Introduction

Welcome to the Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research at UCSF, one of the largest and most comprehensive programs of its kind in the United States.

In some 125 labs, scientists are carrying out studies, in cell culture and animals, aimed at understanding and developing treatment strategies for such conditions as heart disease, diabetes, epilepsy, multiple sclerosis, Parkinson’s disease, Lou Gehrig’s disease, spinal cord injury and cancer.

While the scientific foundation for the field is still being laid, UCSF scientists are beginning to move their work toward human clinical trials. A team of pediatric specialists and neurosurgeons is carrying out the second brain stem cell clinical trial ever conducted in the United States, focusing on a rare disease, inherited in boys, known as Pelizaeus-Merzbacher disease.

Others are working to develop strategies for treating diabetes, brain tumors, liver disease and epilepsy. The approach for treating epilepsy potentially also could be used to treat Parkinson’s disease, as well as the pain and spasticity that follow brain and spinal cord injury.

The center is structured along seven research pipelines aimed at driving discoveries from the lab bench to the patient. Each pipeline focuses on a different organ system, including the blood, pancreas, liver, heart, reproductive organs, nervous system, musculoskeletal tissues and skin. And each of these pipelines is overseen by two leaders of international standing — one representing the basic sciences and one representing clinical research. This approach has proven successful in the private sector for driving the development of new therapies.

The center, like all of UCSF, fosters a highly collaborative culture, encouraging a cross-pollination of ideas among scientists of different disciplines and years of experience. Researchers studying pancreatic beta cells damaged in diabetes collaborate with those who study nervous system diseases because stem cells undergo similar molecular signaling on the way to becoming both cell types. The opportunity to work in this culture has drawn some of the country’s premier young scientists to the center.

While the focus of the science is the future, UCSF’s history in the field dates back to 1981, when Gail Martin, PhD, co-discovered embryonic stem cells in mice and coined the term “embryonic stem cell.” Two decades later, UCSF’s Roger Pedersen, PhD, developed two of the first human embryonic stem cell lines, following the groundbreaking discovery by University of Wisconsin’s James Thomson, PhD, of a way to derive the cells.

Today, the University’s faculty includes Shinya Yamanaka, MD, PhD, of the UCSF-affiliated
J. David Gladstone Institutes and Kyoto University. His discovery in 2006 of a way to reprogram ordinary skin cells back to an embryonic-like state has given hope that someday these cells might be used in regenerative medicine.

Yamanaka’s seminal finding highlights the unexpected and dramatic discoveries that can characterize scientific research. In labs throughout UCSF and beyond, the goal is to move such findings into patients.

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