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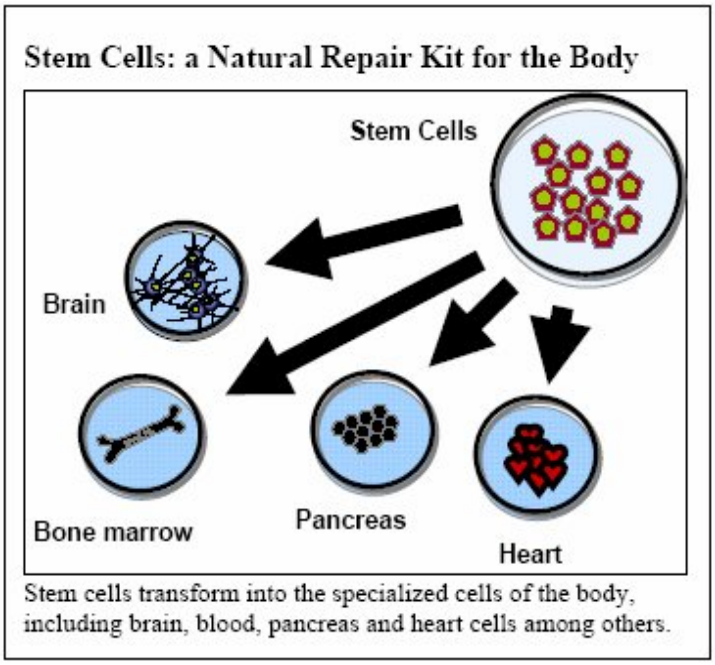
SUE AND BILL GROSS STEM CELL RESEARCH CENTER

STEM CELL BASICS

Stem cells are unspecialized cells that can generate healthy new cells and tissues, lead to improved understanding of disease and potentially foster the development of new, more effective drugs. Importantly, the mainstream medical community agrees that all aspects of stem cell research should be explored - within strict ethical limits - to develop new treatments and cures for devastating diseases and injuries. The Sue and Bill Gross Center for Stem Cell Research at UC Irvine developed this fact sheet to help explain the basics of stem cell research to our neighbors in the Orange County community.

What is a stem cell?

Stem cells are the "master" cells that give rise to each of the specialized cells within our bodies. During organ and tissue development, they transform into a particular specialized cell when prompted by their environment or by genetic programs resident within them. Stem cells also renew themselves indefinitely while maintaining their transformative potential.



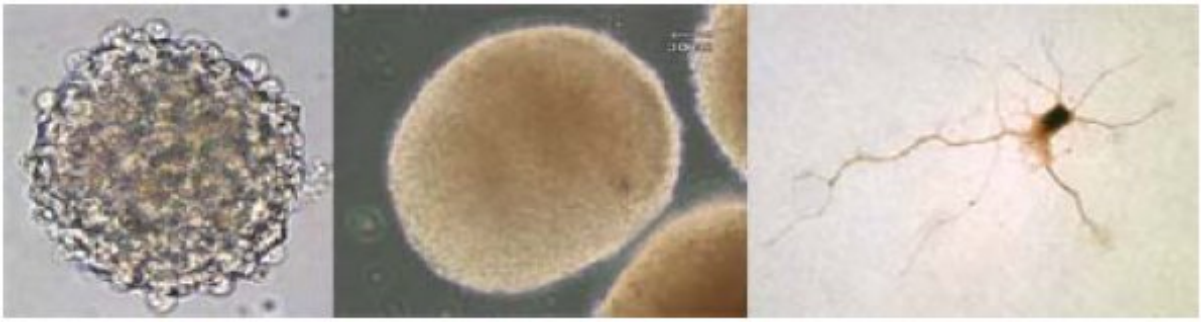
Stem cells transform into the specialized cells of the body, including brain, blood, pancreas and heart cells among others.

In general, scientists categorize stem cells by their tissue of origin. *Adult stem cells* are those found within specific body tissues and typically generate only the cells of that tissue. For example, brain stem cells can only become the specialized cells of the brain. Umbilical cord blood stem cells are a kind of blood-forming adult stem cell. *Embryonic stem cells*, which are found in an early-stage embryo called a *blastocyst* (pronounced *blast-o-sist*), can transform into virtually any type of cell found in the body.

Researchers are now learning to direct the process by which stem cells change into specialized cells. In theory, these methods could be used to repair damaged or diseased tissue by stimulating the regenerative capacity of adult stem cells, or by transplanting specialized cells prepared from embryonic stem cells. Researchers at UCI recently developed a first-of-its-kind way to change human embryonic stem cells into cells (called *oligodendrocytes*) that nourish and protect nerve fibers, possibly leading to new treatments for some neurodegenerative disease

Why are medical researchers interested in stem cell biology?

Stem cell research has often been called "the new frontier of medicine" because of its potential to advance our understanding of the biological processes that keep our bodies healthy and whole. For



Photos depicting transformation of human embryonic stem cells into cells called oligodendrocytes that insulate nerve fibers. *Left*: Cluster of human embryonic stem cells. *Middle*: Cluster of partially specialized cells committed to becoming brain cells. *Right*: a single oligodendrocyte. (Courtesy: Reeve-Irvine Research Center.)

example, investigations of adult stem cell function will illustrate how our tissues repair themselves (or don't) after injury. Research using embryonic stem cells can shed light on the molecular mechanisms governing tissue development, and aid the design of safer, more effective drugs. Stem cells of either type might even be used - in different ways - to repair or replace tissues damaged by disease.

In the years ahead, knowledge gained through stem cell research is expected to improve treatment options for a number of currently incurable diseases and injuries, including spinal cord injury, stroke, heart disease, cancer, diabetes, Parkinson's, multiple sclerosis, severe burns, ALS, Hungtington's Disease, lupus, sickle cell disease, HIV/AIDS, osteoarthritis, rheumatoid arthritis and vision and hearing loss. In addition, stem cells might also provide unique experimental tools that will allow neuroscientists to better understand puzzling brain disorders like autism, Alzheimer's and cerebral palsy.

Where do human embryonic stem cells come from?

Human embryonic stem cells derive from 3-5 day old blastocysts that are produced during fertility treatment and donated for research purposes after treatment concludes. In many cases these surplus blastocysts would be stored frozen indefinitely or discarded as medical waste. Donors must give their informed consent prior to donation.

Some labs have suggested alternative ways of deriving human embryonic stem cells, including via a technique called *somatic cell nuclear transfer* (SCNT or *therapeutic cloning*). SCNT may one day allow researchers to generate replacement cells that escape the problem of immune rejection when transplanted into patients.

Strict Ethical Safeguards Govern Embryonic Stem Cell Work at UCI

UCI abides by all Federal and California statutes governing the donation of biological materials for research (including embryos) in order to protect the health, safety and privacy of the donors. Chief among these are standards requiring that donors give their informed consent, and that these donations are free of coercion or financial incentive. In addition, UCI has established an Embryonic Stem Cell Research Oversight Committee to monitor adherence to these safeguards, as well as to ensure that research involving embryonic stem cells serves important research aims and is conducted according to the highest ethical standards.

Why is research using embryonic stem cells so important? Aren't adult stem cells sufficient?

Different stem cell types harbor different characteristics, and scientists do not know which set of stem cell characteristics will ultimately be needed to cure or treat disease. More importantly, knowledge gained about embryonic stem cells will complement studies of adult stem cells, and vice versa. That's why most scientists believe that they can best work toward therapy advances by responsibly investigating all aspects of stem cell biology.