

STEM CELL GLOSSARY

Term	Definition
Adult stem cells	A commonly used term for tissue-specific stem cells. Stem cells that are found in many of our adult organs and tissues. They can generate some or all of the cell types in that organ or tissue but cannot give rise to every cell type in the adult body. This type of stem cell is responsible for replenishing the cells that make up the particular organ or tissue in which it is found.
Approved stem cell treatments	Treatments that are backed by convincing evidence of efficacy and safety, approved by the appropriate regulatory bodies, and are widely accepted by the global medical community. At this time, beyond the treatment of various cancers of the blood and selected immunological conditions, there are very few conditions for which stem cell-based therapies are established as effective and safe treatments.
Autologous	Cells or tissues from the same individual; an autologous bone marrow transplant involves one individual as both donor and recipient.
Basic Research	Research designed to increase our knowledge and understanding, without necessarily having immediate practical applications or a primary goal to solve a problem.
Biopsy	Sample of tissue or cells that has been removed from the body, primarily to diagnose or treat a disease.
Blastocyte	A very early stage of development that consists of a mainly hollow ball of about 150-200 cells that forms 3-5 days after an egg cell is fertilized by a sperm. In humans, the blastocyte gives rise to every single cell type a human body needs to survive, including the umbilical cord and the placenta.
Blinded clinical trial	Participants do not know if they receive the treatment that is being assessed, a control treatment, or a placebo.
Blood stem cell transplantation/bone marrow transplantation	Bone marrow, blood stem cell transplantation, or hematopoietic stem cell transplantation is one of the only stem cell treatments that has been proven to be effective and safe. Through blood stem cell transplantation, healthy blood stem cells can replace diseased ones, potentially making all future blood and immune cells and treating the patient. Blood stem cell transplantations are most commonly used to treat blood diseases (such as cancers or red blood cell disorders), bone marrow failure diseases, and certain diseases that result from missing or dysfunctional immune cells. Inherited metabolic diseases (deficiencies in breaking down substances in the body) can also be treated by transplantation.
Bone marrow stromal cells	A general term for non-blood cells in the bone marrow, such as fibroblasts, adipocytes (fat cells) and bone- and cartilage-forming cells that provide support for blood cells. Contained within this population of cells are multipotent bone marrow stromal stem cells that can self-renew and give rise to bone, cartilage, adipocytes, and fibroblasts.
Chimera	Persons or animals that have some living cells in their body that came from another person or animal. Chimerism can occur in nature, can be the result of a transplant, or chimeric animals can be created in the lab as part of biomedical research.

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Clinical research	Research that focuses on studying the safety and efficacy of medical treatments (i.e. drugs, devices, medications) and diagnostic tools in human subjects.
Clinical translation	A multi-step process taking scientific research discoveries and findings from the laboratory (basic research) and applying them into real-world clinical settings (clinical research).
Clinical trial	A kind of clinical research designed to answer specific questions about a new treatment or a new way of using a current treatment that also establishes whether potential treatments are effective and safe.
Control group	Group of participants who do not receive the experimental treatment under investigation. Instead, they are given an existing approved treatment, or a placebo. This group is essential to determine the overall efficacy and safety of the experimental treatment under study.
Cord Blood	The blood in the umbilical cord and placenta after a baby is delivered. Cord blood contains all components of whole blood and blood stem cells. Blood stem cells can regenerate the blood and immune system and can be used to treat some blood disorders such as leukemia or anemia.
Differentiation	The process by which cells become increasingly specialized to carry out specific functions in tissues and organs.
Disease modeling	The process of creating in vitro, or lab-based, models that replicate the features and characteristics of a particular disease. These models are used to study the mechanisms of how the disease develops and to develop and test potential treatments.
Drug screening	The process of testing large numbers of potential drug candidates for activity, function and/or toxicity in defined assays.
Efficacy	The ability to produce a desired or intended result. Also called effectiveness.
Embryo	Generally used to describe the stage of development between fertilization and the fetal stage; the embryonic stage ends 7-8 weeks after fertilization in humans.
Embryo Model	Organized 3-D structures created by stem cells that mimic the developmental processes of the early-stage human embryo but do not currently have the features or function required to become a proper human embryo.
Embryonic stem cell (ESC)	Undifferentiated cells derived from the inner cell mass of the blastocyst which give rise to every cell type in the adult body.
Exosomes	Small, sac-like vesicles produced by cells that can contain various molecules, such as proteins and genetical material (like RNA and DNA). Their role is to help cells communicate with each other and transport molecules throughout our bodies. There are currently no FDA-approved exosome products.
Fibroblast	A common connective or support cell found within most tissues of the body.
Hematopoietic	Blood-forming; hematopoietic stem cells give rise to all the cell types in the blood.
Induced pluripotent stem cell (iPSC)	Stem cells that have been made in the lab by converting tissue-specific cells into cells that behave like embryonic stem cells, are pluripotent, and can self-renew.



Informed or Treatment Consent Form	Document that outlines a patient's role in receiving an experimental treatment, and the possible implications of proceeding with the investigational or unapproved treatment. It should provide a clear and detailed description of the treatment or procedure in plain language and explain a patient's options for treatment, their rights and responsibilities, and the risks.
Investigational treatment/Investigational procedure	Terms sometimes used to describe stem cell treatments that are being tested in clinical trials but are not approved as effective and safe. Until formal approval, a stem cell treatment that is being tested in clinical trials is considered investigational. It is important to understand that testing a stem cell treatment in clinical trials does not guarantee that it will become an approved and established therapy.
In vitro	Latin for "in glass." In biomedical research, this refers to experiments that are done outside the body in an artificial environment, such as the study of isolated cells in controlled laboratory conditions (also known as cell culture).
In vivo	Latin for "within the living." In biomedical research, this refers to experiments that are done in a living organism, such as mice or fruit flies.
Mesenchymal stem cells (MSC)	A term used to describe cells isolated from the connective tissue that surrounds other tissues and organs. MSCs were first isolated from the bone marrow and shown to be capable of making bone, cartilage and fat cells. MSCs are now grown from other tissues, such as fat and cord blood. Not all MSCs are the same and their characteristics depend on where in the body they come from and how they are isolated and grown. May also be called mesenchymal stromal cells.
Mitochondrial disease	Disease caused by mutations in mitochondrial DNA that is passed down from parent to child and can lead to a wide variety of symptoms throughout the body.
Mitochondrial replacement therapy (MRT)	A treatment where parental genetic information is transferred to a donor egg that has healthy mitochondria, so that the offspring will not inherit mitochondrial disease from an affected parent but be biologically related to both parents.
Multipotent stem cells	Stem cells that can give rise to several different types of specialized cells in specific tissues; for example, blood stem cells can produce the different types of cells that make up the blood, but not the cells of other organs such as the liver or the brain.
Organ	Group of tissues that structurally form a functional unit that performs a specific function in the body.
Organoid	Self-organized three-dimensional structures that are typically derived from stem cells and mimic key aspects of the functional, structural, and biological complexity of an organ. Organoids are tools for advancing our understanding of biology and are used for a wide variety of applications, such as drug discovery and cell therapy. They are not miniature organs.

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Patient testimonials	A statement based on personal knowledge or belief that endorses the services provided. These statements may be subjective, influenced, and lack the scientific rigor necessary to establish the safety and efficacy of a treatment. It is critical to rely on evidence-based research and consult with healthcare professionals when making personal health decisions
Phase 1 clinical trial	Researchers test an experimental drug or treatment in a small group of people (20-100) for the first time. The researchers evaluate the treatment's safety, determine a safe dosage range, and identify side effects.
Phase 2 clinical trial	The experimental drug or treatment is given to a larger group of people (up to 300) to see if it is effective and to further evaluate its safety.
Phase 3 clinical trial	The experimental study drug or treatment is given to large groups of people (300-3,000). Researchers confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.
Phase 4 clinical trial	Post-marketing studies, which are conducted after a treatment is approved for use by the FDA, provide additional information including the treatment or drug's risks, benefits, and best use.
Placebo	An inactive treatment that is used as a comparison to help researchers determine whether a treatment is more effective than no treatment at all.
Placebo effect	Perceived or actual improvement in symptoms that cannot be attributed to the placebo itself and therefore must be the result of the patient's (or other interested person's) belief in the treatment's effectiveness.
Pluripotent stem cell	Stem cells that can differentiate into any cell in the body. The types of pluripotent stem cells are: embryonic stem cells and induced pluripotent stem cells.
Preclinical research	Builds upon the findings of basic research and involves further laboratory research on cells, tissues, and/or animals to test for safety, toxicity, and efficacy in model systems prior to clinical research in human patients.
Progenitor cells	An intermediate cell type between stem cells and differentiated cells. Progenitor cells have the potential to give rise to a limited number or type of specialized cells and have a reduced capacity for self-renewal. Also called precursor cells.
Randomized clinical trial	Participants are randomly assigned to receive different interventions in order to reduce bias.
Regenerative medicine	An interdisciplinary branch of medicine with the goal of replacing, regenerating or repairing damaged cells, tissues, or organs to restore normal function or structure.
Reprogramming	In the context of stem cell biology, this refers to the conversion of differentiated cells into embryonic-like induced pluripotent stem cells by artificially altering the expression of key genes.
Self-renewal	A special type of cell division in stem cells by which they make copies of themselves.
Stem cells	Unique cells in our bodies defined by two properties; they can divide to make more of themselves, and they can generate specialized cell types such as skin, muscle, or blood cells.

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Stem cell tourism	The travel to another state, region, or country specifically for the purpose of undergoing a stem cell treatment available at that location. This phrase is also used to refer to the pursuit of untested and unregulated stem cell treatments.
Stem cell treatments	Uses stem cells or the specialized cell types that come from stem cells to replace or repair a patient's cells or tissues that are damaged or absent.
Teratoma	A benign tumor that usually consists of several types of tissue cells that are foreign to the tissue in which the tumor is located.
Tissue	A group of cells with a similar function or embryological origin. Tissues organize further to become organs.
Tissue-specific stem cells	Stem cells that are found in many of our adult organs and tissues. They can generate some or all of the cell types in that organ or tissue but cannot give rise to every cell type in the adult body. This type of stem cell is responsible for replenishing the cells that make up the particular organ or tissue in which it is found. Also called adult or somatic stem cells.
Totipotent cells	Cells with the ability to give rise to all the cells of the body and cells that aren't part of the body but support embryonic development, such as the placenta and umbilical cord.
Translational research	Research that focuses on how to use knowledge learned from basic research to prevent, diagnose, and treat diseases.
Unproven stem cell "treatment"	Treatment that has not been tested through formal or regulated clinical trials and has not been shown to be effective or safe. Unproven stem cell "treatments" are currently being marketed and administered by stem cell clinics and providers around the world operating outside accepted ethical standards. Administration of unproven "treatments" can cause physical, psychological, and/or financial harm.
Zygote	A single cell that forms when an egg is fertilized by a sperm. Every cell type in the human body arises from this single cell. Within a few days, that single cell divides over and over again until it forms a blastocyte.