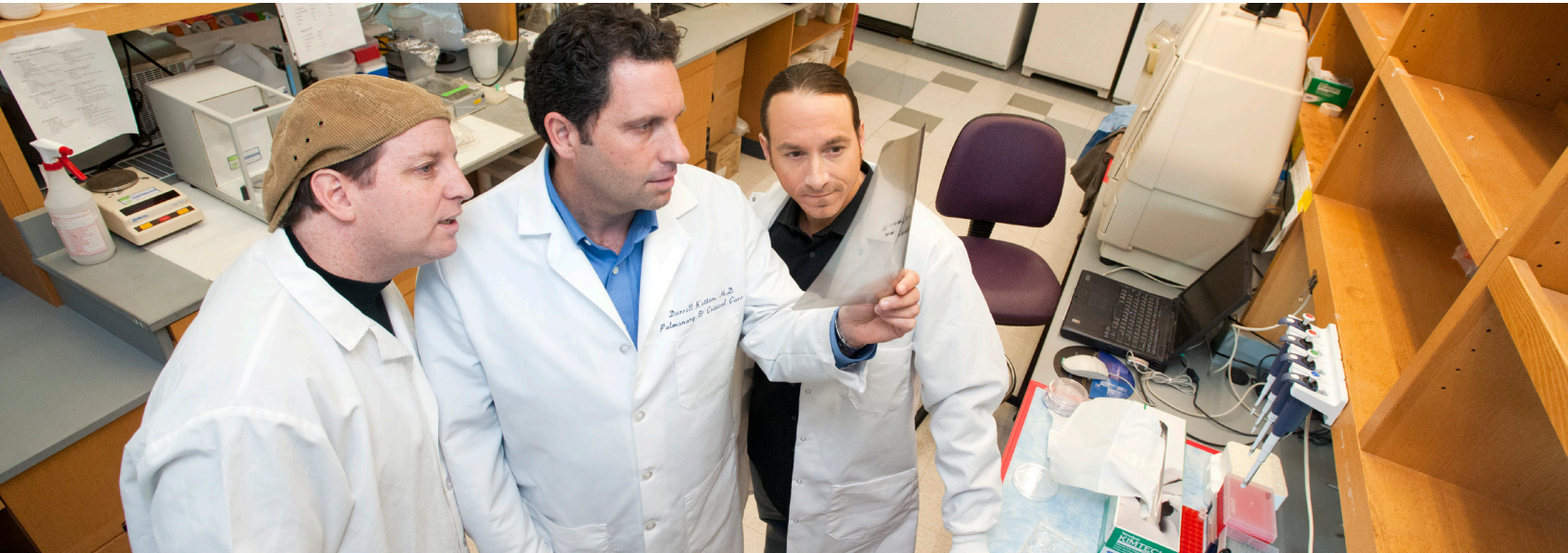


The Center for Regenerative Medicine Advancing Science to Heal the World



OUR GOALS

One- to two-year goals

- A clinical trial in a test tube: using a patient's cells in a test tube to find targeted therapeutics and gauge the medicine's effectiveness before administering it to the patient. This would allow therapeutics to be tested and implemented faster and for less cost than ever before.
- Lab-grown blood for patients with specific anemias: Creating blood in the laboratory will end the need for blood donations and resolve concerns about contamination and supply.
- Fixing defective disease-causing genes in patients through gene editing technologies and using disease models in a lab to design patient-specific medicine to treat diseases that were incurable before.

Ultimate goal

- Creating working bio-artificial organs made from patient-derived cells.

The Center for Regenerative Medicine (CReM) is a multidisciplinary laboratory focused on revolutionizing medicine and research through stem cell technology.

Rather than developing specific treatments for targeted diseases, CReM is focused on methodologies that will change the way medicine is performed and common illnesses diagnosed in the very near future. CReM co-directors Darrell Kotton, MD, Gustavo Mostoslavsky, MD, PhD, and George Murphy, PhD, work with more than 50 other scientists from a variety of fields to discover cell mutations and find out how to generate new cells for patients who are suffering from a variety of common conditions and diseases that currently have no other treatment options.

CReM pursues three major goals:

1. To revolutionize the way medicine is practiced and solve previously untreatable diseases and conditions, while making care more effective, quicker, and more cost efficient for patients.
2. To advance scientific research through open source biology—the selfless sharing of knowledge and cells without restriction or exclusivity, with the ultimate goal of moving science forward more efficiently.
3. To train the next generation of scientists to continue the center's work.

SPECIFIC CLINICAL INTERESTS

CReM focuses on diseases that impact patients at Boston Medical Center, New England's largest safety net hospital, as well as those that affect the global community:

Diseases of the lung

- Lung diseases of newborns and children
- Cystic Fibrosis and Alpha-1 Deficiency
- Emphysema/COPD
- Pulmonary hypertension: primary, sickle cell, or scleroderma

Diseases of the gastrointestinal tract

- Inflammatory bowel disease (Crohn's/ulcerative colitis)
- Hemochromatosis
- Familial Adenomatous Polyposis
- Cirrhosis of the liver

Diseases of the blood

- Sickle cell anemia
- Amyloidosis
- Thrombocytopenia
- Immunodeficiencies

Neurological disease

- Parkinson's Disease
- Alzheimer's Disease
- ALS

Bone regeneration

- Fractures
- Bone loss after trauma

Diabetes

MILESTONES

The rapid evolution of stem cell research over the past decade has brought CReM's research beyond what the investigators could have dreamed at its launch:

- **2004:** Kotton takes on the high-risk mission of generating new lung epithelial cells from stem cells in his Boston University lab. At the time, he had no idea if it were possible or what the body's reaction to the cells would be if he succeeded.
- **2006:** Kotton, Murphy, and Mostoslavsky incorporate a Japanese researcher's discovery of induced pluripotent stem (iPS) cells into their work. For the first time, they can make stem cells that have the potential to differentiate into any type of tissue out of a patient's own cells. No longer do they have to worry about a patient's body rejecting the stem cells.
- **2008:** Cementing their primacy in their field, Kotton, Murphy and Mostoslavsky combine their labs into the Center for Regenerative Medicine, with a focus on using iPS cells to advance regenerative medicine, particularly for diseases commonly seen in their patients in the Boston area.
- **2013:** The vision of CReM is realized and finds a home in a state-of-the-art laboratory on Boston University's campus. Milestones in the center's research include:
 - Developing novel tools for stem cell science, like the "stem cell cassette" that is now used in more than 500 labs worldwide.
 - Creating a bio-artificial rat lung that allowed a rat to breath for six hours and a gene therapy that offered lifetime protection against an inherited form of emphysema in mice.
 - Assembling an international iPS Cell Bank with more than 150 high quality cell lines created from patients with inherited diseases.

Today, it's a historic time in stem cell research, with an inexhaustible supply of patient-derived stem cells available. CReM is one of few groups in the country primarily focused on differentiating patient-derived stem cells into chosen cell or tissue types and manipulating the DNA within these cells to correct genetic defects. CReM is also working on lab-based disease modeling, gaining insight into human disease and testing the effectiveness of novel drugs on patient cells and tissues grown in the laboratory. With growing expertise in these techniques and 13 different lineages of cells, the team is hastening the advent of patient-specific medicine.

CReM BY THE NUMBERS

- 55 publications
- 1 patent
- \$3.7 million of research funding from 15 grants in 2014-15

HOW YOU CAN HELP

While the center receives grants and financial support from institutions including the National Institutes of Health, Alpha-One Foundation, American Thoracic Society, American Lung Association, Coulter Foundation, Boston University and Boston Medical Center, this support is not enough to take the next big step forward.

The upcoming years could prove to be a quantum leap in stem cell therapies as the CReM completes the groundwork for the transition from animal studies and clinical trials to human iPS application.

Your support will allow the CReM to lead the way in this field by giving the scientists the ability to focus on their research. It will help:

- buy state-of-the-art equipment and facilities,
- recruit new researchers from across the nation and around the world,
- development and operation of the iPS Cell Bank, and
- support clinical research aims of different programs.

To learn more, please contact Boston University School of Medicine at 617-638-4570 or busmdev@bu.edu