot in the gene, or indirectly through nearby markers

**DISTRIBUTION VOLUME:** The volume in the body in which a given amount of factor concentrate is distributed

**EMLA-CREAM:** A local anaesthetic in cream form

**EPIDURAL ANESTHESIA:** Spinal anaesthesia

**FACTOR:** Protein substance which helps in blood coagulation

**FACTOR CONCENTRATE:** Purified coagulation factor in a concentrated form for intravenous injection

**PHARMACOKINETIC:** The study of what happens with a medicine in the body

**FIBRIN:** An insoluble protein formed during coagulation

**HAEMATOME:** A solid swelling of clotted blood in the tissues

**HAEMOPHILIA:** A bleeding disorder

**HAEMOSTASIS:** The stilling of blood flow

**HEPATITIS:** Inflammation of the liver

**HIV:** Human immunodeficiency virus

**INHIBITOR:** Antibodies which neutralise the effect of coagulation factor

**IMMUNOLOGY:** The scientific study of immunity

**INDUCE:** Develop, give rise to

**IMMUNE TOLERANCE:** The switching off of antibody production to respective factor

**INTRAVENOUS:** Within, or into a vein

**INTRA-UTERINE:** Within the uterus

**COAGULATION:** A chain reaction where soluble protein changes into insoluble fibre

**COMPLEMENT ACTIVATED:** Complement, a factor in the blood which, if activated, can lead to an allergic reaction, with nettle rash, breathing difficulties and fall in blood pressure

**CONTRACTURE:** The permanent locking of a joint, usually in a dysfunctional position

**CHORION BIOPSY:** Tissue sample from the part of the placenta made up of foetal cells

**JOINT PLASTIC:** Prosthesis operation i.e. reconstructive joint surgery

**MOLECULAR BIOLOGY:** The study of the structure of large molecules associated with living organisms

**MENORRHAGIA:** Abnormally heavy bleeding at menstruation

**MUTATION:** A genetic change leading to illness

**OBSTETRICS:** The branch of medicine and surgery concerned with childbirth

**OSTEOMY:** The surgical cutting or removal of bone

**PORT-A-CATH:** Small implant under the skin facilitating the injection of medicine or the taking of blood samples

**PARACETAMOL:** Pain-relieving substance contained in Alvedon® and Panodil®

**PRENATAL DIAGNOSTICS:** Prenatal diagnostics

**PROPHYLACTIC:** Preventive

**RECESSIVE:** In genetics, incapable of expression unless the responsible allele is carried by both members of a pair of homologous chromosomes

**RECOMBINANT:** Genetically produced

**SOLVENT-DETERGENT:** Substance used to kill hiv and hepatitis in plasma products

**SYNOVECTOMY:** The excision of pathologically altered joint tissue

**TITRE:** Concentration, level

**TRAUMA:** A physical wound or injury

**EXCHANGE:** The percentage of the amount of factor concentrate given that remains in the blood

**WHF:** World Federation of Hemophilia

**WHO:** World Health Organisation

## Further information

### INFORMATION MATERIAL

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**Facts and Advice** from The Information Centre for Haemophiliacs is a series of four page information sheets about haemophilia that has been compiled by haemophilia care services and the Swedish Haemophilia Society. The information sheets provide condensed knowledge about the disorder and are aimed at haemophiliacs themselves, their work colleagues, the authorities, nursing schools and nursing staff.

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## **THE SWEDISH HAEMOPHILIA SOCIETY**

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The Swedish Haemophilia Society is a national interest organisation. The Society organises support for individuals with haemophilia, von Willebrand disease and other bleeding disorders. Health-care questions are a priority in the political sphere of the work done within the society. In co-operation with Sweden's haemophilia health care, the Society works for the improved care and treatment of haemophiliacs. The Society also promotes medical research through the recently formed Arosenius Fund.

The Society is a member of the World Federation of Haemophilia and enjoys good co-operation with sister organisations in Europe and the Nordic countries.

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