

# How are stem cells used?

## Stem Cell & Regenerative Medicine Center University of Wisconsin-Madison



### **W**hy are stem cells so valuable in research?

One reason stem cells have generated excitement in science is their versatility.

Like little Swiss Army knives, stem cells provide the basic tools researchers can apply to help solve many kinds of problems in biology and medicine. The different types of stem cells — embryonic, induced pluripotent, fetal and adult — can all be used in different ways to inform our understanding of human development and disease, devise novel therapies and screen for new therapeutic compounds.

**How are they used in research?** Human stem cells provide a powerful source of cells for basic experimental studies that can be done in a laboratory dish. These range from studying the particular genes expressed in stem cells to characterizing the specialized cell types that can form from stem cells. Stem cells can provide model systems for understanding the mechanisms of a wide variety of diseases.

Perhaps the most important but often the most overlooked role of human embryonic stem (ES) cells is their ability to shed light on the earliest stages of human development. One key to understanding human development is determining how human ES cells morph into any of the 220 types of cells in the body. Before scientists had access to human ES cells, our best window to understanding early development was through studies of mouse ES cells. While the mouse is a powerful research model, the events of early development in humans and mice are significantly different. Using ES cells, scientists can see the very first events in human development to understand how normal development unfolds and how the process may go awry and lead to pregnancy loss, birth defects and other problems.

**Can stem cells be used to find new medicines?** One powerful application of human stem cells will likely be in the arena of drug discovery and safety. The ability to make pure populations of different kinds of human cells means that many thousands of chemical compounds can be quickly tested for efficacy and

safety by exposing the cells to those chemicals. Many such compounds are now tested on animals. While animal models are critically important, the use of human cells to test new medicines offers the opportunity to better determine drug effects in humans and refine both the safety and beneficial effects of new compounds.

**Are stem cells now used for therapy?** Bone marrow transplantation is a well-established therapy for a variety of blood diseases and involves the transplantation of blood stem cells. However, applications using other sources of adult stem cells to treat a range of different diseases are still largely in preclinical studies or early clinical trials. For example, stem cell transplantation is being tested in clinical trials for treating severe forms of heart disease. Although scientists believe many forms of stem cells have great therapeutic potential, they are still relatively new to science and require years of research to demonstrate safety and efficacy before new cellular therapies are widely available to patients.

Directors: Timothy Kamp, M.D.  
Clive Svendsen, Ph.D.

Administrator: Rupa Shevde, D.M.Sc.

Program: Sue Gilbert  
sggilber@wisc.edu

Outreach: Jordana Lenon  
jlenon@primate.wisc.edu

Stem Cell & Regenerative Medicine Center  
1500 Highland Avenue  
Madison, WI 53705  
phone: (608) 263-2982  
fax: (608) 263-5267  
www.stemcells.wisc.edu