

SICKLE CELL DISEASE (SCD) THERAPIES FACT SHEET

Glossary of Terms

Acute – An illness or condition that is sudden, severe, and/or lasts for a short time, such as a cramp or a cold.

Adhesion – An unusual occurrence of body surfaces sticking together, such as magnets or blood cells.

Adverse event – A negative side effect from a treatment.

Administer – To give a treatment or service (i.e., “administer medicine”).

Amino acid – Small **molecules** that combine to form **proteins**.

Anaphylaxis – A severe, possibly deadly allergic reaction that can happen within seconds or minutes after coming into contact with something you are allergic to, such as peanuts, bee stings, or a particular medicine.

Anemia – A condition when the body's number of red blood cells is too low.

Antioxidants – Substances that can stop or slow damage to cells caused by harmful changes in blood oxygen levels.

Anti-P-selectin – If a medicine that is *anti*-p-selectin, it means that the medicine slows or stops blood cells from being as sticky, so blood can flow smoothly. P-selectin is a protein that makes the inner walls of blood vessels sticky. In SCD, p-selectin levels rise as cells break up, causing blood cells to stick to the inside of the **blood vessel** and block blood flow.

Blood vessel wall – The tubes that blood flows through, made of tissue.

Cell – The smallest unit of any living thing, such as a **red blood cell** or a **white blood cell**. Cells can only be seen under a microscope, but when together in large numbers they make up tissues, such as skin, bones, blood, and muscle.

Clinical research trial, or **clinical trial** – A study where people are assigned to receive a new treatment or a **placebo** to see whether the new treatment works.

Complication – A problem that can happen in addition to or as a result of treatment for a disease or condition (i.e., getting an infection after surgery).

Constipation – When it is difficult to have a bowel movement, usually because of hardened stool (poop).

Crescent-shaped – A thin, curved shape that is thicker in the middle and narrows to thin points at each end, like the sliver of the moon.

Endothelium – The thin layer of tissue that lines the heart and blood vessels.

Genes – The information that determines your physical traits. These traits are passed on to you (inherited) from your parents, and give your body instructions for many things, such as eye color, skin color, height, and health and disease. Humans have as many as 25,000 different genes.

Health status – The impact of a disease on a patient, including their symptoms, quality of life, and other factors, as told by the patient.

Hemoglobin (Hb) – A **protein** found in **red blood cells** that carries oxygen to all parts of the body.

Hemolysis – A condition when the **red blood cells** break up. This causes **hemoglobin** to leak into the plasma (the liquid part of your blood) and often leads to **anemia**.

Home Care Agency – A group that hires trained care providers to care for patients in their own homes.

Hydroxyurea – A medicine used to treat many types of blood disorders, including SCD.

Infusion centers – A clinic that provides treatment **intravenously**.

Infusion therapy – Therapy in which the patient receives fluids or medicine **intravenously**, instead of by mouth (orally).

Infusion reactions – A side effect that might happen after the infusion of medicine. The reaction can be mild to severe. Some reactions happen during or within minutes after getting an infusion. Others can happen up to a few days or weeks after getting an infusion. For example, a reaction might make you feel tired or sick to your stomach.

Intravenously – A treatment that is given by inserting a needle or catheter (a thin tube) into a vein.

Medical condition – A disease, illness, or injury that can take place in your body or your mind.

Milligram (mg) – One thousandth (1/1000) of a gram; about the weight of a small feather.

Molecule – A small particle made up of one or more atoms. All things are made of molecules.

Nausea – Feeling the urge to vomit.

Glossary of Terms

Outpatient – A procedure where a patient receives treatment at a hospital or clinic but does not stay there overnight.

Oxygen-binding – Hemoglobin is a molecule that carries most of the oxygen in our body. Each hemoglobin molecule can connect with, or bind to, up to four oxygen molecules. (See **Hemoglobin** and **Molecule**)

Pharmaceutical grade – A medicine that has been found to be very pure and safe for use in humans as decided by the U.S. Food and Drug Administration.

Placebo – A harmless substance (such as a pill) or procedure (such as type of surgery) that deliberately does not work. It is used in research to compare the effect of the treatment being tested, such as a drug, and no treatment at all. Placebos help scientists find out whether a new treatment actually works and if it causes side effects.

Platelets – A very small, round piece of a cell found in large numbers in blood. Platelets are made in the bone marrow. They stick together and help with blood clotting when a blood vessel is damaged. For example, if you cut your knee, platelets help make the clot that stops the bleeding.

Polymerize (polymerization) – The term polymer comes from two Greek words: “poly” and “meros”, which together, mean “many parts.” Polymerization happens when many small **molecules** stick together to make a very large molecule, called a polymer.

Protein – Large **molecules** that help build, maintain, and repair the tissues in your body.

Random – Lacking aim or direction (i.e., flipping a coin, which randomly lands on heads or tails).

Red blood cell – The most common **cell** in the blood. Red blood cells contain **hemoglobin**, which carries oxygen to all of the tissues in the body. A typical red blood cell is shaped like a round disk with thinner section in the middle, like a doughnut without a hole. Red blood cells are made in the bone marrow and last for about 120 days, so they are constantly replaced.

Research or **research study** – The process a scientist follows to learn more about a topic.

Sickle Cell Genotypes – SCD can be caused by different combinations of genes (called “genotypes”):

- **HbSS** – People who have this form of SCD inherit two sickle cell genes (“S”), one from each parent. This is commonly called *sickle cell anemia* and is usually the most severe form of the disease.
- **HbSC** – People who have this form of SCD inherit one sickle cell gene (“S”) from one parent and one abnormal hemoglobin called “C” from the other parent.
- **HbS beta 0-thalassemia (or Sβ0-thalassemia)** – People who have this form of SCD inherit one sickle cell gene (“S”) from one parent and one gene for beta thalassemia, another type of anemia, from the other parent. Individuals with HbS beta 0-thalassemia usually have a severe form of the disease similar to HbSS.
- **HbS beta + thalassemia (or Sβ+thalassemia)** – People who have this form of SCD inherit one sickle cell gene (“S”) from one parent and one gene for beta thalassemia, another type of anemia, from the other parent. Individuals with HbS beta + thalassemia tend to have a milder form of the disease.

Sickle – The shape **red blood cells** become when **hemoglobin** sticks together – fragile, stiff, and **crescent-shaped**.

Vascular – Related to the movement of body fluids, such as blood, through vessels or a system of vessels. Your heart and blood vessels make up your vascular system.

Vasculopathy – Any disease that affects blood vessels.

Vaso-occlusive crises – A common, painful complication of SCD caused by the blockage of blood flow by **sickled** cells.

White blood cell – A type of blood cell that is made in the bone marrow. These cells are part of the body’s immune system, which helps fight against infection and other diseases.