



Bone Marrow or Blood Stem Cell Transplants in Children With Severe Forms of Autoimmune Disorders or Certain Types of Cancer

A Review of the Research for Parents and Caregivers



Is This Information Right for Me?

This information is for you if:

- Your child's doctor has said your child has one of the following diseases.
 - **A very severe form** of one of the following autoimmune disorders in which **no other treatment worked**.
 - Systemic lupus erythematosus
 - Systemic sclerosis
 - Malignant multiple sclerosis
 - Juvenile idiopathic arthritis
 - Crohn's disease
 - One of the following types of cancer.
 - Astrocytoma of an intermediate stage that continued to grow after treatment or came back
 - Rhabdomyosarcoma that has spread to other body parts
 - Retinoblastoma that has spread to the brain and spinal cord
 - Ewing's sarcoma family of tumors
 - Wilms' tumor that came back after treatment
 - Ependymoma
- Your child's doctor has talked with you about a **hematopoietic** (pronounced he-MAH-tuh-poy-ET-ic) **stem cell transplant (HSCT)** that uses your child's own stem cells. It may also be called a blood stem cell transplant or a bone marrow transplant.
- Your child is under the age of 21. The information in this summary is from research on children younger than 21.

Note: A bone marrow or blood stem cell transplant can have severe side effects. In the research for this summary, a stem cell transplant was only done in patients with a very severe form of the disease when no other treatment worked. This summary only discusses stem cell transplants that use a child's own stem cells, **not** stem cells from a donor.

Note: An HSCT may not be helpful for all children with one of these diseases. Go to page 5 (very severe forms of autoimmune disorders) or 7 (types of cancer) to see what researchers found about treating each disease with an HSCT that uses the child's own stem cells.

What will this summary cover?

This summary will cover:

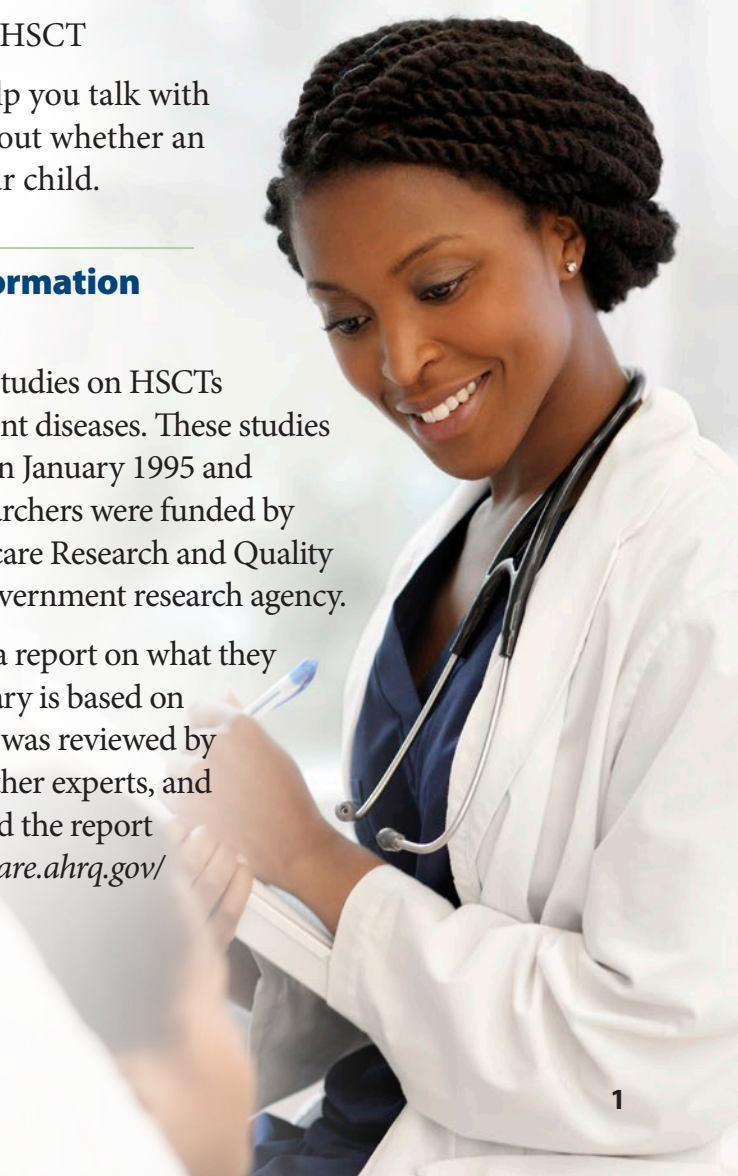
- Information about each autoimmune disorder and type of cancer
- Possible benefits of an HSCT
- What researchers have found about children with a very severe form of one of these diseases who received an HSCT that used their own stem cells
- What an HSCT is and how it is done
- Possible risks of an HSCT

This summary can help you talk with your child's doctor about whether an HSCT might help your child.

Where did the information come from?

Researchers reviewed studies on HSCTs in children with different diseases. These studies were published between January 1995 and August 2011. The researchers were funded by the Agency for Healthcare Research and Quality (AHRQ), a Federal Government research agency.

The researchers wrote a report on what they found, and this summary is based on that report. The report was reviewed by doctors, researchers, other experts, and the public. You can read the report at www.effectivehealthcare.ahrq.gov/stem-cell-children.cfm.



Understanding Your Child's Condition: Very Severe Forms of Autoimmune Disorders

What are autoimmune disorders and how might an HSCT help?

Autoimmune disorders are diseases in which the immune system (the system that protects the body from germs) does not work as it should. In people with an autoimmune disorder, the immune system attacks healthy cells and tissues by mistake. This can damage organs or other parts of the body.

For a child with a very severe form of an autoimmune disease, the purpose of an HSCT is to reset the child's immune system. The hope is that after the HSCT, the immune system will no longer attack healthy cells and tissues in the child's body.

The first step in an HSCT is to collect stem cells from the child's blood or bone marrow. Then, the white blood cells (an important part of the immune system) are destroyed using chemotherapy and sometimes radiation. After that, the child's stem cells are put back into the child's body. The white blood cells that develop from the transplanted stem cells build up to re-form the child's immune system.

The next few pages give a brief description of the autoimmune disorders covered in this summary.

Note: This summary only discusses HSCTs for children with very severe forms of these autoimmune disorders after no other treatment worked. This summary also only discusses HSCTs that use the child's own stem cells, not stem cells from a donor.

Systemic Lupus Erythematosus

Systemic lupus erythematosus (pronounced sis-TEM-ick LOO-pus AIR-uh-THEE-mah-TOE-sis) is a disease that causes damage to the skin, blood vessels, joints, and major organs such as the heart, lungs, kidneys, and brain.

Symptoms can include:

- A red skin rash
- Painful or swollen joints
- Fever
- Extreme tiredness
- Muscle pain
- Abdominal pain
- Shortness of breath
- Seizures
- Chest pain
- Blood in the urine
- Kidney failure
- Stroke

Systemic Sclerosis

Systemic sclerosis (pronounced sis-TEM-ick skluh-ROE-sis) is a disease that causes tissues in the body to become hard or thick. Systemic sclerosis can damage the skin, the tissues under the skin, blood vessels, and major organs such as the heart, lungs, and kidneys. This disease is more common in girls than in boys.

Symptoms can include:

- Tight, itchy patches of skin
- Painful or swollen joints
- Swollen fingers and hands
- Heartburn
- Tiredness
- Whiteness of the fingers, feet, and hands when you feel cold or anxious (called Raynaud's phenomenon)

Malignant Multiple Sclerosis

Malignant multiple sclerosis (pronounced muh-LIG-nant MUL-tuh-pull skluh-ROE-sis) is a rare severe form of multiple sclerosis that gets worse very quickly. Multiple sclerosis is a disease that affects the brain and spinal cord. This disease is more common in girls than in boys.

Symptoms can include:

- Problems with vision
- Weak muscles
- Dizziness
- Trouble with balance and tasks that require coordination
- Feelings of numbness or tingling
- Problems with memory and thinking
- Tiredness

Juvenile Idiopathic Arthritis

Juvenile idiopathic (pronounced id-ee-uh-PATH-ik) arthritis is a disease that causes swelling, pain, and stiffness in the joints of children. This disease can be mild or severe and can damage the joints. It can also affect a child's heart and lungs and can cause growth problems.

Symptoms can include:

- Limping
- Swollen, painful, or stiff joints
- Skin rashes
- Fever
- Swollen glands

Crohn's Disease

Crohn's (pronounced KRONES) disease causes inflammation in the intestines. It can also cause inflammation in the mouth and rectum.

Symptoms can include:

- Cramping or pain in the belly
- Pain when passing stool (waste)
- Diarrhea
- Bleeding from the rectum
- Weight loss
- Fever

What have researchers found about treating children who have very severe forms of autoimmune disorders with an HSCT?

Autoimmune Disorder	Number of Patients Studied in the Research	Researchers found the following about an HSCT using the child's own stem cells to treat a very severe form of the disorder after no other treatment worked:
Systemic lupus erythematosus	17 patients	<ul style="list-style-type: none"> ■ Symptoms improved enough for the child to not take medicines for their disorder for several months up to several years, depending on the disorder. ■ There is not enough research to know how well an HSCT works in the long term to treat children with a severe autoimmune disorder. There also are several possible long-term side effects (see pages 12 and 13).
Systemic sclerosis	43 patients	
Malignant multiple sclerosis	5 patients	
Juvenile idiopathic arthritis	5 patients	
Crohn's disease	7 patients	

Note: It is important to note that these research findings are based on only a very small number of studies in children with very severe forms of these autoimmune disorders after no other treatment worked. Talk with your child's doctor about whether these research findings apply to your child and whether an HSCT may help your child.



What are cancerous tumors and how might an HSCT help?

A tumor forms when cells grow out of control. Some tumors grow only at the place where they form and do not spread to other parts of the body. These are called “benign” (noncancerous) tumors. A tumor that is cancerous may spread to other parts of the body and damage organs, which can be life threatening.

For a child with cancer, the purpose of an HSCT is to allow doctors to use intense chemotherapy and possibly radiation treatment to try to kill the cancerous tumor cells. The chemotherapy and radiation also destroy the child's healthy blood cells. Before the chemotherapy and radiation treatment, stem cells are collected from the child's blood or bone marrow. After the treatment, the child's stem cells are then put back into the child's body to rebuild the blood cells.

This summary discusses HSCTs in children with one of the following types of cancer:

- **Astrocytoma** (pronounced ASS-truh-sie-TOE-mah) is a type of brain or spinal cord cancer. This summary discusses HSCTs in children with astrocytoma that is of an intermediate stage and either continued to grow during treatment or came back after treatment.
- **Rhabdomyosarcoma** (pronounced RAB-doe-my-uh-sar-KOE-mah) is a type of cancer of the muscles, tendons, fat, or blood vessels. This summary discusses HSCTs in children with rhabdomyosarcoma that has spread (or “metastasized”) to other parts of the body.
- **Retinoblastoma** (pronounced RET-in-oh-blas-TOE-mah) is a type of eye cancer. This summary discusses HSCTs in children with retinoblastoma that has spread (or “metastasized”) to the brain and spinal cord.

- **Ewing’s sarcoma** (pronounced YOU-ings sar-KOE-mah) **family of tumors** is a type of bone cancer that occurs either in the bones or another part of the body. This summary discusses HSCTs in children with one of these tumors.
- **Wilms’** (pronounced VILMZ-ez) **tumor** is a type of kidney cancer. This summary discusses HSCTs in children with a Wilms’ tumor that came back after treatment.
- **Ependymoma** (pronounced ep-EN-de-MOE-mah) is a type of cancer that affects the brain or spinal cord. This summary discusses HSCTs in children with this type of cancer.

What have researchers found about treating children who have certain types of cancer with an HSCT?

Type of Rare Cancer	Number of Patients Studied in the Research Who Had an HSCT	An HSCT using a child’s own stem cells:
Astrocytoma that is of an intermediate stage and either continued to grow during treatment or came back after treatment	10 patients	May increase the chance of surviving 5 years when compared with usual treatment*, but more research is needed to know for sure
Rhabdomyosarcoma that has spread to other parts of the body	346 patients	Did not increase the chance of surviving when compared with usual treatment*
Retinoblastoma that has spread to the brain and spinal cord	91 patients	Did not appear to increase the chance of surviving when compared with usual treatment*, but more research is needed to know for sure
Ewing’s sarcoma family of tumors	446 patients	
Wilms’ tumor that came back after treatment	114 patients	
Ependymoma	215 patients	May lower the chance of surviving when compared with usual treatment*, but more research is needed to know for sure

* Usual treatment is different for each type of cancer. Talk with your child’s doctor to see what usual treatment is for your child’s cancer.

Note: It is important to note that these research findings are based only on small studies of children with these types of cancer. Talk with your child’s doctor about whether these research findings apply to your child and whether an HSCT may help your child.

Understanding Hematopoietic Stem Cell Transplants

What are hematopoietic stem cells?

Hematopoietic stem cells are young cells that develop into blood cells. There are three types of blood cells:

- **Red blood cells** carry oxygen throughout your body.
- **White blood cells** are an important part of the immune system, which protects your body from germs.
- **Platelets** help your blood to clot.

Stem cells are found mostly in your bone marrow (the spongy tissue inside your bones). In the bone marrow, stem cells multiply and develop into blood cells. When the blood cells are fully developed, they leave your bone marrow and go into your bloodstream. Some stem cells can also be found in your bloodstream.

Note: Hematopoietic stem cells are not stem cells taken from an embryo (a human egg that has been fertilized by sperm) and are not the same as stem cells used in cloning.



What is the process of getting an HSCT?

Collecting the stem cells

Stem cells can come from your child's blood, bone marrow, or umbilical cord blood.

If the stem cells are taken from your child's blood, your child will need to take a type of medicine that increases the number of stem cells in the blood. This medicine is usually given as a shot every day for several days. Side effects of the medicine may include aching muscles or bones and headaches. Once there are enough stem cells in your child's blood, the doctor puts an intravenous (IV) tube into a large vein in your child's arm or a thin tube called a central line in the chest. Your child may be given general anesthesia so it is not painful. Your child's blood goes through the tube and into a machine that takes out the stem cells. The rest of the blood is then returned to your child through the tube.

If the stem cells are taken from the bone marrow, the doctor puts a needle into the back of your child's hipbone to collect the stem cells. Your child will be given general anesthesia so it is not painful. After the stem cells are collected, your child may have pain for a few days in the place where the needle was stuck in.

Stem cells can also come from umbilical cord blood. When your child was born, you may have had umbilical cord blood collected from your child's umbilical cord and the placenta. If your child's umbilical cord blood was frozen and stored in a cord blood bank, the doctor may be able to use stem cells from your child's cord blood for the HSCT.

Preparing for the HSCT

Before the HSCT, your child will need to undergo what is called “conditioning.” Conditioning consists of intense chemotherapy and sometimes radiation. In children with very severe forms of autoimmune disorders, conditioning is needed to destroy the child’s faulty white blood cells. In children with cancer, conditioning is needed to try to destroy the cancer cells in the child’s body.

Conditioning treatment can make your child feel sick.

Immediate side effects can include:

- Nausea and vomiting
- Diarrhea
- Loss of appetite
- Sores in the mouth
- Feeling tired
- Hair loss
- Infection

Some types of chemotherapy can also affect the heart and lungs.

There are other longer term risks from conditioning. These risks are discussed on pages 12 and 13.

Getting the HSCT

To do the transplant, the doctor will put a thin tube called a central line into a large vein in your child’s chest or an intravenous (IV) tube in your child’s arm. The stem cells go through the tube into your child’s bloodstream. This can take between 1 and 5 hours and is not painful.

Immediate side effects may include:

- A funny taste in the mouth
- Funny smelling breath
- Fever or chills
- Low blood pressure
- Shortness of breath
- Tightness in the chest or chest pain
- Hives

There are other longer term risks from an HSCT. These risks are discussed on pages 12 and 13.

The stem cells travel to your child’s bone marrow, where they will start to multiply and develop into blood cells over the next several weeks.

After the HSCT

After the transplant, your child may need to stay in the hospital for several weeks. It will take a few weeks for the stem cells to get into the bone marrow and start to make new blood cells. During this time, your child will only have a small number of blood cells (red blood cells, white blood cells, and platelets).

Until your child's new blood cells develop, your child may need antibiotics to prevent infection. Your child may also need to be given transfusions (red blood cells and/or platelets from donated blood are given to your child) and nutrition through an IV tube.



What are the risks of an HSCT?

An HSCT comes with many risks. Some of these risks can be life threatening.

Possible Problems Right After the HSCT

- **Infection:** Your child's immune system will be very weak after the HSCT. It can take up to a year or longer for the immune system to return to normal. During this time, your child is at risk for getting a severe infection because the immune system cannot fight off germs very well. Your child's doctor may give your child antibiotics to help prevent infection. A fever is often the first sign of infection. Call the doctor right away if your child has a fever.
- **Bleeding:** After the HSCT, your child will have a low number of platelets (the blood cells that help blood clot). It can take several weeks for new platelets to form. During this time, your child can bleed or bruise easily. If your child's platelet count gets too low, your child may need a platelet transfusion (platelets from donated blood are given to your child through an IV tube).



Possible Problems That Could Show Up Later

- **Relapse:** In children with an autoimmune disorder, symptoms of the disorder could come back. In children with cancer, the cancer could come back.
- **Infertility:** The chemotherapy and/or radiation given to your child during conditioning can cause infertility (not being able to have children later in life). You may wish to talk with your child's doctor about egg harvesting or sperm banking before the HSCT.
- **Damage to organs:** The chemotherapy and radiation given to your child during conditioning may damage your child's organs (such as the kidneys, liver, lungs, heart, or bones). Symptoms of organ damage may not show up right away.
 - The organs that may be damaged depend on the specific type of chemotherapy and the doses of chemotherapy and radiation that your child received. Your child's doctor will discuss the possible long-term side effects to watch for in your child.
- **Growth problems:** An HSCT may stunt your child's growth.
- **Cancer (a new or second cancer):** Anyone who receives an HSCT has a higher risk of developing cancer in their lifetime. The cancer could develop several years after the transplant. In children whose cancer was successfully treated with an HSCT, a new and different type of cancer could develop.

What are the costs of an HSCT?

An HSCT can be very expensive. The costs to you depend on your health insurance. Contact your health insurance provider to find out if your plan covers an HSCT and how much you will need to pay. Also talk with your child's doctor about whether other resources are available to help with these expenses.

Ask Your Child's Doctor

You may want to ask your child's doctor the following questions. Many of these questions may be best answered by a doctor who has experience with HSCTs.

- What are the possible benefits and risks of an HSCT for my child?
- What are the chances that an HSCT could help my child?
- Could an HSCT cure my child or just improve his or her symptoms?
- What are the chances that my child's symptoms (or cancer) will come back after the HSCT?
- How should we prepare for the transplant?
- What should we expect after the transplant?
- How long will my child need to stay in the hospital?
- What serious side effects should we watch for, and when should we call you about them?
- What possible serious problems should we watch for later in my child's life?

Other questions:

Write the answers here:

Sources

The information in this summary comes from the report *Hematopoietic Stem-Cell Transplantation in the Pediatric Population*, February 2012. The report was produced by the Blue Cross and Blue Shield Association Technology Evaluation Center Evidence-based Practice Center through funding from the Agency for Healthcare Research and Quality (AHRQ).

For a copy of the report, or for more information about AHRQ and the Effective Health Care Program, go to www.effectivehealthcare.ahrq.gov/stem-cell-children.cfm.

Additional information came from the MedlinePlus® Web site, a service of the National Library of Medicine and the National Institutes of Health. This site is available at www.nlm.nih.gov/medlineplus.

This summary was prepared by the John M. Eisenberg Center for Clinical Decisions and Communications Science at Baylor College of Medicine, Houston, TX. Parents or caregivers of children who have received a bone marrow or blood stem cell transplant and young adults who received a bone marrow or blood stem cell transplant as teenagers reviewed this summary.

