Your Guide to Understanding Aplastic Anemia


(800) 747-2820  www.AAMDS.org
The Aplastic Anemia & MDS International Foundation, Inc. is an independent nonprofit organization. Our mission is to support patients, families, and caregivers coping with:

- Aplastic anemia
- MDS (myelodysplastic syndromes)
- PNH (paroxysmal nocturnal hemoglobinuria)
- Related bone marrow failure diseases

This booklet offers you information about aplastic anemia and how it is diagnosed and treated. Although the information in this booklet has undergone a thorough, independent medical review to insure its accuracy, this information is not intended to be a substitute for the advice of your doctor. You should always seek medical advice from a qualified physician.

For more information, call us at (800) 747-2820, or visit us online at www.AAMDS.org.

We wish to express special thanks to the following medical professionals and individuals who have assisted with review and editing of this booklet:

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Finding out you have aplastic anemia can be a lot to take in all at once. Reading this booklet will help you and your family get the answers you need. Some people read the book from cover to cover. Others read only the sections they need right now, and then pull the booklet out again at a later date when they want to learn more.

There are many technical medical terms when talking about aplastic anemia (ay-PLAS-tik uh-NEE-mee-uh). We tell you what they mean and how to pronounce them.

For more information about aplastic anemia and related diseases, please call us at (800) 747-2820, or visit us online at www.AAMDS.org.

“This booklet has been a huge help to me and my family. Thank you for putting together the key facts I need – all in one place!”

—Michele
Aplastic anemia is a rare and serious disease. It happens when your bone marrow fails to make enough blood cells (low blood cell counts).

Who Gets Aplastic Anemia?

Each year, between 600 and 900 Americans learn that they have aplastic anemia. The disease can strike people of any age, race, and gender. But it is more common among children, teenagers, and young adults. It is also more likely to strike Asian-Americans.

What Causes Aplastic Anemia?

Most experts believe that aplastic anemia happens when your immune system attacks and kills your bone marrow stem cells. Stem cells are required for blood cell production. When they are killed, your blood counts fall, often to very low levels.

What Are the Symptoms of Aplastic Anemia?

The symptoms of aplastic anemia are caused by low blood cell counts. The symptoms depend on which type of blood cell is affected. Low red blood cell counts cause fatigue and tiredness, low white blood cell counts increase the risk for infections, and low platelet counts cause bleeding and bruising.

How Do I Find Out If I Have Aplastic Anemia?

Your doctor will test samples of your blood and bone marrow.
5 Steps to Taking an Active Role in Your Care

If you’re like most people with aplastic anemia, you want to stay in control as much as possible. Here are some ways to take an active role in your care. Take these 5 steps:

1. Find an expert on aplastic anemia who you can work with.
2. Learn all you can about aplastic anemia and possible treatment options.
3. Talk with your doctor and other healthcare professionals.
4. Write down your medical information in a notebook or on a computer.
5. Work with your doctor to make a treatment plan.

Ways to Treat Aplastic Anemia

These are the most common treatments for aplastic anemia:

- Blood transfusion (trans-FYOO-zhun) involves putting blood from a donor into your body. This raises blood counts.
- Growth factors can help more blood cells to grow and mature. They may be helpful but should not replace immunosuppressive therapy.
- Antibiotics prevent and treat infection.
- Immunosuppressive drug therapy lowers your immune response. This prevents your immune system from attacking your bone marrow, lets stem cells grow back, and raises blood counts.
- Stem cell transplantation involves putting blood-forming stem cells from a healthy donor into your bloodstream. The stem cells travel to your bone marrow and begin making healthy blood cells. This raises blood counts.
A Closer Look at Aplastic Anemia

What Is Aplastic Anemia?

Aplastic anemia is a rare and serious disease. It happens when your bone marrow fails to make enough blood cells (low blood cell counts).

Facts to Know About Blood

In order to understand aplastic anemia, it’s important to know certain facts about blood.

What Is Blood Made Of?

Blood is made of blood cells floating in plasma (PLAZ-muh). The plasma is mostly made of water with chemicals in it. These chemicals include proteins, hormones, minerals, and vitamins.

What Are the 3 Basic Types of Blood Cells?

1. **Red blood cells** are also called erythrocytes (i-RITH-ruh-sites). They make up almost half of blood. Red blood cells are filled with hemoglobin (HEE-muh-gloe-bun). That’s a protein that picks up oxygen in the lungs and brings it to cells all around the body.

2. **White blood cells** are also called leukocytes (LEW-kuh-sites). They fight disease and infection by attacking and killing germs that get into the body. There are several kinds of white blood cells, each of which fights a different kind of germ.

3. **Platelets** are also called thrombocytes (THROM-buh-sites). They are small pieces of cells that help blood clot and stop bleeding.

How Are Blood Cells Formed?

The process of making blood cells is called hematopoiesis (hi-mat-uh-poy-EE-suss). Blood cells are made in the bone marrow. That’s a spongy tissue located inside some bones. It contains young parent cells called stem cells.

These blood-forming stem cells can grow into all 3 types of blood cells. They make copies of (clone) themselves, and they also produce mature blood cells.

When blood cells are fully mature and functional, they leave the bone marrow and enter the blood. Healthy people have enough stem cells to keep making all the blood cells they need every day.
What Causes Aplastic Anemia?

Aplastic anemia is caused by destruction of stem cells in your bone marrow. Stem cells normally develop into three types of blood cells: red blood cells, white blood cells, and platelets.

Most research suggests that stem cell destruction occurs because the body’s immune system attacks its own cells by mistake.

Normally, the immune system attacks only foreign substances. When your immune system attacks your own body, you are said to have an autoimmune (o-toe-im-YOON) disease. Aplastic anemia is generally thought to be an autoimmune disease.

Other autoimmune diseases include rheumatoid arthritis (ROO-ma-toyd ar-THRI-tis) and lupus (LOO-pus).

2 Types of Aplastic Anemia

Aplastic anemia may be acquired or hereditary:

1. Acquired aplastic anemia

Acquired aplastic anemia can begin anytime in life. About 3 out of 4 cases of acquired aplastic anemia are idiopathic. This means they have no known cause.
What Causes Acquired Aplastic Anemia?

About 1 in 4 cases of acquired aplastic anemia can be linked to one of several causes. These include:

- Toxins, such as pesticides, arsenic, and benzene.
- Radiation and chemotherapy used to treat cancer.
- Treatments for other autoimmune diseases, such as lupus and rheumatoid arthritis.
- Pregnancy. Sometimes, this aplastic anemia improves on its own after the woman gives birth.

2. Hereditary aplastic anemia

Hereditary aplastic anemia is passed down through the genes from parent to child. It is usually diagnosed in childhood and is less common than acquired aplastic anemia.

Related Health Problems

People who develop hereditary aplastic anemia usually have other genetic or developmental abnormalities. Some of these include Fanconi’s anemia, Shwachman-Diamond syndrome, and dyskeratosis congenita.

What Does Not Cause Aplastic Anemia?

Aplastic anemia cannot be passed through germs from person to person.

Some other health problems can cause low blood counts, but do not cause aplastic anemia. These conditions include infections, such as HIV, and autoimmune diseases, such as lupus.

The Big Question

Q: I have aplastic anemia. What will happen to me over time? How long can I expect to live?

A: The course of aplastic anemia varies a lot from person to person. You may have only mild symptoms. Or you may have severe symptoms.

Many years ago, there were no treatments for aplastic anemia. It was considered a fatal disease. Today, with standard treatments, between 7 and 9 out of 10 patients can get better. The chance for recovery depends on many factors, including how severe your case is.

The Good News!

New treatments are becoming available. They are helping people with aplastic anemia live longer. To learn about ways to treat aplastic anemia, see page 16.

Review

- Aplastic anemia is a rare and serious disease that happens when your bone marrow fails to make enough blood cells.
- Blood is made of red cells, white cells, and platelets floating in plasma.
- Stem cells in the bone marrow can grow into all 3 types of blood cells.
- Most scientists believe that aplastic anemia happens when your immune system attacks your bone marrow stem cells.
- Aplastic anemia may be acquired or passed down through genes.
What Are the Symptoms of Aplastic Anemia?

“First I was feeling run down – but I figured I just had a cold that wouldn’t go away. Then I started getting short of breath. And my girlfriend noticed I was bruising easily. That’s when I realized something more serious was going on, so I went to the doctor.”

–James

Low Blood Cell Counts

The symptoms of aplastic anemia are caused by low blood cell counts. That means you don’t have enough of one or more types of blood cell.

Low Red Blood Cell Count

A low red blood cell count is called anemia (uh-NEE-mee-uh). Red blood cells carry oxygen from your lungs to the rest of your body.

Low White Blood Cell Count

A low white blood cell count is called neutropenia (noo-truh-PEE-nee-uh). White blood cells fight infections in the body by attacking and killing bacteria and viruses.

Low Platelet Count

A low platelet count is called thrombocytopenia (throm-buh-sie-tuh-PEE-nee-uh). Platelets help blood to clot and stop bleeding.

What Are the Symptoms of Low Blood Cell Counts?

The symptoms of low blood cell counts depend on which type of blood cell is affected. In aplastic anemia, most often all three blood cell types are reduced. Read the section below to see the symptoms for each type of cell. Check off any signs you have, and share this list with your doctor.

You may have many of these symptoms, or just one or two. And you may get a new symptom at any point in the course of your illness.
If you have a **low red blood cell count**, you may:

- Feel a little tired or very tired.
- Feel less alert or have trouble concentrating.
- Have a loss of appetite or lose weight.
- Have paler-than-normal skin.
- Have trouble breathing.
- Have a rapid heartbeat.
- Have reduced ability to exercise or climb stairs.

If you have a **low white blood cell count**, you may:

- Have repeated fevers and infections.
- Get bladder infections that may make it painful to pass urine, or make you urinate more often.
- Get lung infections that cause coughing and difficulty breathing.
- Get mouth sores.
- Get sinus infections and a stuffy nose.
- Get skin infections.

If you have a **low platelet count**, you may:

- Bruise or bleed more easily – even from minor scrapes and bumps.
- Get heavy menstrual periods.
- Get nose bleeds.
- Get tiny, flat red spots under your skin, which are caused by bleeding. These spots are called petechiae (puh-TEE-kee-ie).
- Have bleeding gums, especially after dental work or from brushing your teeth. Check with your doctor before getting any dental work.

**Review**

- The symptoms of aplastic anemia are caused by low blood cell counts.
- The symptoms of low blood cell counts depend on which type of blood cell is affected.
How Do I Find Out If I Have Aplastic Anemia?

“For me, diagnosis took three months. I went from doctor to doctor, trying to find out what was wrong with me. I finally found an expert on blood problems who was able to pin down a diagnosis. So although I’m bummed about the disease, I’m relieved to finally know what I have.”

—Kaitlyn

Aplastic anemia is a complex disease. Diagnosing it can be a complex process. Doctors use a number of tools to help them understand your aplastic anemia better.

Doing a Medical History

To understand what is causing your symptoms and low blood counts, your doctor will take a detailed medical history. He or she may ask you questions like the following:

- What are your symptoms?
- What medicines or herbal supplements have you been taking?
- Have you been exposed to harmful chemicals?
- Did you have chemotherapy or radiation treatments in the past?
- Is your urine dark or tea-colored in the morning?
- Has your liver been inflamed recently?

Giving a detailed health history helps your doctor give you an accurate diagnosis.

Getting Lab Work

When trying to figure out the cause of your symptoms, your doctor will ask for blood samples and a sample of your bone marrow. These samples will be used in a number of tests.

About Blood Tests

The tests on your blood help your doctor find out how severe your aplastic anemia is. Some of the results can be confusing. Look at the chart on page 11. Then compare this chart with your lab report.

One important test is a complete blood count, or CBC for short. The CBC measures the number of each blood cell type in your blood sample. If the CBC shows a low number of red blood cells, white blood cells, or platelets, your doctor may look at the cells under a microscope. This is called a blood smear, and it can show if any blood cells are abnormal.

Your doctor will ask you to get a CBC, and possibly other blood tests, on a regular basis. This will allow your doctor to know if your blood counts are high or low and if they have changed from the last time you got a CBC. If your blood counts are abnormal or have changed, your doctor may try to find out why.
A CBC generally includes the following:

- **White blood cell (WBC) count** measures the actual number of white blood cells in a given volume of blood. It is also called a leukocyte count. A low count can mean you have an increased risk of getting an infection.

- **White blood cell (WBC) differential** looks at the many different types of white blood cells in your blood. Each type protects you from different kinds of infections. Doctors are most concerned about the neutrophil (NOO-truh-fil) count. Neutrophils are the most common type of white blood cell and are the cells most responsible for fighting infection.

- **Red blood cell (RBC) count** measures the actual number of red blood cells in a given volume of blood. A low count is called anemia.

- **Hemoglobin (Hgb)** measures the amount of this oxygen-carrying protein in red blood cells. This level is low in people with anemia.

- **Hematocrit (hi-MA-tuh-crit) (HCT)** measures how much of a given volume of whole blood is made up of red blood cells. It is also called packed cell volume, or PCV. The value is given as a percentage of red blood cells in a volume of blood. This number is about 3 times the hemoglobin.

- **Platelet (thrombocyte) count** measures the number of platelets in a given volume of blood. A low count means you have an increased risk of bleeding.

- **Mean corpuscular volume (MCV)** measures the average size of red blood cells. It is high when red blood cells are larger than normal. It is low when red blood cells are smaller than normal.

### Normal Blood Values for Adults

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<th>Adult Normal Range* (Varies in different clinics or hospitals, or in different parts of the country)</th>
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<td>Red blood cell count</td>
<td>Men 4.4 to 5.8 million cells/mcL&lt;br&gt;Women 3.9 to 5.2 million cells/mcL</td>
</tr>
<tr>
<td>White blood cell count</td>
<td>4.5 to 10 thousand cells/mcL</td>
</tr>
<tr>
<td>Hemoglobin</td>
<td>Men 13.8 to 17.2 grams/dL&lt;br&gt;Women 12.0 to 15.6 grams/dL</td>
</tr>
<tr>
<td>Hematocrit</td>
<td>Men 41 percent to 50 percent&lt;br&gt;Women 35 percent to 46 percent</td>
</tr>
<tr>
<td>Platelets</td>
<td>150 to 450 thousand/mcL</td>
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<tr>
<td>MCV (Mean Corpuscular Volume)</td>
<td>76 - 100 cu µm</td>
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*Normal blood values involve a range rather than a single volume.

**NOTE:** Normal blood counts for children differ slightly from normal adult counts. The normal range for children depends on both their age and gender.
Other Blood Tests

Your doctor may order other blood tests after getting results from your CBC.

EPO Level

EPO, or erythropoietin (i-rith-row-POY-uh-tun), is a protein. Your kidneys make it if you don’t have enough oxygen in your body. EPO causes your bone marrow to make more red blood cells.

A shortage of EPO can cause anemia. A low EPO level may be a sign of a problem other than aplastic anemia.

Iron Level

If you have anemia, your doctor may also check the level of iron in your blood. A shortage of iron can cause anemia.

Vitamin B-12 and Folate Levels

If your red blood cells are abnormally large, your doctor will check the levels of Vitamin B-12 and folate in your blood. A shortage of these vitamins reduces blood cell production in the bone marrow. This causes a drop in the number of white blood cells, red blood cells, and platelets in the blood.

About Bone Marrow Tests

Bone marrow tests are done for several reasons. Most likely your doctor checked your bone marrow to see if you have aplastic anemia. The doctor may also do a bone marrow exam now and again to find out if your aplastic anemia has stayed the same, gotten better, or gotten worse since the last exam.

The bone marrow test shows:

1. Exactly what types and amounts of cells your marrow is making.

2. Levels of bone marrow blasts (immature white blood cells).

3. Damaged chromosomes (DNA) in the cells of your bone marrow. These are called cytogenetic (sie-toe-juh-NEH-tik) abnormalities.

Giving a Bone Marrow Sample

Giving a bone marrow sample is a fairly simple procedure. It can be done in your doctor’s office, or in the hospital. It usually takes about 30 minutes. Here’s what to expect:

■ Your doctor gives you a shot to numb the area near your hipbone.

■ Your doctor makes a small cut in your skin.

Bone Marrow Aspiration (BMA)

■ Your doctor inserts a needle that goes into the marrow of your hipbone. When the needle is all the way in, you may feel a deep, aching pain.

■ Your doctor uses a syringe to draw out about a tablespoon of liquid called bone marrow aspirate. You may feel stinging and pulling in your hip and down your leg.
Bone Marrow Biopsy (BMB)

Your doctor puts the needle in again to take out a piece of solid marrow. You may feel some pressure while the needle is going in.

- Your doctor loosens the marrow sample to get it out. You may feel a little jerk.
- Your doctor takes out about half an inch of bone marrow core.

Risks of Giving a Bone Marrow Sample

When you give a bone marrow sample, you face some small risks. There is a chance that the site of the sample may:

- Bleed
- Feel sore
- Get infected
- Have a bruise

If you feel sore, ask your doctor if you can take Tylenol® (acetaminophen). Your doctor will probably tell you to avoid aspirin and other NSAIDs (non steroidal anti-inflammatory drugs), since they can increase the risk for bleeding.

Any time a needle is put into your skin, infection could occur. However, infection happens very rarely.

Examining Bone Marrow

Liquid (Aspirate)

Your doctor or another doctor called a pathologist (puh-THAH-luh-jist) will examine the liquid bone marrow (aspirate) to look for abnormalities linked to aplastic anemia. Using special tools and tests, he or she will figure out if there are:

- Blood cells with an abnormal shape, size, or look. This is called dysplasia (dis-PLAY-zeeuh).
- Chromosomal (DNA) abnormalities.

- Increased, decreased, or normal levels of iron in your bone marrow.
- Too many young white blood cells (blasts) in your marrow.

Solid (Core)

Your doctor will send the solid (core) sample to a laboratory. Based on the lab report, your doctor will:

- Analyze the quantity (cellularity) of the bone marrow occupied by different cells.
- Check for scarring of the bone marrow (fibrosis, pronounced fie-BRO-suss), which can interfere with making normal red blood cells.
- Look for abnormal cells.

You may hear your doctor talk about some of the specific types of tests used to analyze your bone marrow. These include flow cytometry and FISH.

Flow cytometry (sy-TOM-uh-tree) tests blood cells taken from your bone marrow. The cells are labeled with dyes and run past a laser. This method counts the various types of cells in your bone marrow.

FISH (fluorescence in situ hybridization, pronounced flor-EH-sense in SIT-tyoo hy-bruh-duh-ZAY-shun) directs colored light at specific parts of chromosomes or genes. It tells your doctor if some of your marrow cells have a genetic abnormality.

Your doctor will be able to see if you have any chromosomes that are rearranged or missing, or if there are any extra chromosomes. FISH looks only for the most common genetic abnormalities associated with low blood counts.
How Is Aplastic Anemia Classified?

Doctors divide aplastic anemia into 3 groups:
■ Moderate
■ Severe
■ Very severe

If you have moderate aplastic anemia:
■ You may have low blood cells counts, but not as low as with severe aplastic anemia.
■ You may have few or no symptoms.
■ Your doctor may not recommend treatment. Instead, your doctor may just keep an eye on your blood counts.
■ Your condition may stay the same for many years.

If you have severe aplastic anemia:
The growing cells in your bone marrow (cellularity) occupy less than 25 percent of your bone marrow. Normal bone marrow has a cellularity of around 100 minus your age in years.

At least 2 of the following are true:
■ Your neutrophil count is less than 500 cells per microliter (<500/mm³). Neutrophils are a type of white blood cell that fight bacterial infections.
■ Your platelet count is less than 20,000 per microliter (<20,000/mm³).
■ Your reticulocyte (young red blood cell) count is less than 20,000 per microliter (<20,000/mm³).

If you have very severe aplastic anemia:
■ Your neutrophil count is less than 200 per microliter (<200/mm³).
■ Your blood counts are otherwise like those of someone with severe aplastic anemia.

These categories were defined in 1975 by Dr. Bruce Camitta and his team. Most doctors use them.

Aplastic Anemia is Related to 2 Other Bone Marrow Failure Diseases:
■ PNH, or paroxysmal nocturnal hemoglobinuria (par-uk-SIZ-muhl nok-TURN-uhl hee-muh-gloe-buh-NYOOR-ee-uh)
■ MDS, or myelodysplastic syndromes (my eh-lo-diss-PLASS-tik SIN-dromez)

Like aplastic anemia, these diseases cause the bone marrow to stop making cells properly.
PNH is related to aplastic anemia:

- More than 10 out of every 100 people with aplastic anemia will also have PNH at the time of diagnosis, or will develop it later. Patients can have a mix of these two diseases, with features of both to varying degrees.
- Some people with PNH will develop aplastic anemia.

PNH is different from aplastic anemia:

- In PNH, an abnormal bone marrow stem cell makes abnormal red blood cells. These PNH red blood cells are attacked by proteins and destroyed in the blood stream. In aplastic anemia, bone marrow stem cells make normal blood cells, but not enough of them.
- At least 1 in 3 people with PNH get blood clots. The symptoms depend on where the clots occur.
- People with PNH may have periods when they have dark urine. The color comes from hemoglobin that is released from the destroyed red cells in the bloodstream and filtered out through the kidneys.

MDS is related to aplastic anemia:

- A small number of people with aplastic anemia develop MDS.
- Like aplastic anemia, MDS can cause low blood cell counts. The symptoms depend on which blood cells are affected. (See pages 5, 8, and 9.)

MDS is different from aplastic anemia:

- In MDS, an abnormal bone marrow stem cell makes abnormal blood cells. In aplastic anemia, bone marrow stem cells make normal blood cells, but not enough of them.
- People are more likely to get MDS as they get older, unlike aplastic anemia.
- If you have aplastic anemia, you should get regular tests for both PNH and MDS. To learn more about all 3 diseases, please call the Aplastic Anemia and MDS International Foundation (AA&MDSIF) at (800) 747-2820. Or visit us online at www.AAMDS.org. We can answer your questions and provide you with educational booklets.

Review

- In order to find out if you have aplastic anemia, your doctor may:
  - Take your medical history.
  - Take samples of your blood and bone marrow.
  - Examine the samples.

- Aplastic anemia can be classified into 3 types: moderate, severe, and very severe.

- Aplastic anemia is related to 2 other bone marrow failure diseases: PNH and MDS.
Ways to Treat Aplastic Anemia

“I just found out that my daughter has aplastic anemia. I really wish she didn’t have to go through that. But I’m glad she’s living in a time when there are so many treatment options available. It gives me hope.”

—Steve

Before getting any treatment …

Talk with your doctor about the risks and benefits. That way you can make an informed choice about which treatment is best for you.

The main goal of aplastic anemia treatment is to increase the number of healthy cells in your blood. When your blood counts go up:

- You are less likely to need blood from a donor (transfusion).
- Your quality of life gets better.
- Your symptoms are not as bad.

Issues in Making a Treatment Plan

Your doctor will look at several issues in finding the best treatment plan for you. These include:

- Your symptoms.
- Your age.
- How severe your case is. (See page 14.)
- Other conditions or diseases you have.
- Whether someone (often a family member) is willing and able to donate matching stem cells to you.

Summary of Treatments

These are the most common treatments for aplastic anemia:

- Blood transfusions involve putting blood from a donor into your body. These temporarily increase blood counts until other treatments can work. (See page 17.)
- Iron chelation therapy reduces high levels of iron in your blood. High levels of iron can be caused by getting lots of red blood cell transfusions. (See page 18.)
- Growth factors may help more blood cells to grow and mature. They help some patients for a while, but they do not treat the immune system problem that causes aplastic anemia. So they should not take the place of stem cell
transplantation or immunosuppressive drug therapy. (See page 18.)

- **Antibiotics** prevent and treat infection. (See page 19.)
- **Immunosuppressive drug therapy** prevents your immune system from attacking your bone marrow, lets stem cells grow back, and raises blood counts. This is the standard treatment for older patients or those without a stem cell donor. (See page 19.)
- **Stem cell transplantation** involves putting blood-forming stem cells from a healthy donor into your bloodstream. These cells travel to your bone marrow and start making healthy cells. This is the standard treatment for younger patients with severe aplastic anemia if a donor is available. (See page 21.)

**Supportive Care**

Supportive care helps you manage the symptoms of aplastic anemia. This approach includes the use of:

- Blood transfusion
- Iron chelation to treat iron overload
- Growth factors
- Antibiotics

**Blood Transfusion**

When you receive a blood transfusion, the cell parts of blood from a donor are put into your bloodstream. This can help some patients with low blood counts.

White blood cells live for a very short time. So patients with a low white count rarely get transfusions of white blood cells.

The 2 types of transfusion typically used for aplastic anemia patients are:

- Red blood cell transfusion
- Platelet transfusion

**Red Blood Cell Transfusion**

If you don’t have enough healthy red blood cells, you may get a red blood cell transfusion. It usually takes 2 to 5 hours to complete. Here’s what to expect.

**Before the transfusion:**

- Your blood will be tested to make sure it matches the donor blood. This usually takes about 1 hour.
- Donor blood may be filtered and irradiated (i-RAY-dee-ate-ed) to remove and deactivate certain cells. This process lowers your risk of having a bad immune response to the blood called graft-versus-host-disease (GVHD).
- Your doctor may order Tylenol® and Benadryl® before the transfusion to help prevent fever, chills, and allergic reaction.
- You may get a gel called EMLA to numb the area where the needle will enter.
- You will get an IV in your vein.

**During the transfusion:**

- Your nurse will check your vital signs – temperature, heartbeats per minute, breaths per minute, and blood pressure.
- You will get 1 to 3 units of red blood cells without plasma (packed red blood cells).

**After the transfusion:**

- If you took Benadryl®, you may feel drowsy.

**Side Effects of Red Blood Cell Transfusion**

A red blood cell transfusion carries the risk of certain side effects:

- You may have an allergic reaction.
- You might have a fever.
- You might develop a rash or hives.
- After you get many transfusions iron may build up in your body (iron overload).
Platelet Transfusion

If you don’t have enough healthy platelets in your blood or if you are bleeding, you may get a platelet transfusion. At first, a platelet transfusion helps for about 7 days. But after you have had many platelet transfusions, they may not help as long.

Side Effects of Platelet Transfusion

Platelets are more likely than red blood cells to cause an immune response. So you are more likely to have side effects like chills and fever.

Your body is quicker to develop antibodies against platelets than against red blood cells. This can cause platelet transfusions to fail after repeated transfusions.

Iron Chelation Therapy for Iron Overload

You should get your blood iron level checked regularly if you get red blood cell transfusions often. That’s because the transfusions can cause you to have too much iron in your blood. This can lead to a condition called iron overload, which can hurt your heart and other organs.

Iron overload can start to become a problem after as few as 20 units of red blood cells. A blood iron level, or ferritin (FER-i-tin), of over 1,000 is considered high enough to consider treatment.

If you have iron overload, your doctor may ask you to take an iron chelator to get the extra iron out of your body. The FDA (U.S. Food and Drug Administration) has approved 2 iron chelators to treat iron overload in the U.S.

Deferasirox (Exjade®) is a tablet that you mix with juice or water and swallow. It is taken once a day. Because it can be taken by mouth, it is also called an oral iron chelator.

Deferoxamine (Desferal®) is a liquid that is usually given by a slow infusion under the skin using a portable light-weight infusion pump. It can also be given by infusion into a vein (IV drip) or by injection into a muscle.

An oral iron chelator called diferyprone (Ferriprox®) is approved for use in Europe and elsewhere, but not the U.S. It is usually taken 3 times a day (morning, afternoon, and night). The number of tablets taken per day depends on your weight and how much iron you have in your body.

Growth Factors

Growth factors are chemicals (proteins) made by your body. They cause your bone marrow to make blood cells. Most people with aplastic anemia have higher natural levels of growth factors than healthy people do because their bodies are trying to
stimulate the failing bone marrow to make more blood cells.

For some aplastic anemia patients, taking man-made forms of growth factors can make their bone marrow work better. Growth factors work best in patients with higher blood counts, since they have more bone marrow cells to stimulate.

Red Blood Cell Growth Factors

Erythropoietin (i-rith-row-POY-uh-tun), or EPO for short, is a growth factor made by your kidneys. It causes your bone marrow to make more red blood cells.

Man-made forms of erythropoietin are available. Here are some of them:

- Epogen® and Procrit® are taken at least once a week. These drugs can be given as an injection under the skin or into a vein.
- Darbepoietin (Aranesp®) is taken every 2 to 3 weeks. It is given as an injection under the skin or into a vein.

White Blood Cell Growth Factors

White blood cell growth factors cause your bone marrow to make white blood cells. These are the most common ones:

- G-CSF (granulocyte colony-stimulating factor) is sold under the names Filgrastim® and Neupogen®. It may increase the risk of developing MDS, so it is best reserved for patients with active infection due to low white blood cell counts.
- GM-CSF (granulocyte macrophage colony-stimulating factor) is sold under the names Leukine® and Sargramostim®.

Antibiotics

The most common white blood cells are called neutrophils. They kill bacteria.

If you have a low neutrophil level, your doctor may ask you to take antibiotics. These medicines help to prevent and fight infection.

Immunosuppressive Drug Therapy

Most scientists believe that aplastic anemia happens when your immune system attacks and destroys some of your bone marrow stem cells. Your bone marrow is then no longer able to make enough blood cells.

Immunosuppressive drugs can lower your body’s immune response. This allows your bone marrow stem cells to grow back and make more blood cells.

Benefits of Immunosuppressive Drug Therapy

This therapy is usually the first treatment used with older patients and with all patients who don’t have a matched related bone marrow donor. It has several benefits:

- It usually causes at least partial bone marrow recovery.
- It usually causes few side effects.
- It usually requires only brief hospital stays.

Immunosuppressive Drugs

The most common immunosuppressive drugs used to treat aplastic anemia are:

- ATG (antithymocyte globulin) or ALG (antilymphocyte globulin)
- Cyclosporine
ATG

How ATG Helps
ATG kills specific cells in your immune system called T-lymphocytes — the cells that are attacking your stem cells. This allows your bone marrow to rebuild its supply of stem cells. The stem cells, in turn, make more blood cells.

How ATG Is Given
Usually, ATG is given by IV for 8 to 12 hours a day, for 4 days. The schedule depends on your needs, the type of ATG used, and the methods of your doctor or hospital. No one schedule has been shown to be better than another.

How Well Does ATG Work?
■ When used alone, ATG improves blood counts about half of the time.
■ When used with cyclosporine, ATG improves blood counts in about 7 out of 10 cases.
■ If it works, ATG usually stops the need for transfusions within 3 months. Full success can take at least nine months.
■ Some patients may respond well, but later relapse and need another treatment.

Side Effects of ATG
When getting the IV, you may have:
■ Chills
■ Fever
■ Hives
These side effects go away after treatment.

Less Common Side Effects
Rarely, patients get a severe allergic reaction to ATG. This is called anaphylaxis (an-uh-fuh-LAK-suss), which causes a drop in blood pressure and trouble breathing. It can even cause death.

Before you receive ATG, you should have a skin test to find out if you are likely to develop anaphylaxis to this medicine. If you have a mild allergic reaction to the skin test, you can still take ATG. But you will need to start with a small amount to get your body used to it.

Another side effect of ATG is serum sickness. This happens when your immune system reacts to foreign proteins in the medicine. It causes fever, rash, joint pain, and muscle aches. If you get serum sickness, you will probably notice it 1 to 2 weeks after the first dose of ATG. You can take steroids (prednisone) during ATG treatment and for a few weeks afterward to prevent or treat serum sickness.
Cyclosporine

How Cyclosporine Works
Cyclosporine prevents T-lymphocytes from becoming active. Once the T-lymphocytes are turned off by the cyclosporine, they stop attacking stem cells in the bone marrow. Then the stem cells may be able to grow back.

How Cyclosporine Is Given
Cyclosporine comes in liquid and pill forms. The first dose is based on how much you weigh. Later doses depend on the amount of cyclosporine in your blood. Too little cyclosporine will not work. Too much will cause side effects. If you are responding well to cyclosporine, your doctor may decrease your dose over time.

How Well Does Cyclosporine Work?
- When used alone, cyclosporine is less effective than ATG.
- When used with ATG, cyclosporine improves blood counts in about 7 out of 10 cases.

Side Effects of Cyclosporine
Your doctor should monitor you for side effects. Cyclosporine may cause:
- High blood pressure
- Kidney damage
- Loss of magnesium or potassium
- Liver inflammation
- Increased hair growth
- Gum swelling

These problems can usually be fixed. You can take medicine to lower your blood pressure and to replace magnesium or potassium.

Other Immunosuppressive Drugs
Scientists are studying the use of other immunosuppressive drugs to treat aplastic anemia.

Stem Cell Transplantation

In this procedure, stem cells are taken from a healthy donor. They enter your bloodstream through an IV and make their way into your bone marrow. The donor cells are called a graft.

3 Sources of Stem Cells
The donor stem cells may come from 1 of 3 sources:

1. Bone Marrow
   Stem cells are collected from marrow inside the donor’s hipbone.

2. Cord Blood
   Stem cells are collected from an umbilical cord right after the birth of a baby. They are kept frozen until needed.

3. Circulating (Peripheral) Blood
   The donor is given a white blood cell growth factor. This increases the number of stem cells in the blood. Then stem cells are collected from the donor’s bloodstream.

Factors in Choosing Stem Cell Transplantation
Your doctor will consider several factors in deciding if a stem cell transplant is right for you. These factors include:
- Your physical condition
- How severe your disease is
- What type of donor is available
- Your age

Stem cell transplants have the best chance of bringing blood counts back to normal in children, teenagers, and young adults who have severe aplastic anemia. But there is no absolute upper age limit for stem cell transplantation.
Finding a Donor

Matched Related Donor

Q: My little sister has aplastic anemia and needs a stem cell transplant. How do I find out if I can be his donor?

A: Your sister’s transplant will be more likely to succeed if she can get a matched related donor. This involves finding a close relative – usually a brother or sister – with matched HLA (human leukocyte-associated) antigens. Leukocytes (LOO-kuh-sites) are white blood cells that protect your body from infection and remove harmful substances. HLA antigens are proteins on the surface of these cells. A blood test (or a cheek swab) can be used to see if your HLA antigens match hers.

Matched Unrelated Donor

About 7 out of 10 people can’t find a matched related donor. If you are in this position, your doctor can help you search for a matched unrelated donor, or MUD for short.

If you may be a candidate for a stem cell transplant in the future, start looking for a donor right away. It can take time to find a matched unrelated donor if no related donor is available.

What If There’s No Perfect Match?

Even if you cannot find a perfectly matched donor, you may still be able to receive stem cells from a partially matched donor. It depends on the type of mismatch.

Getting a Stem Cell Transplant

Before the Transplant

- Your remaining sick stem cells are destroyed to make room for healthy stem cells. Your immune system is also killed to prevent it from rejecting the new stem cells.
- Doctors generally give a chemotherapy drug called cyclophosphamide (Cytoxan) together with an immunosuppressive drug called ATG. To learn more about ATG, see page 20. Radiation is also given if the stem cells come from an unrelated donor. These treatments take about a week.
- Stem cells are collected from the donor.

During the Transplant

- Your doctor gives you an IV. The donor stem cells go right into your vein.
- The donor stem cells travel to your bones.

After the Transplant

- In 2 to 4 weeks, the new stem cells start making new, healthy blood cells. This process is called
engraftment. Your doctor keeps an eye on your blood counts to see how well the engraftment is going.

- Your new immune system grows. You take cyclosporine and possibly other medicines to prevent the new immune system from attacking your body. You may also take medicines to protect you from infections.

**How Well Does It Work?**

- About 8 out of 10 transplants with matched related donors succeed.
- About half of transplants with matched unrelated donors succeed.

**Stem cell transplants work best if you:**

- Are younger than 55 years old.
- Have a matched related donor.
- Have no other health problems.

**The chance of success goes down if:**

- There was a big lag in time (more than one year) between diagnosis and transplant.
- You have had many blood transfusions.
- You have had serious infections that weakened your body.

**What Can Go Wrong?**

Moving stem cells from one body into another is a complex process. Here are some things that can go wrong.

**Your Body Rejects the Graft**

In 5 to 10 out of 100 patients, the new stem cells do not grow. This is called graft rejection, or bone marrow failure. It is more likely to happen if many blood transfusions have been given before the transplant or if the graft came from an unrelated donor.

**The Graft Rejects and Attacks Your Organs**

Other transplants can make the patient sick because the donor’s immune system attacks the recipient’s body (usually skin, intestines, or liver). This condition is called GVHD (graft-versus-host disease).

GVHD’s symptoms can range from mild to deadly. In some cases, it can be prevented or treated with medicines that lower your immune response (immunosuppressive therapy).

**GVHD is more likely if:**

- You are an older adult.
- Your donor was not a close relative.
- Your donor was not a perfect match.

**GVHD and Cord Blood**

Even if cord blood is not a perfect match, it carries a lower risk of severe GVHD than mismatched bone marrow or peripheral blood stem cells. But the number of stem cells available in cord blood is small. So normally only people who weigh less than 80 pounds (40 kg) can find a cord blood unit that is big enough (has enough stem cells) for a transplant. Clinical trials are looking into the use of two cord blood units for people who weigh more.

**GVHD and Peripheral Blood Stem Cells**

Some researchers think peripheral blood stem cells are less likely to cause graft rejection, but more likely to cause GVHD.

**Mini (Non-myeloablative) Transplants**

Mini transplants use lower doses of chemotherapy than standard transplants do. This reduces the risk of side effects caused by chemo and the risk of infection during the transplant. This makes mini transplants a better choice for older adults. The procedure may carry a higher risk of graft rejection though, so they are not routinely done for aplastic anemia.
Other Treatments

If these treatments are not available to you or do not work well, you can explore other options. Many of these options are experimental and are available only through clinical trials. To learn more about clinical trials, see page 30.

Review

- Blood transfusion involves putting blood from a donor into your body. This raises blood counts temporarily.
- Iron overload therapy reduces high levels of iron in your blood.
- Growth factors can help more blood cells to grow and mature. This may raise blood counts temporarily.
- Antibiotics prevent and treat infection.
- Immunosuppressive drug therapy lowers your immune response. This prevents your immune system from attacking your bone marrow stem cells, lets stem cells grow back, and raises blood counts. This can cause a remission.
- Stem cell transplantation involves replacing your sick stem cells with healthy stem cells from a donor. This can cure the disease.
If you’re like most people with aplastic anemia, you want to stay in control as much as possible. Here are some ways to take an active role in your care. Take these 5 steps:

1. Find an Expert on Aplastic Anemia Who You Can Work With.

Because you have a rare disease, there are not many doctors who have a lot of experience treating aplastic anemia. Ask your regular doctor or your insurance company for the name of a blood expert who treats people with aplastic anemia. Or call a teaching hospital near you that is linked with a medical school.

Choose a doctor who treats people with aplastic anemia or other bone marrow problems. He or she is more likely to know about new treatments. Make sure your doctor clearly answers your questions, explains all of your treatment options, and includes you in making decisions.

2. Learn All You Can About Aplastic Anemia and Possible Treatment Options.

We congratulate you for reading this booklet and sharing it with your friends and family. To get other free materials on aplastic anemia, call us at (800) 747-2820, or visit us online at www.AAMDS.org.

3. Talk with Your Doctor and Other Healthcare Professionals.

Ask as many questions as you wish. If you are not sure what the doctor meant, ask again. Remember, your doctor is there to help you!

Here are 11 good questions to ask. See page 33 for a tear-out sheet you can bring to your doctor.

**About My Treatment**

1. What are all my treatment options?
2. What treatment option do you recommend for me? Why?
3. How likely am I to get better with the treatment?
4. Has this treatment been used a lot (standard)? Or is it a new or experimental treatment?
5. How long will the treatment take to work? When will I know if it is working?
6. Can my disease return, even after successful treatment?
About My Medicine

7. How do I take the medicine? How often do I need to take it?

8. How long will I need to take the medicine?

9. What are the common side effects of this medicine? What can be done to control them?

10. What are the long-term side effects of this medicine?

11. How much does it cost? Is it covered by my insurance?

4. Write down your medical information.

Keep a notebook with questions to take to your doctor visits. Ask a family member or friend to come along to help you take notes. Or track your health information on a computer. You can also write down your symptoms and treatment details in the back of this booklet.

5. Work with your doctor to make a treatment plan.

Work closely with your doctor to find the best treatment plan for you. Together, look at all the risks and benefits. This way, you can make an informed choice about your treatment.

Review

To take an active role in your health care:

■ Find an expert on aplastic anemia who you can work with.

■ Learn all you can about aplastic anemia and possible treatment options.

■ Talk with your doctor and other healthcare professionals.

■ Write down your medical information in a notebook or on a computer.

■ Work with your doctor to make a treatment plan.
“Ever since I got my diagnosis, my boyfriend has been trying to get me to eat better. For Valentine’s Day, he gave me a beautiful basket of fruit. I thought that was so sweet.”

— Sophie

The choices you make every day play a key role in managing your aplastic anemia. By taking good care of your body and mind, you allow yourself to be as healthy as possible.

Take Care of Your Body

Eat a Healthy Diet

There is no specific diet that makes aplastic anemia better or worse. Experts recommend a well-balanced diet. Your doctor can help you find the best eating plan for you.

Check with your doctor before taking any medicines, supplements, vitamins, or herbs. They may interact with your medicines and prevent them from working or increase the risks for side effects.

Get the Right Amount of Exercise

It’s good for your body to get some form of regular exercise. But since you have aplastic anemia, you may not have much energy to stay active. Plus, you may need to take special precautions. Ask your doctor what amount of exercise and what kinds of activity would work for you.

If you have a very low red blood cell count:

- Avoid any activity that hurts your chest, makes it very hard to breathe, or makes your heart beat fast.

- Don’t go up too high in the mountains or in an airplane. High altitudes can cause a shortage of oxygen and chest pain.

If you have a very low platelet count:

- Avoid any activities that could cause you to be bumped or bruised. You especially want to avoid hitting your head on something. Ask your doctor about safe ways to stay active. Tell your doctor if you get a bad headache or bleeding, or any of the other symptoms mentioned on page 9.

- Use a sponge (toothette) or very soft brush for your teeth often to prevent bleeding gums. Avoid flossing.

If you have a very low white blood cell count, protect yourself from germs:

- Use a sponge (toothette) or very soft brush for your teeth often to prevent bacteria from entering your bloodstream when you brush.

- Keep minor infections from getting serious. Tell your doctor if you have a fever or feel very tired. These can be early signs of infection.
■ Make sure your food is not too hot. Burns can cause infection in your mouth.
■ Stay away from crowds and sick people.
■ Carry hand gel that kills bacteria when you go out. Use it if you can’t find soap and water.

Take Care of Your Mind

Different people react in different ways to having aplastic anemia. Read what these patients have to say about their experience.

“I’m one of those people who has to be doing something. That’s the way I deal with stress. So I’m always on the computer, looking for new research. My doctor says I know almost as much as she does!”
— Roger

“I was feeling really depressed. Then I hooked up with this online support group for teenagers with bone marrow problems. We write to each other every day. They’re the only ones who really understand what I’m going through. It helps a lot.”
— Rashmi

“This disease has been the biggest challenge of my life. I’ve had it since I was 15, and for the past 2 years I’ve had to make some tough choices and look death in the face. I think it’s made me grow up faster and made me a better, stronger person.”
— Lanisha

Special Diet for Low White Count

If your white count is very low, your doctor may ask you to avoid certain foods that can make you sick. This is called a neutropenic (noo-truh-PEE-nik) diet. Take these steps to keep germs out of your food:

■ Cook your food well.
■ Avoid eating raw meats and raw fish, such as sushi.
■ Do not eat any cheeses that say “aged” on the label.
■ Drink only beverages that have been pasteurized to kill germs. Milk you buy from the supermarket is fine to drink.
■ Stay away from fermented drinks, such as homemade wine, cider, root beer, and vinegar.
■ Stay away from buffets, salad bars, and crowded restaurants.
■ Wash and peel fresh fruit and vegetables very well before eating them. Some doctors may ask you to avoid these foods as well unless they can be well cooked.
We Can Help

Aplastic anemia can be hard to cope with, but you don’t have to do it alone. The AA&MDSIF is here to help. Contact us to:

- Link up with other people who have aplastic anemia through the Peer Support Network.
- Get support from our Patient Information Specialists.
- Order our booklets on managing treatment decisions and other subjects related to living with aplastic anemia.

To contact us, call (800) 747-2820. Or visit us online at www.AAMDS.org.

Here are some other ways to take care of your mind:

- Join a support group.
- Learn more about aplastic anemia.
- Speak with a chronic illness counselor.

Review

To live successfully with aplastic anemia:

- Take care of your body.
- Take care of your mind.
About Clinical Trials

“My daughter did not respond well to standard treatments, so her doctor helped her get into a clinical trial. He says it’s her best chance of recovery.”

—Soo Jin

Scientists are always looking for new and better ways to treat aplastic anemia. They are conducting controlled, clinical trials, also called research studies. The studies are often done at university medical research centers around the world.

These research studies:

- Compare new treatments or new combinations of treatments with standard ones.
- Help scientists learn more about standard treatments.
- Test the safety and effectiveness of new treatments.

You may want to explore whether you may be eligible for a clinical trial if:

- Standard treatments have not made your aplastic anemia better.
- Your aplastic anemia came back after standard treatments.
- You are not satisfied with how well standard treatments work for you.

Before entering a clinical trial, discuss the possible risks and benefits with your doctor.

For more information on clinical trials for aplastic anemia patients, visit the AA&MDSIF website at www.AAMDS.org. Or call (800) 747-2820 and ask to speak with a patient education specialist.
Special Issues for People With Aplastic Anemia

“I really want to have children someday, but it looks like I’ll have to put those plans on hold. It just wouldn’t be safe for me – or for my baby.”

—Melinda

Because you have aplastic anemia, everyday events can be more risky for you than for healthy people. Here are some examples.

Airplane Travel and High Altitudes

The farther you get from Earth, the less oxygen there is. If you have aplastic anemia, flying in an airplane or going up high in the mountains may cause a shortage of oxygen. It may also cause chest pain. Changes in cabin pressure can cause bleeding inside the ear if platelets are very low. The recirculated air may increase risk of viral infection.

Before you do either of these things, it’s a good idea to:

■ Get a red blood cell count and a platelet count.
■ Get transfusions if necessary.
■ Ask your doctor if it is safe.

Pregnancy and Aplastic Anemia

Pregnancy is possible for women who have been treated for aplastic anemia. But it carries some significant risks for both mother and child.

There is limited research on pregnancy and aplastic anemia. However, one study done in 2002 showed that 19 percent of women had a relapse of aplastic anemia during pregnancy. This means that their blood counts went down. Some women also needed blood transfusions during childbirth.

This study also showed that you may have an increased risk of problems during pregnancy if:

■ You have a low platelet count.
■ You also have PNH. (See page 15.)

If you have had aplastic anemia and are pregnant or want to get pregnant, find an aplastic anemia specialist and an OB (obstetrician) who specializes in high-risk births. Every person and every pregnancy is different. Make sure you talk with the specialist about your specific case and understand all the risks.

Surgery

Surgery can also be risky for people with aplastic anemia. Surgery can cause serious bleeding in people with a low platelet count. Platelet transfusions may be needed before surgery. The risk for serious infections is also higher.

If you do have surgery, it’s a good idea to make sure your aplastic anemia specialist talks with your surgeon.

Review

Since you have aplastic anemia, these everyday events may be more risky for you:

■ Airplane travel and high altitudes
■ Pregnancy
■ Surgery
More Ways to Get Help

The Aplastic Anemia & MDS International Foundation (AA&MDSIF) is here to help. We provide the following services:

- Free support through our Patient Education Specialists.
- Free educational materials on many topics related to aplastic anemia.
- Online Learning Center with presentations by medical experts.
- Conferences for patients and family members.
- Events and training for healthcare professionals.
- Clinical trials information.
- Newsletters and eBulletins with important information and updates.

Contact us today. Here’s how:

- Call us: (800) 747-2820
- Email us: help@AAMDS.org
- Go to our website: www.AAMDS.org

Remember – you are not alone. We are standing by to support you in any way we can.
11 Good Questions to Ask Your Doctor (tear-out sheet)

About My Treatment

1. What are all my treatment options?

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___________________________________________________________________________________________

2. What treatment option do you recommend for me? Why?

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3. How likely am I to get better with the treatment?

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4. Has this treatment been used a lot (standard)? Or is it a new or experimental treatment?

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5. How long will the treatment take to work? When will I know if it is working?

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6. Can my disease return, even after successful treatment?

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About My Medicine

7. How do I take the medicine? How often do I need to take it?

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8. How long will I need to take the medicine?

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9. What are the common side effects of this medicine? What can be done to control them?

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10. What are the long-term side effects of this medicine?

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11. How much does it cost? Is it covered by my insurance?

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Important Information I Want to Remember:

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The Aplastic Anemia & MDS International Foundation, Inc. is an independent nonprofit organization. Our mission is to support patients, families, and caregivers coping with:

- Aplastic anemia
- MDS (myelodysplastic syndromes)
- PNH (paroxysmal nocturnal hemoglobinuria)
- Related bone marrow failure diseases

This booklet offers you information about aplastic anemia and how it is diagnosed and treated. Although the information in this booklet has undergone a thorough, independent medical review to insure its accuracy, this information is not intended to be a substitute for the advice of your doctor. You should always seek medical advice from a qualified physician.

For more information, call us at (800) 747-2820, or visit us online at www.AAMDS.org.

We wish to express special thanks to the following medical professionals and individuals who have assisted with review and editing of this booklet:

**Ronald L. Paquette, MD**
Adjunct Associate Professor, Department of Medicine
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Fighting Bone Marrow Failure Diseases Through Patient Support & Research Since 1985

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