ADVANCING TREATMENTS FOR HEART DISEASE

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.

FOUNDED IN 2006, the Institute for Stem Cell and Regenerative Medicine (ISCRM) at the University of Washington brings together more than 140 people from various medical disciplines and institutions. Together, they are pursuing the promise of stem cells in repairing or regenerating damaged tissue.

With stem cell medicine, ISCRM scientists see the opportunity to make great strides in treating diseases, injuries and conditions such as heart disease, blindness, cancer, Alzheimer's disease and other conditions that affect millions of people worldwide.

Using Stem Cells to Treat Heart Disease

The cardiovascular team at ISCRM focuses on developing **new treatments for heart disease** — the leading cause of death and hospitalization in the United States. Myocardial infarction (also known as a heart attack) is a common manifestation of heart disease, in which blood flow to the heart is limited, and the heart muscle cells do not receive enough oxygen.

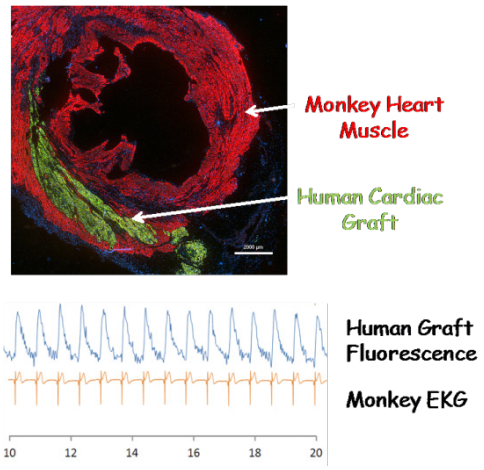
As a result of myocardial infarction, some cardiac muscle cells can die, further weakening the heart's ability to pump blood. Unfortunately, heart muscle is not regenerated in response to damage and cell death, like some other tissues in the body. **Stem cell-based therapies, however, represent a potential tool for replacing cardiac muscle cells when they are lost or damaged from heart disease.**

It has been shown that certain types of stem cells can be differentiated into new cardiac muscle cells in the laboratory. In fact, our experts in cardiovascular health, Drs. Charles Murry, Robb MacLellan and Michael Laflamme, have led the field in defining and refining methods for differentiating human embryonic stem cells into cardiac muscle cells and transplanting those cells into animal models of myocardial infarction. Their published work in this area elegantly demonstrates the therapeutic potential of human embryonic stem cell-derived cardiac muscle cells to repopulate the damaged heart and improve heart function.

Turning Discoveries Into Therapies

Based on the success of these ISCRM investigators, UW Medicine has recently launched a new program, the **Heart Regeneration Program,** to translate this important work into treatments for patients. The goal of this new program is to **develop human embryonic stem cell-derived cardiac muscle cells as a therapy for people with heart disease.**

The first major milestone for the program will be to generate a large data package, which will extensively characterize the potential cell therapeutic and demonstrate its safety profile for the FDA. With UW Medicine's support and philanthropic investments, we anticipate that



Re-growing Heart Muscle

Top image: stem cell-derived human heart muscle graft (green) in infarcted monkey heart (red).

Bottom tracings: fluorescence from flashing human heart muscle cells (blue line) is in sync with monkey electrocardiogram (orange), indicating successful integration of the stem cell graft.

this stage of development will be completed in two to three years. When the FDA is satisfied with that report, and they have designated the cell therapeutic as an investigational new drug (IND), clinical testing can begin.

The first clinical trial will enlist a small number of heart disease patients within UW Medicine's hospitals and other affiliated hospitals to demonstrate the safety of this cell therapeutic in humans. This phase 1 trial will be targeted for completion within one year, and will likely be the first clinical trial in the world of an embryonic stem cell-derived therapeutic for heart disease.

Opportunities for Partnership

Finding ways to enhance the treatment of heart disease is an important part of stem cell research. The Heart Regeneration Program represents a translational effort to bridge the gap between scientific exploration and clinical practice that will benefit patients. Partnerships with members of our community will be tremendously beneficial to our program's progress.

Your Support

If you are interested in helping ISCRM make cardiovascular health a priority, or if you wish to learn more about the institute's work, please contact Jim Boyle, assistant vice president for advancement at boyleje@uw.edu or 206.543.7252. Or visit depts.washington.edu/iscrm. Thank you for your interest.

Key Faculty

Charles E. Murry, M.D., Ph.D., UW Professor, Departments of Pathology, Bioengineering and Medicine-Cardiology; Co-director, ISCRM; Director, Center for Cardiovascular Biology; Arra and Eva Woods Endowed Professor

W. Robb MacLellan, M.D., UW Professor of Medicine and Head, Division of Cardiology; UW Professor, Department of Physiology and Biophysics; Robert A. Bruce Endowed Chair in Cardiovascular Research

Michael A. Laflamme, M.D., Ph.D., UW Associate Professor, Department of Pathology

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Box 358045, Seattle, WA 98195-8045 | 206.543.5686 | writemed@uw.edu | supportuwmedicine.org