



# Myelodysplastic Syndromes (MDS) Diagnosis, Treatments & Support

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LEUKEMIA &  
LYMPHOMA  
SOCIETY®

**fighting blood cancers**

# LLS Mission & Goals

**Our mission.** Cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families.

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# Topics to Cover

- What are myelodysplastic syndromes (MDS)?
- Signs/symptoms
- Diagnosis
- MDS subtypes
- Prognostic scoring systems
- Treatment planning
- Treatment options
- Stem cell transplantation
- Clinical trials
- Side effects
- Follow-up



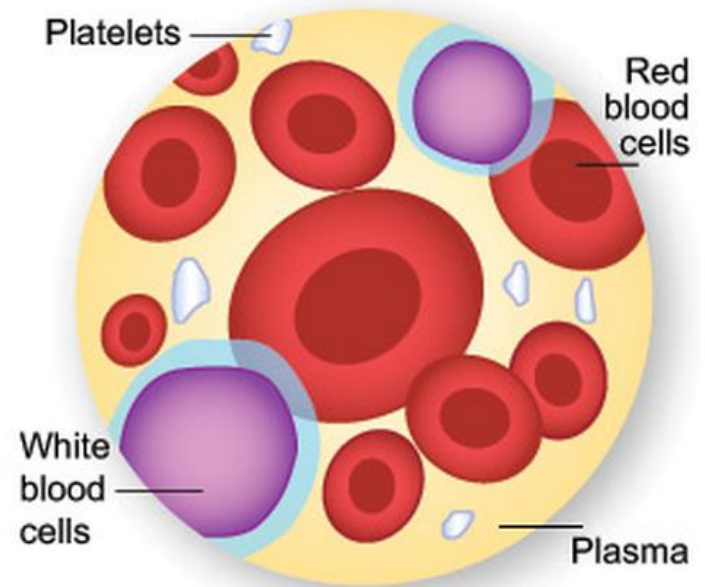
# About Blood

## Blood cells

- White blood cell (fights infection)
- Red blood cell (carries oxygen)
- Platelet (helps blood to clot)

## Plasma

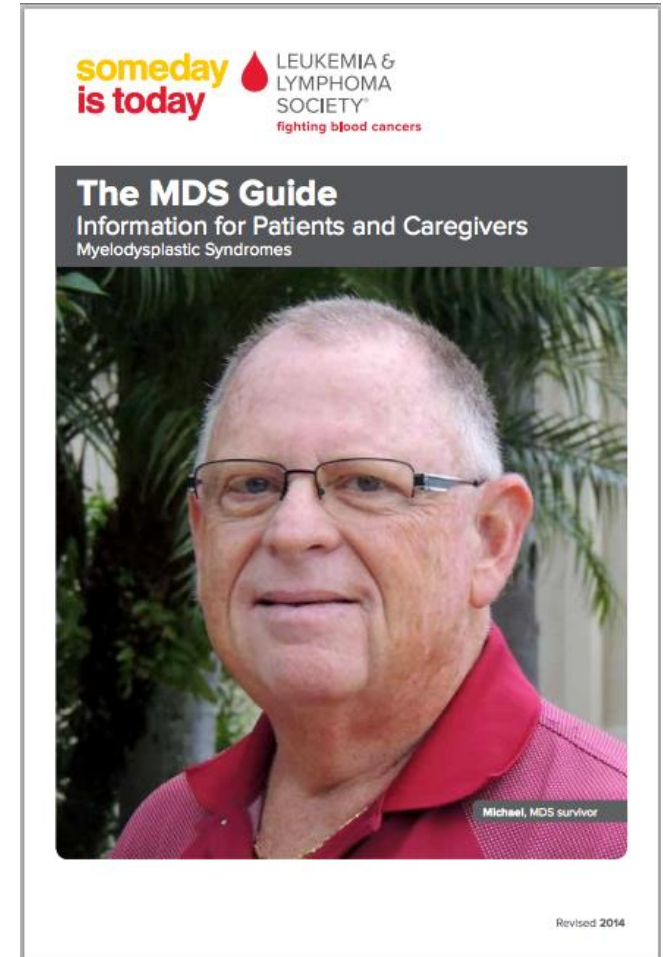
- The liquid part of the blood
- Mostly water
- Vitamins, minerals, proteins, hormones and other natural chemicals



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# What is Myelodysplastic Syndrome (MDS)

Myelodysplastic syndrome (MDS) is a disease of the blood and bone marrow that results when the blood-forming cells in the bone marrow are damaged.



# Is MDS cancer?

MDS is a cancer, which means

- It is caused by a mutation (change) to a normal cell
- This change leads to the development of cells that no longer behave normally.

\*Remember that the course of MDS can be slower and interfere less with quality of life than the course of some other diseases that aren't cancer.

# About MDS

- With MDS, there are changes to the stem cells in the bone marrow.
- Sometimes the stem cells never become mature cells (“blast cells”) and cannot perform the specific function of the mature cell, resulting in a lower than normal number of circulating blood cells.
- Sometimes these blast cells linger in the bone marrow and die before they are released into the blood stream. The bone marrow gets filled with too many cells, and this leaves less room for healthy white blood cells, red blood cells and platelets to form in the bone marrow.

# About MDS (cont'd)

As a result, the number of healthy blood cells (red blood cells, white blood cells and platelets) is usually lower than normal.

- Anemia (low number of red cells)
- Neutropenia (low number of neutrophils, a type of white blood cell)
- Thrombocytopenia (low number of platelets)
- Pancytopenia (low number of all 3 types of blood cells)

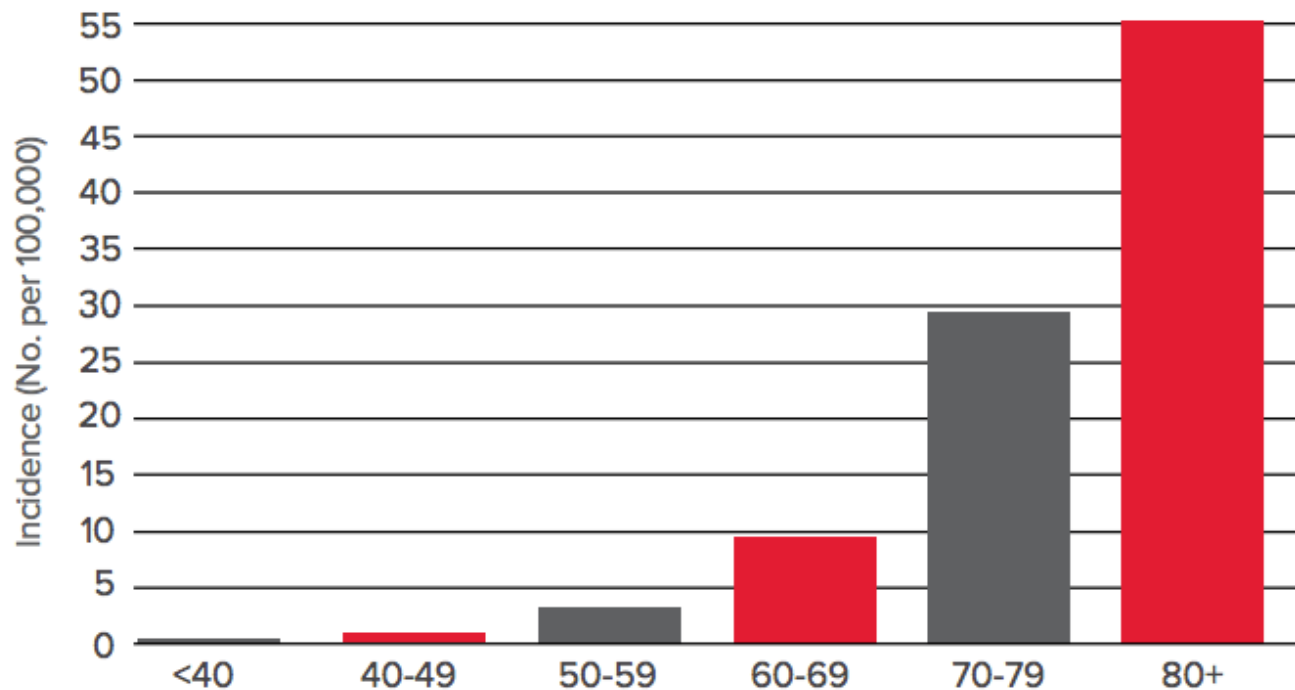


# Causes/Risk Factors

- Primary MDS (de novo-"from the beginning")
  - Most common type of MDS
  - Disease has no known cause
  - One risk factor may be repeated exposure to benzene, most commonly found in cigarette smoke.
  
- Secondary MDS ("treatment related")
  - Less common than primary MDS
  - Chemotherapy and/or radiation therapy poses risk for developing treatment-related MDS

# Incidence

## Myelodysplastic Syndromes: Age-Specific Incidence Rates (2006-2010)



**Figure 1.** | The horizontal axis represents the age of patients from below age 40 years and then in 10-year age increments up to age 80+. The vertical axis shows the incidence of new cases of MDS from 2006 to 2010, per 100,000 people.

Source: Howlader N, Noone AM, Krapcho M, et al. (eds). SEER Cancer Statistics Review, 1975-2010, National Cancer Institute. Bethesda, MD, based on November 2010 SEER data submission, posted to the SEER Web site, 2013.

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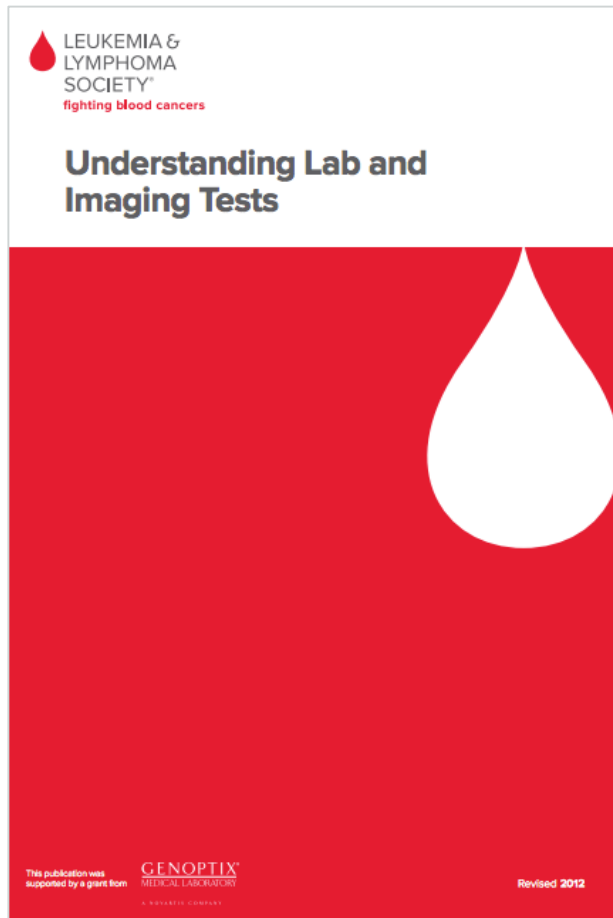
# Signs and Symptoms

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



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# Diagnosis



- Physical Exam
- Blood Tests
  - Complete Blood Count (CBC)
- Bone marrow tests
  - Bone marrow aspiration
  - Bone marrow biopsy
- FISH: Fluorescence in situ hybridization

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# Diagnostic Criteria

An MDS diagnosis is made when at least one of the following characteristics is found in the bone marrow:

- Blast cells (immature cells) making up more than 5% of the bone marrow cells
- Changes to the structure or form of the bone marrow (dysplasia)
- Chromosomal damage to the DNA in the cells (cytogenetic abnormalities)
  - “Simple” means fewer than 3 chromosome are affected
  - “Complex: means 3 or more chromosomes are affected

# MDS Classification

- Historically doctors classified MDS into different subtypes based on the appearance of the patient's marrow and the results of blood cell counts.
- The French-American-British (FAB) Work Group devised a classification of MDS consisting of the following 5 subtypes:
  - Refractory anemia (RA)
  - Refractory anemia with ring sideroblasts (RARS)
  - Refractory anemia with excess blasts (RAEB)
  - Refractory anemia with excess blasts in transformation (RAEB-T)
  - Chronic myelomonocytic leukemia (CMML)

# International Prognostic Scoring System (IPSS) and IPSS Risk Category

The IPSS uses three "prognostic indicators" to predict the course of the patient's disease:

- The percentage of blast cells in the bone marrow
- The type of chromosomal changes, if any, in the marrow cells
- One or more cytopenias (decreases in the number of cells [red blood cells, white blood cells, and/or platelets] circulating in the blood).

Patients are assigned an IPSS Risk Category based on their IPSS score.

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

# International Prognostic Scoring System-Revised (IPSS-R)

- Doctors also use the IPSS-R score, a recent update of the IPSS, assigning patient risk into 5 groups.
- The IPSS-R covers the same disease factors as the IPSS, but the factors are identified in a more detailed way.

The IPSS-R shows 5 disease factors (blasts, cytogenetics, hemoglobin, absolute neutrophil count, and platelet count).

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



# Treatment Planning

A hematologist/oncologist assigns patients to either a low-risk or high-risk category to help determine their treatment plan.

## **The goals for low-risk MDS patients are to**

- Manage the disease by reducing their transfusion needs
- Lower the risk of infection
- Increase the number of good-quality years of life

## **The goals for high-risk MDS patients are to**

- Increase life expectancy, which is much shorter than those with low-risk MDS
- Improve quality of life

# Fast Facts about Treatment Planning

- A person who has MDS is usually treated by a hematologist/oncologist.
- It is essential to seek treatment in a center where doctors are experienced in the care of patients with MDS.
- Talk with your doctor and ask questions about how he or she plans to treat your MDS.

# Some Treatment Approaches for MDS

## The most common treatments for MDS include

- Observation of blood cell counts (watch and wait)
- Transfusions
- Iron chelation therapy
- Erythropoiesis-stimulating agents (ESAs) and other growth factors
- Drug therapy
- Chemotherapy
- Stem cell transplantation
- Clinical trials

# Observation of Blood Cell Counts (Watch and Wait)

- A watch and wait approach allows doctors to monitor a patient's condition and blood counts without initiating treatment until it is needed.
- The advantage of watch and wait is that patients can avoid the potential side effects of therapy until their MDS progresses.

## Patient in watch and wait

- Need to continue to see their doctor
- Undergo regular tests and exams
- Tell their doctor about any changes or symptoms
- Understand that treatment may be needed if there are any signs of disease progression.

# Transfusions

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Blood Cancer Treatment Series

## Blood Transfusion

Revised 2011

## Red blood cells transfusions may

- Improve red blood cell counts
- Relieves symptoms of anemia such as shortness of breath, dizziness, fatigue, chest pain

## Platelet transfusions treat

- Low platelet counts
- Unusual bleeding
- Bruising

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# Iron Chelation Therapy

- Uses medicine (iron chelators) to remove excess iron
  - Deferasirox (Exjade®)
  - Deferiprone (Ferriprox®)
  - Deferoxamine mesylate (DFO; Desferal®)
- May be appropriate for anemic patients who depend on transfusions
- It is not known if iron chelators prolong life

# Erythropoiesis-Stimulating Agents (ESAs) and Other Growth Factors

## Red blood cell growth factors

- Epoetin alfa (Procrit®)
- Darbepoetin alfa (Aranesp®)

## White blood cell growth factors

- Granulocyte-colony stimulating factor (G-CSF)
- Granulocyte macrophage-colony stimulating factor (GM-CSF)

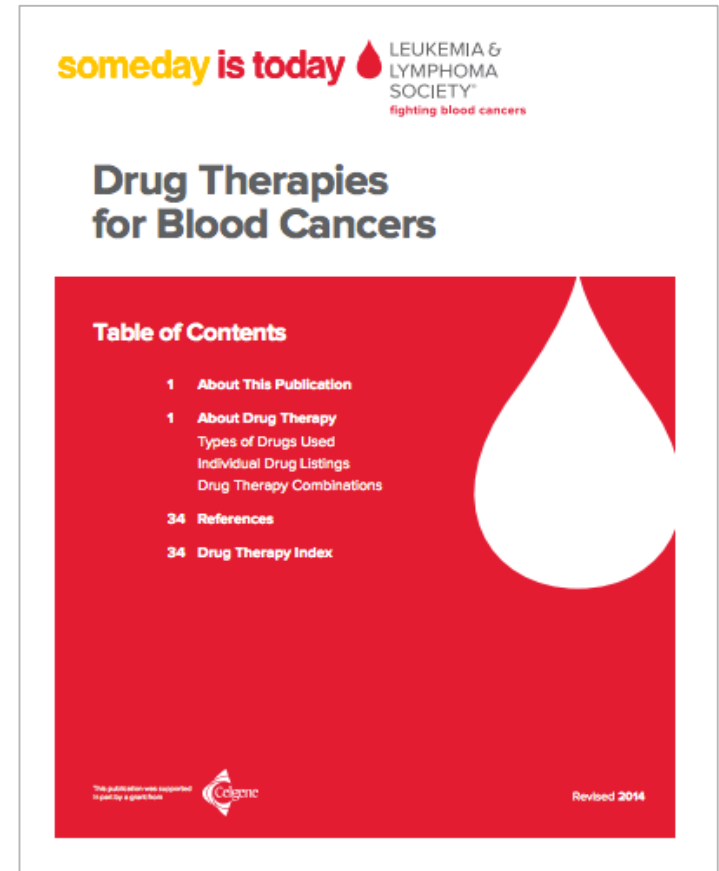
## Platelet growth factors

- Romiplostim (Nplate®)
- Eltrombopag (Promacta®)

# Drug Therapy

Some single-drug approaches that have been FDA approved for MDS are

- Azacitidine (Vidaza®)
  - For low- and high-risk patients
  - Improves bone marrow function
  - Kills unhealthy cells
  - Given intravenously (IV) or subcutaneously (SC)





# Drug Therapy (cont'd)

- Decitabine (Dacogen®)
  - For low- and high-risk patients
  - Administered intravenously
  - Treatment leads to fewer numbers of transfusions
  - Improved blood cells counts in some patients
- Lenalidomide (Revlimid®)
  - Preferred therapy for patients with transfusion-dependent anemia due to low- or intermediate-1-risk MDS
  - Works best for those with certain cytogenetic changes
- Imatinib mesylate (Gleevec®)
  - FDA-approved for patients with MDS associated with platelet-derived growth factor receptor (PDGFR) gene rearrangements.

# Chemotherapy

## These drugs may include

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

\*In clinical trials, newer drugs, such as clofarabine (Clolar®) are being investigated.

# How Patients Receive Treatment

## Central Line

- A thin tube that is put under the skin and into a large vein in the chest
- Also called a “indwelling catheter”

## Port

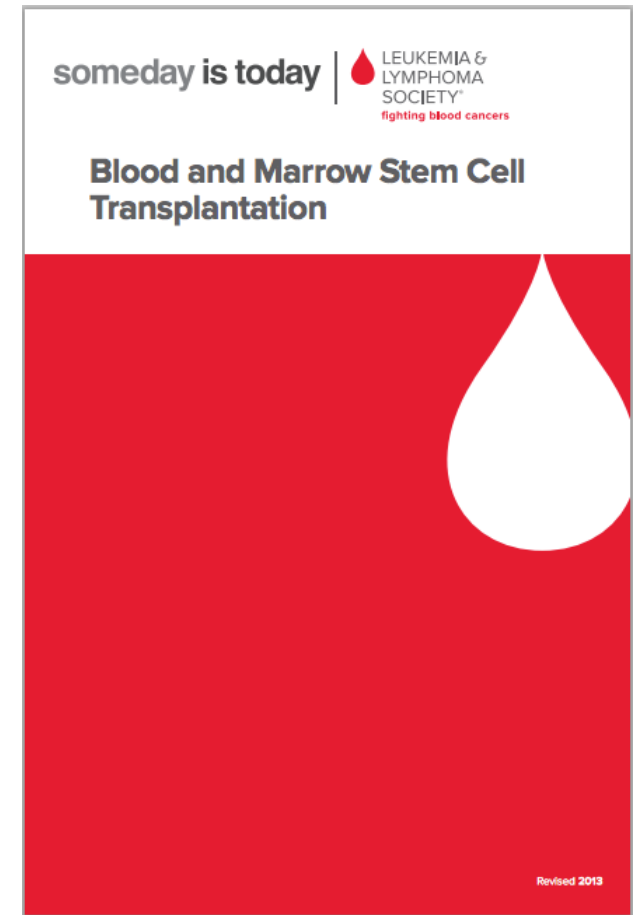
- A small device used with a central line, placed under the skin of the chest
- No dressing changes or home care needed

## PICC or PIC Line

- Inserted through a vein in the arm.

# Stem Cell Transplantation (SCT)

Stem cell transplantation may provide a cure for some patients with MDS.



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# Stem Cell Transplantation (con't)

## **Allogeneic stem cell transplantation is for:**

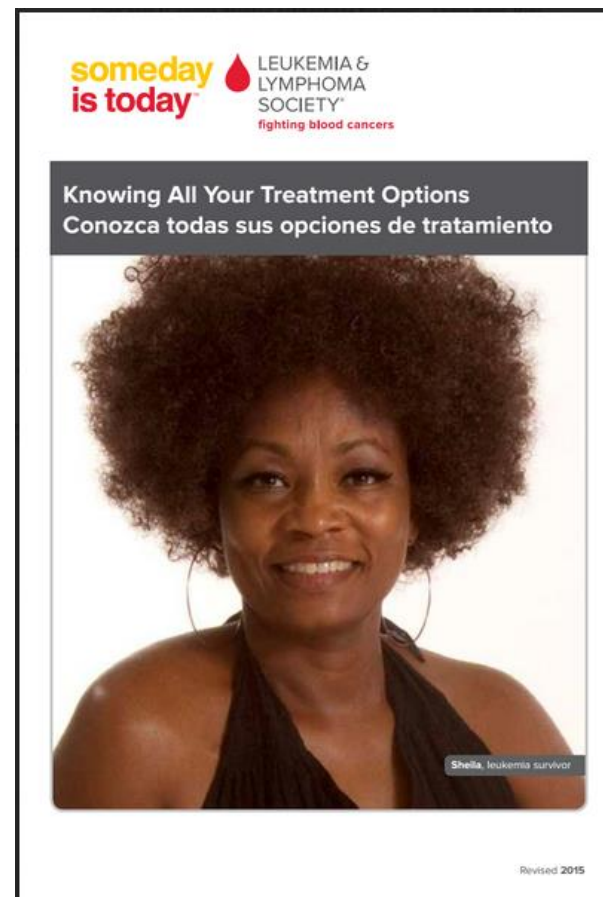
- Younger patients
- Patients in their 60s or 70s in otherwise good health
- Patients who are in either IPSS Intermediate-2 or the IPSS high-risk category
- Patients who have stem cell donor (sibling or unrelated)

## **Reduced-intensity allogeneic stem cell transplantation:**

- Involves lower intensity conditioning therapy, either with or without radiation
- Almost as effective as standard allogeneic SCT
- May have lower mortality rates
- May be an option for patients older than 70, the majority of MDS patients

# About Clinical Trials

- A carefully controlled research study conducted by doctors to
  - improve treatment options
  - increase survival
  - improve quality of life
- Designed to give patients the safest, potentially most effective therapies



# More About Clinical Trials

## Who should participate?

- Trials are not only for people with the most advanced disease.
- Patients should not wait for standard treatment to fail before asking about trials.
- Trials can be designed to test new treatments that improve response rates or improve quality of life of patients with newly diagnosed or very limited disease as well.

**Want more information?**



You can view, print or order the free LLS publications *Understanding Clinical Trials for Blood Cancers* and *Knowing All Your Treatment Options* at [www.LLS.org/publications](http://www.LLS.org/publications). Or, contact our Information Specialists for copies.

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## **Trials Currently Available**



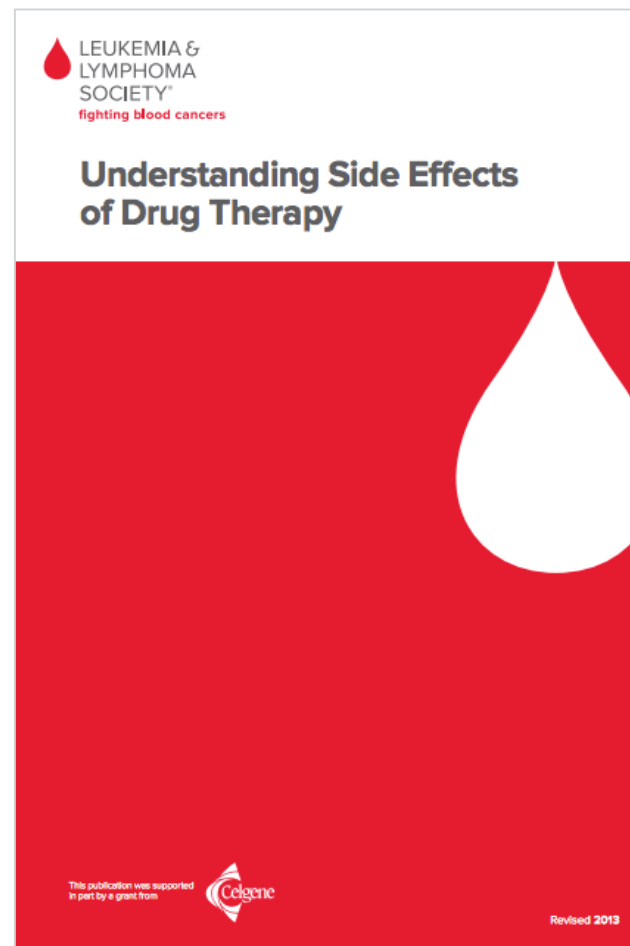
# Potential Treatment Side Effects/Complications

## Chemotherapy-related side effects

- Low blood counts (anemia, neutropenia, thrombocytopenia)
- Mouth sores
- Hair loss
- Rashes
- Nausea/Vomiting/Diarrhea

## Possible signs of infection

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



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# Treatments, Management and Coping with Side Effects



- Antibiotics
- Medication for nausea/vomiting
- Hand-washing to prevent infection
- Keep the patient's central-line clean
- Practice good dental hygiene

# Follow-up

- Track each visit and record what was discussed
- Ask your doctor if and why certain tests are being done and what to expect
- Ask for copies of lab reports
- Discuss test results
- Find out if and when follow-up tests are needed
- Seek medical and psychosocial support for fatigue, depression and other long-term effects if needed.



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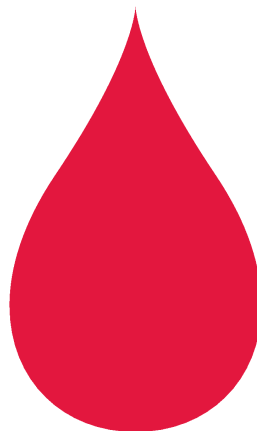
# Here to Help

Support

Resources

Advocacy

Research



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# Here to Help

Support

- **Patti Robinson Kaufmann *First Connection Program*:**  
[www.LLS.org/firstconnection](http://www.LLS.org/firstconnection)
- **Online Chats:**  
[www.LLS.org/chat](http://www.LLS.org/chat)
- **Discussion Boards:**  
[www.LLS.org/discussionboards](http://www.LLS.org/discussionboards)
- **Patient and Family Support Groups:**  
[www.LLS.org/supportgroups](http://www.LLS.org/supportgroups)

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# Here to Help

## Resources

- **Patient Education Programs**
  - National and Local
  - [www.LLS.org/programs](http://www.LLS.org/programs)
- **Free Education Materials**
  - Booklets and Fact Sheets
  - [www.LLS.org/booklets](http://www.LLS.org/booklets)
- **Education Videos**
  - [www.LLS.org/videos](http://www.LLS.org/videos)

# Here to Help

## Resources

- **Information Specialist**
  - (800) 955-4572
- **Local Chapters**

[www.lls.org/chapterfind](http://www.lls.org/chapterfind)

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We have one goal: **A world without blood cancers**

Questions &  
Answers

Thank you

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