

The Power of Stem Cells

En Español

Stem cells have the potential to treat a wide range of diseases. Here, discover why these cells are such a powerful tool for treating disease—and what hurdles experts face before new therapies reach patients.

- How can stem cells treat disease?
- What diseases could be treated by stem cell research?
- How can I learn more about CIRM-funded research in a particular disease?
- What cell therapies are available right now?
- What about the therapies that are available overseas?
- Why does it take so long to create new therapies?
- How do scientists get stem cells to specialize into different cell types?
- How do scientists test stem cell therapies?
- Can't stem cell therapies increase the chances of a tumor?
- Is there a risk of immune rejection with stem cells?
- How do scientists grow stem cells in the right conditions?

How can stem cells treat disease?

When most people think about about stem cells treating disease they think of a stem cell transplant.

In a stem cell transplant, stem cells are first specialized into the necessary adult cell type. Then, those mature cells replace tissue that is damaged by disease or injury. This type of treatment could be used to:

- Replace neurons damaged by spinal cord injury, stroke, Alzheimer's disease, Parkinson's disease or other neurological problems;
- Produce insulin that could treat people with diabetes or cartilage to repair damage caused by arthritis; or
- Replace virtually any tissue or organ that is injured or diseased.

But stem cell-based therapies can do much more.

- Studying how stem cells develop into heart muscle cells could provide clues about how we could induce heart muscle to repair itself after a heart attack.
- The cells could be used to study disease, identify new drugs, or screen drugs for toxic side effects.

Any of these would have a significant impact on human health *without transplanting a single cell*.

What diseases could be treated by stem cell research?

In theory, there's no limit to the types of diseases that could be treated with stem cell research. Given that researchers may be able to study all cell types they have the potential to make breakthroughs in any disease.

How can I learn more about CIRM-funded stem cell research in a particular disease?

CIRM has created disease pages for many of the major diseases being targeted by stem cell scientists. You can find those disease pages [here](#).

You can also sort our [complete list of CIRM awards](#) to see what we've funded in different disease areas.

What cell therapies are available right now?

While there are a growing number of potential therapies being tested in clinical trials there are only a few stem cell therapies that have so far been approved by the FDA. Two therapies that CIRM provided early funding for have been approved. Those are:

- Fedratinib, approved by the FDA in August 2019 as a first line therapy for myelofibrosis (scarring of the bone marrow)
- Glasdegib, approved in November 2016 as a combination therapy with low dose ara-C for patients 75 years of age and older with acute myelogenous leukemia

Right now the most commonly used stem cell-based therapy is bone marrow transplantation. Blood-forming stem cells in the bone marrow were the first stem cells to be identified and were the first to be used in the clinic. This life-saving technique has helped thousands people worldwide who had been suffering from blood cancers, such as leukemia.

In addition to their current use in cancer treatments, research suggests that bone marrow transplants will be useful in treating autoimmune diseases and in helping people tolerate transplanted organs.

Other therapies based on adult stem cells are currently in clinical trials. Until those trials are complete we won't know which type of stem cell is most effective in treating different diseases.

Find Out More:

Read the top ten things to know about stem cell treatments (from ISSCR)



Alan Lewis talks about getting an embryonic stem cell-based therapy to patients (3:46)

What about stem cell therapies that are available overseas?

Many overseas clinics - and a growing number here in the U.S. too - advertise "miraculous" stem cell therapies for a wide range of incurable diseases. This phenomenon is called stem cell tourism and is currently a source of concern for reputable stem cell scientists. These predatory clinics are offering therapies that have not been tested to prove they are effective or even safe. In recent few years, some patients who visited those clinics have died, others have been left blind or had serious infections as a result of receiving unproven and untested stem cells.

Find Out More:

Learn more about the issue on our Stem Cell Tourism page.



Jeanne Loring discusses concerns about stem cell tourism (3:38)



CIRM/ISSCR panel on stem cell tourism

Why does it take so long to create new stem cell therapies?

Stem cells hold the potential to treat a wide range of diseases. However, the path from the lab to the clinic is a long one. Before testing those cells in a human disease, researchers must grow the right cell type, find a way to test those cells, and make sure the cells are safe in animals before moving to human trials.

Find Out More:



Hans Keirstead talks about hurdles in developing a new therapy (5:07)

How do scientists get stem cells to specialize into different cell types?

One of the biggest hurdles in any stem cell-based therapy is coaxing stem cells to become a single cell type. The vital process of maturing stem cells from one state to another type is called differentiation.

Guiding stem cells to become a particular cell type has been fraught with difficulty. For example, stem cells growing in a developing embryo receive a carefully choreographed series of signals from the surrounding tissue. To create the same effect in the lab, researchers have to try and mimic those signals. Add the signals in the wrong order or the wrong dose and the developing cells may

choose to remain immature—or become the wrong cell type

Many decades of research has uncovered many of the signals needed to properly differentiate cells. Other signals are still unknown. Many CIRM-funded researchers are attempting to differentiate very pure populations of mature cell types that can accelerate therapies.

Find Out More:



Mark Mercola talks about differentiating cells into adult tissues (3:37)

How do scientists test stem cell therapies?

Once a researcher has a mature cell type in a lab dish, the next step is to find out whether those cells can function in the body. For example, embryonic stem cells that have matured into insulin-producing cells in the lab are only useful if they continue producing insulin once transplanted inside a body. Likewise, researchers need to know that the cells can integrate into the surrounding tissue and not be rejected by the body.

Scientists test cells by first developing an animal model that mimics the human disease, and then implanting the cells to see if they help treat the disease. These types of experiments can be painstaking—because even if the cells don't completely cure the disease, they may restore some functions that would still be of enormous benefit to people. Researchers have to examine each of these possible outcomes.

In many cases testing the cells in a single animal model doesn't provide enough information. Most animal models of disease don't perfectly mimic the human disease. For example, a mouse carrying the same mutation that causes cystic fibrosis in humans doesn't show the same signs as a person with the disease. So, a stem cell therapy that treats this mouse model of cystic fibrosis may not work in humans. That's why researchers often need to test the cells in more than one animal model.

Can't stem cell therapies increase the chances of a tumor?

The promise of embryonic stem cells is that they can form any type of cell in the body. The trouble is that when implanted into an animal they do just that, in the form of tumors called *teratomas*. These tumors consist of a mass of many cell types and can include hair cells and many other tissues.

These teratomas are one reason why it is necessary to mature the embryonic stem cells into highly purified adult cell types before implanting into humans. Virtually all evidence has shown that the mature cells are restricted to their one identity and don't appear to revert to a teratoma-forming cell.

Find Out More:

UC Davis researcher focuses on stem cell safety (from UC Davis)



Paul Knoepfler talks about the tendency of embryonic stem cells to form tumors (4:10)

Is there a risk of immune rejection from stem cells?

Transplanted stem cells, like any transplanted organ, can be recognized by the immune system as foreign and then rejected. In organ transplants such as liver, kidney, or heart, people must be on immune suppressive drugs for the rest of their lives to prevent the immune system from recognizing that organ as foreign and destroying it.

The likelihood of the immune system rejecting a transplant of embryonic stem cell-based tissue depends on the origin of that tissue. Stem cells isolated from IVF embryos will have a genetic makeup that will not match that of the person who receives the transplant. That person's immune system will recognize those cells as foreign and reject the tissue unless a person is on powerful immune suppressive drugs. The same is true for adult stem cells from a donor.

Stem cells generated through SCNT or iPS cell technology, on the other hand, are a perfect genetic match. The immune system would likely overlook that transplanted cells, seeing it as a normal part of the body. Still, some suggest that even if the cells are perfectly matched, they may not entirely escape the notice of the immune system. Cancer cells, for example, have the same genetic make up as surrounding tissue and yet the immune system will often identify and destroy early tumors. Until more information is available from animal studies it will be hard to know whether transplanted patient-specific cells are likely to call the attention of the immune system.

Find Out More:



Jeffrey Bluestone talks about immune rejection of stem cell-based therapies (4:05)

How do scientists grow stem cells in the right conditions?

In order to be approved by the FDA for use in human trials, stem cells must be grown in *good manufacturing practice (GMP) conditions*. Under GMP standards, a cell line has to be manufactured so that each group of cells is grown in an identical, repeatable, sterile environment. This ensures that each batch of cells has the same properties, and each person getting a stem cell therapy gets an equivalent treatment.

Updated 2/16

Source URL: <https://www.cirm.ca.gov/patients/power-stem-cells>