

THE INSTITUTE FOR STEM CELL AND REGENERATIVE MEDICINE (ISCRM) *at the University of Washington*



Located at UW Medicine at South Lake Union, ISCRM brings together researchers from many disciplines for collaboration and discovery.

A Quick Primer: Stem Cells

Because stem cells can develop (differentiate) into other, more specific cells, such as heart cells or neuro-retinal cells, they show great medical potential for replenishing and repairing tissues throughout the body. There are several types, including:

Human embryonic stem cells: derived from fertilized eggs left unused after *in vitro* fertilization. These cells have a great capacity for differentiation.

Adult stem cells: unspecialized cells found in low numbers within many adult tissues. Compared to embryonic cells, their ability to differentiate is more limited.

Induced pluripotent stem cells (iPSCs): adult stem cells that, with the addition of genes or drugs, can be induced to resemble embryonic stem cells and then differentiate into any cell type.

REGENERATIVE MEDICINE is a revolutionary new field made possible by rapid advances in our understanding of the biology of stem cells, which have the capability to form all the tissues of the body. Scientists see the opportunity to use stem cells to treat a variety of conditions, such as heart disease, diabetes, cancer, hearing loss, Alzheimer's disease, Parkinson's disease, rheumatoid arthritis and blindness, as well as spinal cord and traumatic brain injuries.

Seeing the potential of this kind of research, the University of Washington formed the Institute for Stem Cell and Regenerative Medicine (ISCRM) in 2006. What follows is a brief overview of stem cell medicine, a look at some of the institute's projects, and a request for support that will help UW Medicine develop stem cell and gene therapies for patients worldwide.

The Importance of Stem Cells

Stem cells have the remarkable ability to change or differentiate into other types of cells that replenish and repair tissues throughout the body. The first stem cells to be used therapeutically were part of a bone marrow transplant, a procedure first performed in Seattle. These cells were known as hematopoietic, or blood-forming, stem cells.

Can stem cells and regenerative medicine cure diseases like heart failure, diabetes and blindness? Scientists think so. These diseases are caused by cells that are damaged or deficient: heart muscle cells that can no longer pump blood, for example, islet cells that are not producing the proper amount of insulin, or neuroretinal cells that can no longer sense light. As scientists learn how to transform stem cells into other cell types, they are developing therapies to treat such diseases. Research suggests that advances for some of these conditions could be achieved in just a few years.

Creating a Leader in Stem-Cell Research: ISCRM

When the University of Washington created ISCRM, their intention was to break down walls between researchers in different fields in order to make significant advances in stem cell and regenerative medicine. Institutes eliminate the compartmentalized faculty model, instead bringing together the diverse, interdisciplinary expertise needed to make rapid progress in new and complex areas of research.

In addition to fostering collaboration and speeding the development of stem-cell science, ISCRM also takes advantage of UW Medicine's considerable strengths in basic research and its track record of translating research into therapies.

Philanthropy: Powering the Cores

One of ISCRM's strengths is its cores — high-tech equipment, funded by local philanthropists, that speeds the work of ISCRM's researchers and their collaborators in the community.

The Tom and Sue Ellison Stem Cell Core Lab

This state-of-the-art facility is the only academic venue in the region where scientists can receive training in stem-cell work, and it is one of the largest facilities of its kind in the country, surpassing what is available at Harvard, Stanford and other leading institutions.

The Quellos High-throughput Screening Facility

This facility allows researchers to screen for candidate drugs as well as probe the functions of every gene in a cell. It is the only academic facility of its kind in the state and one of only a few on the West Coast.

The Lynn and Mike Garvey Imaging Center

This center provides leading-edge, advanced microscopy equipment to researchers, which allows them to visualize stem cells both in lab cultures and within living animals.

Faculty and funding

ISCRM includes more than 140 faculty members from 34 UW departments and five institutions, many of whom are housed at UW Medicine at South Lake Union. Their work is made possible by the infusion of approximately \$26 million a year, an amount that includes federal research funding, private grants and invaluable philanthropic gifts from individuals and organizations.

Education and outreach

In addition to conducting research, ISCRM's faculty members are dedicated to education and outreach. They teach graduate students about the basic biology of adult and embryonic stem cells and the cells' therapeutic uses. Researchers from all over the world visit ISCRM to deliver public lectures and study at its facilities, encouraging collaboration and cross-pollination of ideas. And while ISCRM's reach is global, it is also rooted in our community. ISCRM researchers give presentations at local schools and host students and teachers in our facilities, giving them hands-on experiences in stem-cell science.

The number of researchers at ISCRM, the internationally recognized impact of their work, the institute's level of funding and its commitment to outreach and education make ISCRM a leader in the field of stem-cell and regenerative medicine.

Groundbreaking Progress

It takes years of careful work to translate discoveries in the lab into therapies that benefit patients. One of the important steps along the way is the clinical trial, where patients participate in testing new drugs. Although ISCRM was founded only a few years ago, some of the institute's research projects are likely to be ready for clinical trials within the next few years.

Promising projects are being led by ISCRM researchers in the following areas:

Cancer. Scientists are developing a number of new therapies that may lead to cures for the deadliest cancers. One therapy focuses on disrupting the survival signals cancer stem cells receive from surrounding non-cancerous cells that allow them to resist treatments like chemotherapy and radiation. Another therapy focuses on screening for drugs that may be useful in suppressing the growth of glioblastomas, a particularly aggressive and malignant form of brain tumor.

Cardiovascular health. ISCRM has one of the world's leading programs in heart repair and regeneration. We are exploring two approaches to prevent and treat heart disease. The first approach uses stem cells to grow new heart muscle, while the second utilizes gene therapy to help existing heart muscle pump blood more effectively. Both of these approaches are showing promise in pre-clinical trials with animals.

Diabetes. Scientists are researching how immature stem cells mature into healthy, insulin-producing cells (known as islet cells) and how immune cells and blood vessel cells interact with transplanted islet cells. Understanding these processes will make it possible to vastly improve the treatment of diabetes by developing an unlimited source of islet cells that can be transplanted into patients with the disease.

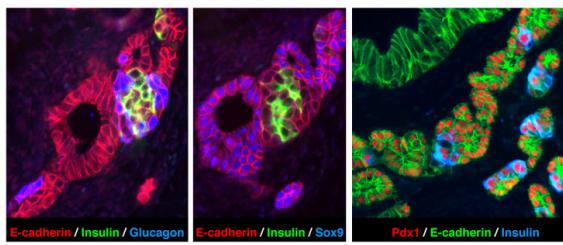
Muscular dystrophy. Researchers are pursuing the development of a gene therapy that can reverse the effects of Duchenne muscular dystrophy (DMD). They have found that damaged genes leading to DMD could be replaced in all muscles of the body using a non-infectious virus — work that is currently in pre-clinical trials with animals. The team is also in the process of developing alternative drug therapies for DMD that would reduce muscle degeneration and correct heart failure, a fatal side effect of the disease.

Spinal cord regeneration. ISCRM's Center on Neural Cell Engineering and Personalized Therapy (CONCEPT) is exploring a number of different ways to cure paralysis caused by chronic spinal cord injuries. One approach central to the team's effort uses cell reprogramming to develop and then transplant healthy cells into an injured spinal cord. Cell reprogramming uses a patient's own tissue to create induced pluripotent stem cells, which can become any cell in the body. Ultimately, these cells should make it possible to replace damaged nerves causing paralysis.

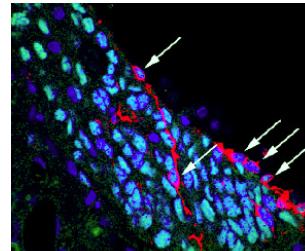
Therapeutics. The research team is developing a variety of drugs and therapeutic agents to improve the treatment of cancer. One approach the scientists are exploring is how signaling pathways between cells can regulate the way cancer cells, specifically melanoma cells, react to drugs. They have found that enhancing these pathways improves the effectiveness of certain drugs, some already FDA approved to treat other diseases.

Join Us

If you would like to support ISCRM's ground-breaking research or learn more about the institute, please contact Jim Boyle at boyleje@uw.edu or 206.543.7252 or visit depts.washington.edu/iscrm/. Thank you for your interest in our work.



The images above show immunofluorescent staining of stem cells, transplanted into mice, that are advancing work on creating insulin-producing cells.



Pictured above are human skin cells that have been coaxed through cell reprogramming to become neural stem cells. The green nuclei are transplanted cells that have integrated with an injured spinal cord in a rat. Their integration into the site of a chronic injury results in significant improvement in hand function.

ISCRM Leadership



Randall T. Moon, Ph.D., Founding Director

UW Professor of Pharmacology; Howard Hughes Medical Institute Investigator; the William and Marilyn Conner Professor for the Institute for Stem Cell and Regenerative Medicine; Fellow, American Academy of Arts and Sciences. Dr. Moon received a Ph.D. from UW Medicine and did post-doctoral training at Caltech. He has published more than 200 research articles and is a member of the Washington State Academy of Sciences.

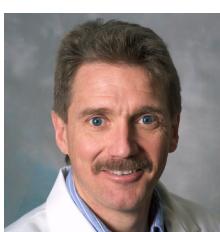
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