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The Rockefeller University Hospital:  
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## **The Rockefeller University Hospital: Transforming Medicine**

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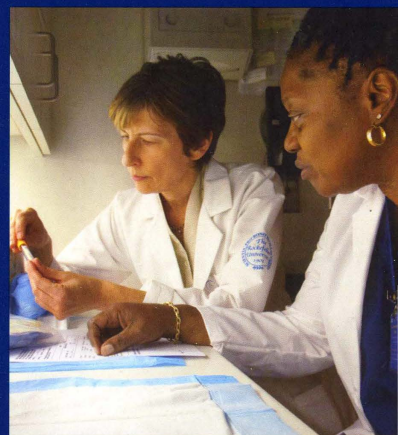
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THE ROCKEFELLER UNIVERSITY HOSPITAL

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# TRANSFORMING MEDICINE



**On the Cover**

**Left:** Physician Mina Pastagia, a Clinical Scholar who is studying a novel treatment for staphylococcal skin infections in psoriasis patients.

**Center:** Virologist Charles Rice, a leader in the study of hepatitis C, with postdoctoral investigator Catherine Murray.

**Right:** Melissa Offenhartz (at left), director of nursing and patient care services, and nursing assistant Olga Ford.

## A REVOLUTIONARY IDEA

For more than a century, The Rockefeller University has stood at the forefront of biomedical discovery. Founded in 1901 as the nation's first medical research center, Rockefeller has long been a world leader in the effort to understand human health and disease. Rockefeller's research hospital, which opened its doors in 1910, was the country's first clinical research hospital—and the birthplace of clinical research in the United States. A hospital unlike any other at the time, this facility was devoted to clinical investigation and to translating laboratory discoveries into new approaches to diagnosing, treating, and preventing disease.

The revolutionary idea on which the Hospital was founded is as alive today as it was in 1910. The rise of molecular biology, born in the Rockefeller Hospital in 1944, has opened previously unimagined therapeutic possibilities. Thus, translating basic discoveries into medical treatments and preventions is more important than ever. More and more, both basic scientists and physician-scientists are exploring medical applications of their discoveries. The Hospital's expert and dedicated staff and I are doing all we can to support the efforts of these gifted investigators and to train the next generation of clinical research leaders.



Barry S. Collier, M.D.  
*Physician-in-Chief*  
*The Rockefeller University Hospital*

## EXTRAORDINARY BREAKTHROUGHS

The Rockefeller Hospital has been the site of myriad breakthroughs that have advanced the treatment and prevention of disease, including the discovery that DNA is the carrier of genetic information—the finding that launched the modern biological revolution. Rockefeller researchers were also the first to:

- demonstrate that a virus can cause cancer
- confirm the connection between cholesterol and heart disease
- create effective vaccines for meningitis and pneumonia
- develop animal models for the study of heart disease and multiple sclerosis
- discover blood types and ways to preserve whole blood, making blood banks possible
- pioneer clinical studies based on the dendritic cell, a key immune system cell discovered at Rockefeller
- demonstrate that the body's metabolism adjusts to maintain a set weight, helping to explain why weight loss is so difficult
- develop drug combinations that offered the first life-extending treatments for HIV and AIDS
- discover an obesity gene and a weight-regulating hormone, leptin
- devise methadone treatment to manage heroin addiction
- develop a rationally designed antiplatelet therapy, which improved the results of coronary artery stenting





Above: The Rockefeller University Hospital, which has been extensively renovated over the past decade, houses a 20-bed inpatient unit and an outpatient center, as well as state-of-the-art laboratories for principal investigators.

Right: World-renowned AIDS investigator David Ho administers a new vaccine under study to a volunteer at the Hospital.



## LEADING THE WAY IN CLINICAL RESEARCH AND MOLECULAR MEDICINE

Biomedical scientists associated with the Hospital conduct research that is innovative and influential. They are creating new “smart” drugs, designing and testing therapeutic and preventative vaccines, and introducing a host of other medical interventions that emanate from the latest laboratory findings. Working at the cutting edge of molecular medicine and clinical investigation, the University’s researchers are developing:

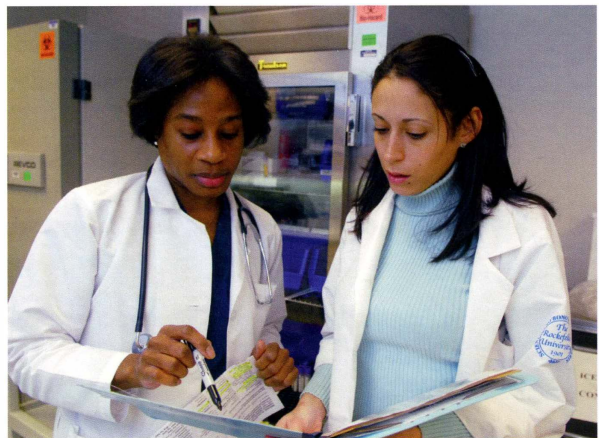
- Precise molecular markers that can be used to plan cancer treatment, based on a Rockefeller scientist’s discovery of several microRNAs that disable tumor metastasis. MicroRNAs, tiny molecular entities that were unknown 20 years ago, are now recognized as determinants of health and disease.
- New methods using microRNAs to identify disease-relevant genetic variations in individuals with fragile X syndrome, which is strongly associated with autism. These technologies will eventually contribute to custom-designed treatments for a range of disorders.
- A “nanosyringe” for intracellular delivery of protein therapeutics, re-engineered from a mechanism that some bacteria use to inject toxins into host cells.
- New ways to prevent and treat rare childhood infections as well as global diseases such as tuberculosis, based on a Rockefeller scientist’s revolutionary work that is revealing inborn immune deficiencies that confer vulnerability to specific microorganisms.
- New ways to fight MRSA and other antibiotic-resistant bacteria with an enzyme isolated from a phage—a virus that kills bacteria. This could dramatically reduce the incidence of resistant infections acquired in hospitals, a leading cause of death in the elderly.
- New therapeutic regimens for melanoma based on knowledge acquired from state-of-the-art molecular genetic and imaging studies.



- Recommendations regarding the effectiveness of vitamin D in preventing cardiovascular disease.
- A broad-spectrum therapy to suppress autoimmunity, the harmful attack by the immune system against the body's own cells that can result in rheumatoid arthritis, type 1 diabetes, and lupus.

**Right: The Hospital's nursing staff plays an essential role in clinical investigations.**

**Below: Physician-scientist Agata Smogorzewska with a member of her laboratory. A Rockefeller alumna, she returned to the University in 2009 as an assistant professor.**





## CLINICAL SCHOLARS

The Clinical Scholars Program at The Rockefeller University Hospital addresses one of the most pressing needs in biomedicine: the training of physician-scientists who are qualified to conduct research in both the laboratory and the clinic. The three-year program is designed to provide an optimal environment and mentoring structure in which young physicians gain the experience and capabilities necessary to initiate independent careers as patient-oriented and translational investigators.

Most applicants to the program are physicians who have recently completed their residency or subspecialty fellowship. Each Clinical Scholar

becomes affiliated with at least one Rockefeller research group and carries out patient-oriented translational research under the mentorship of the head of laboratory. This independent research is complemented by a rigorous core curriculum in clinical and translational investigation. The program leads to a master's degree in clinical and translational research.

Current and recent Clinical Scholars conduct or have conducted innovative biological research on a broad spectrum of medical problems, including tumor immunity and

tumor immunotherapy, the regulation of dietary cholesterol absorption, platelet physiology and thrombosis, the autoimmune basis of psoriasis, the role of the hormone leptin in obesity, HIV infection and treatment,



the development of vaccines for HIV, immune dysregulation associated with hepatitis C, the molecular and genetic bases of addiction, and a range of questions in basic immunology.

Although most Clinical Scholars are associated with Hospital-based laboratories, several Scholars have joined basic research groups to initiate and carry out projects that integrate recent findings in biology with patient-based studies. Topics addressed by these Clinical Scholars include

liver inflammation, the cause of chronic lymphocytic leukemia and lymphomas, and the application of RNA biology to kidney disease and neurodegenerative disorders.

**Left: Clinical Scholar Dana Orange with Robert Darnell, head of the Laboratory of Molecular Neuro-oncology.**

**Below: Immunologist Marina Caskey (at right) with colleagues. A former Clinical Scholar, Dr. Caskey continues to conduct HIV vaccine research at Rockefeller under a career development award from the National Institutes of Health.**



## **DISTINGUISHED FACULTY**

Faculty currently affiliated with the Rockefeller Hospital include such outstanding investigators as:

**Jan L. Breslow, M.D.**, former president of the American Heart Association, who has pioneered the use of DNA technology to study heart disease.

Senior Attending Physician, Frederick Henry Leonhardt Professor  
Laboratory of Biochemical Genetics and Metabolism

**Jean-Laurent Casanova, M.D., Ph.D.**, who received the 2008 Richard Lounsbery Award for discoveries about genetic vulnerabilities to viral and bacterial diseases of childhood.

Senior Attending Physician, Professor  
St. Giles Laboratory of Human Genetics of Infectious Diseases

**Barry S. Coller, M.D.**, a leader in vascular biology, who developed an F.D.A. approved antiplatelet therapy and created a test to monitor the effectiveness of aspirin and clopidogrel (Plavix) in individual patients.

Physician-in-Chief, Vice President for Medical Affairs  
David Rockefeller Professor  
Allen and Frances Adler Laboratory of Blood and Vascular Biology

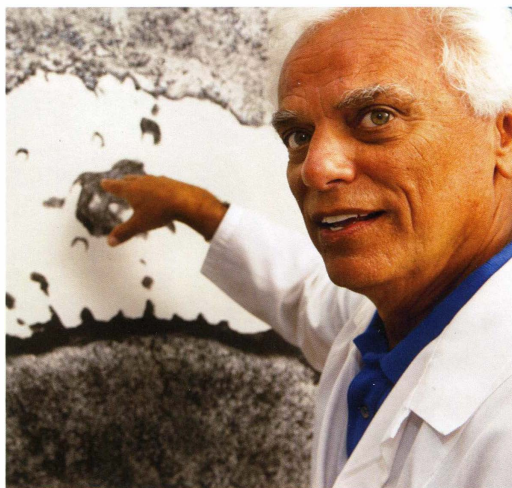
**Robert B. Darnell, M.D., Ph.D.**, whose work is shedding light on autoimmunity, neurodegenerative diseases, and cancer.

Senior Attending Physician, Robert and Harriet Heilbrunn Professor  
Laboratory of Molecular Neuro-oncology  
Investigator, Howard Hughes Medical Institute

**Vincent A. Fischetti, Ph.D.**, who is developing novel antibacterial strategies against MRSA, strep, anthrax, and other pathogens.

Professor  
Laboratory of Bacterial Pathogenesis and Immunology





**Top: National Medal of Science recipient Elaine Fuchs with President Barack Obama.**



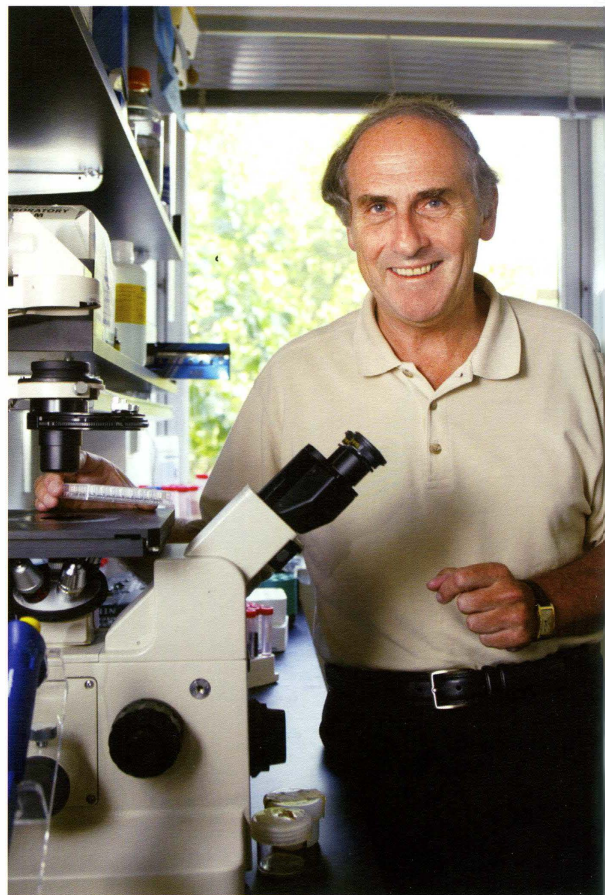
**Above: Physician-scientists Jean-Laurent Casanova (at left) and Sohail Tavazoie, who recently established new laboratories at the University.**

**Above: Microbiologist Vincent Fischetti.**



Albert Lasker Award recipients Jeffrey Friedman, below, and Ralph Steinman, right. Dr. Friedman discovered the appetite-regulating hormone leptin. Dr. Steinman identified dendritic cells, key components of the body's immune system.

Mary Jeanne Kreek, bottom at center, has been an international leader in establishing addiction studies as a vital branch of biomedical science.



**Jeffrey M. Friedman, M.D., Ph.D.**, who received a 2010 Albert Lasker Award for his discovery of the obesity gene and the weight-regulating hormone leptin.

Marilyn M. Simpson Professor  
Laboratory of Molecular Genetics  
Director, Starr Center for Human Genetics  
Investigator, Howard Hughes Medical Institute

**Elaine Fuchs, Ph.D.**, recipient of a National Medal of Science in 2009 for her innovative research on skin stem cells.

Rebecca C. Lancefield Professor  
Laboratory of Mammalian Cell Biology and Development  
Investigator, Howard Hughes Medical Institute

**David D. Ho, M.D.**, who is at the forefront of efforts to develop HIV vaccines and other prevention strategies.

Senior Physician, Irene Diamond Professor  
Scientific Director and Chief Executive Officer, Aaron Diamond AIDS Research Center

**Mary Jeanne Kreek, M.D.**, a pioneer researcher in addiction biology who helped develop methadone maintenance therapy for heroin addiction.

Senior Attending Physician, Patrick E. and Beatrice M. Haggerty Professor  
Laboratory of the Biology of Addictive Diseases

**James G. Krueger, M.D., Ph.D.**, noted for his discoveries about psoriasis, who also heads a major initiative on melanoma.

Senior Attending Physician, D. Martin Carter Professor  
Laboratory for Investigative Dermatology  
Director, Milstein Medical Research Program

**Michel C. Nussenzweig, M.D., Ph.D.**, who is designing immuno-therapies for HIV, cancer, diabetes, and other disorders.

Senior Physician, Sherman Fairchild Professor  
Laboratory of Molecular Immunology  
Investigator, Howard Hughes Medical Institute



**Jeffrey V. Ravetch, M.D., Ph.D.**, who studies immune mechanisms and is developing new therapies for autoimmune disorders.

Theresa and Eugene M. Lang Professor  
Leonard Wagner Laboratory of Molecular Genetics and Immunology

**Charles M. Rice, Ph.D.**, who is a leading authority on the hepatitis C virus, one of the main causes of liver cancer.

Maurice R. and Corinne P. Greenberg Professor  
Laboratory of Virology and Infectious Disease  
Director, Center for the Study of Hepatitis C

**Agata Smogorzewska, M.D., Ph.D.**, who uses a hereditary genome instability syndrome, Fanconi anemia, as a model for understanding the cellular aging process and cancer.

Assistant Professor  
Laboratory of Genome Maintenance

**Ralph M. Steinman, M.D.**, who received the 2007 Albert Lasker Award for his discovery of the dendritic cell, which is now a focus of study in laboratories worldwide.

Senior Physician, Henry G. Kunkel Professor  
Laboratory of Cellular Physiology and Immunology  
Director, Christopher H. Browne Center for Immunology and Immune Diseases

**Sohail Tavazoie, M.D., Ph.D.**, who studies small molecules that block tumor metastasis in an effort to improve cancer diagnosis and treatment.

Senior Attending Physician, Leon Hess Assistant Professor  
Elizabeth and Vincent Meyer Laboratory of Systems Cancer Biology

**Leslie B. Vosshall, Ph.D.**, whose studies of human olfaction are leading to a deeper understanding of neurosensory disorders.

Robin Chemers Neustein Professor  
Laboratory of Neurogenetics and Behavior  
Investigator, Howard Hughes Medical Institute



## FROM THE BOARD CHAIR

I first became involved with The Rockefeller University because of the Hospital. Some years ago my daughter was seen by Rockefeller doctors. She suffered from psoriasis—not a life-threatening condition but decidedly a quality-of-life-threatening one. At the time, I knew little about the University. I observed that she received excellent care. In return, my family had the satisfaction of knowing that her participation was contributing to research that might one day provide a cure.

**Above: Board Chair  
Russell Carson with  
Professor Leslie VossHall.**

My curiosity aroused, I began attending events at the University to find out more about it. What I learned so impressed me that I eagerly accepted an invitation to join the Board in 1994. In the years since, as my knowledge has deepened, so has my appreciation—indeed, my awe—of the University and its remarkable team of scientists and physicians. Their contributions to medical science have been profound.

**Russell L. Carson**  
*Chair, The Rockefeller University*

## **OVERVIEW OF THE ROCKEFELLER UNIVERSITY**

Scientifically and geographically, The Rockefeller University Hospital stands at the heart of a world-renowned center for biomedical investigation and graduate education. The Rockefeller University is dedicated to research that will improve the understanding of life for the benefit of humanity.

Founded in 1901 as The Rockefeller Institute for Medical Research, the University was the nation's first institution devoted exclusively to biomedical science. It was renamed The Rockefeller University in 1965.

### **A Community of Scholars**

Today, the University comprises more than 70 independent laboratories. The Rockefeller community includes 300 research and clinical scientists, 350 postdoctoral investigators, 200 graduate students, and 1,050 technical and administrative personnel, all working at the University's 14-acre campus.

### **A Unique Open Structure**

Since its founding, The Rockefeller University has embraced an open structure to encourage collaborations between disciplines and empower faculty members to take on high-risk, high-reward projects. No formal departments exist, bureaucracy is kept to a minimum, and scientists are given resources, support, and unparalleled freedom to follow their research wherever it leads.

### **Areas of Investigation**

Rockefeller's scientists conduct research in six broad areas: biochemistry and structural biology; molecular, cell, and developmental biology; medical sciences and human genetics; immunology, microbiology, and virology; neuroscience; and physics and mathematical biology. In addition, eight interdisciplinary centers focus on Alzheimer's disease, cancer, hepatitis C, and other biomedical concerns.





### **Award-Winning Science**

Rockefeller University's unique approach has led to some of the world's most revolutionary contributions in biology and medicine. Over the years, 23 Nobel laureates have been associated with Rockefeller, including two alumni. In addition, the University has been home to 21 recipients of the Albert Lasker Medical Research Award, 14 National Medal of Science recipients, and 14 Gairdner Foundation International award-ees. Thirty-five members of the current faculty have been elected to the National Academy of Sciences.

### **An Exceptional Place to Learn**

More than 1,000 scientists have received Rockefeller Ph.D. degrees since the graduate program began in 1955. Providing opportunities to conduct the highest quality science, the program attracts exceptional

students from around the world. The Rockefeller University also sponsors one of the nation's top M.D.-Ph.D. programs with neighboring Memorial Sloan-Kettering Cancer Center and Weill Cornell Medical College. In addition, at any one time, several hundred postdoctoral investigators receive advanced training and conduct research in Rockefeller's laboratories.

### **Funding for New Science**

At Rockefeller, the research enterprise and educational programs are supported by government and private grants and contracts, private philanthropy, and income from the endowment. The University's annual operating budget totals more than \$310 million.

### **Key Partnerships**

Rockefeller has close ties to Memorial Sloan-Kettering Cancer Center, Weill Cornell Medical College, New York-Presbyterian Hospital, and other leading institutions. In addition, 14 University scientists are Howard Hughes Medical Institute Investigators—the highest percentage of HHMI Investigators on any biomedical faculty in the country. Rockefeller also forges collaborations with biotech and pharmaceutical companies to speed research findings into development and improve patient care.



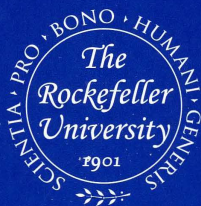
**Rockefeller University Trustee Judith Roth Berkowitz, a member of the Board's Hospital Committee, with Physician-in-Chief Barry Collier.**

For more information, please contact:

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SCIENCE FOR THE BENEFIT OF HUMANITY