



B2B

Speaking From Experience

Hemophilia B in Early Childhood



F O R E W O R D

The B2B (hemophilia B patient to hemophilia B patient) series began in 2005 as a vehicle to empower individuals with hemophilia B through peer support and education. The objective of the program remains to address the various challenges of having hemophilia B, especially those encountered when transitioning from one life stage to another. Another goal of the B2B program is to help strengthen the internal support system and educational network within the hemophilia B community. The B2B program is a sharing of firsthand accounts about everyday life from those living with hemophilia B.

The three previous B2B books, *Young Adults and Hemophilia B*, *Learn From Experience: A Guide for Mature Adults*, and *Navigating the Preteen Years*, presented peer-to-peer life experiences from young adults and mature adults with hemophilia B. In the third book, families of preteens provided a heads up about the future when raising preteens with hemophilia B. The fourth B2B book in this series, *Hemophilia B in Early Childhood*, offers tips from parents who have raised infants, toddlers, and preschoolers with hemophilia B, as well as insight from medical professionals who treat children with hemophilia B.

On behalf of Pfizer Inc, The Coalition for Hemophilia B, Inc., and the B2B Advisory Board, we would like to extend our gratitude to the members of the hemophilia B community who contributed to this book. Your time, knowledge, and personal stories about life with hemophilia B are greatly appreciated.

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We would also like to thank the professionals involved with hemophilia care who shared their insights about hemophilia B.

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The narratives and statements from health care professionals in this book were provided prior to its initial publication in 2011.

The views and opinions expressed in this book are those of patients, parents, and hemophilia care specialists who are members of an advisory board within the hemophilia B community and not of Pfizer.

The information in this book should in no way replace the advice of your health care professional. Be sure to talk with your physician, nurse, or hemophilia treatment center (HTC) staff regarding any form of medical advice or treatment.

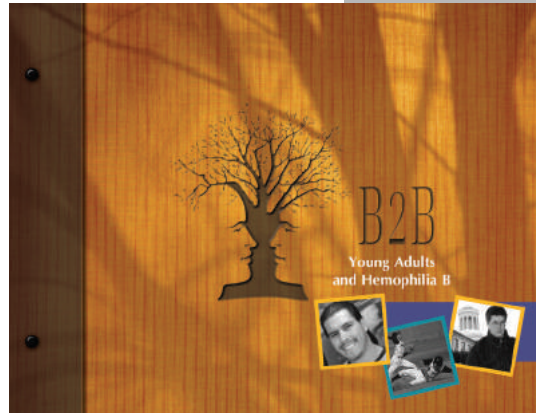


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I N T R O D U C T I O N

The early childhood years, from birth through age 6, often encompass a tremendous amount of developmental change for a child, beginning from day 1 of life, when an infant's body movements are simple reflex actions. From that day forward, it may seem that new physical and social milestones are reached daily as continuous muscle development takes place and alertness and curiosity increase.

The first 12 months of life can be an exciting time for parents/caregivers as they watch their infant change faster and work harder than imaginable. Parents/caregivers often begin to notice better and better muscle control at about 6 months of age when an infant may start to move around on his or her own. Typically, by the end of the first year, the infant may stand without assistance and begin to become a sociable person, distinguishing familiar people from strangers. For the next few years, the toddler years, stages of development seem to come at a very fast pace. For the parents/caregivers of a young child with hemophilia B, these stages of increased mobility and exploration can possibly result, either directly or indirectly, in a bruising or bleeding episode.

Dealing with a child's hemophilia B can be challenging for any parent/caregiver. As children become more and more curious about the world around them, it will become more and more important that they are not kept from learning about their environment in an effort to protect them from bruising and bleeding.

Parents/caregivers may find it tricky to manage the child's needs with those of the other family members. Because parents are the most critical influence in a child's life, it's important that they be aware of how hemophilia B may affect them and other family members emotionally and how they can work to keep it from interfering with parenting/caregiving.

Fortunately, guidance and support are available to families of a child with hemophilia B through medical professionals, hemophilia treatment centers (HTCs), and other families who have experienced similar situations. With their help, parents/caregivers can learn how to cope with their child's hemophilia B, how to meet their own needs and the needs of other family members, how to organize their time, and how to forgive themselves for not being perfect.

Hopefully, you will find this book and the listed resources to be valuable support for you and your family now and in the future, as you raise your child with hemophilia B.

The objectives of this book are to:

- Provide an overview of hemophilia B that includes treatment issues and parenting/caregiving situations that may arise during early childhood years
- Offer recommendations from hemophilia B community members and medical experts for meeting the challenges of everyday living for your family and your child with hemophilia B
- Suggest resources for parents/caregivers of infants and toddlers with hemophilia B to help manage specific psychosocial and/or treatment issues that may arise during this time

WHAT IS HEMOPHILIA?



Hemophilia is a congenital bleeding disorder. About 20,000 people in the United States have hemophilia, and each year another 400 babies are born with the disorder. Hemophilia usually occurs only in males; however, there are exceptions.^{1,2}

The term bleeding disorder refers to a wide range of medical conditions that lead to poor blood clotting and continuous bleeding. You may hear them referred to as coagulopathies or clotting disorders. A person with a bleeding disorder tends to bleed for a longer period of time following an injury to a blood vessel than someone who does not have a bleeding disorder.²

People born with hemophilia have little or none of a protein needed for normal blood clotting. The missing protein is called a clotting factor, which works together with other proteins and platelets to help blood clot. Platelets are small blood cells that are formed in the bone marrow. When blood vessels are injured, clotting factors help the platelets stick together to plug cuts and breaks at the site of the injury to stop the bleeding. Without clotting factors, normal blood clotting cannot take place.²

There are two main varieties of hemophilia:

- Hemophilia A—the most common type of hemophilia
 - The body has little or no clotting factor VIII²
 - About 8 out of 10 people with hemophilia have hemophilia A²
- Hemophilia B—the second most common type of hemophilia, is also known as factor IX deficiency, or Christmas disease
 - The body has little or no clotting factor IX²
 - Hemophilia B occurs in about 1 in 25,000 male births³

Hemophilia can range from mild to severe.⁴

- People with mild hemophilia (5% to 40% factor level) usually have problems with bleeding only after serious injury, trauma, or surgery. In many cases, mild hemophilia is not discovered until an injury or surgery or tooth extraction results in unusual bleeding. The first episode may not occur until adulthood⁵
- People with moderate hemophilia (1% to 5% factor level) tend to have bleeding episodes after injuries. They may also experience occasional bleeding episodes without obvious cause. These are called “spontaneous bleeding episodes”⁵
- People with severe hemophilia (less than 1% factor level) have bleeding following an injury and may have frequent spontaneous bleeding episodes, often into the joints and muscles⁵

There are different levels of hemophilia; each is based on the amount of clotting factor in the blood.

Prenatal/Neonatal Considerations in a Family With a History of Hemophilia

When there is a family history of hemophilia, pregnancy should be managed by an obstetric unit having experience with hemophilia and access to both laboratory monitoring and appropriate factor replacement therapy. If possible, delivery should take place in an obstetric unit associated with an HTC.⁶

A key aspect in the delivery of a child with known or suspected hemophilia is avoiding the use of instrumentation, which increases the risk for intracranial hemorrhage (ICH). Hemophilia carrier status itself is not a contraindication to vaginal delivery, but elective Caesarean section may be considered in an attempt to reduce the risk for an ICH in the baby.⁶

Many people who have or have had family members with hemophilia will ask that their baby be tested for the disorder soon after birth. Ideally, this testing should be planned before the baby’s delivery so that a sample of blood can be drawn from the umbilical cord (which connects the mother and baby before birth) immediately after delivery and tested to determine clotting factor levels. Testing umbilical cord blood avoids potential trauma to the baby, but care should be taken to avoid contamination of the sample with maternal blood.^{6,7}

It is worth noting that umbilical cord blood testing is better at finding low levels of factor VIII (the clotting factor that is defective in patients with hemophilia A) than it is at finding low levels of factor IX. This is because factor IX levels take more time to develop in newborns, and do not reach normal levels until a baby is at least 6 months of age. Therefore, a mildly low level of factor IX at birth does not necessarily mean that the baby has hemophilia B. A repeat test when the baby is older might be needed in some cases.⁷ Be sure to talk to your hematologist if you are a carrier for hemophilia B and are pregnant or considering becoming pregnant.

In approximately one-third of patients, there is no family history of hemophilia and the occurrence of hemophilia represents a new genetic event or mutation.⁸ When hemophilia is suspected on the basis of either clinical findings or a positive family history, diagnostic studies may be done to confirm the diagnosis.⁹

If your pediatrician or family physician suspects that your child may have hemophilia, he or she may order tests.

These may include⁷:

- Complete Blood Count (CBC): This common test measures the amount of hemoglobin (the red pigment inside red blood cells that carries oxygen), the size and number of red blood cells, and the number of different types of white blood cells and platelets in the blood. The CBC is normal in people with hemophilia, but the amount of hemoglobin and the number of red blood cells may be low in a hemophilia patient experiencing a severe bleeding episode
- Activated Partial Thromboplastin Time (APTT): This test determines how long it takes for blood to clot. It measures the clotting ability of factors VIII, IX, XI, and XII. If the levels of any of these clotting factors are too low, it takes longer than normal for the blood to clot. The results of this test will show a longer clotting time among people with hemophilia A or B
- Prothrombin Time (PT): This test also measures the time it takes for blood to clot. It measures primarily the clotting ability of factors I, II, V, VII, and X. The results of this test will be normal among most people with hemophilia A and B
- A clotting factor XIII or IX assay can reveal the type of hemophilia and its severity (Table 1)

Table 1. Levels of Factor IX in the Blood of Normal People and People with Hemophilia of Different Severities⁷

Severity	Level of factor IX in the blood
Normal (person who does not have hemophilia)	50% to 100%
Mild hemophilia	5% to 40%
Moderate hemophilia	1% to 5%
Severe hemophilia	Less than 1%

Severe hemophilia causes severe bleeding throughout life, usually beginning soon after birth. In many babies, hemophilia is suspected with the appearance of a scalp hematoma (blood that has collected outside of a blood vessel) after delivery or when a routine circumcision (removal of the foreskin of the penis) results in excessive bleeding.¹⁰ Toddlers are at particular risk because they fall frequently and may bleed into the soft tissue of their arms and legs. These small bleeds result in bruising and noticeable lumps, but do not usually require treatment. As a child becomes more active, bleeding may occur into the muscles, a much more painful and debilitating situation.⁴

The age when hemophilia B is first diagnosed in a child who does not have a family history of the disorder and the frequency of bleeding episodes the child experiences are generally related to the factor IX clotting activity. In any affected person, bleeding episodes may be more frequent in childhood and adolescence than in adulthood.⁵

There are several important considerations when caring for a person who has hemophilia. Prevention of bleeding episodes should be a primary goal. The second goal involves treating bleeding episodes early and aggressively. Additionally, supportive and adjunctive measures for each bleeding episode in the context of a multidisciplinary team approach should be used.¹¹

Standard treatment for a bleeding episode is infusion of a factor IX concentrate to replace the defective clotting factor. The amount infused depends on the severity of bleeding, the site of the bleeding, and the weight and height of the patient.¹²

Following an infusion, the health care professional (HCP) may perform a blood test to measure the level of circulating factor IX activity in a patient's blood; this is called recovery. Because recovery varies for each patient,¹³ knowing this important recovery value helps the HCP figure out the proper dose of factor needed.

What Are the Symptoms of Hemophilia B?

An accurate diagnosis of hemophilia is the first essential step to hemophilia care, so it is important to assess any symptom that a child is experiencing.

Bleeding is the most common symptom of hemophilia, especially into the joints and muscles. For a child with hemophilia, small cuts or surface bruises are usually not a problem, but deeper injuries may result in bleeding episodes that could cause serious problems and lead to permanent disability unless treated promptly.⁸

MASAC Recommendations¹⁴

In 1954, the National Hemophilia Foundation (NHF) formed a medical advisory council comprising scientists, physicians, and other treatment professionals who are regarded as experts in the field of bleeding disorders to advance clinical care and promote hemophilia research. This group is now known as the Medical and Scientific Advisory Council (MASAC), and it establishes the quality-of-care guidelines for the treatment of hemophilia. The recommendations issued by MASAC are guidelines that are intended to set the standard of care and are referred to by physicians, medical schools, pharmacists, emergency room personnel, insurance companies, and others.

The symptoms of hemophilia bleeding depend on where the bleeding occurs. Young infants may have bleeding from their mouth when they are cutting teeth or if they bite their tongue or tear tissue in their mouth.⁸

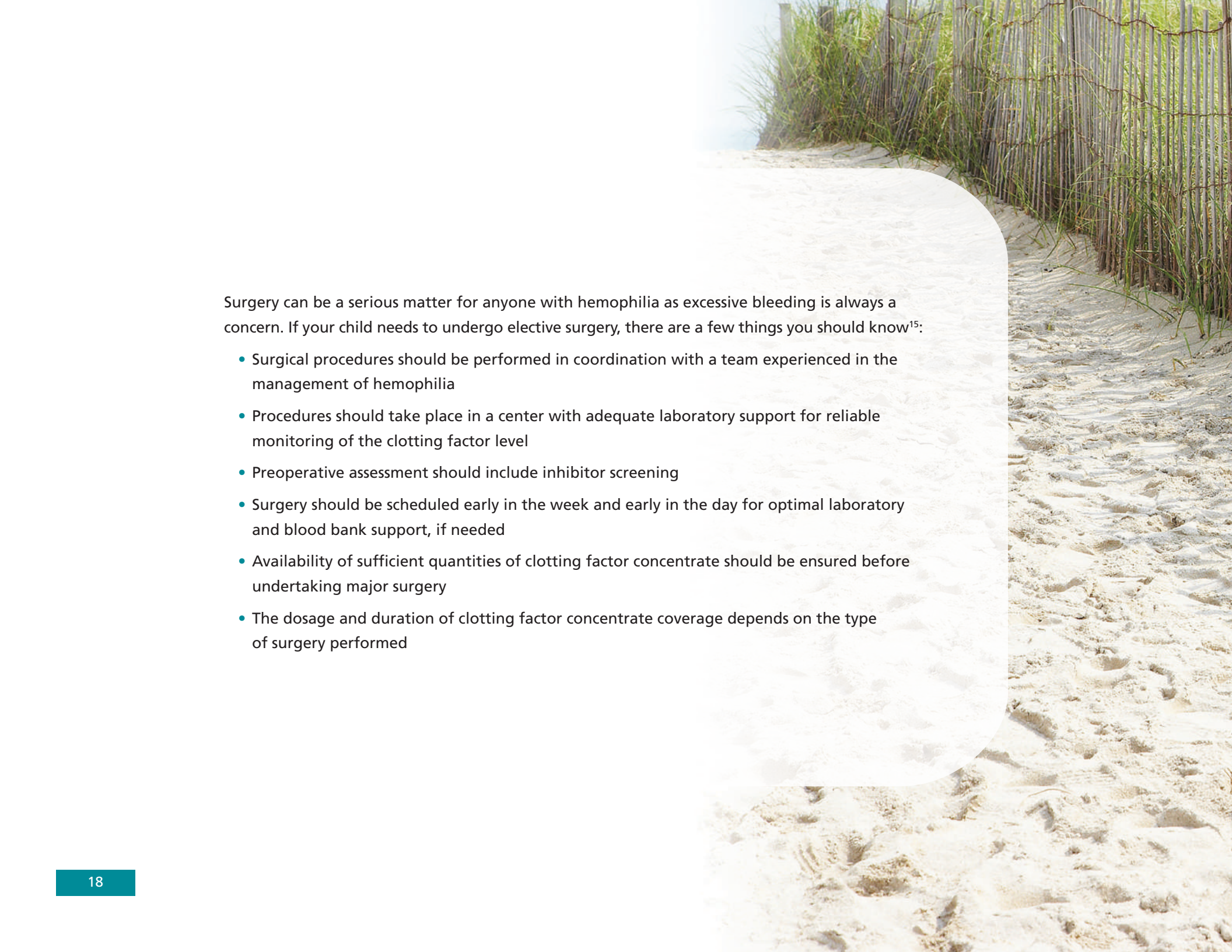
Toddlers and older children commonly have bleeding into their muscles and joints. The symptoms of these types of bleeds include⁸:

- Pain
- Swelling
- Loss of range of motion
- Inability to move or use the affected arm or leg

In joint bleeds there is usually no bruising or discoloration of the skin to indicate that the swelling and pain are due to bleeding.⁸

Bleeding into joints is considered one of the most common and serious complications of hemophilia B, and it is very important to treat these events promptly in your child. The earliest clinical signs of a joint bleed are increased warmth over the area and discomfort with movement, particularly at the end of range of motion. Symptoms that occur later often include pain at rest, swelling, tenderness, and extreme loss of motion.¹⁵

When there is bleeding into a joint, the blood is gradually resorbed over 3 to 4 weeks, and significant damage is unlikely. However, if such bleeding occurs repeatedly, materials contained in red blood cells (iron and other substances) begin to accumulate in the joint. These materials promote long-lasting inflammation and changes in the structure of the joint, such as loss of cartilage. Repeated bleeding into a joint may also decrease the activity of cells that form new bone, a process that normally occurs throughout life and keeps joints healthy.¹⁶

A photograph of a sandy path leading to a dune. The path is made of light-colored sand and has several footprints. To the right of the path, there is a metal fence made of vertical poles and horizontal wires. Behind the fence, there are tall, green grasses. The sky is visible in the background, appearing clear and blue.

Surgery can be a serious matter for anyone with hemophilia as excessive bleeding is always a concern. If your child needs to undergo elective surgery, there are a few things you should know¹⁵:

- Surgical procedures should be performed in coordination with a team experienced in the management of hemophilia
- Procedures should take place in a center with adequate laboratory support for reliable monitoring of the clotting factor level
- Preoperative assessment should include inhibitor screening
- Surgery should be scheduled early in the week and early in the day for optimal laboratory and blood bank support, if needed
- Availability of sufficient quantities of clotting factor concentrate should be ensured before undertaking major surgery
- The dosage and duration of clotting factor concentrate coverage depends on the type of surgery performed

Inhibitors—A Special Problem in Hemophilia

It is possible that after a few treatments, the factor IX concentrate used to control bleeding in your child may no longer be effective. This occurs in about 3% of all people with hemophilia B and is due to the development of molecules called inhibitors. In a small number of people with hemophilia B, the immune system mistakenly identifies replacement factor IX as a molecule against which it should make antibodies. In some instances these antibodies block the activity of factor IX concentrate, and it loses its effectiveness for stopping bleeds.^{17,18}

HOW DID MY CHILD GET HEMOPHILIA B?



It is often difficult to understand how your child can have hemophilia B when you, the parent, do not have hemophilia B. However, you may have been told that you are the carrier of the disorder.

Hemophilia B is a sex-linked disease passed on from a female to her male offspring. As previously discussed, hemophilia B is caused by a deficiency in factor IX, resulting from a genetic defect in the factor IX gene, which is carried on the X chromosome.

Each person has two chromosomes that determine his or her sex: a female has two X chromosomes and a male has an X and a Y chromosome. A female child always receives two X chromosomes; if she receives one abnormal X chromosome (that is to say, an X chromosome that has an abnormal factor IX gene), in most cases she will still be capable of producing a sufficient quantity of factor IX. A person who has one abnormal chromosome but does not actually suffer from the disorder is called a carrier.¹⁹

If, however, she has a son who receives her abnormal X chromosome, he will be unable to produce the right quantity of factor IX, and he will suffer some degree of hemophilia.¹⁹ In rare cases, a girl may be born with very low clotting factor and, like boys with hemophilia, have a greater risk for bleeding.¹⁹ Please see Figures 1 and 2 on page 22.

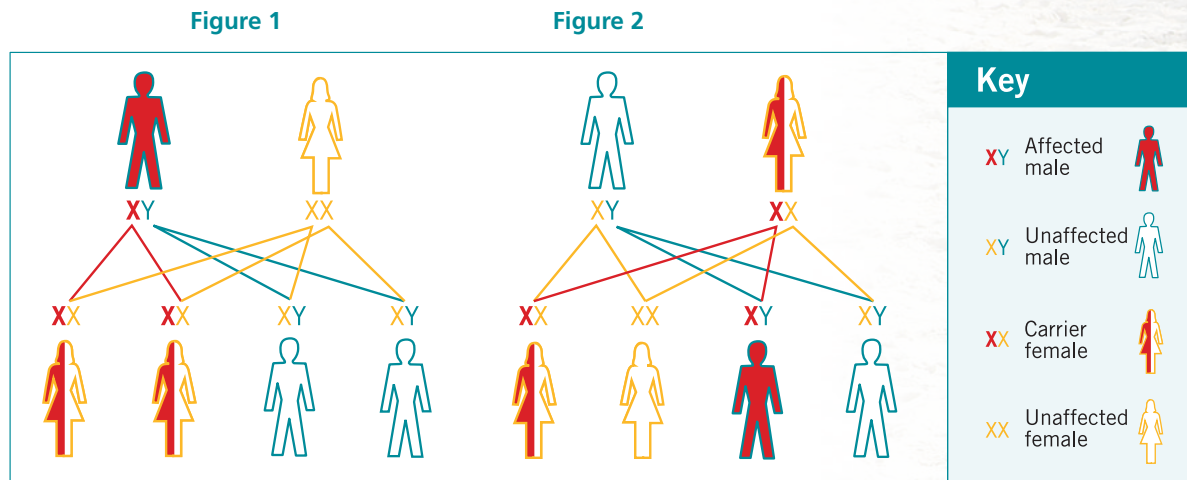


Figure 1 A father with hemophilia will pass the defective gene to his daughters, making them carriers. His sons, however, will not be affected because they get their X chromosome from their unaffected mother.¹⁹

Figure 2 In a mother who is a carrier, there is a 50% chance her daughter will be a carrier also. If she has a son, there is a 50% chance he will have hemophilia.¹⁹

About one-third of all people with hemophilia B are the first member of their family in whom the disorder has been detected, resulting from a spontaneous change in the factor IX gene that occurs for unknown reasons.²⁰ Once such a spontaneous change takes place, children of the affected person can inherit the abnormal gene.



THE IMPORTANCE OF COMPREHENSIVE CARE



What is Comprehensive Care?

Comprehensive hemophilia care is a multidisciplinary team approach to treat the whole person, and the family, through continuous supervision of the medical and psychosocial aspects of the disorder. This type of care addresses physical, emotional, educational, and vocational needs.²¹

In 1973, the NHF launched a campaign to establish the creation of a nationwide network of hemophilia diagnostic and treatment centers. The aim was for these centers to provide comprehensive services for patients and families within one treatment facility.²²

To sufficiently offer comprehensive care, typical resources should include²³:

- Diagnostic services
- Established treatment protocols
- Direct psychosocial and educational services
- Consult for surgery support and blood-borne diseases
- Genetic counseling
- Research programs

The World Federation of Hemophilia (WFH) defines the functions of a comprehensive care program as one that¹⁵:

- Carries out all tests necessary for the definitive diagnosis of hemophilia and other inherited bleeding disorders
- Educates patients and parents regarding safety precautions for the prevention and early identification of bleeds
- Manages bleeding episodes with appropriate treatment products and first aid
- Promotes regular exercise to maintain muscle and joint health and provides rehabilitative services for restoring function following bleeds
- Develops and reviews a management plan for each patient
- Monitors and manages the complications of hemophilia and its treatment, such as arthropathies, inhibitors, and transfusion-transmitted infections

- Provides genetic counseling and genetic diagnostic services for patients and family members
- Educates, advises, and counsels patients, family members, health care workers, educators, and employers to ensure that the patients' needs are met
- Conducts research to further knowledge and improve the management of bleeding disorders, often in collaboration with national and international hemophilia research centers

Comprehensive care has resulted in significant improvement in the health of persons with hemophilia, as well as reducing the amount of health care utilization. A Centers for Disease Control and Prevention (CDC) study of approximately 3,000 people with hemophilia A and hemophilia B showed that those who used an HTC were 40% less likely to die of hemophilia-related complications compared to those who did not receive care at an HTC.²⁴

The focus of the comprehensive care team at the HTC is the prevention of severe and costly medical complications, such as progressive joint disease. The members of the team are committed to assisting patients and families with diagnosis and assessment. They also help with education, management of acute bleeding episodes, initiating and providing home infusion therapy, routine follow-up, and preoperative and postoperative management when surgery becomes necessary.

Finding an HTC

A network of approximately 141 federally funded HTCs²² across the country offers excellent treatment, education, and support services to individuals with hemophilia and their families.

The CDC supports and funds the national network of HTCs, and provides an interactive directory that can be found at the following site:

Hemophilia Treatment Center (HTC) Directory²⁵

www2a.cdc.gov/ncbddd/htcweb/dir_report/dir_search.asp

Finding an HTC

To take advantage of the services at an HTC, register at one near you. To find an HTC near you, contact: handi@hemophilia.org

The Hemophilia Comprehensive Care Team

Team members may include¹⁵:

- Pediatric/adult hematologists (have expert knowledge about hemophilia and other bleeding disorders)
- Nurse coordinators (play a key role and serve as a link between the family and the HTC comprehensive team members)
- Social workers (provide support to patients and families, and assist in identifying barriers to care and strategies to improve access to care)
- Physiatrists (physical therapist specialists who analyze the impact of the disease on body functions and structures and assess the functional abilities of the patient in activity, exercise, and rehabilitation)

- Dentists (work closely with the HTC team by providing routine checkups and oral hygiene)
- Orthopedists (specialize in managing joint disease resulting from repeated bleeding episodes)
- Occupational therapists (assist in maintaining activities of daily living)
- Laboratory services (perform essential blood and other laboratory tests to determine the type and severity of the bleeding disorder, measure factor levels, and check for the presence of inhibitors)
- Genetic counselors (provide education and information regarding the inheritance pattern of the disorder)

The staff at the HTC needs your input to develop a plan of care that will help keep your child healthy, active, and able to live successfully with the challenge of hemophilia²²

Many families utilize the resources provided by HTCs because the staff understands their unique needs and takes the time to develop treatment plans. State-of-the-art medical care is provided and many benefit from the skills and experiences provided by the team.





Do the 5!

- “Do the 5!”—the theme of the National Prevention Program (NPP)—is a simple, helpful way to remember some of the most important things you can do to take care of your child²⁶
- “Do the 5” is a collaborative effort by the NHF, CDC, NPP, and members of the bleeding disorders community²⁷

What Are the “5”?²⁶

1. Get your child an annual, comprehensive checkup at a treatment center
2. Get your child vaccinated (hepatitis A and hepatitis B are preventable through vaccination)
3. Treat bleeds early and adequately
4. Have your child exercise to maintain a healthy weight and protect the joints (speak with your HCP about the type of exercise program that may be right for your child)
5. Get your child tested regularly for blood-borne infections

MANAGING YOUR CHILD'S HEMOPHILIA B
DURING EARLY CHILDHOOD



Infancy is a spectacular time of life. The infant has so much to learn: to reach, to grasp, to recognize, to smile, to laugh, to roll over, to sit, and to maybe even stand alone and walk. These are just a few of the things your baby will work at doing before the end of the first year.²⁸

Parents/caregivers are the most critical influence in a baby's first year of life. The parent/caregiver is the primary nurturer, teacher, and protector. The quality of parenting/caregiving and the interactions between parents/caregivers and baby in the earliest weeks substantially determine how far development in the first year will progress. Clearly, children will fare the best when their environment allows them to develop to their fullest intellectual potential and provides a happy, stimulating, and healthy childhood in which the capacity to love and to be loved is learned.²⁸

How Will the Development Stages That Occur in All Infants Affect Your Child With Hemophilia?

Parents/caregivers of a child with hemophilia may find it easier to care for their child if they understand the stages of physical, emotional, and mental growth all children go through. The sequence is mostly the same for everyone, but the timing is purely personal.²⁸

Development follows a head-down-to-toes direction. Eye muscles come under control first, then the facial muscles, neck muscles, and the trunk and the legs. At the same time, a center-outward development is occurring in the fingertip direction.²⁸ When your child is about 6 months old, all of these components may begin to come together and your child may become more and more mobile, which is likely to increase the chances for bruising or bleeding. Your child may begin crawling, getting up on his hands and knees, and even standing while holding on to something. While it is important that you allow your child to explore and expand this newly acquired mobility, it's also important that the environment is safe. This will be a challenging time for you, as your tendency may be to be overprotective.

The following is an overview of developmental milestones for infants, toddlers, and preschool children. Knowing ahead of time what to expect as your child with hemophilia grows may help you to gauge the types of safety measures that you may want to put in place during these life stages.



Developmental Milestones Infants Ages 6 to 12 Months

Gross Motor²⁹

- Voluntary crawling begins
- Turns, twists in all directions
- Rolls from back to stomach
- Creeps, propels self on tummy
- Stands with substantial support

Social²⁹

- Alternates hand with object in mouth
- Turns when he hears his name

Special Considerations³⁰

- Crawling and walking are important for muscle development despite possible increased bleeding
- Frequent use of playpens is discouraged
- Bleeding episodes that need factor concentrate seldom occur in the first year
- Infants with hemophilia may experience more bruising than other infants
- Head injuries should be reported to an HCP immediately

Immunizations

As with any other child, your child with hemophilia requires a range of immunizations against different diseases. Your child may develop bruises at the site of a shot. The HCP may suggest giving some shots with a small needle under the skin or giving the child a factor treatment before the shots. Most immunizations are performed subcutaneously (beneath the skin) to avoid bleeding. You should check with your HCP or HTC professional to find out whether this is possible.³¹

Safety Measures

- Enroll child in MedicAlert® system;³² to order an emblem (a bracelet or necklace for older children) call MedicAlert at 888.412.9033 or visit www.medicalert.org/
- Always strap baby into a car seat³⁰
- Never leave infant alone in a bathtub, on a bed, or on a changing table³⁰
- Put gates across stairways³⁰
- Use nonskid strips on the bathtub floor³⁰
- Remove sharp or breakable utensils from lower cupboards³⁰
- Remove any furniture that can be easily tipped over³⁰
- Do not use baby walkers because they can be dangerous⁸
- Put baby carriers on the floor instead of on top of furniture in case they are tipped over³⁰
- Pad coffee table corners and other pointed edges³⁰



Developmental Milestones

The Toddler Years

Gross Motor³³

- Cruises about while holding on to an object or person
- Walks when supported
- Can pull self up to standing position
- Steps off low object
- May begin walking without support, but clumsy at running and climbing; requires watching
- Gets self to stand by flexing knees
- Standing, pivots body 90 degrees
- Walks, but prefers crawling
- Climbs up and down stairs
- May climb out of crib or playpen

Social³³

- Expresses emotions and recognizes them in others
- Distinguishes self from others
- Mimics actions of others

Special Considerations

- More prone to accidents due to increased mobility and lack of judgement³⁰
- Mouth and soft tissue bleeds are common³⁰
- Head bumps are common³⁰
- Head injuries need to be reported to HCP immediately³⁰
- May associate infusion with something he has done or view it as a punishment⁸
- Good time to explain to the child that infusions will make him feel better⁸
- Parents' anxiety about bleeds may cause them to be overprotective⁸

Safety Measures

- Seek input from your hemophilia treatment team when making health and safety decisions or in the treatment of injuries⁸
- Lower child's crib mattress to its lowest level to discourage climbing out of crib or put mattress on the floor⁸
- Discourage unsupervised climbing and jumping off high places or furniture³⁰
- Always strap child into an approved car seat per your state law³⁰
- Avoid excessive roughhousing⁸
- Use a helmet when skating, biking, etc⁸



Developmental Milestones

The Preschool Years

Gross Motor³⁴

- Walks with an agile, almost adult style
- Runs around obstacles
- Running is more controlled; can start, stop, and turn
- Turns somersaults; hops on one foot; gallops
- Can easily catch, throw, and bounce a ball
- Catches large balls and throws overhead
- Climbs ladders; uses slide independently
- Rides a tricycle
- Alternates feet when climbing stairs
- Can brush teeth, comb hair, wash, and dress with little assistance

Dental care is a particularly important issue for both children and adults with hemophilia. Good oral hygiene is essential to prevent gum bleeding and the need for extensive dental work.⁸

Dental care should start for your child as soon as the baby teeth start to erupt. Teeth should be brushed twice a day with a soft brush and flossed regularly.⁸ Toothpaste containing fluoride should be used in areas where natural fluoride is not present in the water supply. Fluoride supplements may also be prescribed if appropriate.¹⁵ Flossing may cause a small amount of blood to ooze from the gums at first, but as the gums get healthier, the oozing stops.⁸

It is important to inform your dentist of your child's hemophilia and make sure that he or she understands the special needs of a child with hemophilia or is willing to learn about them. It is also important to contact your HCP or HTC before any dental procedures (to coordinate treatment). If your child has severe hemophilia, the HCP may want him to have factor IX treatment before the dental procedure.⁸

Social³⁴

- Follows simple directions; enjoys helping with household tasks
- Begins to recognize personal limits
- Likes to play alone, but near other children
- Can now make choices between two things
- Begins to notice other people's moods and feelings
- Thinks literally; starting to develop logical thinking
- Expresses anger verbally rather than physically
- Distinguishes right from wrong and honest from dishonest, but does not recognize intent

Special Considerations⁸

- Child begins to understand infusions are necessary to relieve pain
- Child may dislike venipuncture and not tell parents or staff
- Child should be encouraged to participate in his care
- It is helpful for medical staff to explain what is being done and to name equipment used
- Child can participate in medical care by choosing a venipuncture site, dissolving factor, holding pressure on the site, etc

Safety Measures

- Seek input from your hemophilia treatment team when making health and safety decisions or in the treatment of injuries⁸
- Use ice to help reduce bruising and ease discomfort⁸
- Help your child stay fit and trim; extra weight puts stress on joints⁸
- Teach child to floss regularly and to brush teeth with a soft brush⁸
- Inform dentist of child's hemophilia⁸
- NHF recommends that your child receive the hepatitis B vaccine (recommended for all children) and the hepatitis A vaccine (above 2 years old)³⁵

RECOGNIZING AND TREATING BLEEDS IN EARLY CHILDHOOD



What Are the Types of Bleeds?

Hemorrhages or “bleeds” may be caused by injury or may occur spontaneously (without any apparent cause).³⁶ Bleeds can begin in infancy, childhood, adolescence, or adulthood.⁴ The most common types are deep bleeding into the joints and muscles.⁹

Newborn males may bleed following circumcision (removal of the foreskin from the penis).¹⁰ During infancy, one of the most common bleeding signs is easy bruising.⁹ Another common place where bleeding can occur is in the mouth when biting the tongue⁸ or injuring the small piece of skin that attaches the center of the lips to the top and bottom of the mouth (the frenulum).⁴ Infants with hemophilia may also bleed under the skin or into the muscle after getting a shot or injection.⁴

During the toddler years, when children begin to move around more and more, they may experience bleeds into their joints. These types of bleeds are called hemarthroses, and they often occur in the knees, elbows, and ankles.⁹ These bleeds happen less frequently in the shoulders, wrists, and hips.⁴

Minor head bumps can be frustrating because it’s hard to know whether to treat with clotting factor or not. Head bumps are especially common in young children at the toddler stage (ages 1 to 2 years) who are just learning to walk and run and who are unsteady on their feet. These children often bump into doors, walls, and furniture. Many times the child is not upset by the injury—he doesn’t even cry—and often there is no bruise or cut caused by the bump. If you are not sure, you should speak to the nurse coordinator or medical director of your child’s HTC.³⁷

If any of the following symptoms occur, you must seek medical assistance immediately: headache, blurred vision, nausea or vomiting, mood or personality changes, drowsiness, loss of balance or coordination, weakness or clumsiness, stiffness of the neck, loss of consciousness, or seizures.³⁷

A photograph of a sandy path leading to a dune. The path is made of light-colored sand and has several footprints. To the right of the path, there is a metal fence made of vertical poles and horizontal wires. Behind the fence, there are tall, green grasses. The sky is visible in the background, showing a clear blue color.

R.I.C.E.³⁷

Bleeds in the joints, muscles, or soft tissues can be treated by using a form of first aid called R.I.C.E. (Rest [“R” can also mean Replacement of clotting factor], Ice, Compression, Elevation).

During a bleed, the affected area should be rested—no walking if the bleed is in the knee, no lifting if the bleed is in the elbow. To lessen pain or swelling, apply ice to the affected area—10 to 15 minutes every 2 hours is recommended. Applying pressure (compression, such as using an elastic bandage) to the area can also help to slow the bleeding. Always check with your local HTC for the proper way to apply the bandage. Elevating or raising the injured limb (arm or leg) above the heart will help to slow the bleeding.

Be sure to check with your HCP about the best way to handle a bleed.

Serious Bleeds

Examples of sites of bleeding episodes in hemophilia that threaten life, limb, or function include:

- Intracranial or head bleeds³⁸
- Neck or throat bleeds³⁸
- Gastrointestinal tract bleeds³⁸
- Abdominal bleeds³⁸
- Kidney or bladder bleeds⁸
- Ocular (eye) bleeds³⁸
- Deep cuts or lacerations³⁸

All of these bleeds require a call to your HCP, immediate intervention, or a trip to your local emergency room (ER).

Other Bleeds

Some bleeds may require medical attention and others may not. Speak with an HCP to learn when to seek medical care.

Urinary Tract Bleeds

Approximately 66% to 90% of people with hemophilia have bleeding in the urinary tract, also called hematuria, at least once in their lives. A symptom to watch for includes reddish-brown urine.⁴

Iliopsoas Bleeds

Iliopsoas bleeds occur in the muscle of the pelvic area, near the hip joint. This type of bleed can damage the nerves of the thigh muscle, thereby limiting a person's movement. If an iliopsoas bleed is left untreated, it can cause heavy blood loss and permanent damage.⁴

Compartment Bleeds

Compartments are closed-in spaces, such as in the forearm muscles. When a person bleeds deep inside these closed spaces, the blood settles in this area and puts pressure on the nerves and blood vessels within the muscle. If left untreated, compartment bleeds can cause permanent nerve damage and sometimes a loss of limb. Symptoms to watch for include pain and tingling in the fingers or toes.³⁷

Mouth Bleeds

If and when a child has a mouth bleed, it may be difficult for a clot to form. The inside of the mouth is wet and always moving. Because of this, it is difficult for an injury to heal. If a clot does form, it may detach or fall off before the injury is healed. Speak with an HCP if this happens.³⁷

National Hemophilia Foundation

Publications from the National Hemophilia Foundation contain informative resources for people with bleeding disorders and their families. The list of available publications can be accessed at www.hemophilia.org.

Bruising

Bruises are another common bleeding symptom in people with hemophilia. Some bruises can be mild and heal on their own with ice, and others may not. Please seek medical attention for bruises that are very painful, grow larger over time, limit movement, or affect sensitive critical areas.³⁷

Mouth Bleeds

Mouth bleeds are a common occurrence in the infant and toddler age group.⁴ This type of bleed can be caused by teething and may begin in a child as young as 3 months. The emergence of baby teeth can be uncomfortable for a child. The child may chew or bite anything within reach, such as toys, in an effort to feel better. Chewing on toys can cut the gums and cause them to bleed. Often the bleeding can be kept under control by applying pressure to the area for 5 to 10 minutes. (This may not be an easy task, as the mouth of an infant or toddler is very small and the child may not cooperate.) If the bleeding does not stop, call the child's HCP.³⁷

Mouth bleeds, such as those caused by biting the lip or tongue, a torn frenulum, new teeth coming in, or a dental procedure, are very common in people with hemophilia.⁹ They can be very serious because persistent mouth bleeding can cause severe anemia.³⁷

Preparing for Emergencies

It can be difficult for patients with hemophilia to achieve and maintain a normal level of factor to prevent all potential hemorrhages. People with hemophilia are at risk for severe bleeding that may lead to serious or life-threatening circumstances requiring emergency care.³⁹

People with hemophilia B or parents of children with hemophilia B are in the best position to manage their health or their child's health.

- Learn as much as possible about hemophilia
- Learn what to do if a bleeding situation may be happening

HCPs in the ER will ask to be provided with information on the hemophilia patient's past and current medical history. Be prepared to answer their questions.

Keeping an Infusion Log

It's a good idea to keep an infusion log of your child's treatments, including dates and times of the infusions, as well as emergency visits to the hospital. Make sure to include the site of the bleed, the date and time of the infusion, the child's weight, how much factor was infused, and the side effects, if any. Bring this infusion log when you and your child visit the HTC or an ER, as it will help to speed up care and also may be needed by your insurance company.

See Sample Infusion Log on page 44.

Smartphone Apps

With today's technology, smartphone apps serve many purposes. For example, HemMobile® is a free app that lets you keep track of your infusions and any bleeds you might have. This information may be helpful to share with your doctor at your next appointment.

Sample Infusion Log

Be sure to write the date and time for each infusion.

Date _____ Time _____

Weight _____ Product _____

Place the vial stickers here so you have a record of the lot number, expiration date, and the number of units per vial.

Place stickers here

Total # units _____

Write down the units in each vial and the number of units used. This tracks how much factor was needed for each bleed.

___ Prevention Activity/Event _____

___ Bleed/Injury Location _____

___ Bleeding Symptom(s) _____

___ Follow-up Scheduled _____

Check the reason for your infusion.

Describe how long after the bleed your treatment began. You can also note if you had any reaction to the factor.

Notes _____



Be Proactive When Treating Hemophilia B in Early Childhood

Thomas Truncale, DO, MPH
Associate Professor of Medicine
Director, USF Occupational Medicine Residency Program
Department of Environmental and Occupational Health
University of South Florida

Parent of two sons with hemophilia

Thomas Truncale, DO, MPH, is a pulmonary and critical care physician at the University of South Florida. He and his wife have four children (three boys and a girl). Two of their sons have hemophilia: a 10-year-old and a 9-month-old. There is no family history of hemophilia that they have been able to identify, and his wife has three brothers. Their 10-year-old son was first diagnosed with hemophilia when he was 15 months old and beginning to walk. Dr. Truncale points out that in many cases, infants don't show signs of hemophilia until they are older and starting to become more mobile. At that time, bruising and joint swelling may appear and hemophilia may become apparent.

As a parent of children who have hemophilia, Dr. Truncale has made several observations. With infants, toddlers, and preschoolers, he notes that nail clipping is an area where oozing might be noticed if the skin is accidentally cut. He also cautions that any head bump or head injury can be a serious issue, especially if an abnormal appearance or odd behavior is noticed in the child. "At the very least, the doctor should be called or the child taken to the emergency room." This is also the case if the child has a permanent intravenous catheter (used for infusions of factor IX) and develops a fever. If this should happen, the child should immediately be taken to the hospital. Dr. Truncale stresses the importance of discussing any concerns or questions regarding your child's care with the child's physician.

As a parent, Dr. Truncale ranks proactivity number one in managing hemophilia in treatment, education, and parenting. As soon as his children became active, Dr. Truncale began discussions with their doctor about the best treatment plan. They immediately sought out a source for comprehensive care, such as an HTC, where they could find all of the resources his children needed, including physicians, nurses, social workers, dentists, and other families who had experienced the same challenges. Dr. Truncale recommends locating an HTC where your child can be taken on a regular basis for medical care. "Regularity is very important," advises Dr. Truncale. "Establish a method of doing things and stick to it. Develop an association with the home care nurse and have both parents learn infusion. Infusion treatment requires a lot of stuff. Set aside a specific area in the home for the treatment where all of the supplies can be kept organized and ready for use. Check expiration dates on all medicines constantly. Provide a separate refrigerator for the medicines so they are not mixed in with food. Maintain regularity in this space."

Proactivity should extend beyond the home into the day care center, preschool, church, and community. Dr. Truncale recommends that all families of a child with hemophilia put together a package of educational materials for the school nurse, the teachers, church personnel, babysitters, and other family members. The information in the package should include a personal card with important contact information. Most HTCs have educational materials available for this purpose. Don't forget to add your own contact information to this package.

Your Personal Business Card

Prepare a personal business card to distribute to any people your child may come in contact with, such as parents of friends, relatives, teachers, church staff, sports staff, day care staff, sitters, and HTC personnel. Make sure to include your phone numbers (all of them) and other family phone numbers, as well as your child's doctors' numbers and HTC personnel numbers.

EMERGENCY CONTACT CARD

Jane Mother

732.272.1234 (cell)
732.272.1234 (home)
732.272.1234 (office)

Joe Father

732.272.1234 (cell)
732.272.1234 (home)
732.272.1234 (office)

Relative

732.272.1234 (cell)
732.272.1234 (home)
732.272.1234 (office)

How Will You Recognize an Emergency Situation?

There may be no visible signs of bleeding in a person with hemophilia, but bleeding issues such as joint hemorrhages, head injuries, muscle bleeds, and trauma can be serious. Emergency bleeding events require recognition and immediate intervention with factor replacement products to replace the missing factor (factor IX) in the blood and restore normal blood clotting.⁴⁰

The following situations require factor replacement therapy:

- Suspected bleeding in the brain. Such bleeding is life-threatening and requires immediate emergency care⁴¹
- Suspected bleeding into a joint or muscle⁴²
- Significant injury to the head, neck, mouth, or eyes, or evidence of bleeding in those areas⁴²
- New or unusual headache, particularly one following trauma⁴²
- Severe pain or swelling at any site⁴²
- Open wounds requiring surgical closure, wound adhesive, or Steri-Strips™⁴²
- History of an accident or trauma that might result in internal bleeding⁴²
- Invasive procedure or surgery⁴²
- Heavy or persistent bleeding from any site⁴²
- Gastrointestinal bleeding⁴²
- Acute fractures, dislocations, and sprains⁴²
- Limited motion, pain, or swelling of any joint⁴¹



What to Take With You When You Go to the Emergency Room

- Clotting factor IX and infusion supplies
- For a joint bleed, an ice pack (if readily available) to begin icing the bleeding joint immediately
- Physician's phone number in case the ER personnel need to speak to him or her
- Information about hemophilia B—the ER staff may have little experience with hemophilia B and may ask you about your child's treatment
- Your child's infusion log (if readily available)

Note: You may also want to carry a letter from your child's HCP describing your child's hemophilia and treatment. It's also a good idea to find out in advance where to go for care when you are out of town.

Important Points to Remember When Emergency Care Is Needed

- Factor IX replacement therapy is used in patients with hemophilia B for acute bleeding episodes or presumed acute bleeding episodes
- Have an emergency dose of clotting factor concentrate in your home at all times if advised by your HTC
 - Clotting factor concentrates might not be kept on hand at all hospitals. If you do not have clotting factor IX with you, the ER personnel may have to identify another hospital to best deal with the emergency. This will increase the time it takes until treatment is provided
- Take your child's factor IX with you when you travel and/or if you go to the ER
 - The ER may ask you if you have your child's factor IX with you, and they may ask you to infuse the dose for your child

NUTRITION AND EXERCISE



Helping Your Child to Eat Right

Part of staying fit includes eating right—eating a well-balanced diet that includes plenty of fresh fruits and vegetables. Check with your local HTC for more information about nutrition. Maintaining a healthy weight is important for anyone with hemophilia, not just children. Being overweight can put additional pressure and stress on joints, such as knees and ankles. The additional pressure and stress can cause damage to the padding between the joints, or cartilage. For additional information about nutrition, visit www.health.gov/dietaryguidelines.

Staying Fit

Maintaining physical activity is important for all children. It is especially important for children with hemophilia because building strong muscles can help protect joints from bleeds. Exercise helps to build strength and flexibility, both of which aid in preventing injuries. It is also good for the mind and assists in building a child's confidence.⁴³

FINDING THE RIGHT DAY CARE OR PRESCHOOL



What You Need to Know About Choosing a Day Care or Preschool Program for Your Child With Hemophilia B

When considering child care, it is important to know the kinds of care available in your community. Remember, your child is a person who has a bleeding disorder. When choosing a center, keep in mind your child's habits, personality, needs, likes, and dislikes; these needs are similar to those of the child's peers who do not have a bleeding disorder. Gather as much information as possible about a child care center before choosing a program. You will want to consider whether to have someone come to your home (eg, a nanny, relative, or au pair) or choose a child care setting near your home or work.⁴⁴

Needless to say, how much the family can afford for child care influences, or even controls, your choice. When you are ready to look for care—whether an in-home babysitter, a family day care home, or a center-based program—talk with relatives, friends, neighbors, and coworkers. They are likely to know about resources in the community. They may even have recommendations for specific providers. Remember, your HTC and local NHF chapter can link you to other families who have young children with bleeding disorders, and they may have helpful advice.⁴⁴



Resources for Finding Out-of-Home Child Care⁴⁴

There are several different types of child care programs. These include:

- Licensed child care homes (eg, family day care homes) are homes that may be licensed to care for a limited number of children of varying ages
- License-exempt child care (eg, a family home where a parent is allowed to care for one or two unrelated children in addition to his or her own; also, governmental, university, church, synagogue, public school, and hospital-based programs)
- Center-based child care (eg, group care where there is trained and paid staff)

To find out about programs where you live, contact:

- Child Care Aware, a program of the National Association of Child Care Resource and Referral Agencies (NACCRRA) at 1.800.424.2246. Child Care Aware also has a website: www.childcareaware.org

To check on licensing of these facilities, call your Department of Children and Family Services, Department of Public Welfare, Department of Public Health, or your school district office.⁴⁴

Helping Day Care or Preschool Staff to Understand

In a child care facility, it is likely the caregivers will, at first, feel nervous about their responsibility for your child. Provide as much information about your child's bleeding disorder as you believe is necessary for his safety. Be open to answering questions, even those that are asked over and over. Remember that most people know little or nothing about bleeding disorders.⁴⁴

Make sure that the person in charge knows what to do if your child should become injured. Ensure that this person has a general understanding of what hemophilia is and the type(s) of bleeds your child may have. Write down all instructions you believe are important, and advise the person NOT to give your child any prescription or nonprescription medications without your approval. The instructions you leave may include the following:

- The symptoms of a bleed
- Your child's limitations—what he can and cannot do
- Number(s) where you can be reached in case of an emergency
- Emergency contact names and phone numbers—your HCP and the local HTC⁴⁵

Some HTCs make day care center visits to educate the staff about hemophilia and other bleeding disorders. There are also publications available from HANDI, the information service of NHF, by calling 1.800.42.HANDI.⁴⁴

Things You May Want the Day Care or Preschool Staff to Be Aware of:

- Any physical restrictions or limitations your child may have
- Your child's medications and how they are used
- Symptoms of a bleed and how to treat it
- Where you can be reached during the day⁴⁵

FAMILY ISSUES IN THE EARLY CHILDHOOD YEARS



Teaching Your Child About Hemophilia

An important family issue is when and how to teach your child about hemophilia. The NHF has provided some excellent advice about both of these issues. They have suggested that it may be very difficult to help preschoolers understand their hemophilia. At this age, children cannot clearly understand the inside of their body because they cannot see it, and concepts such as the clotting cascade may not be possible for them to understand. By the time the child is 7 to 11 years of age, he can think about hemophilia in a more step-by-step way and may know that his disease involves blood and a “factor” that makes bleeding stop. Simple concrete explanations may be useful at this age.⁴⁶

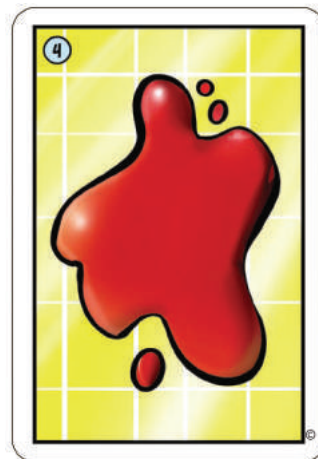
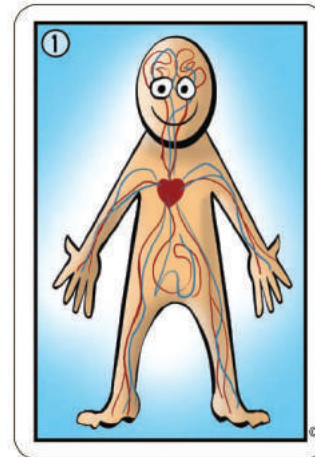
Frederica Cassis and the World Federation of Hemophilia have developed a set of cards called “HemoAction,” designed to be used in games, which may also be employed to help explain hemophilia to school-aged children. Several of these cards are illustrated in Figure 3.⁴⁷

Figure 3. HemoAction Cards⁴⁷

Look at this!

Our body is all linked together by internal tubes: they're the blood vessels!

These vessels carry blood to our entire body and are divided into three types: arteries, veins, and capillaries.



And do you know what runs inside your veins? Blood!

Blood is a red liquid that has a lot of important functions in your body.

One of them is "clotting," which stops the bleeding when we hurt ourselves.

Source: HemoAction Playing Cards, World Federation of Hemophilia and Frederica Cassis, 2009.

Psychosocial Implications of Hemophilia in Early Childhood

Two key issues with hemophilia that may impact parents/caregivers early on are coping with the diagnosis and balancing vigilance and overprotectiveness.⁴⁸

- A diagnosis of hemophilia can cause emotions ranging from acceptance to denial, confusion, anger, guilt, and fear for the future. These feelings can complicate or contradict the joy of the baby's arrival. The sooner these issues are confronted, the easier the adjustment will be—this is why the first years of interaction with the HTC are so important for families. For parents, the HTC should be a place where trust is built, and reliability and mutual education are assured
- Parents may sometimes feel guilt at having passed on hemophilia to their child. They may be disappointed and angry that dreams for their child may not be fulfilled. Anxiety over access to treatment or cost of treatment and concern about venous access for the delivery of factor replacement may occur. Anxiety about family disruption and sibling rivalry over the attention spent on the child with hemophilia may develop along with fears about treatment and care

Hemophilia affects not only the individual, but the whole family. Siblings should be included in counseling sessions and be given a basic understanding of hemophilia.⁴⁸

Family Dynamics in the Hemophilia Home

Edward Kuebler, LMSW-ACP
Gulf States Hemophilia and Thrombophilia Center
Houston, Texas

Edward Kuebler, a member of the comprehensive care team at Gulf States Hemophilia and Thrombophilia Center in Houston, Texas, works with people to help them understand that hemophilia affects not just the individual, but the whole family. When a child is diagnosed with hemophilia, the family unit needs to reevaluate how they will raise the child from this day forward. This thought often puts families into upheaval, and for this reason, Edward states, "It is very important that families immediately get involved with an HTC where they can find health professionals to help them assess their situation and identify approaches that will work for them. Many families simply don't know what to do."

At the HTC, they will have access to resources where all members of the comprehensive care team begin the process of education that will help families gain a better understanding of the bleeding disorder. Here they can meet other families with similar situations who can share their experiences.

One of the areas Edward focuses on is helping parents identify when they are being overprotective of their child with hemophilia. He works with them to change this behavior by:

- Recognizing and talking about their fears
- Guiding them to understand that overprotection may hinder the child's emotional, social, and physical development

Edward feels that it is important to watch for signs of difficult adjustment, such as parents rejecting or distancing themselves from the child, blaming the other (female) parent, feeling shame, or intense conflict in the family. "Parents don't trust themselves to parent a child with hemophilia for the first year or two." He works with families to achieve acceptance and to recognize that the dream they once had for their baby is now changed. Today, he helps them to move forward by learning to face each life stage as it arrives.

Edward feels that the Internet is a positive motivator in helping parents understand hemophilia because families are getting more information earlier. However, he does note that peer-to-peer communication is the most valuable resource for parents.

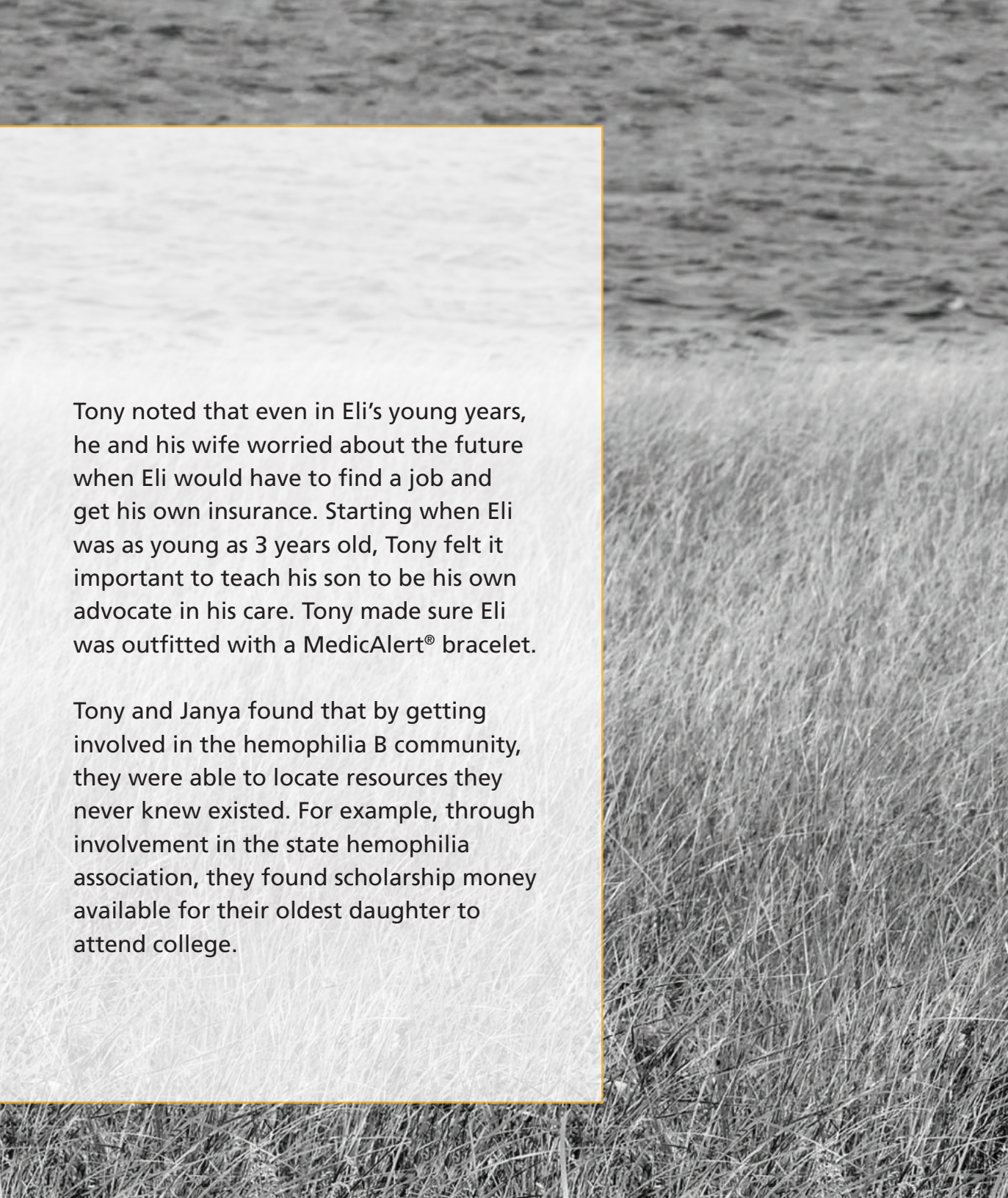
TONY'S Perspective

Get Involved Early in the Hemophilia B Community

Tony R. is the father of Eli, who has hemophilia B. Here, Tony recalls Eli's early childhood years and talks about what he found helpful in his experience with a child with hemophilia.

Tony believes it's necessary that members of a family take part in the treatment regimen as often as possible. He and his wife, Janya, as well as their oldest daughter, asked their home care nurse to teach them how to infuse, and eventually they all became involved in Eli's care.

When asked what he thought was the most important thing he taught Eli about his hemophilia B early on in life, Tony said that teaching him to take care of himself by telling his parents when something hurts was a big step.



Tony noted that even in Eli's young years, he and his wife worried about the future when Eli would have to find a job and get his own insurance. Starting when Eli was as young as 3 years old, Tony felt it important to teach his son to be his own advocate in his care. Tony made sure Eli was outfitted with a MedicAlert® bracelet.

Tony and Janya found that by getting involved in the hemophilia B community, they were able to locate resources they never knew existed. For example, through involvement in the state hemophilia association, they found scholarship money available for their oldest daughter to attend college.

JENNIFER'S Perspective

We Haven't Stopped for Hemophilia

Jennifer M. and her husband, Matthew, are the parents of four children: Kaitlyn, Megan, Emily, and Nicholas. Megan is a special needs child with Williams syndrome and Nicholas has hemophilia B. Before Nicholas, there was no previous confirmed diagnosis of hemophilia in the family, although Jennifer's brother died from an intracranial hemorrhage weeks after birth. A diagnosis of hemophilia was not expected when Nicholas was born until testing after his birth showed a factor level of less than 1%, and a confirmative diagnosis of severe hemophilia B.

Jennifer and her family have been extremely pleased with all the resources available to them through the hemophilia B community. The family attends numerous events and conferences every year, and their oldest daughter, Kaitlyn, has become involved with other siblings of young children with hemophilia B. She has even attended "advocacy days" in their home state of Maryland with her parents, where she spoke to a local congressman about resources for children with hemophilia.

When Jennifer was asked how she managed having both a child with hemophilia and one with special needs, she replied, "We haven't stopped for hemophilia. We use all of the resources in the hemophilia B community and as a family we have made many friends." She feels very strongly that every family with a child with hemophilia should get involved with an HTC as soon as possible and take advantage of the education and socialization available to the entire family.

To help educate the school on hemophilia B, Jennifer holds an education session with Nicholas' teachers and school staff. The family has also attended camp with Nicholas.



BRAD'S Perspective

Erasing the Fear of the Future Through Understanding

Brad and Lisa S. are the parents of twin boys, Eli and Max. Both boys have hemophilia B. Early on, Lisa and Brad had the support of their family; however, they did not know a lot about the disorder even though Lisa's father had hemophilia. As a result of this lack of knowledge, they decided to become educated immediately. So, they joined an HTC and their local hemophilia chapter. Through newly formed peer-to-peer relationships, a world opened up for them that began erasing some of the apprehension they initially had because of a lack of understanding of the disorder and fear of the future.

Brad believes that parents need to understand the process of a bleed and what can be done to prevent injuries that inevitably happen. He recalls in particular the boys' toddler years, and despite growing confidence and strength in knowledge about hemophilia, he and Lisa were still nervous about injury when the boys started walking. Lisa sewed extra cushioning into their clothing to help soften any falls.

Brad does have concerns for the future as well. "We do have anxiety about what is ahead. We are concerned about how we will manage treatments, both in terms of how the boys will respond and working out the logistics. We've made many trips to the ER for infusions and it has been stressful at times. We also have concerns about keeping them safe while not being too overprotective, especially when they are outside of our home." Brad and Lisa know how important socialization and exploration are to a child's growth, but admit that there are many stresses that go with this.

Brad and Lisa both work and had difficulty making a decision to find daycare. They did find a very capable nanny who became very involved and knowledgeable in the treatment of Eli and Max.



CONCLUSION AND ACKNOWLEDGMENTS



Parenting infants, toddlers, and preschoolers is no easy matter and a constant challenge under the best of circumstances. In a family in which a young child has hemophilia B, the stress, worry, and challenges are probably even greater. In many cases, the family is not prepared to manage a bleeding disorder and the changes it inevitably brings to the family unit. Among many families with young children who have hemophilia B, there seems to be a common statement from most of them: "Get involved in the hemophilia B community." Families benefit from the extensive resources in the community, including printed materials, family get-togethers, educational conferences, emotional support, and most of all, comprehensive hemophilia care at a local HTC. Within the hemophilia B community, families are certain to find others with similar situations who can offer guidance and support, as well as companionship for all members of the family during these early childhood years and for many years to come. The best protection and guidance for the children is complete involvement in their well-being.

Morris Green, MD, FAAP, Perry W. Lesh Professor Emeritus of Pediatrics at Indiana University Medical Center said, "It has become increasingly clear that a child's first 3 years of life largely determine his or her future developmental trajectory. To a large extent, these early years set the stage for later outcomes in personal health, emotional development, educational attainment, social competence, self-confidence, self-reliance, and positive human relationships. Parental investment in the coin of nurturance, care, love, and understanding during this formative age period brings both short- and long-term dividends."⁴⁹

Resources

[Pfizer Hemophilia Connect](#)

We're committed to helping the hemophilia community.

For the past two decades, Pfizer has been a part of the hemophilia community. We recognize it can be difficult to understand what support is available to help families with bleeding disorders, and to help address this challenge, we created Pfizer Hemophilia Connect.

Pfizer Hemophilia Connect is a one-stop destination to access all of our resources for eligible patients.

[Soozie Courter Hemophilia Scholarship Program](#)

Pfizer provides scholarships to students with hemophilia A or hemophilia B who are high school seniors, have a graduate equivalency diploma (GED), or are currently enrolled in an accredited junior college, college (undergraduate or graduate), or vocational school. Awards are based on academics, recommendations, and a personal statement from the student.

Visit www.HemophiliaVillage.com to download an application.

[HemophiliaVillage.com](#)

The Pfizer-sponsored website, www.HemophiliaVillage.com, provides information for the hemophilia community. Consumers and professionals alike can find product information and learn about programs and services.

[B2Byourvoice.com](#)

The Pfizer B2B Consumer Advisory Board was developed to directly connect Pfizer to hemophilia B patients and caregivers in order to gain firsthand feedback from the hemophilia B community. As a result, the B2B program has created tools and resources to support the community and continues to evolve to address needs as they change. Visit www.b2byourvoice.com for more and to check out a series of books, videos, patient stories, and other resources for people living with hemophilia B.

References

1. Hemophilia Federation of America. What is hemophilia? www.hemophiliafed.org/bleeding-disorders/hemophilia/. Accessed October 18, 2017.
2. National Heart, Lung, and Blood Institute. What is hemophilia? <https://www.nhlbi.nih.gov/health/health-topics/topics/hemophilia/>. Accessed October 18, 2017.
3. Zaiden RA, Nagalla, S. Hemophilia B: epidemiology. Medscape website. <http://emedicine.medscape.com/article/779434-overview#a5>. Accessed October 18, 2017.
4. Lozier JN, Kessler CM. Clinical aspects and therapy of hemophilia. In: Hoffman R, Benz EJ Jr, Shattil SJ, et al, eds. *Hematology: Basic Principles and Practice*. 4th ed. Philadelphia, PA: Elsevier/Churchill Livingstone;2005:2047-2069.
5. Konkle BA, Huston H, Fletcher SN. Hemophilia B. *Gene Reviews*. <https://www.ncbi.nlm.nih.gov/books/NBK1495/>. Accessed October 18, 2017.
6. Chalmers E, Williams M, Brennand J, Liesner RI, Colins P, Richards M; on behalf of the Paediatric Working Party of the United Kingdom Doctors' Organization. Guideline on the management of haemophilia in the fetus and neonate. 2011;154:208-215. <http://onlinelibrary.wiley.com/doi/10.1111/j.1365-2141.2010.08545.x/epdf>. Accessed October 18, 2017.
7. Centers for Disease Control and Prevention. Hemophilia diagnosis. www.cdc.gov/ncbddd/hemophilia/diagnosis.html. Accessed October 18, 2017.
8. National Hemophilia Foundation. Caring for your child with hemophilia. <https://www.hemophilia.org/sites/default/files/document/files/Caring%20for%20Your%20Child.pdf>. Accessed October 18, 2017.
9. Montgomery RR, Gill JC, Di Paola J. Hemophilia and von Willebrand disease. In: Orkin SH, Nathan DG, Ginsburg D, Look AT, Fisher DE, Lux SE, eds. *Nathan and Oski's Hematology of Infancy and Childhood*. 7th ed. Philadelphia, PA: Saunders/Elsevier;2009:1487-1524.
10. The Merck Manual for Health Care Professionals. Coagulation disorders: hemophilia. www.merckmanuals.com/professional/hematology_and_oncology/coagulation-disorders/hemophilia.html. Accessed October 18, 2017.
11. Dunn AL, Abshire TC. Recent advances in the management of the child who has hemophilia. *Hematol Oncol Clin N Am*. 2004;18:1249-1276.
12. MedlinePlus Medical Encyclopedia. Hemophilia B. www.nlm.nih.gov/medlineplus/ency/article/000539.htm. Accessed October 18, 2017.
13. Poon M-C, Lillcrap D, Hensman C, Card R, Scully M-F. Recombinant factor IX recovery and inhibitor safety: a Canadian post-licensure surveillance study. *Thromb Haemost*. 2002;87:431-435.
14. National Hemophilia Foundation. Medical and Scientific Advisory Council (MASAC). www.hemophilia.org/Researchers-Healthcare-Providers/Medical-and-Scientific-Advisory-Council-MASAC. Accessed October 18, 2017.
15. World Federation of Hemophilia. Guidelines for the management of hemophilia. Montréal, Québec: World Federation of Hemophilia; 2012.
16. Simpson ML, Valentino LA. Management of joint bleeding in hemophilia. *Expert Rev Hematol*. 2012;5(4):459-468.
17. DiMichele D. Inhibitor development in haemophilia B: an orphan disease in need of attention. *Br J Haematol*. 2007; 138:305-315.
18. Canadian Hemophilia Society. Inhibitors. www.hemophilia.ca/en/bleeding-disorders/hemophilia-a-and-b/the-complications-of-hemophilia/inhibitors/#c259. Accessed October 18, 2017.
19. National Heart, Lung, and Blood Institute. What causes hemophilia? www.nhlbi.nih.gov/health/health-topics/topics/hemophilia/causes.html. Accessed October 18, 2017.
20. Freedman SJ, Furie B. Biochemistry of factor IX and molecular biology of hemophilia B. In: Hoffman R, Benz EJ Jr, Shattil SJ, et al, eds. *Hematology: Basic Principles and Practice*. 4th ed. Philadelphia, PA: Elsevier/Churchill Livingstone;2005:2031-2045.
21. Evatt BL. The natural evolution of haemophilia care: developing and sustaining comprehensive care globally. *Haemophilia*. 2006;12(suppl 3):13-21.
22. National Hemophilia Foundation. Comprehensive medical care: HTC's. <https://www.hemophilia.org/Researchers-Healthcare-Providers/Comprehensive-Medical-Care-Hemophilia-Treatment-Centers>. Accessed October 18, 2017.
23. Evatt BL, Black C, Batorova A, Street A Srivastava A. Comprehensive care for haemophilia around the world. *Haemophilia*. 2004;10(suppl 4):9-13.
24. Centers for Disease Control and Prevention. Hemophilia treatment centers (HTCs). <https://www.cdc.gov/ncbddd/hemophilia/htc.html>. Accessed October 18, 2017.
25. Centers for Disease Control and Prevention. Hemophilia Treatment Center (HTC) Directory. https://www2a.cdc.gov/ncbddd/htcweb/dir_report/dir_search.asp. Accessed October 18, 2017.
26. National Hemophilia Foundation. National Prevention Program. <https://stepsforliving.hemophilia.org/next-step-kids/maintaining-a-healthy-body-kids/do-the-5-kids>. Accessed October 18 2017.

27. Nazzaro A-M, Crudder S. NHF National Prevention Program: how young people with hemophilia Do the Five. *HemAware*. 2002;7:14-19.
28. Caplan F, Caplan T. Introduction. In: *The First Twelve Months of Life: Your Baby's Growth Month by Month*. New York, NY: Bantam Books;1993:xv-xx.
29. Caplan F, Caplan T. The sixth month. In: *The First Twelve Months of Life: Your Baby's Growth Month by Month*. New York, NY: Bantam Books;1993:221-247.
30. Hemophilia of Georgia. Safety Issues. <http://www.hog.org/handbook/section/5/safety-issues>. Accessed October 18, 2017.
31. National Hemophilia Foundation. MASAC recommendations on administration of vaccines to individuals with bleeding disorders. <https://www.hemophilia.org/Researchers-Healthcare-Providers/Medical-and-Scientific-Advisory-Council-MASAC/MASAC-Recommendations/MASAC-Recommendations-on-Administration-of-Vaccines-to-Individuals-with-Bleeding-Disorders>. Accessed October 18, 2017.
32. MedicAlert Foundation. <http://www.medicalert.org/>. Accessed October 18, 2017.
33. Caplan F, Caplan T. The twelfth month. In: *The First Twelve Months of Life: Your Baby's Growth Month by Month*. New York, NY: Bantam Books;1993:357-379.
34. Destefanis J, Firchow N. Developmental milestones: ages 3 through 5. GreatSchools. <http://www.greatschools.org/gk/articles/developmental-milestones-ages-3-through-5/>. Accessed October 18, 2017.
35. National Hemophilia Foundation. MASAC recommendations for hepatitis A and B immunization of individuals with bleeding disorders. <https://www.hemophilia.org/Researchers-Healthcare-Providers/Medical-and-Scientific-Advisory-Council-MASAC/MASAC-Recommendations/MASAC-Recommendations-for-Hepatitis-A-and-B-Immunization-of-Individuals-with-Bleeding-Disorders>. Accessed October 18, 2017.
36. HIP Working Group. Hemophilia in Pictures. Montréal, Québec: World Federation of Hemophilia;2005.
37. Rivard G-E, Blanchette V, Hillard P, Mulder K, Zourikan N. Management of bleeds. In: *All About Hemophilia: A Guide for Families*. Montréal, Québec: Canadian Hemophilia Society;2001:5-1-5-28.
38. National Hemophilia Foundation. Types of bleeds. <https://www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeds>. Accessed October 18, 2017.
39. Hoots WK. Emergency Care Issues in Hemophilia. Treatment of hemophilia, No. 43. Montréal, Québec: World Federation of Hemophilia, 2007.
40. Kessler CM, Asatiani E. Acquired inhibitors to factor VIII. In: Lee CA, Berntorp EE, Hoots WK, eds. *Textbook of Hemophilia*. Malden, MA: Blackwell Publishing Ltd;2005:86-90.
41. National Heart, Lung, and Blood Institute. What are the signs and symptoms of hemophilia? <https://www.nhlbi.nih.gov/health/health-topics/topics/hemophilia/signs>. Accessed October 18, 2017.
42. National Hemophilia Foundation. MASAC Document #252. Guidelines for emergency department management of individuals with hemophilia. <https://www.hemophilia.org/sites/default/files/document/files/252%20ER%20Management.pdf>. Accessed October 18, 2017.
43. Zourikian N, Jarock C, Mulder K. Physical activity, exercise and sports. In: *All About Hemophilia: A Guide for Families*. Montréal, Québec: Canadian Hemophilia Society;2010:12-1-12-18.
44. Steinhart B, Fung EH. The Child With a Bleeding Disorder: Guidelines For Finding Childcare. New York, NY: National Hemophilia Foundation;2000.
45. Amesse C, Douesnard S, Rivard G-E, Zereik H. Growing with hemophilia. In: *All About Hemophilia: A Guide for Families*. Montréal, Québec: Canadian Hemophilia Society;2010:10-1-10-35.
46. Gorlin JB. Teaching children about bleeding disorders. In: *Nurses' Guide to Bleeding Disorders*. New York, NY: National Hemophilia Foundation;2013. <https://www.hemophilia.org/sites/default/files/document/files/Nurses-Guide-Chapter-13b-Teaching-Children-about-Bleeding-Disorders.pdf>. Accessed October 18, 2017.
47. Cassis FRMY. HemoAction: Playing and Learning About Hemophilia. Montréal, Québec: World Federation of Hemophilia;2009.
48. Cassis FRMY. Psychosocial Care for People With Hemophilia. Montréal, Québec: World Federation of Hemophilia;2007.
49. Green M. Foreword. In: Murkoff H, Eisenberg A, Hathaway S. What to Expect®: The Toddler Years. New York, NY: Workman Publishing;1994:xvi-xvii.



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