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National Alliance of Healthcare Purchaser Coalitions
Driving Innovation, Health and Value

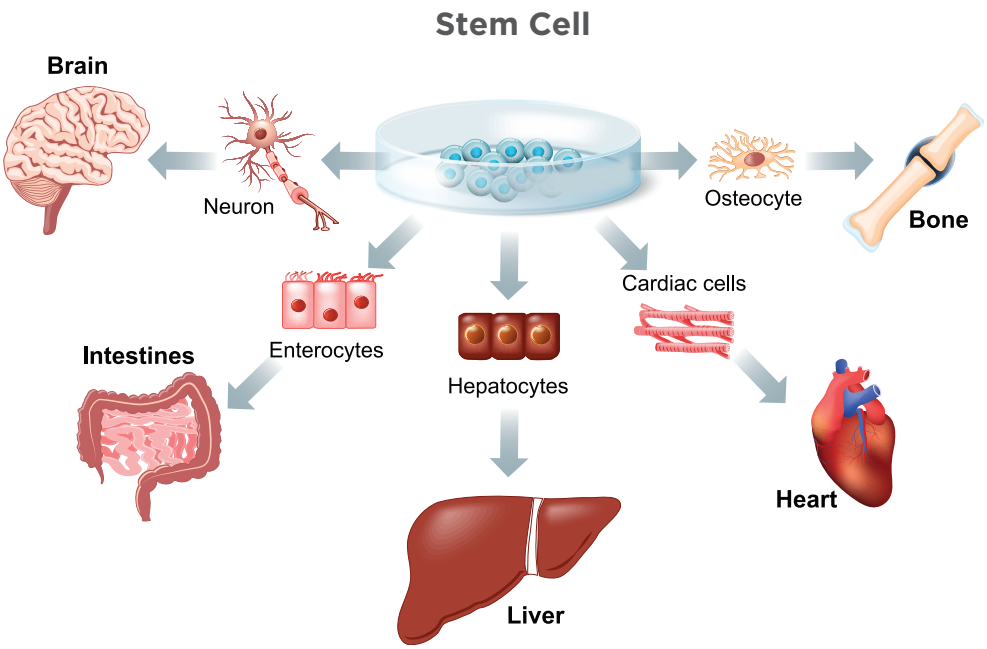
Clinical Brief

Regenerative Medicine

A topic of emerging importance for employers is the value and use of stem cell therapies. Also known as **regenerative medicine**, these therapies offer great promise for new medical treatments. As employers determine whether and how to include them as covered benefits, this topic will remain a subject of debate in board rooms and break rooms. To gain insight, it's important to first understand the basics.

What are stem cells?

Stem cells are the body's raw materials —cells from which all other cells with specialized functions are generated. Stem cells are undifferentiated, meaning they're capable of developing into cells that serve numerous functions in different parts of the body. Most cells in the body are differentiated, or specialized cells. These cells can only serve a specific purpose in a particular organ. For example, red blood cells are specifically designed to carry oxygen, and cells lining the GI tract are responsible for absorption of nutrients. However, under the right conditions in the body or a laboratory, stem cells can become new stem cells (self-renewal) or become specialized cells (differentiation) with a more specific function, such as blood cells, brain cells, heart muscle cells, or bone cells. No other cell in the body has the natural ability to generate new cell types.



Stem cell therapy promotes the repair response of diseased, dysfunctional or injured tissue using stem cells or their derivatives.

What is stem cell therapy?

The underlying premise (and promise) of stem cell therapy is that stem cells can be directed into becoming specific, specialized cells that can regenerate and repair diseased or damaged tissues in people. Use of stem cell therapy is currently under consideration for treatment of a number of medical conditions, as shown in Table 1.

Stated somewhat differently, stem cell therapy promotes the repair response of diseased, dysfunctional or injured tissue using stem cells or their derivatives. This treatment approach represents the next chapter in organ transplantation and uses cells instead of donor organs, which are limited in supply.

Stem cells may have the potential to be grown to become new tissue for use in transplant and regenerative medicine. Researchers continue to advance the knowledge about stem cells and their applications in transplant and regenerative medicine.

Where do stem cells come from?

- **Adult stem cells.** These stem cells are found in small numbers in most adult tissues, such as bone marrow or fat, and are in current clinical use for bone marrow transplants. Compared with embryonic stem cells (described below), adult stem cells have a more limited ability to be transformed into various cells of the body.

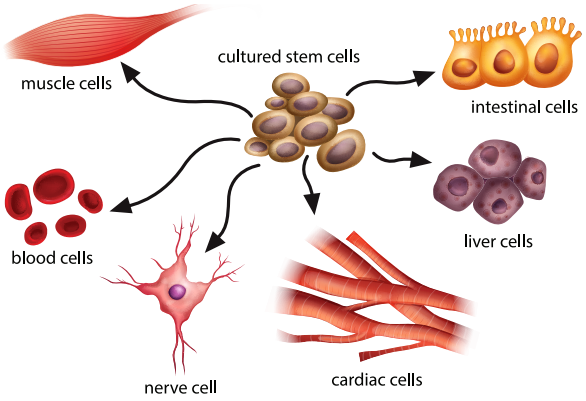
  Although research into adult stem cells is promising, adult stem cells may not be as versatile and durable as embryonic and cord blood stem cells. Adult stem cells may not be able to be manipulated to produce all cell types, which limits their use in disease treatment, but their value appears to have greater potential than initially surmised. Until recently, researchers thought adult stem cells could create only similar types of cells. For instance, researchers thought that stem cells residing in the bone marrow could give rise only to blood cells. However, emerging evidence suggests that adult stem cells may be able to create at least some other cell types. For instance, bone marrow stem cells may be able to differentiate into bone or heart muscle cells. Early-stage clinical trials are underway to evaluate usefulness of these cells in treatment. Another important consideration is that adult stem cells also are more likely to contain abnormalities due to environmental hazards, such as toxins, or from errors acquired by the cells during replication. These limitations may well impact the potential value of adult stem cells in development of treatments.

- **Embryonic stem cells.** These stem cells come from embryos that are three to five days old. Unlike adult stem cells, embryonic stem cells can divide into more stem cells or can become virtually every type of cell in the body. This versatility allows embryonic stem cells to be used to regenerate or repair diseased tissue and organs. While there is certainly great clinical treatment potential for the use of these cells, two important barriers exist. First, it is impossible for an individual to receive his or her own embryonic cells. Another important challenge of using embryonic stem cells, particularly in the research setting, is assuring access due to ethical and legal considerations relating to the status of embryos.

- **Perinatal stem cells.** Umbilical cord blood has been identified as a rich source of stem cells, having a wide capability to differentiate into an array of different cell types. Umbilical cord blood samples can be readily obtained at the time of birth, and the process requires only minimal additional expertise. Importantly, stem cells in umbilical cord blood can be frozen and stored virtually indefinitely until a need for their use arises. These cells have been used extensively over the past 30 years in stem cell transplant and are currently being investigated in human clinical trials for additional clinical applications.

  Researchers have discovered stem cells in amniotic fluid. These stem cells also have the ability to change into specialized cells. Amniotic

Potential Application of Human Stem Cells



3 Ways to Donate Marrow

fluid fills the sac that surrounds and protects a developing fetus in the uterus. Stem cells have been identified in samples of amniotic fluid drawn from pregnant women to test for abnormalities — a procedure called amniocentesis. While this approach holds some promise for ensuring access to stem cells, amniocentesis is not without risk to the fetus. Newer genetic tests may also reduce the need for amniocentesis in detecting genetic or other abnormalities in the fetus, effectively limiting access to these stem cells.

What are current uses of stem cell therapy?

The most successful stem cell therapy — bone marrow transplant — has been around for more than 60 years. Stem cells are infused to replace the patient's own bone marrow cells that have been damaged by disease and/or chemotherapy or serve as a way for the donor's immune system to fight some types of cancer and blood-related diseases, such as leukemia, lymphoma, neuroblastoma and multiple myeloma. Though commonly called "bone marrow transplant," the procedure is technically a hematopoietic stem cell transplant and adult stem cells or umbilical cord blood cells are used.

Another FDA-approved use of stem cell therapy involves the growth of skin grafts for treatment of significant burns. Stem cells are induced to differentiate into skin cells and can replace those lost as a result of serious burn injuries.

Lastly, in Europe, stem cell treatment of corneal injuries has recently received regulatory approval for use. While this indication for use has not yet received FDA approval in the US, it is an indication of the growing scope of potential applications for this type of treatment.

Ongoing research efforts regarding stem cell use in treatment of diseases

If researchers can find a reliable way to direct the differentiation of stem cells, they may be able to use the cells to treat certain diseases. The implications of this treatment approach are profound. For example, by directing the cord blood stem cells to turn into insulin-producing cells, researchers may be able to transplant the cells into people with type 1 diabetes.

In California, the state's own Stem Cell Agency provides a detailed list of the disease programs and clinical trials currently underway in stem cell research. Examples of such research activities include:

- injecting modified stem cells directly into the brain after a stroke
- using stem cells to replace damaged cells in the inner ear that detect sound, helping to restore hearing
- altering the genes of stem cells to make them resistant to diseases, such as AIDS, and then inserting them into people with the disease
- cultivating stem cells to repair the fragile bones of people with osteoporosis

Table 1. Representative medical conditions that may potentially be treated with stem cells include:

autism spectrum disorder

heart disease

primary immune diseases

traumatic spinal cord injury

hemoglobin disorders

retinal disease

stroke

hearing loss

Huntington's disease

rheumatoid arthritis

multiple sclerosis

Parkinson's disease

Significance for employers

Stem cell-based treatments hold promise for effective treatment of many chronic conditions, particularly those that are progressive, including diabetes, central nervous system disorders, cancers, and autoimmune disorders. All of these conditions are associated with ongoing healthcare costs that are often recurrent, increasing and substantial. Use of stem cell treatments may interrupt the progressive nature of these conditions, and significantly reduce future associated healthcare expenditures.

Employer considerations

- Understand the current and potential value of stem cell-derived treatments in terms of current and future applications. Importantly, use of a patient's own cells instead of a donor's cells or organ can virtually eliminate concerns of immune reaction against the implanted tissue.
- Be aware of evolving science and potential applications for stem cell therapy. As a rapidly developing field of research, it is virtually certain that new treatments for an array of conditions will become available.
- Appreciate that health plan clinical coverage policies for stem cell therapy are reviewed and updated on an ongoing basis, with changes impacting coverage scope and consequently, potential employer financial exposure.
- At the patient level, understand the role and future potential value of cord blood stem cells, and ongoing approaches to ensuring future access.

Resources

Mayo Clinic: Stem Cells: What They Are and What They Do

US News & World Report: Before You Undergo Stem Cell Treatment

Wikipedia: Stem-cell Therapy

California Stem Cell Agency

WebMD: Should You Bank Your Baby's Cord Blood?

Pregnancy and Cord Blood Banking

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