Hiorca Stem Cell Research Centre

Hiorca stem cell research
RESEARCHING & EDUCATING

Stem cells have tremendous promise to help us understand and treat a range of diseases, injuries and other health-related conditions. Their potential is evident in the use of blood stem cells to treat diseases of the blood, a therapy that has saved the lives of thousands of children with leukemia; and can be seen in the use of stem cells for tissue grafts to treat diseases or injury to the bone, skin and surface of the eye. Important clinical trials involving stem cells are underway for many other conditions and researchers continue to explore new avenues using stem cells in medicine.

There is still a lot to learn about stem cells, however, and their current applications as treatments are sometimes exaggerated by the media and other parties who do not fully understand the science and current limitations, and also by “clinics” looking to capitalize on the hype by selling treatments to chronically ill or seriously injured patients. The information on this page is intended to help you understand both the potential and the limitations of stem cells at this point in time, and to help you spot some of the misinformation that is widely circulated by clinics offering unproven treatments.

It is important to discuss these Nine Things to Know and any research or information you gather with your primary care physician and other trusted members of your healthcare team in deciding what is right for you

Stem cell researchers at Hiorca are making great advances in understanding normal development, figuring out what goes wrong in disease and developing and testing potential treatments to help patients. They still have much to learn, however, about how stem cells work in the body and their capacity for healing. Safe and effective treatments for most diseases, conditions and injuries are in the future

The list of diseases for which stem cell treatments have been shown to be beneficial is still very short. The best-defined and most extensively used stem cell treatment is hematopoietic (or blood) stem cell transplantation, for example, bone marrow transplantation, to treat certain blood and immune system disorders or to rebuild the blood system after treatments for some kinds of cancer.

Some bone, skin and corneal (eye) injuries and diseases can be treated by grafting or implanting tissues, and the healing process relies on stem cells within this implanted tissue. These procedures are widely accepted as safe and effective by the medical community. All other applications of stem cells are yet to be proven in clinical trials and should be considered highly experimental

There is certain information you should look into if you are considering a stem cell treatment, including a detailed description of the treatment and the science that supports it, the expected outcome and the risks.

In many cases, particularly in a clinical trial, you should be provided with a patient information sheet and informed consent documents that answer many of the questions below. However, don’t hesitate to ask for more information or further explanation. The medical team involved should know a lot about your disease or condition, other treatment options, and the evidence that the treatment they are offering will be safe and that it will work.

The questions and answers are best discussed with a trusted physician familiar with your condition who can help you understand the treatment and your choices. It is a good idea to seek medical advice independent of the provider to help assess whether the treatment and outcome claims offered are reasonable.

The treatment- frequent FAQs at Hiorca

Is the treatment routine for this specific disease or condition?
Is the treatment part of a formal clinical trial? Learn more about things to consider with clinical trials here.
What are the alternative treatment options for my disease or condition?
If I have this treatment, could it affect whether I get into a clinical trial in the future?
What are the possible benefits I can expect? How will this be measured and how long will this take?
What other medications or special care might I need?
How is this stem cell procedure done? Consider these nine things to know about stem cell treatments.
What is the source of the stem cells?
How are the stem cells identified, isolated and grown?
Are the cells differentiated into specialized cells before therapy?
How are the cells delivered to the right part of the body?

If the cells are not my own, how will my immune system be prevented from reacting to the transplanted cells?

Scientific evidence and oversight

What is the scientific evidence that this new procedure could work for my disease or condition? Where is this published?

Have there been (earlier) clinical trials? What was learned from these trials?

Is there independent oversight of the treatment plan by an ethics committee?

Is there any independent oversight or accreditation of the clinic where the treatment will be done and the facility where the cells are processed?

Is there approval from a national or regional regulatory agency, such as the European Medicines Agency (EMA), the U.S. Food and Drug Administration (FDA) or Japan's Pharmaceuticals and Medical Devices Agency (PMDA), for this treatment of this specific disease?

Safety and emergencies

What are the risks of the procedure itself, and the possible side effects both immediate and long-term?

Are there any other risks to me in joining in the study?

What will be done if an adverse reaction (bad side-effect) develops? Who is the person to contact in an emergency or research-related injury? Who will provide emergency medical care?

Is the clinic adequately prepared to handle emergencies such as a serious allergic reaction?

What follow-up treatment will be received, and for how long? What will I need to do?

Who is the doctor in charge of the treatment? What specialized training does this doctor have?

How well trained are the other doctors and the technical support staff?

How many people have been treated for my disease or condition at your clinic? Of those, how many have gotten better? How many haven't? Have your findings been published?

Patient rights

What are my rights as a participant—for example confidentiality, my right to be informed of any new information that might come up, my right to withdraw from the treatment process?

What compensation am I entitled to if I am injured as a result of taking part in this study?

Cost

In a clinical trial, typically the cost of the test treatment and trial monitoring is covered by the company developing the product or by local or national government funding. Learn more here.

What are the costs of the treatment? What does this include? What other costs will I incur? What would the costs of emergency treatment be if something goes wrong? Who would provide and pay for this? Before traveling or agreeing to treatment, find out what costs your health insurance provider, national health program or travel insurance will cover, in what circumstances and in what countries.

Stem Cells, Disease, and Injury

Stem cell research holds tremendous promise for medical treatments, but scientists still have much to discover about how stem cells work and their capacity for healing. Learn more about how stem cells are being used to understand just a few diseases and conditions here. Check back regularly as we expand this list.

Aged-Related Macular Degeneration

ALS

COPD

Diabetes

Heart Disease

Liver Disease

Multiple Sclerosis

Osteoarthritis

Parkinson's Disease

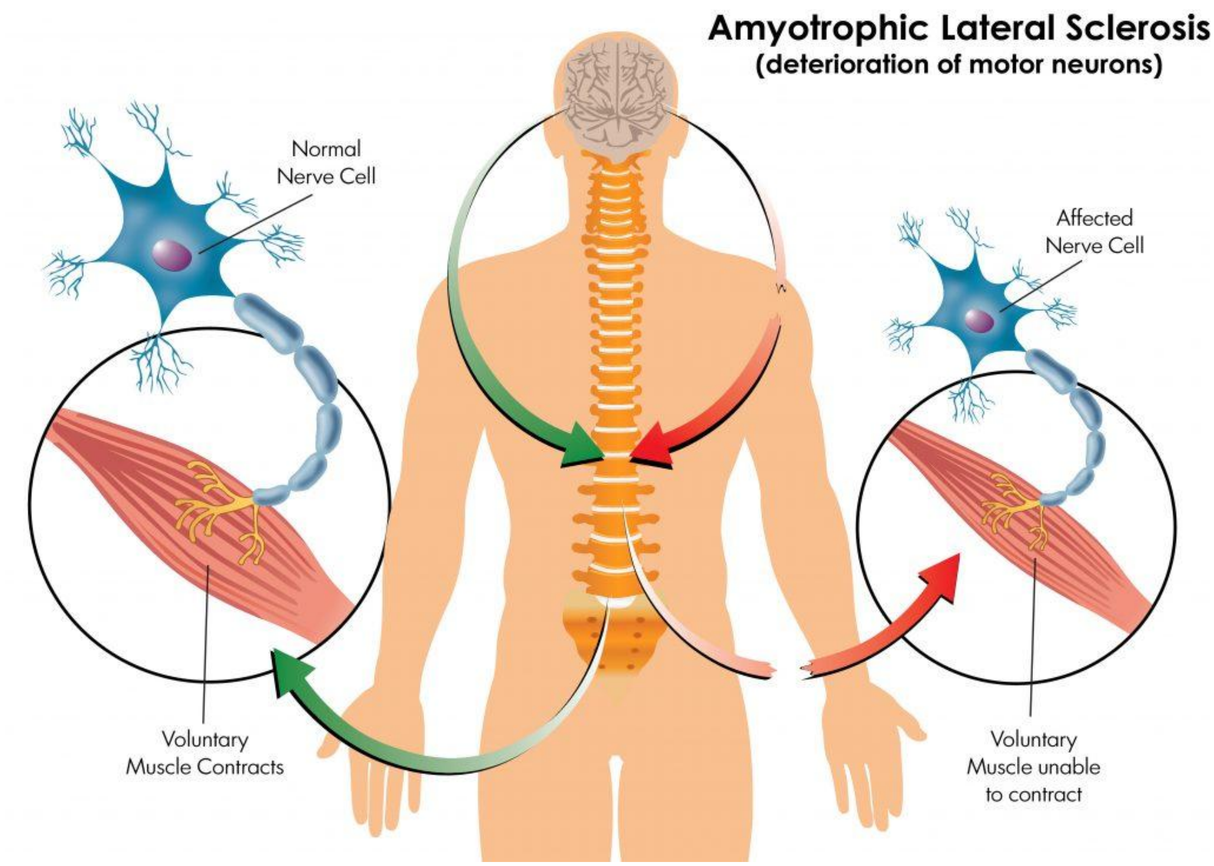
AMD
Age-related macular degeneration (AMD) is a common eye disease that can blur the sharp, central vision that you need for activities like reading and driving.

Cure:
Scientists at Hiorca are using stem cell research to better understand the cells in the eye and how they become diseased, to make cells that could replace damaged cells in the eye, and to identify new drugs that could treat AMD.

To understand the cells in the eye and how they become diseased
Scientists at Hiorca study stem cells in the lab to help them understand how the different cell types in the retina develop and function together, which has led to more sophisticated ways to model normal eye development in the lab and to understand how healthy eye tissue becomes diseased.

ALS

Amyotrophic lateral sclerosis (ALS) is a progressive nervous system disease where the nerve cells in the brain and spinal cord that control the muscles die, causing an increasing loss of muscle control and ultimately death due to loss of control of muscles required to breathe.



Cure using stem cells

he cells affected by ALS are located in the brain and spinal cord and are difficult to access and examine closely in people living with the disease. Scientists have used stem cells to develop ways to recreate, or “model” ALS in the Hiorca lab.

In particular, induced pluripotent stem cells, or iPS cells, are being used in many laboratories studying ALS. Cells from the skin or blood can be reprogrammed or “induced” to an embryonic-like state and can then be coaxed to become any cell type in the body, including motor neurons and other cell types found in the brain and spinal cord. Scientists have made iPS cells from many different patients with ALS as well as from healthy individuals. These iPS cells grow indefinitely in the lab and can be used to generate large numbers of motor neurons with the same genetic makeup as the individual they were made from.

Scientists are using stem cells in Hiorca lab to:

Understand ALS and its causes: Scientists are studying what is happening inside and around the motor neurons that causes them to deteriorate and die in ALS and how this damage might be prevented or halted. They are studying why particular gene mutations cause ALS and what is taking place in patients with sporadic ALS, where there is not a clear genetic cause. Scientists are also examining how different cells of the nervous system influence each other as well as how they connect with muscle cells.

Improve diagnostic tools: Researchers at Hiorca have derived iPS cells from many different ALS patients, some with known genetic causes, others where there is no clear cause. Scientists are searching for what is common and what is unique about different patients. This information may help find ways to diagnose ALS more quickly and to better predict how an individual's disease may progress or respond to particular treatments.

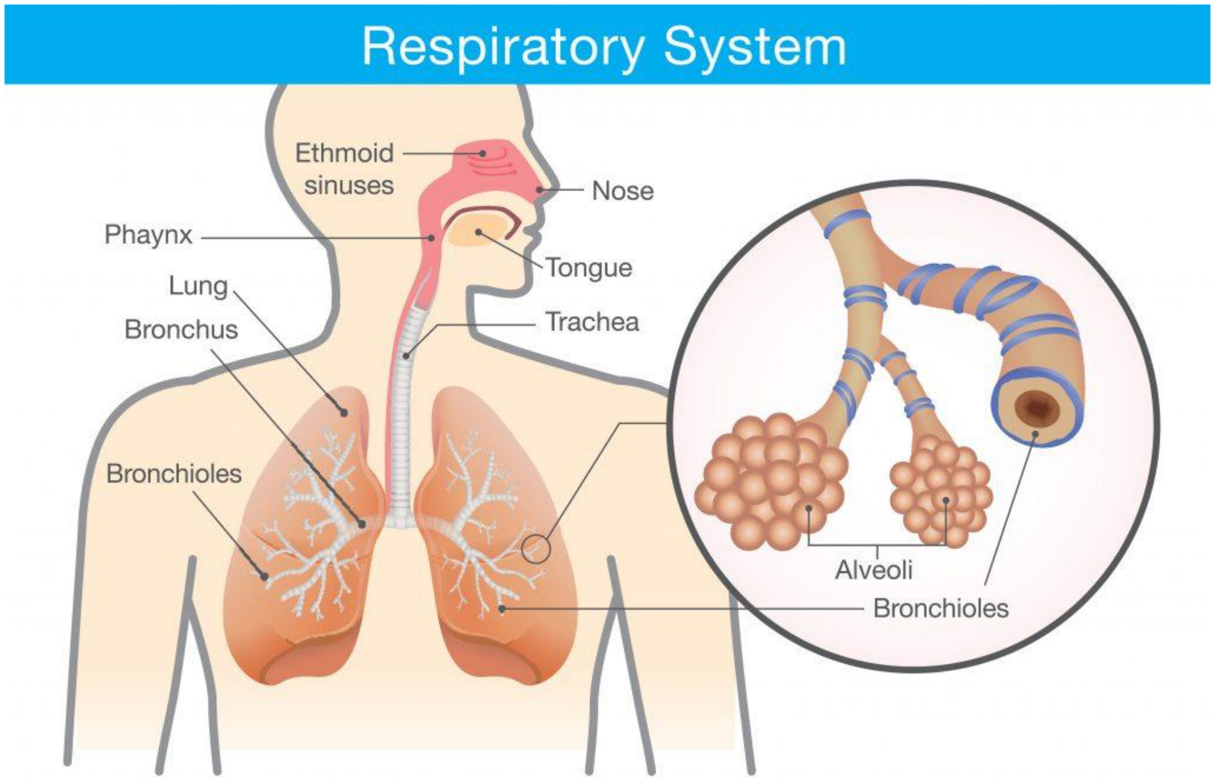
Identify and test new drugs: Motor neurons can be generated from iPS cells in large numbers, which allows scientists to screen for new drugs to treat ALS. They can test whether a drug slows down the deterioration of the motor neuron cells and compare this effect between patients with different underlying causes of ALS.

Develop cell-based approaches for the treatment of ALS: With the great progress in being able to generate neurons in the lab, scientists and doctors have explored how to generate healthy neurons to replace those that deteriorate in ALS. But there are major hurdles to this approach. The motor neurons would need to be delivered to the right places in the brain and along the spinal cord, and once there, they would need to connect to the existing network of neurons as well as signal to the muscles.

Given these challenges, the cell-based approaches being tested at the moment aim to slow down the progression of ALS by providing nourishment and protection to the existing motor neurons or by reducing inflammation in the brain and spinal cord tissue.

COPD

Chronic Obstructive Pulmonary Disease (COPD) is a chronic, progressive respiratory disease that blocks airflow making it difficult to breathe.



Cure using stem cells

The lung has a complex three-dimensional structure, which is critical for its function. It is made up of many different specialized cell types that each perform their own job but work together to carry out the main lung function—to bring oxygen into the body and send carbon dioxide out. It is critical that oxygen is delivered to our cells so that they can produce the energy that is necessary for them to properly function.

JUST SOME OF THE 40+ CELL TYPES IN THE LUNG

In the bronchial tubes, there are cells that make and secrete mucus, and there are other cells with tiny hair-like structures ("cilia") on the surface that sweep the mucus, along with trapped dust, viral particles, or bacteria, back up and out of the lungs. In the air sacs, there are cells involved in oxygen exchange, interspersed with cells that make lung surfactant that prevents the alveoli from collapsing when we breathe out. In addition to these cells that make up the lining of the airways and air sacs, the lung contains blood vessel, muscle, and immune cells.

Researchers are studying how our lungs first develop, how they are maintained and repaired throughout our life, and what goes wrong during the initiation and progression of lung diseases such as COPD.

Three-dimensional (3D) organ-like structures grown in the lab called "organoids" that mimic essential aspects of lung airway function are a particularly valuable tool for these studies, allowing researchers to study human lung development and adult lung function in greater depth. Lung organoids can be grown from several different cell sources:

Adult lung stem or progenitor cells from small pieces of lung tissue (e.g. from biopsies from patients)
Embryonic stem (ES) cells, cells derived from embryos that can give rise to all cells in the body
Induced pluripotent stem (iPS) cells, adult tissue-specific cells that are reprogrammed in the lab to behave like embryonic stem cells. These cells have the capacity to become any cell type in the body.
Researchers are exploring how different specialized lung cells develop and how they talk to each other. Lung organoids are also being used to find new drugs to treat lung disease.

As organoids grown from biopsy and iPS cells are genetically identical to the patient or healthy donor, they can help researchers understand how genetic differences lead to variations in people's susceptibility to disease or how they respond to different drugs.

Further, stem cell research is providing insight into how the lung normally responds to maintenance needs or injury. Researchers have identified cells in the lining of the bronchial tubes and air sacs that can serve as "localized stem cells," that is, these cells can self-renew (make copies of themselves) and generate the specialized lung cell types found nearby. This work suggests that there are several different types of lung stem or progenitor cells in adults that are important for repairing different types of damage.

These findings have important implications for developing new stem cell and regenerative medicine approaches for lung diseases like COPD, that aim to halt or reverse the damage to the lungs.

What is the potential for stem cells to treat COPD?

Researchers are exploring the following stem cell and regenerative medicine approaches for the treatment of lung diseases such as COPD:

Harnessing native repair mechanisms. Researchers are looking for ways to take advantage of the lung's existing repair mechanisms. They are studying the signals that switch on lung repair pathways in a normal lung with the ultimate goal to find new treatments that could activate this process in patients with lung diseases. Given the complexity of the lung and its repair mechanisms, and the complexity of diseases like COPD, there is still a lot to learn.

Cell-based therapies replacing lung cells lost and damaged in disease. In COPD, the cells that line the bronchial tubes and the thousands of tiny air sacs ("alveoli") in the lungs are damaged and do not function properly. The idea of a cell-based therapy would be to generate replacement cells in culture and deliver these to the lungs.

Researchers are rapidly improving ways to generate these cells. It will also be important to deliver cells to the lung in a way that will allow them to integrate into the lining of the lung airways and air sacs to carry out their job.

Addressing chronic inflammation. Airway inflammation is a consistent feature of COPD and is linked to the disease getting worse. Researchers are working to better understand how inflammation is triggered, why it persists, and what can be done to break the cycle.

Some clinical trials have used Mesenchymal Stromal Cell-based therapies (MSCs; sometimes referred to as Mesenchymal Stem Cells) to try to reduce inflammation in lung diseases. As noted below, it is not clear yet whether this approach will be effective.

Building replacement lung tissue. There have been exciting advances in bioengineering lung tissue—that is, using various engineering and/or cell biology methods to grow three-dimensional lungs in the lab—with the eventual hope to make tissue robust enough for transplantation.

Engineers and scientists at Hiorca are using scaffolds to recreate the lung's shape and adding lung cells that can grow on this. They continue to address the challenges in fully recreating the complex structure of the lung with its with its many different cell types and connections to other tissues, that all play a role in effective lung function.

Diabetes
Nearly 400 million people worldwide are living with diabetes, and that number is expected to jump to almost 600 million by 2035, according to the International Diabetes Federation. For many people, diabetes can be controlled with diet, exercise and, often, insulin or other drugs. However, complications from diabetes can be serious and include kidney failure, nerve damage, vision loss, heart disease and a host of other health issues.

Cure using stem cells
Stem cells are being used for ongoing research to help us explore the intricate ways in which our bodies process sugar and answer some important questions about the root causes of diabetes, such as:

In type 1 diabetes, why does the immune system begin to attack beta cells and not other cells in the pancreas or in other organs or tissues?
In type 2 diabetes, what causes the resistance to insulin?
Recently, there has been great progress in generating beta cells from embryonic stem cells (ESCs) and induced pluripotent stem (iPS) cells. Laboratory studies help us better understand disease progression, what the potential genetic causes may be, and similarities and differences between patients. Researchers are using this information to try to diagnose people earlier, prevent disease progression, and more effectively treat diabetes.

iPS-derived beta cells could also be used for beta cell replacement therapy, providing what could potentially be a cure if they can be successfully transplanted into patients in our lab. As iPS cells could be made from the individual patient, the resulting beta cells would avoid transplant rejection, but recipients with type 1 diabetes would still face autoimmune attacks on their beta cells.

Heart disease

Acute myocardial infarction, or a heart attack, occurs when the blood vessels that feed the heart are blocked, often by a blood clot that forms on top of a plaque, a build-up composed of fat, cholesterol, calcium, and other elements found in the blood. Without oxygen and other nutrients from the blood, heart cells die and large swaths of heart tissue are damaged.

After a heart attack, scar tissue often replaces the damaged part of the heart muscle, and this scar tissue impairs the heart's ability to keep beating normally and pumping blood efficiently. The heart ends up working harder, which weakens the remaining healthy sections of the heart and over time, the patient experiences more heart-related health issues.

Cure using stem cells

Researchers are using stem cells in two important ways to improve cardiac health. First, they are turning stem cells into "heart muscle in a dish." If patients have genetic causes of heart disease, their stem cell-derived heart muscle also will have this disease and this heart muscle can be used to discover new drugs. Second, stem cells offer ways to replace damaged heart tissue. Using cellular therapy, researchers hope to repair or replace heart tissue damaged by congestive heart failure and heart attacks. Unlike the treatments listed above, cellular therapy could provide a durable treatment for heart deficiencies, rather than symptom-focused treatment.
Your heart is constructed of several types of cells. For mending damaged heart tissue, researchers generally focus on three specific heart cell types:

Cardiomyocytes, the beating muscle cells that make up the atria, the chambers where blood enters the heart, and the ventricles, where blood is pumped out of your heart. Cardiomyocytes are currently being targeted in cellular therapies for heart disease.
Cardiac pacemaker cells, which send and receive electrical signals to keep your heart beating in rhythm.
Endothelial cells, which line blood vessels and help deliver oxygen to the cardiomyocytes.

Researchers at Hiorca are experimenting with a variety of stem cell therapies to transplant new cells, use tissue engineering to improve the survival or function of transplanted cells, and stimulate existing cells to generate new cardiomyocytes.

Researchers can grow cardiomyocytes in the Hiorca lab from the following sources:

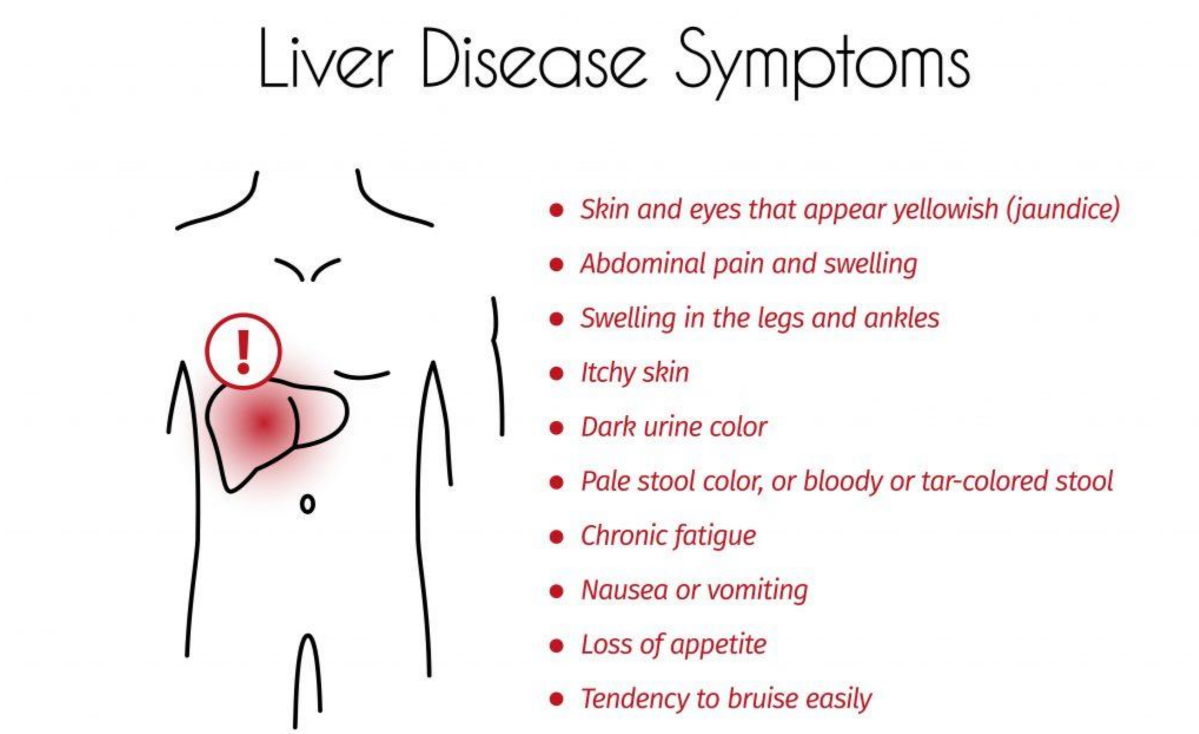
Embryonic stem (ES) cells, cells derived from embryos that can give rise to all cells in the body.

Induced pluripotent stem (iPS) cells, adult tissue-specific cells that are reprogrammed in the lab to behave like embryonic stem cells and which have the capacity to become any cell type in the body, including cardiomyocytes.

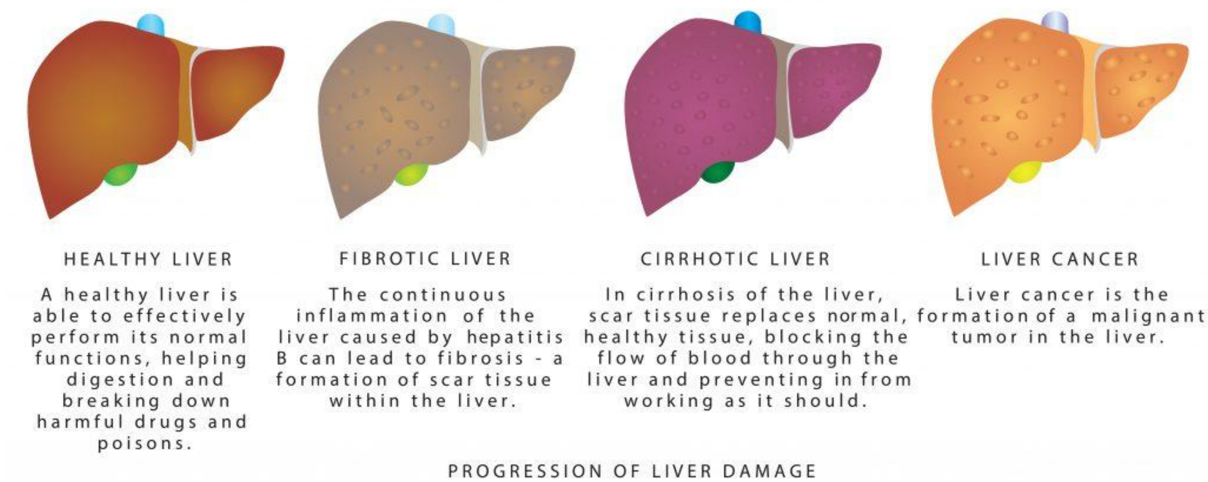
Cardiac cells made from iPS cells are also incredibly useful for creating human models of heart disease to better understand exactly what goes wrong and for testing different drugs or other treatments. They can also be used to help predict which patients might have toxic cardiac side effects from drugs for other diseases such as cancer.

Liver disease

Liver diseases can have multiple causes and symptoms (see Figure 1). Liver diseases can be inherited (have genetic causes) or can be caused by external factors. While there are multiple types of liver disease, liver damage often progresses in similar ways.



Cure using stem cells

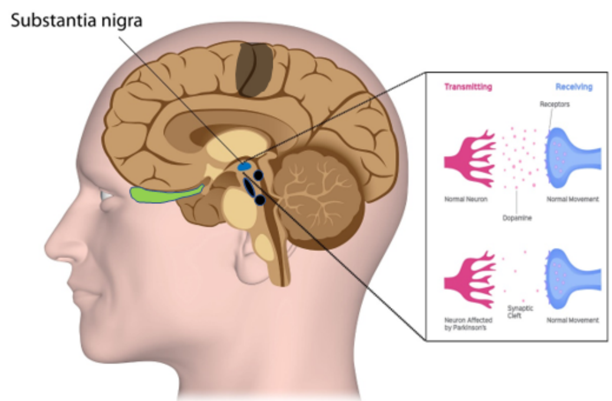


Liver Regeneration

The liver is our only internal organ that can regenerate after damage. This regeneration is partially due to the major cell type in the liver, the hepatocyte. Hepatocytes are critical for synthesizing proteins and lipids, metabolizing carbohydrates, and detoxifying functions of the liver. Hepatocytes can normally divide to replace cells that die and they can even repair some damage to the liver. Another cell type known as hepatic biliary cells, which drain bile, can also orchestrate liver repair. Liver disease that causes damage to hepatocytes or biliary cells reduces the liver's ability to recover. In cases of severe injury, specialized cells known as hepatic progenitor cells are thought to take over regeneration. The formation of scar tissue can limit the ability of the liver to repair itself. Studies are ongoing to determine whether there are stem cells in the adult liver, and if there are, how they function and replenish the liver.

Parkinson's disease

Parkinson's disease (PD) is a progressive neurodegenerative disorder that affects more than 10 million people worldwide. The symptoms of the disease worsen over time, and include shaking, difficulty walking, rigidity, and other debilitating motor and non-motor problems. PD is a particularly active area of stem cell research around the world and scientists are using stem cells to better understand the disease and to find potential new therapies.



Cure using stem cells

Modeling PD in the Hiorca lab

Neurons are difficult to study within the brains of living people, and they cannot be extracted and grown in the lab for research purposes. Based on a Nobel prize-winning scientific breakthrough from 2006, scientists have another way to study neurons from patients. Cells from the skin or blood can now be reprogrammed to an embryonic-like state, called induced pluripotent stem (iPS) cells, which are then capable of becoming any cell type in the body, including neurons of the brain. For example, blood cells taken from a patient with PD can be reprogrammed into iPS cells in a lab. Those cells can in turn be made into a virtually unlimited supply of dopamine-producing and other types of neurons that can be closely studied by scientists.

Since these cells have the same genetic makeup as the patient, they can exhibit the same disease-processes as the cells in the patient. By comparing the development of iPS cell-derived neurons from PD patients to those from healthy individuals, researchers are studying what goes wrong in PD neurons, and what genes may be involved in the development of the disease. Scientists can use these neurons to further understand disease progression as well as discover new drugs that might be effective at delaying or reversing the disease.
Stem cells are therefore allowing researchers to model and study neurons from PD patients in the lab to identify the genetic and environmental causes of PD with a goal of better understanding how to prevent or treat the disease. However, while exciting, this modelling is still in its early stages and is not yet able to accurately replicate all aspects of the disease.

Stem cells as a source of healthy neurons to treat PD

There is strong evidence to suggest that replacing the dopamine-producing neurons that die in the midbrain could significantly help PD patients. Implanting newly generated dopamine-releasing cells in the brain of patients with PD at the site where dopamine normally signals may give a clinical response that is equivalent to that seen with dopamine drugs, with the advantage that the grafted neurons would release dopamine in a physiological way at the site where it is needed, and by so doing should avoid the side effects seen with the drugs.

Beginning in the 1980s, attempts have been made to repair the PD brain using dopamine-producing cells, the most successful attempts being transplants of dopamine cells from fetal tissue. These cells, when grafted into patients with PD, can survive long-term in large numbers, release dopamine, integrate and function in the host brain, and significantly improve PD for years. This approach, while demonstrating proof-of-principle, however, was found to have technical, logistical, and quality control issues, mostly due to the fetal cell sources: inconsistent or poorly-defined cells, undesirable side effects in some trials, and policy issues in the U.S. around the use of fetal cells. Therefore, a more standardized source of dopamine cells is needed.

Over the last 10 years, technologies and protocols have evolved to the point that a virtually limitless supply of midbrain dopamine neurons can now be made from both embryonic stem and iPS cell sources which show good survival in animal models of PD and demonstrate functional benefits. This work has now progressed to the point that first in-human clinical trials are beginning to take place.