

MDS GLOSSARY

COMMONLY USED BONE MARROW FAILURE TERMS



GENERAL

Acute

Sudden, such as a sudden onset of symptoms or diseases.

Acute Myeloid Leukemia (AML)

A cancer of the blood cells. AML happens when very young blood cells (blasts) in the bone marrow fail to mature normally. More blastcells are produced than needed, so there is not enough room within the marrow for other normal blood cells to develop, such as red blood cells or platelets. Some cases of MDS may develop into AML. However, most do not.

Synonyms: acute myeloblastic leukemia, acute myelocytic leukemia

Anaphylaxis

A very severe allergic reaction to a foreign protein, such as in a bee sting, or to a medicine. This reaction causes the blood pressure to drop and may cause difficulty breathing. Emergency treatment is required to manage these symptoms. If very severe, anaphylaxis can progress to shock.

Synonym: anaphylactic shock

Anemia

A condition in which the number of red blood cells is below normal. This may result in fatigue, generalized weakness and shortness of breath.

Antibiotics

Medications used to treat bacterial infections and other similar microorganisms.

Antibodies

Proteins produced by plasma cells in response to foreign substances in the body.

Apheresis

A procedure in which blood is taken from a person, and part of that blood component (such as white blood cells, red blood cells, or plasma) is separated out, and the remaining blood components are reinfused back into the donor.

Aplastic Anemia

A rare and serious condition in which the bone marrow does not make enough blood cells: red blood cells, white blood cells, and platelets. The term aplastic is a Greek word meaning not to form. Anemia is a condition that happens when the red blood cell count is low. Most scientists believe that aplastic anemia happens when the immune system attacks the bone marrow stem cells. Aplastic anemia can be acquired (begin any time in life) or can be hereditary (less common, passed down from parent to child).

Synonyms: acquired aplastic anemia, hereditary aplastic anemia

Apoptosis

Death of a cell as a part of the normal lifecycle.

Autoimmune Disease

Any condition that happens when the immune system attacks the body's own normal tissues. The immune system is a complex organization within the body that is designed normally to "seek and destroy" invaders of the body, including infectious agents. Patients with autoimmune diseases frequently have unusual antibodies circulating in their blood that target their own body tissues.

Basic Research

The study of a subject to increase knowledge and understanding about it. The goal of basic research in medicine is to better understand disease. In the laboratory, basic research scientists study changes in cells and molecules linked to disease. Basic research helps lead to better ways of diagnosing, treating, and preventing disease.

Benzene

A chemical that is widely used by the chemical industry to make plastics, resins, nylon and synthetic fibers. Benzene is found in tobacco smoke, vehicle emissions, and gasoline fumes. Exposure to benzene may increase the risk of developing a bone marrow failure disease. Benzene can affect human health by causing bone marrow stem cells not to work correctly.

Biologic Agent

A substance made from a living system, such as a virus, and used to prevent or treat disease. Biological drugs include antibodies, globulin, interleukins, serum, and vaccines. Also called a biologic or biological drug.

Synonyms: biologic, biological drug

Blast Cells

Immature blood cells that would normally become fully functional mature red cells, white cells, or platelets. The number of blast cells in the bone marrow helps define how severe MDS is in a person. When 20 or more out of 100 cells in the bone marrow are blasts, this is considered acute myeloid leukemia (AML).

Synonym: precursor cell

Blood Clot

A clot or small cluster of blood cells that forms when platelets stick together. A combination of platelets and fibrin that form a mesh with the intention of preventing bleeding in response to an injury or illness. The term thrombus describes a blood clot that develops and attaches to a blood vessel. Blood clots are more common in Paroxysmal Nocturnal Hemoglobinuria (PNH) or in people with blood clotting disorders.

Synonym: thrombus

Blood Tests

Blood samples drawn from the arm that are evaluated for cell counts (red cells, white cells [and their subtypes], and platelets). The blood is also evaluated for the shape and size of the different blood cells and for how various organs are functioning such as the kidneys and liver.

Blood Thinner

A medicine used to treat or prevent blood clots. Also called anticoagulants or blood thinners. Some common blood thinners are enoxaparin or clexaine (Lovenox or Clexane), heparin (Calciparine or Liquaemin), and warfarin (Coumadin).

Synonyms: anticoagulant, anti-clotting

Blood Transfusion

A procedure in which whole blood or one of its components is given to a person through an intravenous (IV) line into the bloodstream.

A red blood cell transfusion or a platelet transfusion can provide temporary relief for some patients with low blood counts.

Bone Marrow

The soft, sponge-like tissue in the center of bones that functions like a factory to produce white blood cells, red blood cells, and platelets.

Bone Marrow Aspirate

The bone marrow aspirate is a sample of the liquid portion of the bone marrow. It is used to

obtain spicules — a small collection of blood forming cells. This provides information about the shape of the cells (morphology), how the cells are maturing (differentiation) and the number of blasts (immature cells) in the bone marrow. The aspirate may also be used for additional testing that may help to determine the cause of the cytopenias, such as cytogenetics.

Bone Marrow Aspiration

The process of removing bone marrow from a specific area using a small needle and syringe. Used for diagnostic purposes. Tests may also be run on the bone marrow cells to look for any genetic abnormalities.

Bone Marrow Biopsy

The bone marrow biopsy is a small core (the shape and size of a medium pencil lead) of the spongy center of the bone marrow. It provides information about the cellularity of the bone marrow (crowded=hypercellular, empty=hypocellular). It will also provide useful information about iron storage, scarring (fibrosis), and the presence of any other abnormal cells.

Synonym: Bone Marrow Trephine Biopsy

Bone Marrow Failure

A condition that occurs when the bone marrow stops making enough healthy blood cells. The most common of these rare diseases are myelo-

dysplastic syndromes (MDS), aplastic anemia, and paroxysmal nocturnal hemoglobinuria (PNH). Bone marrow failure can be acquired (begin any time in life) or can be hereditary (less common, passed down from parent to child).

Cellularity

How much of the bone marrow volume is occupied by various types of blood cells.

Chemotherapy

The use of medicines that kill cells (cytotoxic agents). People with high-risk or intermediate-2 risk myelodysplastic syndrome (MDS) may be given chemotherapy. Chemotherapy may also hurt healthy cells causing side-effects. If chemotherapy works in controlling abnormal cells, then relatively normal blood cells will start to grow again. Chemotherapy agents include: cytarabine (Ara-C) and hydroxyurea (Hydrea), daunorubicin (Cerubidine), idarubicin (Idamycin), and mitoxantrone (Novantrone).

Chronic Illness

A medical condition that lasts a long time. A chronic illness can affect a person's lifestyle, ability to work, physical abilities and independence.

Chromosomes

A structure that contains your genetic information, or DNA. Normally each person has 23 pairs of chromosomes.

Clinical Trial

A type of research study that tests how a drug, medical device, or treatment approach works in people. There are several types of clinical trials. Treatment trials test new treatment options. Diagnostic trials test new ways to diagnose a disease. Screening trials test the best way to detect a disease or health problem. Quality of life (supportive care) trials study ways to improve the comfort of people with chronic illness. Prevention trials look for better ways to prevent disease in people who have never had the disease.

Trials are in four phases:

- Phase I tests a new drug or treatment in a small group to see if it is safe.
- Phase II expands the study to a larger group of people to find out if it works.
- Phase III expands the study to an even larger group of people to compare it to the standard treatment for the disease.
- Phase IV takes place after the drug or treatment has been licensed and marketed to find out the long-term impact of the new treatment.

Clone

To make copies. Bone marrow stem cells clone themselves all the time. The cloned stem cells

become mature blood cells that leave the bone marrow and enter the bloodstream. Abnormal clones are associated with cancers, such as MDS.

Coagulate

To thicken.

Normal blood platelets cause the blood to coagulate and stop bleeding.

Combination Chemotherapy

The use of more than one drug during cancer treatments.

Complete Blood Count (CBC)

The CBC measures the number of white blood cells (WBC) and the number and size of red blood cells, the total amount of hemoglobin, and the fraction of the blood made up of red blood cells.

Cytogenetics

Testing that is performed on bone marrow samples and examines the chromosomes of the cells.

Your cytogenetic results are used to identify the type of MDS you have and to calculate the International Prognostic Scoring System (IPSS) and the revised IPSS (IPSS-R) risk category.

Common abnormalities include:

1. Deletion 5q- – deletion of chromosome 5
2. Deletion 20 – deletion of chromosome 20
3. Deletion Y – deletion of the Y chromosome

4. Monosomy 7 – loss of one of the two 7 chromosomes

5. Trisomy 8 – addition of a third chromosome 8

Synonyms: Chromosomes, Karyotype, DNA

Cytogenetic Remission

No sign of previously detected abnormal chromosomes are found. This represents a response to treatment. When a bone marrow test is performed on a patient with 5q deletion MDS, and there are no signs of an abnormal chromosome 5, then that patient has achieved a cytogenetic remission. Also called cytogenetic response.

Cytopenia

A deficiency of (or too few) mature cells in the blood. Deficiencies can occur in red cells, white cells, and/or platelets.

Cytotoxic Agent

A medicine that kills certain cells. Chemotherapy for MDS patients often involves the use of cytotoxic agents.

De Novo

The original source of disease, something present at the start. MDS may be de novo, the original source of disease, or treatment related, caused from chemotherapy or radiation given for other forms of cancer.

Differentiation

The process of cells maturing to become healthy adult cells of a particular type (i.e. red cells, white cells, and platelets).

Dietary Supplement

Vitamins, minerals, herbs and other substances meant to improve your nutritional intake. Dietary supplements are taken by mouth in the form of a pill, capsule, tablet or liquid.

Dysplasia

Abnormal shape and appearance or morphology, of a cell.

Synonym: dysplastic

Embolus

A blood clot or other foreign matter that gets into the bloodstream and gets stuck in a blood vessel.

Epidemiology

The study of patterns and causes of disease in groups of people. Researchers who study how many people have a disease, how many new cases are diagnosed each year, where patients are located, and environmental or other factors that influence disease, are known as Epidemiologists.

Erythropoietin (EPO)

A protein substance manufactured by the kidneys in response to low oxygen levels in body tissues.

Erythropoietin stimulates the production of red blood cells in the bone marrow.

Erythropoietin-stimulating Agent (ESA)

A medicine used to help the bone marrow make more red blood cells. Epoetin alfa (Epogen, Procrit) and darbepoetin alfa (Aranesp) are erythropoiesis-stimulating agents that can help boost the red blood cell count of some bone marrow failure patients. Also called red blood cell growth factor.

Etiology

The cause or origin of a disease.

FAB Classification

A criteria used for classifying different types of myelodysplastic syndromes (MDS). The FAB (French, American, British) Classification System was developed by a group of French, American and British scientists. This system is based on 2 main factors: the percentage of blast cells in bone marrow, and the percentage of blast cells in the bloodstream. The FAB system is somewhat outdated, but is still used by some doctors today. The World Health Organization (WHO) Classification System has largely replaced the FAB Classification System.

Fanconi Anemia

A rare inherited disorder that happens when the bone marrow does not make enough blood cells: red cells, white cells, and platelets.

Fanconi anemia is diagnosed early in life. People with Fanconi anemia have a high likelihood of developing cancer. Genetic testing is used to diagnose Fanconi anemia.

Fatigue

A feeling of low or no energy, general feeling of tiredness with normal activity. Rest does not necessarily resolve fatigue.

Synonyms: tired, exhaustion, lethargy, malaise

Ferritin

A protein inside of cells that stores iron for later use by your body. Sometimes ferritin is released into the blood. The ferritin level in the blood is called serum ferritin.

Fibrosis

Scarring of tissue. Fibrosis of the bone marrow is a feature seen in some types of unclassified myelodysplastic syndrome (MDS).

Flow Cytometry

A laboratory test that gives information about cells, such as size, shape, and percentage of live cells. Flow cytometry is the test doctors use to assess for specific proteins on the surface of blood cells. It is

the standard test for confirming a diagnosis of paroxysmal nocturnal hemoglobinuria (PNH).

Synonyms: Flow, Immunophenotyping Fluorescence-activated cell sorting (FACS)

Fluorescence In Situ Hybridization (FISH)

An important laboratory test used to help doctors look for chromosomal abnormalities and other genetic mutations. Fluorescence in situ hybridization, also called FISH, directs colored light under a microscope at parts of chromosomes or genes. Missing or rearranged chromosomes are identified using FISH.

Folate

A B-vitamin that is found in fresh or lightly cooked green vegetables. It helps the bone marrow make normal blood cells. Most people get enough folate in their diet. Doctors may have people with paroxysmal nocturnal hemoglobinuria (PNH) take a man-made form of folate called folic acid.

Hematocrit (HCT)

Percent of the total blood volume that is made up of red blood cells. In men a normal hematocrit is 40–52% while in women the normal is 36–46%. Hematocrit is part of a complete blood count. Also called HCT, packed cell volume, PCV. (see red blood cells)

Synonyms: packed cell volume, PCV

Hematologist

A doctor who specializes in the diseases and disorders of blood.

Hematopoiesis

The formation and development of blood cells.

Hemochromatosis

A condition that occurs when the body absorbs and stores too much iron. This leads to a condition called iron overload. In the United States, hemochromatosis is usually caused by a genetic disorder. Organ damage, particularly to the liver and heart, can occur if iron overload is not treated.

Hemolysis

The destruction of red blood cells.

Hypercellular

A condition in which there are too many cells within the bone marrow.

Hypocellular

A condition in which there are too few cells, within the bone marrow. Patients with aplastic anemia have hypocellular bone marrow.

Hypomethylating Agent

A hypomethylating agent is a drug that inhibits DNA methylation. Works by preventing certain genes involved in controlling cancer from being silenced, allowing for the normal functioning of the tumor suppressor genes.

Synonym: demethylating agent

Idiopathic

Usually refers to any condition with no known cause.

Immature Blood Cells

May be called stem cells, progenitor cells or blasts.

Immune Deficiency

A decreased ability of the immune system to fight infection.

Immune System

The complex group of organs and cells that defend the body against infection and disease.

Immunocompromised

Occurs when the immune system is not functioning properly, leaving the patient open to infection. A person can be immunocompromised due to low white blood cell count or due to some medicines.

Synonym: immune compromised

Immunosuppressive Drug

Drugs that lower the body's immune response in autoimmune diseases. These drugs may be used to allow the bone marrow stem cells to grow and make new blood cells. ATG (antithymocyte globulin) or ALG (antilymphocyte globulin) with cyclosporine are used to treat bone marrow failure in aplastic anemia. Immunosuppressive drugs may help some patients with myelodysplastic syndromes (MDS) and paroxysmal nocturnal hemoglobinuria (PNH).

Intravenous Infusion

A method of getting fluids or medicines directly into the bloodstream over a period of time. Also called IV infusion.

Synonym: IV infusion

IPSS/IPSS-R

An International Prognostic Scoring System – system for grading the severity of MDS. The system turns patient data into a score. The score tells how quickly a myelodysplastic syndrome (MDS) case is progressing and helps predict what may happen with the patient’s MDS in the future.

Iron Chelation Therapy

A drug therapy to remove extra iron from the body. Patients with high blood iron (ferritin) levels may receive iron chelation therapy. The U.S. Food and Drug Administration (FDA) has approved two iron chelators to treat iron overload in the U.S.: deferasirox, an oral iron chelator, and deferoxamine, a liquid given by injection, these may differ depending on which country you live in.

Iron Overload

A condition that occurs when too much iron accumulates in the body. Bone marrow failure disease patients who need regular red blood cell transfusions are at risk for iron overload. Organ damage can occur if iron overload is not treated.

Ischemia

Occurs when the blood supply to specific organ or part of the body is cut off, causing a localized lack of oxygen.

Lymphatic System

A network of organs, lymph nodes, lymph ducts, and lymph vessels that help keep the body's fluids in balance and help the body fight infection.

Monosomy 7

Describes the loss of one of the two number 7 chromosomes. "Mono" means one and "somy" comes from the word chromosome. Bone marrow samples are used to detect monosomy 7 and other genetic abnormalities. Monosomy 7 can occur in adult patients with MDS and can occur in childhood bone marrow failure diseases.

Mutation

Any change or alteration in a gene. A mutation may cause disease or may be a normal variation.

Morphology

The study of the structure and form of an organism or one of its parts.

Myelo

A Greek word meaning marrow.

Myelodysplastic Syndromes (MDS)

The Myelodysplastic Syndromes (MDS) are a group of bone marrow failure disorders. Myelo

refers to the bone marrow. Dysplastic means abnormal growth or development. In MDS, the bone marrow does not make blood cells normally. The result is too few cells or low blood counts (cytopenias) and cells that do not function properly.

The most common cytopenias include:

Anemia: low red blood cells (oxygen carrying cells)

Thrombocytopenia: low platelets (cells that help to clot the blood)

Leukopenia: low white blood cells (WBC) (help to fight infection)

Neutropenia: low neutrophils (most important type of WBC for fighting infection)

Natural Killer Cells

A type of cell that lacks B-cell and T-cell receptors and attacks mutant and virus-infected cells.

Neutropenia

A deficiency (below-normal number) of mature white blood cells called neutrophils that assist in fighting bacterial infections.

Neutropenic Diet

A diet for a patient with very low white blood cell count. A neutropenic diet avoids the use of certain foods that can contain bacteria or fungus, such as raw meats, unpasteurized dairy products, aged cheeses, fermented drinks, and unwashed fruits and vegetables.

Oncologist

A doctor who specializes in the treatment and prevention of cancer.

Over-the-Counter (OTC) Medicine

A medicine that is available without a prescription from the doctor. Also called OTC medicine.

Packed RBCs

A concentrated blood product in which most of the plasma, the fluid part of blood, is removed to make red blood cell transfusions easier and faster.

Pancytopenia

A reduced number of all types of blood cells – red blood cells, white blood cells, and platelets.

Paroxysmal Nocturnal Hemoglobinuria (PNH)

A rare and serious blood disease that causes red blood cells to break apart. Paroxysmal means sudden and irregular. Nocturnal means at night. Hemoglobinuria means hemoglobin in the urine. Hemoglobin is the red part of red blood cells. A person with PNH may have episodes of dark urine in the morning, but this symptom is not present in all PNH patients.

Pathophysiology

Abnormal function or processes that cause or are associated with disease or injury.

Pediatric MDS

MDS is rare in children; but it does happen. Most patients are 60 years old or older.

Peripheral Blood Stem Cell (PBSC) Transplant

A procedure where stem cells are collected from the donor's circulating (peripheral) blood. These stem cells are then given to the patient through an intravenous (IV) line. In time, donated stem cells start making new, healthy blood cells. Also called PBSC transplant.

Petechiae

Small, flat red or purplish spots caused by pinpoint bleeding into the skin. It is often a sign of a low platelet count.

Pharmacist

A highly trained and licensed professional whose job concerns the preparation, distribution, and use of prescription drugs. A pharmacist also advises patients, as well as physicians and other health practitioners, on the selection, dosages, interactions, and side effects of medications.

Placebo

A placebo is an inactive pill, liquid, or powder that has no treatment value. Placebo use in clinical trials is extremely uncommon today.

Platelets

Irregularly shaped, colorless cells that are present in blood. Their sticky surface lets them, along with other substances, form clots to stop bleeding. Also called thrombocytes.

Platelet Transfusion

A procedure in which platelets are given to a person through an intravenous (IV) line into the blood-stream. Platelets are more likely than red blood cells to cause an immune response, such as chills and fever. The use of platelets from one donor (apheresis) reduces the chance of reaction to transfused platelets. Transfused platelets increase the blood platelet count and help control bruising and bleeding.

Prophylactic

Something that prevents or protects. For example, blood thinners may be given as a prophylactic measure to prevent blood clots in high risk patients.

Pure Red Cell Aplasia (PRCA)

A condition that occurs when the bone marrow stem cells do not make red blood cells. Red blood cell counts are low. White blood cell and platelet counts are normal.

Red Blood Cell (RBC) Transfusion

A procedure in which packed red blood cells are given to a person through an intravenous (IV) line into the bloodstream. Transfused red blood cells

increase the blood count and help improve symptoms of anemia. Before transfused blood is given, donated blood is typed and cross matched to the recipient's blood. Also called RBC transfusion.

Refractory

Not responsive to treatment or cure. For example, refractory anemia is a low red blood cell count that doesn't respond to standard treatments.

Reticulocyte

An immature red blood cell. Reticulocytes are normally found in the bone marrow. They are present in the bloodstream only in very low numbers.

Remission

Disappearance of the signs and symptoms of cancer. A remission may be complete (CR) or partial (PR).

Ring Sideroblast

A red blood cell that has too much iron. The iron typically forms a ring around the cell's nucleus.

Secondary MDS

A type of MDS that is caused by a previous treatment for another disorder or disease. Treatments typically associated with secondary MDS include radiation therapy and chemotherapy used to treat cancer. Also called therapy-related MDS, T-MDS.

Synonyms: T-MDS, therapy-related MDS

Serum Erythropoietin

Amount of erythropoietin that is present normally in an individual's blood.

Serum Sickness

An immune system reaction to foreign proteins in certain medicines. Serum sickness can be a side effect of ATG, a medication used in the treatment of aplastic anemia, causing fever, rash, joint pain, and muscle aches.

Stem Cells

Cells in the body that develop into other cells. Adult stem cells in the body repair and maintain the organ or tissue in which they are found. Blood-forming (hemapoietic) stem cells are found in the bone marrow. These cells make copies of themselves and develop into red cells, white cells, and platelets. Embryonic stem cells come from human embryos and may be used in medical research.

Subcutaneous Injection

A method of giving medicine in the fatty tissue area under the skin using a short needle.

Synonyms: shot, injection

Supportive Care

Care given to improve the quality of life, or comfort, of a person with a chronic illness. Supportive care treats the symptoms rather than

the underlying cause of a disease. The goal is to help the patient feel better. Patients with low blood counts may be given blood transfusions as supportive care to help manage the symptoms of their disease. Also called palliative care, symptom management.

Synonyms: palliative care, symptom management

Therapy Related MDS

See Secondary MDS

Thrombus

A blood clot that develops and attaches to a blood vessel.

Thrombosis

The process of forming a blood clot.

Thrombocytopenia

A condition in which the number of mature platelets, or thrombocytes, is below normal. When severe, the tendency to bruise and bleed more easily can occur.

Transfusion

Process by which blood or one of its components (e.g., red blood cells, plasma, platelets) is delivered directly into the bloodstream by vein (intravenous or IV), similar to other IV medications.

Transfusion Independence

No longer needing any type of blood transfusion.

White Blood Cells (WBC)

Cells produced in the bone marrow and lymph nodes. White cells are key cells in the immune system that prevent or fight infection.

World Health Organization (WHO) Classification

The most current system for classifying leukemia and myelodysplastic syndromes (MDS), it was developed by the World Health Organization (WHO). This system is based on patient data from around the world and on the most up-to-date knowledge of MDS. WHO Classification of MDS consists of many subtypes based on tests of the blood and bone marrow.

RED BLOOD CELLS

Erythrocyte

A red blood cell. It carries oxygen to body cells and carbon dioxide away from the cells.

(See red blood cells)

Red Blood Cells (RBC)

These are cells that carry oxygen to your tissues.

(See erythrocyte)

WHITE BLOOD CELLS

ANC (absolute neutrophil count)

A measure of the actual number of mature neutrophils in a given volume of blood.

Basophil

Type of white blood cell that plays a role in allergic reactions and asthma.

Eosinophil

Type of white blood cell that kills parasites and plays a role in allergic reactions.

Granulocyte

A term for any of the white blood cell types that have granules containing enzymes to help fight infection: neutrophils, eosinophils and basophils.

Lymphocytes

Small white blood cells produced in the lymphoid organs (the lymph nodes, spleen, thymus, and tonsils) or bone marrow that are essential for normal function of the immune system.

Monocyte

A white blood cell that helps the body fight infections from some bacteria such as tuberculosis.

Neutrophil

A type of white blood cell that functions to destroy bacteria. When the number of neutrophils is too low, the body is at greater risk for developing an infection.

BONE MARROW BIOPSY

Biopsy

A medical procedure to remove a small piece of solid bone marrow using a needle that goes into the marrow of the hip bone. The solid bone marrow is examined for cell abnormalities, the number of different cells, and checked for scarring of the bone marrow.

Iliac Crest

The hip bone area from which bone marrow samples are most commonly taken.

BONE MARROW TRANSPLANT

Allogeneic Stem Cell Transplantation

A procedure in which matched bone marrow or peripheral blood stem cells from a donor (usually related) are collected, stored, and infused into a patient (recipient) following high-dose chemotherapy with or without radiation therapy. In time, donated stem cells given to the patient begin making new, healthy blood cells (known as engraftment).

Allograft

An allogeneic stem cell collection used for transplant.

Autograft

An autologous stem cell collection used for transplant.

Autologous Stem Cell Transplantation

A procedure in which a patient's own stem cells from bone marrow or peripheral blood are collected, stored, and reinfused following high-dose chemotherapy or radiation therapy. In time, donated stem cells given to the patient begin making new, healthy blood cells (known as engraftment).

Bone Marrow Transplant

A procedure in which high doses of chemotherapy or radiation therapy are used to eradicate disease in the bone marrow and lymphatic system and then are replaced with healthy bone marrow from a donor or the patient.

Cord Blood Transplant

A procedure where umbilical cord stem cells are given to the patient through an intravenous (IV) line. Stem cells are collected from an umbilical cord right after the birth of a baby. They are kept frozen until needed. In time, donated stem cells given to the patient begin making new, healthy blood cells.

Engraftment

Refers to how well the donor cells (graft) are accepted by the patient's immune system (host)

after a bone marrow or stem cell transplant. Several factors contribute to better engraftment: physical condition of the patient, how severe the disease is, type of donor available, age of patient. Successful engraftment results in new bone marrow that produces healthy blood cells (new white blood cells, red blood cells, and platelets).

Graft-Versus-Host Disease (GVHD)

GVHD is a common complication of allogeneic bone marrow/stem cell transplantation. It is caused when the donor's immune cells, now in the patient, begin to see the patient's body as foreign and mount an immune response. GVHD most commonly affects the recipient's skin, intestines, or liver. Severity can range from mild to very severe. In some cases, GVHD can be prevented or treated with specific drugs to suppress the body's immune cells (immunosuppressive drug therapy).

Human Leukocyte Antigen (HLA)

One of a group of proteins found on the surface of white blood cells and other cells. These antigens differ from person to person. A human leukocyte antigen test is done before a stem cell transplant to closely match a donor and a recipient.

Matched Related Donor

Bone marrow/stem cell donor that is a sibling or another blood relative to the patient.

Mini-Transplant

See Non-Myeloablative Transplant

Myeloablation

The killing of bone marrow by radiation or chemotherapy. This term usually refers to the complete or near-complete destruction of the bone marrow.

Non-Myeloablative Transplant

Type of allogeneic stem cell or bone marrow transplant that uses lower doses of chemotherapy. This reduces side effects caused by chemotherapy, making it more tolerable for older adults. It does not reduce the risk of graft-versus-host disease. Also called nonmyeloablative transplant or reduced intensity transplant.

Reduced Intensity Transplant

Procedure similar to standard bone marrow transplant. The reduced intensity transplant uses a mild form of chemotherapy pre-treatment. This reduces side effects caused by chemotherapy, making it more tolerable for older adults. It does not reduce the risk of graft-versus-host disease. Also called nonmyeloablative transplant.

Unrelated Donor

A donor that is not a sibling or other familial relation of the patient (recipient).

MEDICATIONS

Adverse Event (AE)

Any undesired actions or effects of a drug or treatment.

Synonyms: side effect, toxicity

Antibiotic Therapy (AB)

Used to treat bacterial infections or prevent recurrence of bacterial infections.

Antithymocyte Globulin (ATG)

An immunosuppressive medication that eliminates abnormally proliferating white blood cells called T lymphocytes which disrupt normal blood cell growth. This may restore normal production of red blood cells which may lead to transfusion independence. The three brand-name drugs are Thymoglobulin[®], Lymoglobulin[®], and Atgam[®].

Colony-Stimulating Factor (CSF)

Protein that stimulates the development and growth of blood cells; sometimes called growth factor. Granulocyte colony-stimulating factor is a CSF that is used to stimulate stem cells from the bone marrow into the bloodstream prior to apheresis.

Corticosteroids

Also called “steroids,” corticosteroids are powerful anti-inflammatory medicines used to treat many diseases and conditions. They are similar to a protein called cortisol that is made

in the adrenal glands. Names of corticosteroids include prednisone and dexamethasone.

Synonym: steroids

Dacogen™ (decitabine)

A medication used in treating some types of MDS and AML. Dacogen works by preventing certain genes involved in controlling cancer from being silenced, allowing for the normal functioning of genes within the body. It is a DNA hypomethylating agent that is administered intravenously (IV).

Desferal® (deferoxamine)

A medication that binds to iron and promotes its removal from the body for treatment of transfusion-dependent iron overload. It is an iron-chelating drug that is administered subcutaneously (under the skin).

Erythropoietin (EPO)

A “recombinant” form of a natural growth factor used to treat symptoms associated with anemia. It stimulates the bone marrow to produce red blood cells. The three brand-name drugs are Aranesp®, Epogen®, and Procrit®. These drugs are administered intravenously or subcutaneously.

Exjade® (deferasirox)

A medication that binds to iron and promotes its removal from the body for treatment of transfusion-dependent iron overload. It is an iron-chelating drug that is administered orally.

Growth Factors (hematopoietic)

A substance made by the body that stimulates the bone marrow to produce blood cells. Some growth factors are man-made in the laboratory and used for treating low blood counts. These include red blood cell growth factors called erythropoietin (EPO) and darbepoetin, and white blood cell growth factors called granulocyte colony stimulating factors (GCSF) and granulocyte macrophage colony stimulating factors (GM-CSF). Also called cytokines.

Synonym: cytokine

Hycamtin® (topotecan hydrochloride)

A chemotherapy agent that may result in remission of MDS. It is administered intravenously.

JADENU™ (deferasirox)

A medication that binds to iron and promotes its removal from the body for treatment of transfusion-dependent iron overload. It is an iron-chelating drug that is administered orally. It is a new oral formulation of Exjade tablets. Whereas the Exjade tablet must be mixed in liquid and taken on an empty stomach, Jadenu can be taken in a single step, with or without a light meal, simplifying administration of treatment for chronic iron overload.

Leukine® (sargramostim)

A growth factor, granulocyte macrophage colony-stimulating factor (GM-CSF), used for the treatment of neutropenia. It increases

white cell production, which may help to reduce the likelihood of additional infection. It is administered subcutaneously.

Neupogen® (filgrastim)

A growth factor, granulocyte colony-stimulating factor (G-CSF), used for the treatment of neutropenia. It increases white cell production, which may help to reduce the likelihood of additional infection. It is administered subcutaneously.

Prednisone

A corticosteroid that is used for many reasons. It is prescribed when the body is not producing enough of this chemical on its own. It is sometimes prescribed with ATG treatment to reduce the risk of anaphylaxis or serum sickness. It helps by reducing the antibody production of the immune system and in treating various allergic conditions. There are many brand names of prednisone. (*See corticosteroid*)

Pyridoxine (Vitamin B₆)

A vitamin needed to make red blood cells. It can be useful in improving red blood cell counts in sideroblastic anemia by increasing the red blood cell production.

Revlimid® (lenalidomide)

A medication that works by stimulating the immune system, preventing new blood vessel growth, and stimulating cell death. It is categorized as an immunomodulatory agent and is taken orally.

Telintra™ (TLK199)

A medication that inhibits a key enzyme (glutathione S-transferase P1-1 or GST P1-1) involved in cell growth and proliferation; this results in normal blood cell production. It is given intravenously (IV).

Thalomid® (thalidomide)

A medication that reduces the blood supply in the marrow, thereby working to limit the growth of abnormal blood cells. It also acts to interfere with other proteins (cytokines) that promote premature death of cells in the bone marrow. It is taken orally.

Trisenox® (arsenic trioxide)

A medication that inhibits new blood vessel growth and stimulates cell death of abnormal cells. It may increase transfusion independence. It is administered as an intravenous infusion (IV).

Vidaza™ (azacitidine, 5-azacytidine)

A medication that works by preventing a cellular process (methylation) that silences the genes involved in controlling the development of cancer. It may increase red blood cells, transfusion independence, hemoglobin, white blood cells, platelets, and/or decreases the amount of blast cells within the bone marrow. It is categorized as a DNA hypomethylating agent and can be administered intravenously (IV) or subcutaneously (under the skin).

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