

Just the Facts:
The Science
and Potential
of Cell Donation
and Banking

Transforming Scientific Research



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Cell and Tissue Donation: Transforming Scientific Research

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To understand human disease, researchers must study human cells. While studying cells in the human body presents practical and ethical limitations, donated cells can be studied more easily in a controlled laboratory environment.

The way diseases and their treatments affect individuals varies, in part due to diversity in sex, race, and/or other genetic factors. For this reason, it is important to study cells donated by people from many different backgrounds to help identify why certain diseases and their treatments impact people and populations differently.

In this section, we provide information and address considerations regarding three types of donation for research purposes:

- Donating Cells for Induced Pluripotent Stem (iPS) Cell Research (page 2)
- Donating Cells for Embryonic Stem (ES) Cell Research ([page 3](#))
- Donating Tissue Samples and Biopsies ([page 4](#))

DONATING CELLS FOR INDUCED PLURIPOTENT STEM (iPS) CELL RESEARCH

What are iPS cells and why is donating cells for iPS cell research important?

Donated cells, such as those found in blood samples or skin biopsies, can be engineered by scientists in the lab to become a type of stem cell, termed “[induced pluripotent stem \(iPS\) cells](#).” Similar to [Embryonic Stem \(ES\) cells](#), iPS cells can then become any cell type in the human body. These stem cells can be converted in the lab to many different cell

types, such as neurons found in your brain or spinal cord, liver cells, or kidney cells. Studying how stem cells work and the different cell types they can become helps scientists learn more about cell and tissue development. Making iPS cells from donors who are healthy, suffering from disease, and who have known genetic mutations, is important to improve our understanding of disease and are critical tools that are helping scientists develop and test new drugs and therapies.

What are the different ways in which my iPS cells could be used?

iPS cells are used in numerous ways to help unlock the mysteries of human biology and advance medicine and biotechnology:

- **Identifying new drugs to treat diseases.** Two obstacles to discovering new disease-treating drugs include: (1) limited numbers of appropriate cell types for testing thousands of different potential treatments, and (2) making sure candidate drugs are not toxic to healthy human cells. To help address both of these concerns, scientists are using iPS cells in the lab to create a virtually unlimited supply of cells upon which thousands of drugs can be tested simultaneously, as well as cells that can be used to test whether or not new candidate drugs will be toxic to other cell types.
- **Personalizing medicine.** Since every person is unique, their iPS cells have unique genetic variations and mutations as well. This means that multiple drugs and treatment combinations can be tested in a lab on each patient’s unique cells to determine the optimized treatment.
- **Discovering how organs form and function.** By studying how iPS cells become mature cells, like liver or lung cells, researchers can better understand how cells form normally during embryo and fetal development, and what can go wrong. Further, when iPS cells are grown and developed into clumps of cells in 3-dimensions, called [organoids](#), they can be used to model human organs to study how they form and function.

- **Making new replacement cells for damaged tissues and organs.** iPS cells can be used to make many different cell types in the lab. These newly produced healthy cells potentially could be transplanted into patients to replace cells lost to injury or disease. For example, it could be possible one day to replace insulin-producing cells of the pancreas in patients with diabetes, or dopamine-producing neurons in patients with Parkinson’s disease, with healthy ones made from iPS cells.

Currently, clinical applications of iPS cell technologies are experimental, meaning there is no treatment using iPS cells that has completed all necessary clinical trials or gained regulatory approval. Research continues to evolve and, one day, may yield effective new methods and therapies to treat patients.

What are important questions to ask before donating cells for iPS cell research or medicine?

All cell or tissue donations should begin with a complete understanding of “informed consent.” This means that donors receive information on the process, procedure, and uses of the donated cells or tissue. Donors also should be given the opportunity to ask questions, so that they can make informed decisions about the use of their cells (see [panel 4](#) of “Process of Donating Cells for iPS Cell Research”). These forms can vary substantially from country to country, and even between hospitals, universities, and health care facilities, but they all share important issues you should clarify before you donate cells, including:

- Who will have access to the cells I donate or the resulting iPS cells?
- How will the cells I donate or the resulting iPS cells be used?
- How will the privacy of my personal information be handled?
- If my cells are sequenced to determine my genetic code, who will have access to my genetic information? Will this information be shared publicly?
- Will information about my cells’ genetic

information or responses to drugs be disclosed to me, as they are revealed through research?

- Will I have access to a genetic counselor or ethics expert I can talk to?
- Will I have the option to change my mind at any time?

A more detailed list of questions can be found in the [ISSCR Patient Handbook](#).

It is important to read through the informed consent information carefully and ask your doctor or medical professional to clarify any questions before signing any forms.

What is the procedure for donating cells, and how will they be turned into iPS cells?

Samples of cells are collected in several ways, most commonly through a blood sample or skin biopsy. These cells then are processed in a lab and reprogrammed into iPS cells. This commonly is done by adding or “turning on” certain genes, or by growing them in specific conditions in a lab (see [panel 2](#) of “Process of Donating Cells for iPS Cell Research”).

Both the donated cells and the transformed iPS cells may be stored indefinitely for future use, as described in your informed consent document.

DONATING CELLS FOR EMBRYONIC STEM CELL RESEARCH

Where do Embryonic Stem (ES) cells come from?

Most human ES cell are derived from donated three-to-five day-old fertilized embryos. The inner mass of cells of the embryo is removed and grown under special conditions in a laboratory that allows the cells to be grown indefinitely.

For individuals undergoing in vitro fertilization (IVF), the process often involves the production of more embryos than can be implanted. These additional embryos either can be stored for future fertility treatments, donated for embryo adoption, discarded, or donated for scientific research, including embryonic stem (ES) cell research. To learn more about these options, talk with your doctor or medical professional.

If iPS cells and ES cells are similar, why is there still a need for ES cell research?

The continued use of ES cells in research labs around the world is vital to understanding early development, disease, and discovering new therapies. Additionally, there are some specific distinctions between ES and iPS cells including the following:

- **ES cells are not identical to iPS cells.** While iPS cells share many of the same properties and characteristics as ES cells, researchers continue to study the similarities and differences between them in order to advance scientific discovery.
- **iPS cells have been reprogrammed (they began as adult cells).** Unlike ES cells, which have never developed into specialized cell types (like blood or skin) and consequently have maintained their “blank slate,” iPS cells have undergone biological aging. iPS cells may therefore retain genetic memory of their age and tissue of origin as well as any mutations accumulated over time.
- **ES cells are the “gold standard” for pluripotent stem cell research.** ES cells were initially isolated from embryos and therefore remain essential to compare and contrast with iPS cells, setting the bar for determining what a completely reprogrammed cell looks like for future research and clinical purposes.

If some embryonic stem cells already exist, why is there a need for more?

It is valuable to study ES cells with different genetic backgrounds in order to investigate variations in disease and to test how genetic differences impact the effectiveness of potential treatments.

Most importantly, many new discoveries come from unexpected places. By studying cells that come from genetically different embryos, scientists may uncover unanticipated breakthroughs. ES cells that come from embryos of diverse genetic backgrounds often reveal important insights into the roles that different gene combinations play in development and disease.

DONATING TISSUES AND BIOPSIES FOR MEDICAL RESEARCH

How do biopsies help with research?

A biopsy is a sample of tissue or cells that has been removed from the body, primarily to diagnose or treat a disease. Occasionally, samples can be donated, with proper informed consent, for further scientific research. By studying donated healthy and diseased tissue samples, researchers learn different aspects of disease across the human population. These studies also allow researchers to address important questions about normal cell behavior and how cells transform and malfunction.

When conducting cancer research, scientists study biopsy cells to understand how cancer cells grow, spread, and change into different cell types. By studying these cells in the lab, researchers learn new ways to target and fight these cancerous cells. Tissue samples can also be used to make [organoids](#), clumps of cells that model human organs, to better understand the biology of the tumor and to screen for new cancer drugs.

Biopsies from a variety of organs and tissues affected by a variety of diseases offer important insights into disease mechanisms and help identify potential future treatments.

What should be included in the informed consent form for donating my biopsy sample(s)?

Similar to donating cells for iPS cell research, discussions surrounding donating tissue samples from biopsies should begin with informed consent. Review these sample questions and discuss them with your doctor before signing informed consent forms. Biopsy-specific donation questions to consider include:

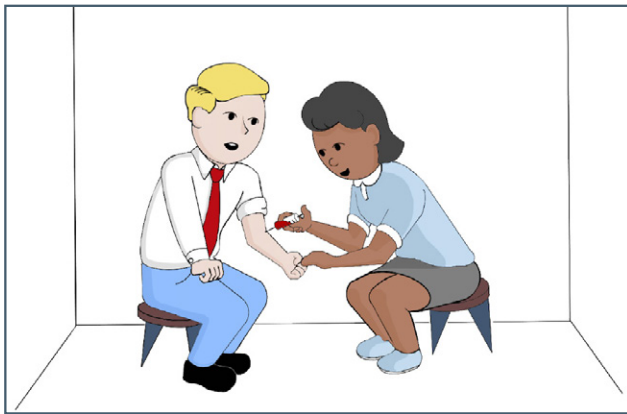
- Will my clinical lab results be shared with the researchers? If so, how will my name and privacy information be handled?
- Will the information about my sample’s genetic information or responses to drugs be shared with me or my medical team to improve my treatment options in the future?

CONCLUSION

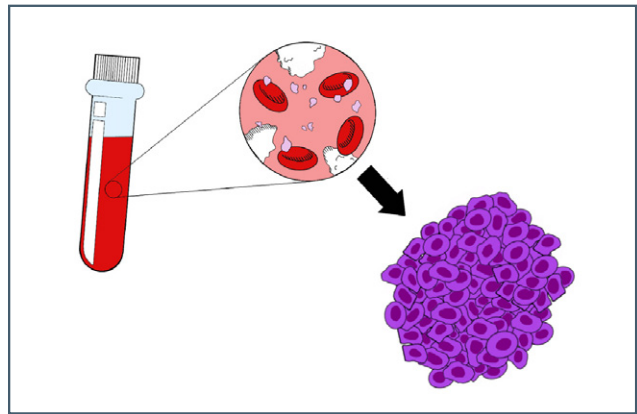
Cell, tissue, and embryo donation are a personal choice. When deciding whether or not to donate, it is essential that proper ethical standards involving responsible informed consent procedures are followed. Visit closerlookatstemcells.org for more information about cell donation and banking, stem cell basics, and disease specific resources.

THE PROCESS OF DONATING CELLS FOR iPS CELL RESEARCH

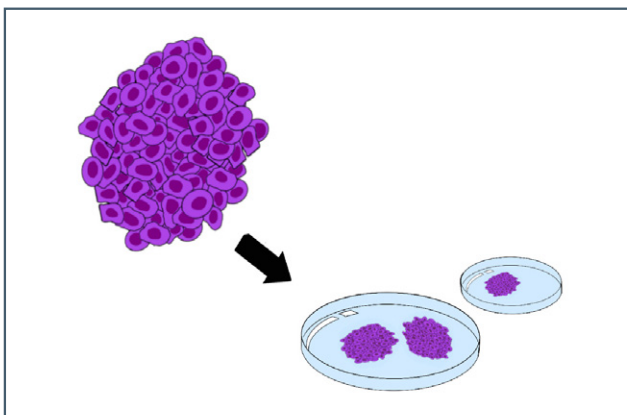
Figure legend: This series of illustrations is an overview of how blood cells are donated for the generation of induced pluripotent (iPS) cells. The figure shows how these stem cells are used in academic and clinical research to better understand human disease and potentially identify new therapies.



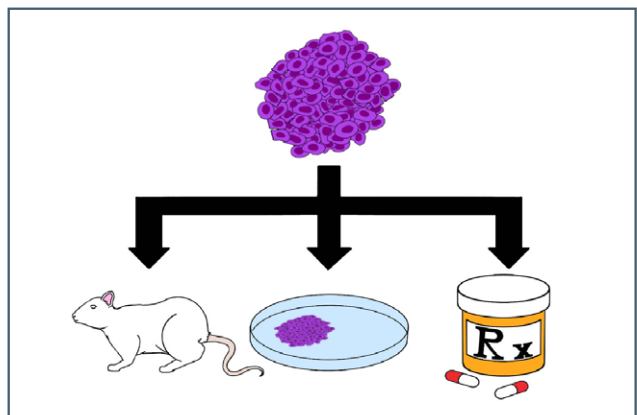
1. You will be asked for a blood sample



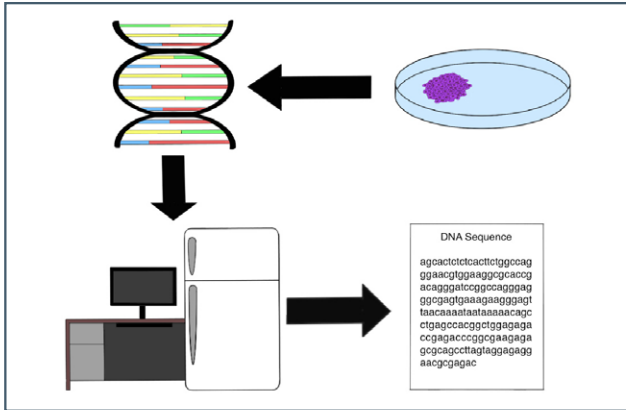
2. The blood cells in your sample will be turned into induced pluripotent stem cells in the lab. These stem cells can turn into any type of cell in the body. Your stem cells and any cells that they turn into will have your DNA.



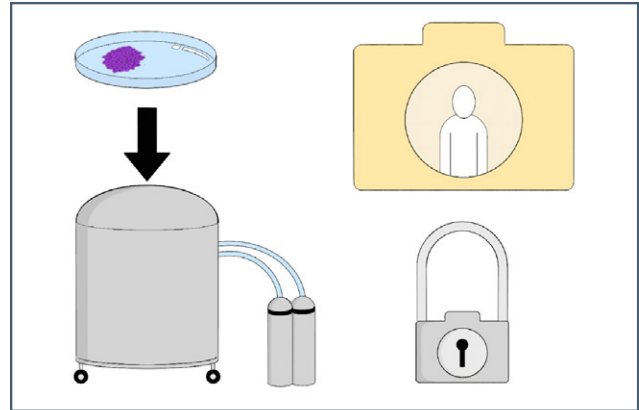
3. Stem cells can keep growing in a dish so that researchers can keep using them for a long time. Your stem cells may be preserved for as long as researchers need to use them or as described in the informed consent document.



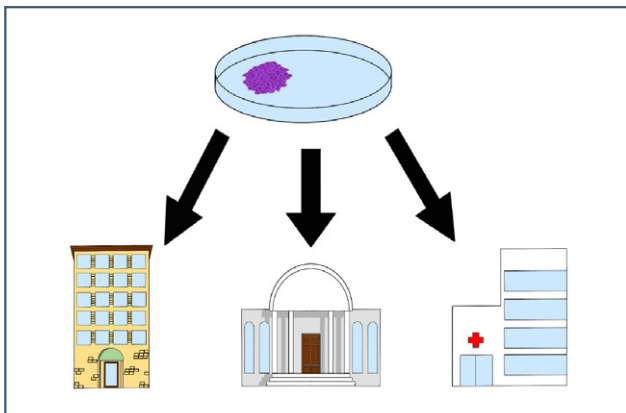
4. Your stem cells may be used in many different ways, such as modeling human diseases in laboratory animals or in a dish, as well as developing new drugs or treatments.



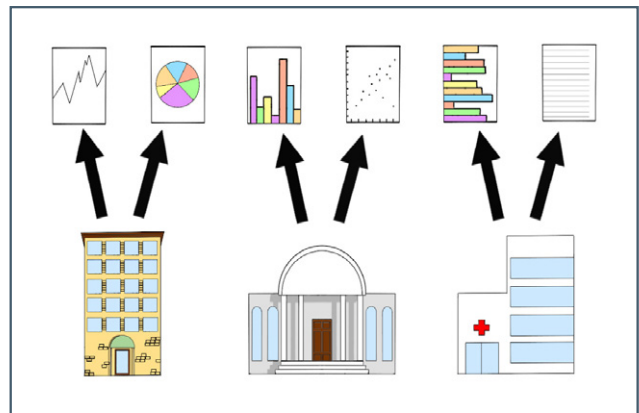
5. The usefulness of stem cells can be improved if scientists sequence their whole genome, or all of the DNA inside the cells. Whole genome sequencing allows scientists to read your entire genetic code to compare it with other people's genomes.



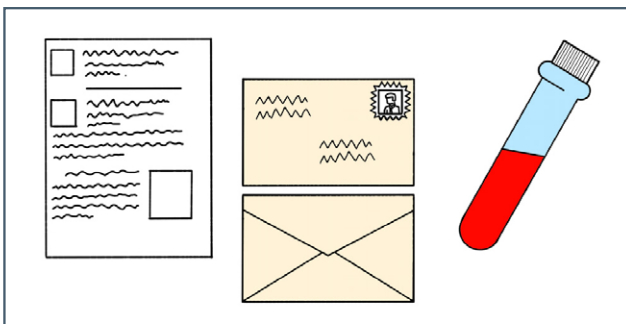
6. Your stem cells may be kept in storage for future use and research. Your name and personal information may be kept separately from the stem cells in a password-protected database that only authorized people will be able to access.



7. Your stem cells may be shared with researchers at other academic institutions or private companies. Depending on the signed informed consent agreement, you may be able to ask for your stem cells in the original storage site and/or other sites to be destroyed.



8. By sharing your stem cells with other researchers, more data might be collected that could reveal new information about your health or new insights into diseases.



9. With your permission, researchers may recontact you for additional samples or for updates on your health.

Panels created by Dylan Hyun, graphics by Matthew Yoder