LEUKEMIA &
LYMPHOMA
SOCIETY®

PROVIDING THE LATEST INFORMATION
FOR PATIENTS & CAREGIVERS

The MDS Guide:

Information for Patients and Caregivers

Revised **2020**

Support for this
publication provided by

Bristol-Myers Squibb Takeda | ONCOLOGY

A six-word narrative about living with blood cancer from patients in our LLS Community

Stay strong and keep moving forward. Find the positive in every day. Be your own best patient advocate. Changed my life for the better. **Accept, learn and focus on present. Learning to live a different life. Sudden and life changing—be positive. Waiting, worrying, anxiousness/happy I'm alive! Embrace a new normal each day.** 5 years, 41 infusions, constant fatigue. **Patience, positive attitude, hope and faith. Test to test, I will survive! Treatment, fatigue, treatment, fatigue and survival. Love life, live better every day.** I don't look back only forward. **So far, so good, live life. Meditation, mindfulness, wellness, faith, nutrition and optimism. Finding the joy while living with uncertainty. Watch, wait, treat, regroup, rest, re-energize.** Blessed to be doing so well! **Eye opening needed learning and healing. Feel great: uncertain travel plans annoying. Renewed faith, meditation, diet, mindfulness, gratitude. Watchful waiting can be watchful worrying.** Scary, expensive, grateful, blessings, hope, faith. **Thank god for stem cell transplants! Do not know what to expect. Extraordinarily grateful, I love my life. Diagnosed; frightened; tested; treating; waiting; hoping.** I'm more generous, impatient less often. **Embrace your treatment day after day. Live today, accept tomorrow, forget yesterday. Strength you never realized you had. Challenging to our hearts and minds.** Life is what we make it. **Live life in a beautiful way.**

Discover what thousands already have at **www.LLS.org/Community**

Join our online social network for people who are living with or supporting someone who has a blood cancer. Members will find

- **Thousands of patients and caregivers sharing experiences and information, with support from knowledgeable staff**
- **Accurate and cutting-edge disease updates**
- **The opportunity to participate in surveys that will help improve care.**

Inside This Guide

Question Guides:

New treatments may have been approved since this book was printed.
Check www.LLS.org/DrugUpdates or call (800) 955-4572.

Introduction

Myelodysplastic syndromes (MDS) are a group of blood diseases that start in the marrow. There are many types of MDS. An MDS is a type of blood cancer.

Between 2011 and 2015, there were about 70,000 new cases of MDS in the United States (an average of about 14,000 cases per year).

Today, there are many treatments to help people who have MDS. Yet there is more work to be done. New treatment options are available through clinical trials.

WANT MORE INFORMATION?

You can view, print or order the free LLS booklet *Myelodysplastic Syndromes* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

New treatments may have been approved since this book was printed.
Check www.LLS.org/DrugUpdates or call (800) 955-4572.

Feedback. Visit www.LLS.org/PublicationFeedback to make suggestions about the content of this booklet.

PART 1 Understanding MDS

Overview of This Section

- Blood cells are made inside the bones in the bone marrow. They begin as stem cells. Stem cells develop in the bone marrow and normally become healthy red blood cells, white blood cells or platelets. Then they leave the bone marrow and enter the bloodstream.
- MDS affects the blood and the bone marrow.
- MDS is diagnosed with blood and bone marrow tests.
- The International Prognostic Scoring System (IPSS) and other scoring systems are used as guides for doctors to determine the best treatment for a person who has been diagnosed with MDS.

About Bone Marrow, Blood and Blood Cells

The general descriptions below may help you understand the information in the rest of this Guide.

Bone marrow is the spongy center located inside the bone where blood cells are made.

Blood cells begin as stem cells in the bone marrow. Stem cells grow and mature into different types of cells: red blood cells, white blood cells and platelets. After the cells have matured, the red blood cells, white blood cells and platelets enter the bloodstream.

Red blood cells carry oxygen around the body. When the number of red blood cells is below normal, a condition called **anemia** usually develops. Anemia may make you feel tired or short of breath. It may make your skin look pale.

White blood cells fight infection in the body. There are two major types of white blood cells: lymphocytes and germ-eating cells.

- Lymphocytes are infection-fighting cells called
  - B cells
  - T cells
  - NK cells
- Germ-eating cells are called
  - Neutrophils
  - Monocytes

Platelets help stop bleeding by clumping together **(clotting)** at the site of an injury.

Plasma is the liquid part of the blood. Although mostly water, plasma also has some vitamins, minerals, proteins, hormones and other natural chemicals in it.

Normal Blood Cell Count Fast Facts

The ranges of blood cell counts below are for adults. They may be a little different from lab to lab and for children and teens.

Red blood cell (RBC) count

- Men: 4.5 to 6 million red cells per microliter of blood
- Women: 4 to 5 million red cells per microliter of blood

Hematocrit (the part of the blood made up of red cells)

- Men: 42% to 50%
- Women: 36% to 45%

Hemoglobin (amount of the red cell pigment that carries oxygen)

- Men: 14 to 17 grams per 100 milliliters of blood
- Women: 12 to 15 grams per 100 milliliters of blood

Platelet count (PLAT C)

- 150,000 to 450,000 platelets per microliter of blood

White blood cell (WBC) count

- 4,500 to 11,000 white cells per microliter of blood

Differential (also called **diff)**

- Shows the part of the blood made up of different types of white cells.
- The types of white cells counted are neutrophils, lymphocytes, monocytes, eosinophils and basophils.
- Adults usually have about 60% neutrophils, 30% lymphocytes, 5% monocytes, 4% eosinophils and less than 1% basophils in the blood.

About MDS

An MDS starts in the bone marrow. There is no way to prevent MDS and you cannot catch it. MDS is a cancer. This means:

- A normal cell changes (mutates).
- Changed cells act differently in the body.

Doctors also believe MDS are more frequently found in people who:

- Are older
- Have had certain types of cancer treatment, such as chemotherapy and/or radiation
- Have rare inherited bone marrow disorders, such as Fanconi anemia, aplastic anemia or dyskeratosis congenita

There are two types of MDS: primary (no cause can be identified) and secondary (treatment-related and less common). Most people diagnosed with MDS have primary MDS.

Signs and Symptoms

Healthy people often have a sign or a symptom when they get an illness or a disease.

- A sign is a change in the body that the doctor sees in an exam or a test result.
- A symptom is a change in the body that the patient can see or feel.

Diagnosing MDS can sometimes be hard. Often the signs and/or the symptoms can also be caused by another disease.

Signs and symptoms of MDS may include

- Fatigue
- Shortness of breath during physical activity
- Pale skin
- Dizziness
- Increased risk of bleeding and bruising
- Infection

Diagnosing MDS

There are many tests that will confirm a diagnosis of MDS.

These tests may include:

- A physical exam.
- Blood tests. One common blood test is called a complete blood count (CBC). This lab test counts the number of red blood cells, white blood cells and platelets in the blood. Usually, patients with MDS have lower-than-normal numbers of red blood cells, neutrophils (a type of white blood cell) and platelets.
- Bone marrow tests. There are two tests used most commonly to find the percentage of abnormal blood cells in the bone marrow and to confirm a diagnosis of MDS. These two tests are bone marrow aspiration and bone marrow biopsy.
- FISH (fluorescence in situ hybridization). This is a test that uses light and probes to identify damaged DNA in chromosomes. FISH is done using a blood or bone marrow sample.
- Molecular testing. These tests can be done on either a sample of blood or bone marrow to look for mutations in genes that are associated with MDS. Certain mutations are linked with either a better or a worse outcome. Doctors use the results of molecular testing to help plan treatment.

How Are the Blood and Bone Marrow Tests Done?

Blood Test — A small amount of blood is taken from the patient's arm with a needle. The blood is collected in tubes and sent to a lab.

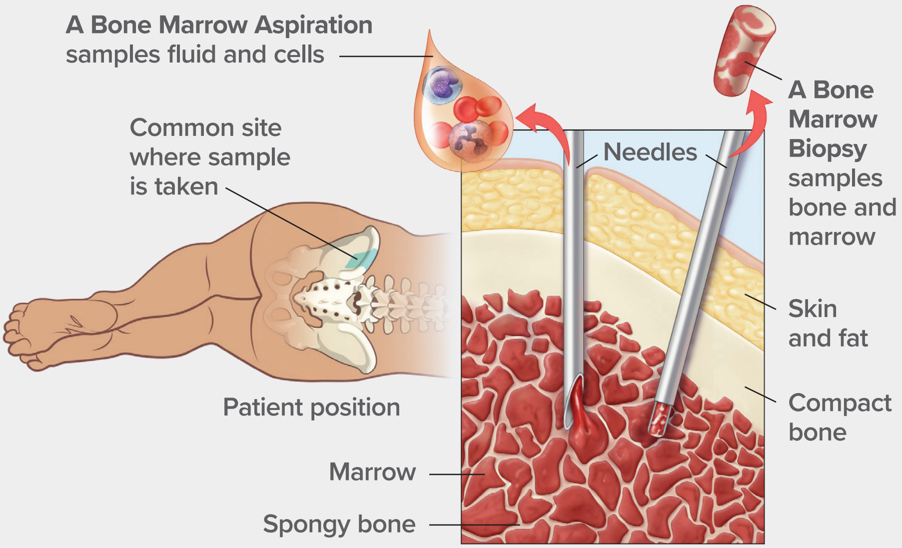
Bone Marrow Aspiration — The removal of a sample of fluid with cells from the bone marrow.

Bone Marrow Biopsy — A very small amount of bone filled with marrow cells is taken from the body.

Both bone marrow tests are done with a special needle. Some patients are awake for the procedure. They get medication first to numb the part of the body that will be used to take the sample of cells. Some patients are given a drug that makes them sleep during this procedure. The sample of cells is usually taken from the patient's hip bone.

Blood and marrow tests may be done in the doctor's office or in a hospital. A bone marrow aspiration and biopsy are almost always done at the same visit.

Bone Marrow Aspiration and Biopsy



Left: The place on the back of the patient's pelvic bone where a bone marrow aspiration or biopsy is done. **Right:** Where one needle goes into bone marrow to get a liquid sample for aspiration and the other needle goes inside the bone for a bone biopsy. The needles are different sizes.

Here are some questions you may want to ask your healthcare team. See pages 33-38 for a full list of questions.

1. What kind of testing will be done to diagnose my disease and to monitor my treatment?
2. How long does it take to get the results?
3. How are the results communicated to me?
4. How often will testing be needed?
5. Where will the testing be done?

Once all the tests are complete, a **hematopathologist** (a doctor or scientist who studies the blood cells and blood tissues to identify disease) will review the results.

It is important to talk to your doctor about the results of the tests. The results are needed for treatment planning.

A diagnosis of MDS requires that you have at least one low blood cell count (of red blood cells, white blood cells or platelets), and one of the following problems must be present in your bone marrow:

- Blast cells (immature cells) make up more than 5 percent of the marrow cells
- Damage to the chromosome DNA
- Changes to the structure or form of the bone marrow

The International Prognostic Scoring Systems and MDS Classification

Doctors often use different types of scoring systems for people who have MDS. These scoring systems were created to figure out the severity of a patient's disease.

The International Prognostic Scoring System (IPSS) is a guide, used along with your doctor's observation, to determine how to best treat you. It is the guide most commonly used by doctors. Although the IPSS does not take into account many factors, such as aging and its effect on the treatment of older patients, it does assign a patient's risk. The risk tells the doctor how quickly the disease will progress into one of four categories:

- Low
- Intermediate-1
- Intermediate-2
- High

Doctors also use The International Prognostic Scoring System-Revised (IPSS-R) score, a recent update of the IPSS. It assigns patient risk into five groups:

- Very low
- Low
- Intermediate
- High
- Very high

The classification of MDS was originally created by the French-American-British (FAB) Work Group, which classified MDS into five subtypes. In 2001, the World Health Organization (WHO) proposed another classification that was modified from the original FAB, incorporating molecular and cytogenetic factors. Since then, the WHO classification has been updated twice, once in 2008 and again in 2016.

The current WHO classification guidelines identify six subtypes of MDS based on the results of tests of the blood and bone marrow. The subtypes are:

1. MDS with single lineage dysplasia (MDS-SLD)
2. MDS with ring sideroblasts (MDS-RS)
    - Single lineage dysplasia (MDS-RS-SLD)
    - Multilineage dysplasia (MDS-RS-MLD)

3. MDS with multilineage dysplasia (MDS-MLD)
4. MDS with excess blasts (MDS-EB)
    - MDS with excess blasts-1 (MDS-EB1)
    - MDS with excess blasts-2 (MDS-EB2)
5. MDS with isolated del(5q)
6. MDS unclassifiable

For definitions of these subtypes, see *Health Terms* on page 27.

WANT MORE INFORMATION?

You can view, print or order the free LLS booklet *Myelodysplastic Syndromes* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

Tracking Your MDS Tests

These tips may help you save time and learn more about your health:

- Ask your doctor why certain tests are being done and what to expect.
- Discuss test results with your doctor.
- Ask for and keep copies of lab reports in a folder or binder. Organize the reports by date.
- Find out if and when follow-up tests are needed.
- Mark appointments that are coming up on your calendar.

WANT MORE INFORMATION?

You can view, print or order the free LLS booklet *Understanding Lab and Imaging Tests* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

Overview of This Section

- People with MDS should choose a doctor who is a specialist in both diseases of the blood and cancer. This type of doctor is called a **hematologist-oncologist.**
- Ask questions about your treatment choices and do not be afraid to be involved in making decisions about your own care. See the question guides *My First Doctor's Visit* and *Treatment and Follow-Up Care* on pages 33-38.
- People diagnosed with MDS can be treated by observation (watch-and-wait approach), clinical trials, supportive care, drug therapy or allogeneic stem cell transplantation.

Finding the Right Doctor

Choose a doctor who specializes in treating MDS and knows about the most up-to-date treatments. This type of specialist is a **hematologist-oncologist.** A hematologist is a doctor who has special training in disorders of the blood. An oncologist is a doctor who has special training in cancer. A hematologist-oncologist has special training in blood cancers.

Your local cancer specialist may work with an MDS specialist. Always check to see if the doctor's affiliated hospital or your chosen hospital is covered under your health insurance plan.

How to Find an MDS Specialist

- Ask your primary care doctor for a recommendation.
- Contact your community cancer center.
- Reach out to doctor and/or health plan referral services.
- Call an LLS Information Specialist at (800) 955-4572.
- Use online doctor-finder resources, such as
    - The American Medical Association's (AMA) "DoctorFinder" online at https://doctorfinder.ama-assn.org/doctorfinder/
    - The American Society of Hematology's (ASH) "Find a Hematologist" online at https://www.hematology.org/Patients/FAH.aspx

When you meet with the specialist, ask questions to get a better idea of the doctor's experience and to understand how the office works. Some sample questions are on page 11. See pages 33-34 for a full list of questions.

1. How many patients have you treated who have this disease?
2. What problems or symptoms should be reported to the nurse or doctor right away?
3. Is there a release form available so my family/caregiver can be given medical information?

Make sure you feel comfortable interacting with the doctor and the rest of the staff. You will be spending a lot of time speaking with this staff and others at this treatment center.

WANT MORE INFORMATION?

You can view, print or order the free LLS booklet *Choosing a Blood Cancer Specialist or Treatment Center* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

Ask Your Doctor

Talk with the doctor and ask questions about how he or she plans to treat your MDS. This will help you become actively involved in making decisions about your care.

When you meet with your doctor

- Ask questions. Below are a few questions to ask. See pages 33-38 at the end of this Guide for a full list of questions. Visit www.LLS.org/WhatToAsk to find other healthcare question guides.
  - What are my treatment choices?
  - Are there any clinical trials that I can join?
  - When do you think treatment should begin?
  - How long will treatment last?
- Take notes. It may be helpful to write down the answers to your questions and review them later.
- Audio record information from the doctor and then listen to the recording later on. Ask the doctor and staff if recording is okay (cell phones have a "record" function; ask someone how to use it).
- Bring a caregiver, friend or family member who can listen to the doctor along with you, take notes and offer support.
- Make sure you understand what the doctor is saying. If you do not understand, ask the doctor to explain it again.

If you need more information or are not sure about your treatment choices, consider getting another opinion (a "second opinion") from a different qualified doctor. If you are unsure or feel uncomfortable about how to tell your doctor you are getting a second opinion, call our Information Specialists at (800) 955-4572 to discuss a way that makes you comfortable. You may also want to check with your health insurance plan to ensure that a second opinion will be covered.

Treatment Planning

There are many treatments for MDS. Your doctor will create a plan that is best for you.

Treatment goals for lower-risk MDS patients include:

- Managing the disease by reducing transfusion needs
- Improving blood counts
- Decreasing the risk of infection
- Increasing the number of good-quality years of life

Treatment goals for higher-risk MDS patients include:

- Increasing life expectancy (shorter for high-risk than it is for low-risk MDS patients)
- Slowing or preventing MDS from progressing to acute myeloid leukemia (AML), another type of leukemia

About MDS Treatments

New treatments may have been approved since this book was printed.
Check www.LLS.org/DrugUpdates or call (800) 955-4572.

Work with your doctor to create a treatment plan. Make sure you understand it, and if you don't, ask questions. Call our Information Specialists to help you know what questions to ask. You may be treated with drugs that are different from the ones you see in this MDS Guide and still be getting good treatment.

Here are some questions you may want to ask your healthcare team. See pages 33-38 for a full list of questions.

1. What is the subtype?
2. What are the treatment options, including clinical trials?
3. What is the goal of treatment?

4. What are the benefits and risks of these treatments?

5. Is one treatment recommended over others?

Treatment options include:

- Observation with regular blood counts (watch and wait)
- New approaches under study (clinical trials)
- Supportive therapy
  - Blood transfusions
  - Iron chelation therapy
  - Blood cell growth factors
  - Managing infections
- Drug therapy
- Allogeneic stem cell transplantation

Observation (Watch and Wait). Sometimes patients do not need treatment right away. A "watch-and-wait" approach allows the doctor to check the patient's condition and blood count, but not to actually start treatment with drugs or other therapy until it is needed. Patients may think that they should have treatment right away. But watch-and-wait with monitoring allows the patient to avoid the side effects of therapy until treatment becomes necessary. Patients in watch-and-wait protocols:

- Need to continue to see their doctor regularly
- Should undergo regular tests and exams, as advised
- Tell their doctor about any changes or symptoms
- Understand that if there are signs that their MDS is progressing, treatment may become necessary

Blood Transfusions. Some patients with symptoms are treated with either transfusions (the process of taking blood from a donor and giving it to the patient intravenously) or with growth factors. Both these processes increase low blood counts and can help make patients feel better.

Before a blood transfusion, doctors will:

- Check a patient's hemoglobin levels (the protein in the red blood cells that carries oxygen)
- Look for symptoms (such as fatigue or shortness of breath) and possibly identify other health problems, such as heart disease

Platelet transfusions may also be needed.

WANT MORE INFORMATION?

You can view, print or order the free LLS booklet *Blood Transfusion* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

Iron Chelation Therapy. Iron chelation therapy uses medicine to remove the extra iron in the body, a condition called "iron overload." Because anemic patients get many blood transfusions, they may be at risk for iron overload, a disorder that can damage the heart and liver. This therapy may be used for anemic patients, who depend on frequent transfusions.

The common FDA-approved drugs used for iron overload include:

- **Deferasirox (Exjade® or Jadenu®)**—This oral medication is in pill form and taken daily. Jadenu and Exjade are the same medication, but Jadenu may be easier to digest.
- **Deferoxamine mesylate (DFO; Desferal®)**—This drug is administered as a slow subcutaneous (under-the-skin, called SC) or intramuscular (into a muscle, called IM) infusion.

Blood Cell Growth Factors. Agents called growth factors help your body make blood cells. There are red blood cell and white blood cell growth factors.

Red Blood Cell Growth Factors

Erythropoietin (EPO) is a hormone created in the kidneys. It helps the production of red blood cells when your body has low oxygen levels. A shortage of EPO can also cause anemia.

Erythropoiesis-stimulating agents (ESAs) are red blood cell growth factors that are man-made versions of natural EPO. They are used to treat MDS patients who have anemia associated with low EPO levels. Examples of these include **darbepoetin alfa (Aranesp®)** and **epoetin alfa (Procrit®).**

Most patients with MDS do not have low EPO levels, so administration of erythropoiesis-stimulating agents (ESAs) is not useful in treating their anemia. Nonetheless, all MDS patients should have their EPO levels checked.

White Blood Cell Growth Factors

- Granulocyte-colony stimulating factor (G-CSF) is a hormone that increases white blood cell production. Some MDS patients with low EPO levels may not benefit from an ESA alone, but if an ESA is given along with a granulocyte-colony stimulating factor (G-CSF), the combination may increase a patient's hemoglobin levels. Examples of these drugs include **filgrastim (Neupogen®)** and **pegfilgrastim (Neulasta®).**

- Granulocyte macrophage-colony stimulating factor (GM-CSF) is another growth factor. **Sargramostim (Leukine®)** is also an example of a GM-CSF medication.

These drugs can help patients with low neutrophil (white blood cell) counts who develop infections.

Managing Infections. A low number of white blood cells can increase a patient's risk for infection. In some cases, infection occurs frequently and it can sometimes be severe. It is important for patients to pay close attention to any infection or unexplained fever, because antibiotics or antiviral drugs may be needed.

Drug Therapy. The following drugs have been approved by the FDA for patients who have MDS:

- **Azacitidine (Vidaza®)**—This drug, given by intravenous (IV) infusion or subcutaneously (under the skin), is approved for the treatment of both low-risk and high-risk patients. It helps the bone marrow begin to work more normally. It also kills the unhealthy cells in the bone marrow. In studies, treatment with azacitidine has led to improved quality of life along with blood transfusions, as needed. It has also reduced the need for transfusions.
- **Decitabine (Dacogen®)**—This drug, given through a slow IV infusion and must be given in a hospital setting, is approved for the treatment of both low-risk and high-risk patients. In studies, decitabine reduced the need for blood transfusions and improved blood counts in some patients.
- **Lenalidomide (Revlimid®)**—This drug, taken by mouth, is approved for the treatment of patients with transfusion dependent anemia caused by low-risk or intermediate-1–risk MDS. This drug works best for patients who have certain cytogenetic changes. Talk to your doctor for more information.
- In specific situations, **imatinib mesylate (Gleevec®)**, taken by mouth, is FDA-approved for adult patients with myelodysplastic syndromes and myeloproliferative neoplasms (MDS/MPN) associated with platelet-derived growth factor receptor (PDGFR) gene rearrangements.

Chemotherapy regimens may consist of a single drug or combinations of two or three different drugs. Chemotherapy is treatment with drugs that kill or damage cancer cells. Each drug type works in a different way. Newer drugs are being tested in clinical trials.

Patients with intermediate-2 and high-risk International Prognostic Scoring System (IPSS) categories may need treatment similar to that given to patients who have acute myeloid leukemia (AML).

Some of these drugs include:

- **Cytarabine (cytosine arabinoside, ara-C; Cytosar-U®)**
- **Idarubicin (Idamycin®)**
- **Daunorubicin (Cerubidine®)**
- **Mitoxantrone (Novantrone®)**

WANT MORE INFORMATION?

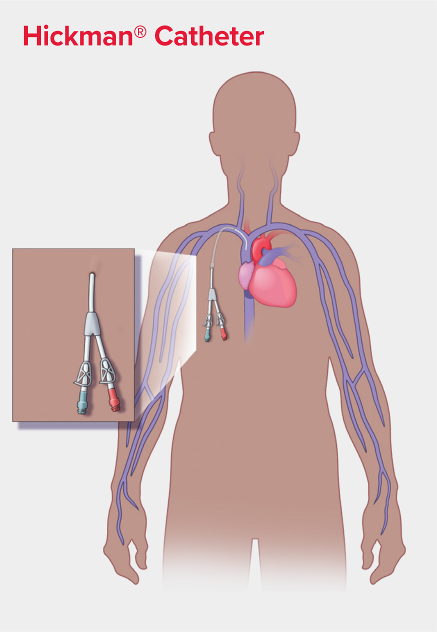
You can view, print or order the free LLS fact booklets *Myelodysplastic Syndromes* and *Acute Myeloid Leukemia* at **www.LLS.org/booklets,** or contact our Information Specialists for copies.

Central Lines, Ports and PICCs

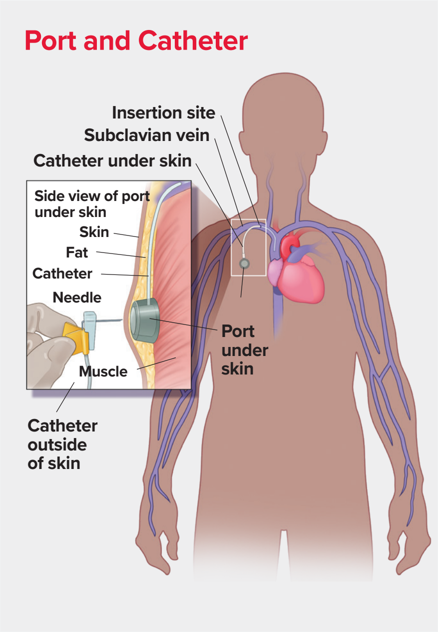
Central Line (catheter) — A thin tube that is put under the skin and into a large vein in the chest. The central line stays firmly in place. “Indwelling catheter” is another word for central line.

Port — This is a small device that is used with a central line. The port is placed under the skin of the chest. After the site heals, no dressings are needed and no special home care is required. The doctor or nurse can give medicines or nutrition to the patient or take blood samples from the patient using the port. A needle is simply inserted through the skin into the port. A numbing cream can be put on the skin before the port is used.

PICC or PIC Line (short for “percutaneously inserted central venous catheter”) — A PICC or PIC (not shown) is inserted through a vein in the arm.


Hickman® Catheter

Hickman® Catheter: An example of a type of central line.


Port and Catheter

Port: A port used with a central line.

© Fran Milner 2018

Allogeneic Stem Cell Transplantation. Your doctor will talk with you about whether stem cell transplantation is a treatment option for you. Intensive radiation and/or chemotherapy, followed by an allogeneic stem cell transplantation, has the best-known potential to cure MDS. This treatment is not for all patients and it has a high risk of serious complications. Talk to your doctor to see if stem cell transplantation is a treatment option for you.

In allogeneic stem cell transplantation, stem cells from another person (a donor) are infused into your body. The donor may be a brother or sister. Or the donor can be an unrelated person with stem cells that “match” the patient’s. Stem cells may also come from cord blood (the blood in the umbilical cord retrieved after a baby’s birth).

The goals of an allogeneic transplant are to:

- Restore the body’s ability to make healthy blood cells after high-dose chemotherapy
- Cure the patient by killing remaining MDS cells

This procedure may be done in the hospital. First, the patient is given high-dose chemotherapy and/or radiation therapy. Stem cells are collected from a donor. The donor stem cells are given to the patient through an IV (intravenous) line or a central line. The donor stem cells go from the patient’s blood to the marrow. They help start a new supply of healthy red blood cells, white blood cells and platelets.

Stem cell transplant may be an option for MDS patients if:

- They are either IPSS intermediate-2 or high-risk category, or if they have therapy-related (secondary) MDS
- The expected benefits of an allogeneic transplant exceed the risks
- There is a stem cell donor

Reduced-Intensity Allogeneic Stem Cell Transplantation. Your doctor will talk to you about whether a reduced-intensity allogeneic stem cell transplant is a treatment option. This procedure uses lower doses of chemotherapy than the doses that are used in a standard allogeneic transplant. Some older and sicker patients may be helped by this treatment. The goal of a reduced-intensity allogeneic transplant is to cure the patient of his or her MDS by killing remaining MDS cells and to start a new supply of healthy cells.

WANT MORE INFORMATION?

You can view, print or order the free LLS publications *Blood and Marrow Stem Cell Transplantation Guide* and *Cord Blood Stem Cell Transplantation Facts* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

PART 3 Clinical Trials

About Clinical Trials

Doctors may recommend that a patient join a clinical trial. Clinical trials are careful studies done by doctors to test new drugs or treatments, or to test new uses for approved drugs or treatments. For example, changing the amount of the drug or giving the drug along with another type of treatment might be more effective than a standard treatment. Some clinical trials combine drugs for MDS in new sequences or dosages.

There are clinical trials for:

- Newly diagnosed MDS patients
- MDS patients of all ages
- Patients who have relapsed, or patients who do not get a good response to treatment

A carefully conducted clinical trial may provide the best available therapy.

Here are some questions you may want to ask your healthcare team. See pages 33-38 for a full list of questions.

1. Is a clinical trial a treatment option?
2. How can I find out if insurance covers the cost of the clinical-trial treatment and treatment-related costs such as testing?
3. Who pays for the travel costs to get to the clinical trial?

Ask your doctor if treatment in a clinical trial is right for you. For more information, please call (800) 955-4572 to speak with an LLS Information Specialist who can provide more information about clinical trials and help search for a clinical trial for you. When appropriate, patients and caregivers can work with Clinical Trial Nurse Navigators who will help find clinical trials and assist throughout the entire process. **Visit our Clinical Trial Support Center at www.LLS.org/CTSC for more information.**

WANT MORE INFORMATION?

You can view, print or order the free LLS publication *Knowing All Your Treatment Options* and *Understanding Clinical Trials for Blood Cancers* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

Overview of This Section

- A side effect is an unintended result from a treatment or a drug.
- Treatment side effects vary depending on the type of treatment.
- Some of the side effects of treatment for MDS may include: fever, chills, coughing, sore throat, pain when urinating, frequent loose bowel movements and other side effects.
- MDS patients will meet with their primary care doctor and hematologist-oncologist for follow-up care.

Side Effects of MDS Treatment

A side effect is an unintended result from a treatment or a drug. Sometimes the side effects are bad, but often they go away once the treatment ends. Ask your doctor about what side effects to expect from your treatment. Learn what they are. Side effects can be managed.

Here are some questions you may want to ask your healthcare team. See pages 33-38 for a full list of questions.

1. What side effects should be reported to the team right away?
2. What are the common side effects of this treatment?
3. How long will the side effects last?
4. How can potential side effects be prevented or managed?

Side effects that affect your blood counts include

- Anemia. A health condition caused by a reduction in the number of red blood cells. People with severe anemia may be pale, weak, tired, and become short of breath very easily. To help increase the red blood cell count, doctors often use a red blood cell transfusion, a process in which red blood cells are donated and given to the patient intravenously.
- Thrombocytopenia. A health condition caused by a drop in the number of platelets. To help increase the number of platelets, doctors often do a platelet transfusion, a process during which platelets are donated and given to the patient intravenously.
- Neutropenia. A health condition diagnosed when there is a big drop in the number of white blood cells (WBCs) called neutrophils. A large decrease

in the number of neutrophils may result in an infection. The doctor may talk about the “absolute neutrophil count” (ANC), which is the number of neutrophils a person has for fighting infections. Infections are usually treated with antibiotics. MDS patients are more likely to get infections than other people.

Look out for the following symptoms and tell your doctor right away if you experience any of them:

- Fever
- Chills
- Coughing
- Sore throat
- Pain when urinating
- Frequent, loose bowel movements

To lower the risk of infection:

- Use good handwashing skills—patients, their visitors and medical staff need to wash their hands well.
- Keep the patient’s central line clean.
- Practice good dental hygiene—patients should take good care of their teeth and gums.

Chemotherapy-Related Side Effects. Chemotherapy affects the parts of the body where new cells are forming quickly. This includes the inside of the mouth and the bowel as well as the skin and hair. These associated side effects are common during chemotherapy:

- Mouth sores
- Diarrhea
- Hair loss
- Rashes
- Nausea
- Vomiting

Not all patients have these side effects. Treatment to prevent or manage nausea, vomiting, diarrhea and other side effects can help patients feel more comfortable.

WANT MORE INFORMATION?

You can view, print or order the free publication *Dental and Oral Complications of Cancer Treatment Facts* and the series about *Side-Effect Management* at **www.LLS.org/booklets,** or contact our Information Specialists for a copy.

Follow-Up Care

Patients should see their primary care doctors and their hematologist-oncologist (cancer specialist) regularly for follow-up care. Ask your doctor about how often you should have follow-up visits. At these visits, the doctor will check you and learn how you are doing. Additional blood tests or even bone marrow tests may be needed to evaluate your treatment progress and to learn if more treatment is needed. The doctor may advise longer periods of time between follow-up visits; however, follow-up visits should be ongoing.

You can also ask the doctor what tests will be needed, and find out how often you should have these tests. It is important to get and keep a record of your cancer treatment, including the drugs you receive and the time period you receive them, so that your doctor can follow up on specific long-term effects that may be associated with your treatment. See page 39 for a place that you can list your treatments.

Here are some questions you may want to ask your healthcare team. See pages 33-38 for a full list of questions.

1. Who will I work with to ensure life-long follow-up care?
2. Will I continue to see this healthcare team?
3. What information can be given to my primary doctor about past treatment and what may be needed in the future?

Take Care of Yourself

Whether you still have MDS or if you have been treated for it, it is very important that you take good care of yourself. Review the following tips to keep yourself healthy and happy:

- Keep all doctors' appointments.
- Talk about how you feel with the doctor at each visit.
- Ask any questions you may have about side effects.
- People with MDS may have more infections. Follow the doctor's advice for preventing infection.
- Receive certain vaccinations including flu, pneumonia and shingles vaccines. Vaccines should not be "live vaccines" and your doctor will know the difference. For example, patients who have MDS can receive the shingles vaccine Shingrix® because it is an "inactivated" rather than a "live" vaccine. Speak to your doctor for more information.
- Eat healthy foods each day. It is okay to eat four or five smaller meals instead of three bigger ones.

- Contact the doctor about tiredness, fever or other symptoms.
- Do not smoke. Patients who smoke should get help to quit.
- Get enough rest.
- Exercise, but first talk with your doctor before starting an exercise program.
- Keep a healthcare file with copies of lab reports and treatment records.
- Have regular cancer screenings.
- See your primary care doctor to keep up with other healthcare needs.
- Talk with family and friends about how you feel. When family and friends know about MDS and its treatment, they may worry less.
- Seek medical advice if you feel sad or depressed and your mood does not improve over time. If you feel sad or depressed every day for a 2-week period, seek help. Depression is an illness. It should be treated even when a person is being treated for an MDS. Treatment for depression has benefits for people living with cancer.

Resources and Information

LLS offers free information and services for patients and families affected by blood cancers. This section of the booklet lists various resources available to you. Use this information to learn more, to ask questions, and to make the most of your healthcare team.

For Help and Information

Consult with an Information Specialist. Information Specialists are master's level oncology social workers, nurses and health educators. They offer up-to-date information about disease, treatment and support. Language services are available. For more information, please

- Call: (800) 955-4572 (Monday through Friday, 9 am to 9 pm ET)
- Email: infocenter@LLS.org
- Live chat: www.LLS.org/InformationSpecialists
- Visit: www.LLS.org/InformationSpecialists

Clinical Trials (Research Studies). Research is ongoing to develop new treatment options for patients. LLS offers help for patients and caregivers in understanding, identifying and accessing clinical trials. When appropriate, patients and caregivers can work with Clinical Trial Nurse Navigators who will help find clinical trials and personally assist throughout the clinical trial process. Visit our Clinical Trial Support Center at www.LLS.org/CTSC for more information.

Free Information Booklets. LLS offers free education and support booklets that can either be read online or ordered. Please visit www.LLS.org/booklets for more information.

Telephone/Web Education Programs. LLS offers free telephone/Web and video education programs for patients, caregivers and healthcare professionals. Please visit www.LLS.org/programs for more information.

Financial Assistance. LLS offers financial support, including insurance premium and medication co-pay assistance, to eligible individuals with blood cancer. For more information, please

- Call: (877) 557-2672
- Visit: www.LLS.org/Finances

LLS Health Manager™ App. This free mobile app helps you manage your health by tracking side effects, medication, food and hydration, questions for your doctor, and more. Export the information you've tracked in a calendar format and share it with your doctor. You can also set up reminders to take medications, hydrate, and eat. Visit www.LLS.org/HealthManager to download for free.

One-on-One Nutrition Consultations. Access free one-on-one nutrition consultations provided by a registered dietitian with experience in oncology nutrition. Dietitians assist callers with information about healthy eating strategies, side effect management, and survivorship nutrition. They also provide additional nutrition resources. Please visit www.LLS.org/nutrition for more information.

Podcast. *The Bloodline with LLS* is here to remind you that after a diagnosis comes hope. Listen in as patients, caregivers, advocates, doctors and other healthcare professionals discuss diagnosis, treatment options, quality-of-life concerns, treatment side effects, doctor-patient communication and other important survivorship topics. Visit www.LLS.org/TheBloodline for more information and to subscribe.

Suggested Reading. LLS provides a list of selected books recommended for patients, caregivers, children and teens. Visit www.LLS.org/SuggestedReading to find out more.

Community Resources and Networking

LLS Community. The one-stop virtual meeting place for talking with other patients and receiving the latest blood cancer resources and information. Share your experiences with other patients and caregivers and get personalized support from trained LLS staff. Visit www.LLS.org/community to join.

Weekly Online Chats. Moderated online chats can provide support and help cancer patients reach out and share information. Please visit www.LLS.org/chat for more information.

LLS Chapters. LLS offers community support and services in the United States and Canada including the *Patti Robinson Kaufmann First Connection Program* (a peer-to-peer support program), in-person support groups and other great resources. For more information about these programs or to contact your chapter, please

- Call: (800) 955-4572
- Visit: www.LLS.org/ChapterFind

Other Helpful Organizations. LLS offers an extensive list of resources for patients and families. There are resources that provide help with financial assistance, counseling, transportation, patient care and other needs. For more information, please visit www.LLS.org/ResourceDirectory to obtain the directory.

Advocacy. The LLS Office of Public Policy (OPP) enlists volunteers to advocate for policies and laws to speed new treatments and improve access to quality medical care. For more information, please

- Call: (800) 955-4572
- Visit: www.LLS.org/advocacy

Additional Help for Specific Populations

Información en Español (LLS information in Spanish). Please visit www.LLS.org/espanol for more information.

Language Services. Let members of your healthcare team know if you need translation or interpreting services because English is not your native language, or if you need other assistance, such as a sign language interpreter. Often these services are free.

Information for Veterans. Veterans who were exposed to Agent Orange while serving in Vietnam may be able to get help from the United States Department of Veterans Affairs. For more information, please

- Call: the VA (800) 749-8387
- Visit: www.publichealth.va.gov/exposures/AgentOrange

World Trade Center Survivors. People involved in the aftermath of the 9/11 attacks and subsequently diagnosed with a blood cancer may be able to get help from the World Trade Center (WTC) Health Program. People eligible for help include

- Responders
- Workers and volunteers who helped with rescue, recovery and cleanup at the WTC-related sites in New York City (NYC)

- Survivors who were in the NYC disaster area and those who lived, worked or were in school in that area
- Responders to the Pentagon and the Shanksville, PA, crashes

For more information, please

- Call: WTC Health Program at (888) 982-4748
- Visit: www.cdc.gov/wtc/faq.html

People Suffering from Depression. Treating depression has benefits for cancer patients. Seek medical advice if your mood does not improve over time, for example, if you feel depressed every day for a two-week period. For more information, please

- Call: The National Institute of Mental Health (NIMH) at (866) 615-6464
- Visit: NIMH at www.nimh.nih.gov and enter “depression” in the search box

Health Terms

Absolute neutrophil count (ANC). The number of neutrophils (a type of white blood cell) that a person has to fight infection.

Acute myeloid leukemia (AML). A fast-moving cancer that starts with the change of a young cell in the bone marrow.

Anemia. A health condition caused by a lower-than-normal number of red blood cells in the blood.

Antibiotics. Drugs to treat infections. Penicillin is a type of antibiotic.

Blast cells. Early (or immature) bone marrow cells. About 1 to 5 percent of all normal marrow cells are blast cells.

Blood cell count. A laboratory test that uses a small blood sample to measure the number and types of cells circulating in the blood. The term complete blood count (CBC) is often used to refer to this test.

Bone marrow aspiration. A procedure to remove liquid from the bone marrow. Experts examine these cells under a microscope to look for abnormal cells.

Bone marrow biopsy. A procedure to remove a small amount of bone along with bone marrow cells. Experts examine this sample under a microscope to look for abnormal cells.

Central line. A special tube (called a catheter) is put into a large vein in the patient's upper chest. It is used to give medicines, fluids or blood products, and to take out blood samples. Also called indwelling catheter.

Chemotherapy. The use of chemicals to kill cancer cells.

Chromosomes. Threadlike structures in each cell that carry a person's genes. Genes tell the cells what to do. In each cell, there are 23 basic structures (chromosomes). When a patient has blood cancer, the number or shape of chromosomes may be changed.

Clinical trials. Studies done by healthcare experts who test new drugs or treatments and explore new uses and combinations of drugs with the hope of either finding a cure or improving quality of life.

Combination chemotherapy or drug therapy. Using two or more drugs together to fight MDS and other cancers.

Diagnose. To detect a disease from a person's signs, symptoms and test results. Doctors diagnose patients.

DNA. The genetic matter found in all cells.

FDA. The short name for the US Food and Drug Administration. The FDA is a federal agency that assures the safety and security of drugs, medical devices and the US food supply.

Growth factor. A chemical used to help your body make blood cells. There are red blood cell and white blood cell growth factors.

Hematologist. A doctor who has special training in treating blood cell diseases.

Hematologist-Oncologist. A doctor who has special training in treating blood cancers.

Hemoglobin. The part of the red blood cell that carries oxygen.

Leukemia. A cancer of the marrow and blood.

Marrow. The spongy material in the center of bones. All blood cells are made in the bone marrow.

MDS, unclassifiable (MDS-U). An MDS subtype that includes patients who do not have refractory anemia or any other MDS subtype.

MDS with excess blasts (MDS-EB). A type of MDS that shows signs of leukemic blast cells when the blood or marrow is examined. There may only be a small number of these blast cells in the marrow, but their presence indicates that leukemia cells are developing.

MDS with isolated del(5q). A subtype of MDS that causes refractory (treatment-resistant) anemia that is associated with a deletion of the long arm (q) of chromosome 5, designated "del(5q)."

MDS with multilineage dysplasia (MDS-MLD). A subtype of MDS in which there are too few of at least two types of blood cells (red blood cells, white blood cells, or platelets). In the bone marrow, those same types of cells look abnormal under the microscope.

MDS with ring sideroblasts (MDS-RS). This type of MDS shows signs of blast cells in the blood and marrow.

MDS with single lineage dysplasia (MDS-SLD). A subtype of MDS in which the marrow shows abnormal changes in only one of the three types of blood cells; there are one or two low blood cell counts of red blood cells, white blood cells and platelets in the blood; and there is less than 5 percent of blasts cells (immature cells) in the bone marrow.

Oncologist. A doctor who has special training in treating cancer.

Plasma. The liquid part of the blood.

Platelet. A type of blood cell that helps the body stop bleeding. Platelets cause plugs (by clotting) to form in the blood vessels at the site of an injury.

Radiation therapy. Treatment with x-rays or other high-energy rays.

Signs and symptoms. Changes in the body that show disease. A sign is a change that the doctor sees in an exam or as a result of a lab test. A symptom is a change that a patient can see or feel.

Stem cell. A type of cell in marrow that makes red blood cells, white blood cells and platelets.

My Healthcare Team Contact List

Use this list to remember names and contact information for members of your healthcare team.

CAREGIVER NAME:

Address: ____
Phone Number/Fax number: ______________________________
Email address: _____________________________________
Additional information: _________________________________

PRIMARY CARE DOCTOR NAME:

Address: ____
Phone Number/Fax number: ______________________________
Email address: _____________________________________
Additional information: _________________________________

PHARMACY NAME:

Address: ____
Phone number/Fax number: _______________________________
Additional information: _________________________________

Information Specialists:
Phone: 1-800-955-4572
Email: infocenter@LLS.org
Website: www.LLS.org/InformationSpecialists

HEMATOLOGIST-ONCOLOGIST NAME:

Address: ____

Phone number/Fax number: ____

Email address: ____

Website/Portal: ____

Additional information: ____

NURSE/NURSE PRACTITIONER NAME:

Phone number/Fax number: ____

Email address: ____

Additional information: ____

SOCIAL WORKER NAME:

Address: ____

Phone number/Fax number: ____

Email address: ____

Additional information: ____

INSURANCE CASE MANAGER/
CARE COORDINATOR NAME:

Address: ____

Phone number/Fax number: ____

Website or email address: ____

Additional information: ____

PHYSICIAN ASSISTANT NAME:

Address: ______

Phone number/Fax number: ______

Email Address: ______

Additional information: ______

NURSE NAVIGATOR NAME:

Address: ______

Phone number/Fax number: ______

Email address: ______

Additional information: ______

OTHER:

Address: ______

Phone number/Fax number: ______

Email address: ______

Additional information: ______

OTHER:

Address: ______

Phone number/Fax number: ______

Email address: ______

Additional information: ______

Question Guide: My First Doctor's Visit

Asking questions will help you take an active role in managing your (or your loved one's) care. If you do not understand any part of the information your healthcare provider gives you, ask him or her to explain it in another way. The following are questions you may want to ask your healthcare team.

When you meet with the doctor, nurse and healthcare team, ask a few questions to get a better idea of the doctor's experience and to understand how the office works.

(Note: The use of "I (we)" and "me (us)" in lists of questions is used for situations in which the patient may not be old enough or able to make his or her own decision. A parent, relative, or caregiver may be assisting or making the decision.)

Questions for the Doctor

DOCTOR'S NAME: ____

Date of appointment or phone call:____________________________________

1. How many patients have you treated who have this disease?
2. What problems or symptoms should be reported to the nurse or doctor right away?
3. How long does it normally take to receive a return phone call?
4. How can you be contacted when there are questions?
5. How can you be contacted at night? On weekends? On holidays?
6. Who are the other members of the team that I should be aware of?
7. Is there a release form available so my family/caregiver can be given medical information?

Questions for the Nurse

NURSE OR OTHER HEALTHCARE TEAM MEMBER'S NAME:

____

1. How long would I (we) have to wait for appointments?
2. What problems or symptoms should be reported to the nurse or doctor right away?
3. How long does it usually take to receive a return phone call?
4. Will there be nurses, social workers and case managers available to help with support needs and quality-of-life concerns?
5. Does your office accept my (our) insurance? Is it considered in-network?

To print copies of other question guides, go to www.LLS.org/WhatToAsk or call (800) 955-4572.

Question Guide: Treatment and Follow-Up Care

Talk with the doctor and ask questions about how he or she plans to treat your (your loved one's) MDS. This will help you and your loved one to be actively involved in making decisions about medical care. The following are questions you may want to ask your healthcare team.

(Note: The use of "I (we)" and "me (us)" in lists of questions is used for situations in which the patient may not be old enough or able to make his or her own decision. A parent, relative, or caregiver may be assisting or making the decision.)

DOCTOR'S NAME: ____

Date of appointment or phone call: ________________________________

Write down your diagnosis:

____

____

____

Write down your subtype of MDS:

____

____

____

Before Treatment Begins

1. Will this treatment affect the ability to have a child in the future?
2. If yes, are there other treatment options available?
3. What are the options for preserving fertility?
4. How much time do I (we) have to make decisions?
5. What is the subtype?
6. What is the goal of treatment?
7. What are the treatment options?
8. Are there any clinical trials I could join?
9. When do you think treatment should begin?
10. How long will treatment last?
11. What are the benefits and risks of these treatments?
12. Is there one treatment recommended over the others?
13. How can potential side effects be prevented or managed?

Testing

1. What kind of testing will be done to monitor the disease and treatment?
2. How long does it take to get the results?
3. How are the results communicated to me (us)?
4. How often will testing be needed?
5. Where will the testing be done?

Treatment

1. Will this be an in-hospital or an outpatient treatment?
    1a. Is it alright to attend work or school during treatment?
    1b. Will someone be needed to drive me home after treatment?
2. What kind of testing will be done to monitor this disease and treatment? How often will testing be needed? Where is the testing done?
3. How will I (we) know if the treatment is effective? What options are available if the treatment is not effective?
4. What is the likely outcome of the disease (prognosis)?

Side Effects

1. What are the common side effects for this treatment?
2. What side effects should be reported to the healthcare team right away?
3. How long will the side effects last?
4. How can potential side effects be prevented or managed?
5. How should I report side effects (phone call, at the office visit, etc)?

Social/Financial Concerns

1. Are there any side effects that will affect appearance or ability to do a job/go to school?
2. What kind of financial and social support services are available to me and my family?
3. How can I (we) find out if insurance will cover the costs of the treatment or the study (clinical trial) treatment?
4. Who is the best person to speak to about bills and insurance coverage?
5. If I (we) do not have insurance coverage, how can the healthcare team help get the treatment needed? Is there someone I can speak to for assistance?
6. If I (we) get in a study treatment (clinical trial), will I (we) be responsible for paying treatment-related costs, such as tests, travel or for clinical-trial drugs?
7. How can I (we) find out if the insurance covers the cost of the clinical trial treatment and treatment-related costs such as testing?

Follow-Up Care and Long-Term and Late Effects

1. Who should I (we) work with to ensure life-long follow up?
2. Will I (we) continue to see this healthcare team?
3. How can I (we) be monitored for long-term and late effects of treatment?
4. What types of long-term and late effects should be brought to the healthcare team's attention?
5. If there are side effects later, how can the healthcare team be reached?
6. What information can be provided to a primary doctor about this treatment?

To print copies of other question guides, go to www.LLS.org/WhatToAsk or call (800) 955-4572.

My List of Treatments

Use this space to list your treatments and when you took them.

DATE:

Treatment:

DATE:

Treatment:

DATE:

Treatment:

DATE:

Treatment:

DATE:

Treatment:

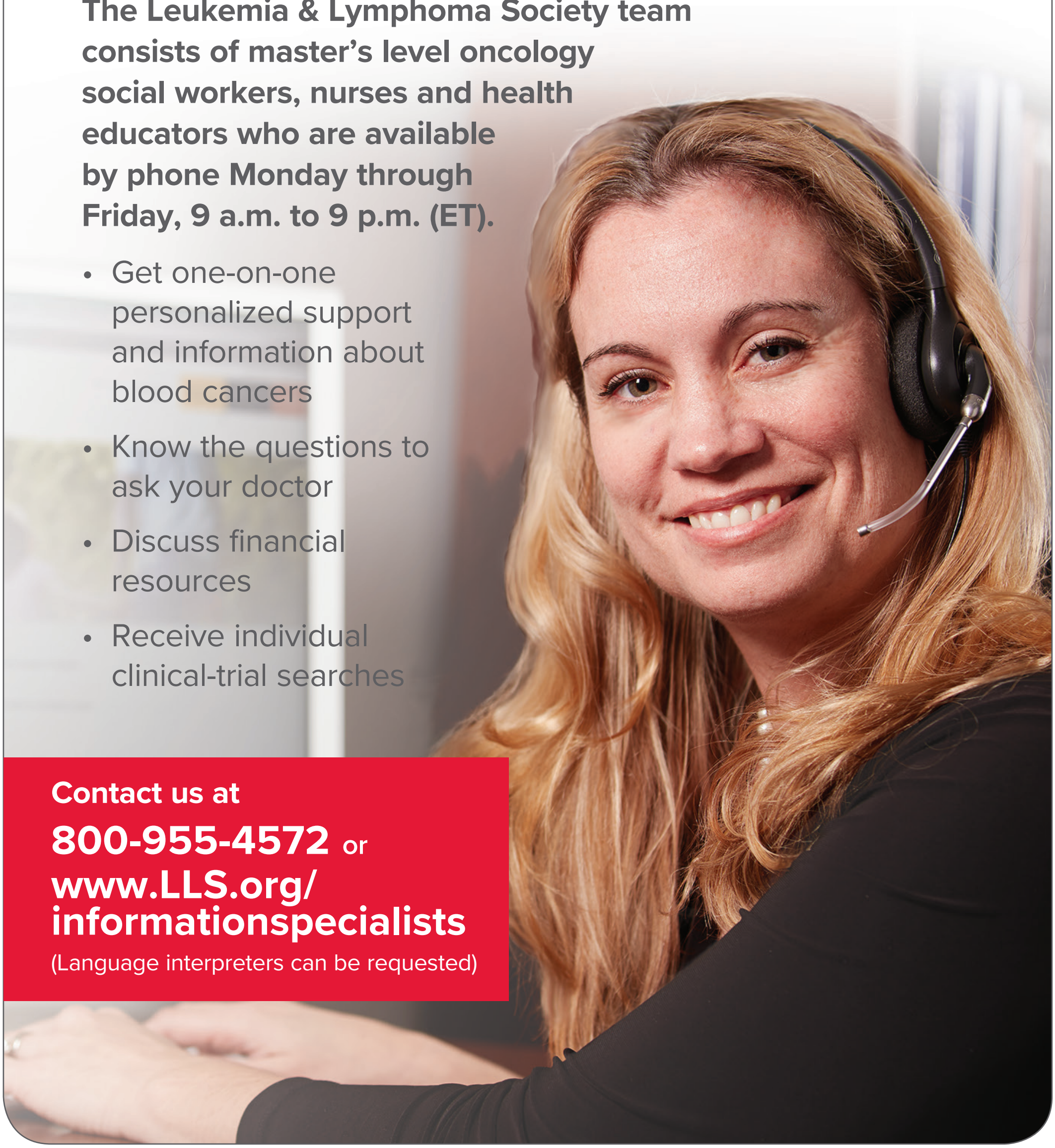
DATE:

Treatment:

NOTES

LEUKEMIA &
LYMPHOMA
SOCIETY®

Get support.
Reach out to our
INFORMATION SPECIALISTS



The Leukemia & Lymphoma Society team consists of master's level oncology social workers, nurses and health educators who are available by phone Monday through Friday, 9 a.m. to 9 p.m. (ET).

- Get one-on-one personalized support and information about blood cancers
- Know the questions to ask your doctor
- Discuss financial resources
- Receive individual clinical-trial searches

Contact us at

800-955-4572 or
www.LLS.org/informationspecialists

(Language interpreters can be requested)

LEUKEMIA &
LYMPHOMA
SOCIETY®

For more information, please contact our Information Specialists **800.955.4572** (Language interpreters available upon request).

BEATING CANCER IS IN OUR BLOOD.

National Office 3 International Drive, Suite 200 Rye Brook, NY 10573

The mission of The Leukemia & Lymphoma Society (LLS) is to cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families. Find out more at **www.LLS.org**.

PS20 20M 2/20