



Harvard Stem Cell Institute
2012 **Annual Report**

HSCIdea

HSCInnovate

HSCInvest

HSCInteract

HSCIncubate

HSCInfluence

HSCInspire

HSCImpact

From the Directors



From left: Douglas A. Melton, David T. Scadden, and Brock C. Reeve

This is the time of year when we look back, before forging ahead. Eight years ago, when we founded the Harvard Stem Cell Institute, we came together with a shared vision of a radically new approach to advancing our understanding of human development and disease, of discovering treatments for the most pressing medical challenges facing society, and of the very way to conduct scientific research. We were and remain convinced that half-century-old model for discovering and developing medical advances was broken. And we believed, and our belief has been borne out countless times these past eight years, that the stem cell—the building block of every cellular and organ system—had to be the focus of a new approach to curing disease.

As we consider the accomplishments of HSCI's eighth year, we are looking back, and moving forward, through the prism of a series of words beginning with the letter I, the first of which is **Idea**. For it was a grand idea that launched HSCI, the idea that we literally could reinvent the way biological problems are approached, scientific research is undertaken, and cures and treatments for diseases are discovered and developed by forming a community of scientists who shared this idea and the belief that stem cells are the foundation on which this innovative change could be built.

From the moment of its creation, to the present day, HSCI has been a unique entity, a scientific collaborative bringing together first a handful and now more than 1000 scientists, in all stages of their careers, in more than 90 laboratories in four schools and the dozen Harvard-affiliated hospitals and research institutions, all of which are part of the vibrant Boston-Cambridge ecosystem of academic and corporate science. This unusual and most effective team of researchers is focused on a broad range of diseases, approaching them from an eclectic collection of scientific disciplines. But all 1000 scientists share the belief that the key to understanding growth, regeneration, and repair processes—in both normal and disease states—is based on understanding the basic unit of life, the living cell. And HSCI has assembled these scientists into teams of cell biologists, chemists, geneticists, computational scientists, clinicians and bioengineers, all of whom believe that if one is going to cure human disease, one needs to study the disease in human cells derived from patients afflicted with the disease being studied. Since we have scientists whose expertise crosses so many disciplinary boundaries, whenever a new problem arises, we can bring experts to bear not only to explicate the problem, but more importantly, to manipulate, to change, the biological situation creating it.

In the following pages, and in the video interviews available online, you will see how the grand **Idea** called the Harvard Stem Cell Institute has proven itself as a platform for **Innovation** and collaborative **Interaction**. We continue to **Invest**, as what might be called “intellectual venture capitalists,” serving as a breakthrough scientific **Incubator**, **Influencing** science and the careers of scientists, **Inspiring** the next generation of researchers, and, ultimately, **Impacting** the future of science, medicine and public health.

With thanks,



Douglas A. Melton, PhD and David T. Scadden, MD
Co-Directors
Harvard Stem Cell Institute



Brock C. Reeve, MPhil, MBA
Executive Director
Harvard Stem Cell Institute

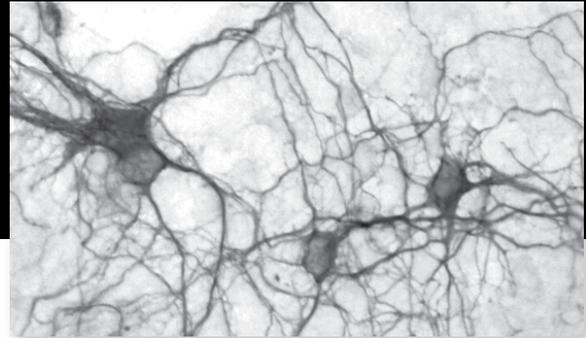
HSCIdeas



The Idea that became HSCI

The idea of an interdisciplinary, cross-institutional collaborative research organization with a unique breadth and depth of expertise focused both on advancing science and curing disease has become reality. HSCI has grown from a concept to become a vibrant, world-leading institution that has led to the development of many new ideas both in science as well as in how to do science.

HSCIInnovate



Platform for Innovation

HSCI's strategy of bringing together scientists and clinicians from multiple disciplines and institutions has led to new breakthroughs. One example of interdisciplinary innovation is this application of a new analytical technology from the world of physics to stem cell biology. By creating a new way to visualize and prove the origins and behavior of selected cell populations, we not only understand more about cell and organ growth and repair, but can also devise new therapeutic strategies.

HSCIinvest



Investing in Basics and Beyond

As an "intellectual venture capitalist," HSCI invests its money and know-how in important work that in many cases would not otherwise be funded. Some of the returns on these investments are farther off, some are very close to the clinic, but all are measured in many tangible ways - attaining follow-on funding, publishing in leading journals, developing and licensing intellectual property, forming new companies and accelerating the careers of our scientists.

HSCIInteract



Collaborative Interaction

From its inception, HSCI realized the importance of close and open collaboration in order to solve the big scientific and clinical challenges that are inherent in taking a fundamental new technology and applying it to tackle curing disease. Sharing perspectives, resources and experimental results has made teams and individuals more successful than they would be otherwise and created a culture of team-based science at HSCI from the very first stages of individuals' careers.

We invite you to watch and listen online to short videos telling our story. This printed guide is a preview of the online presentations.

HSCI**Incubate**



A Breakthrough Incubator

Many early-stage ideas are risky and we don't always know when a brand-new idea will be sufficiently substantive to form the foundation of a company, but the HSCI environment allows us to see those ideas early on, be able to quickly decide how best to support and advance them, and to work with leaders in the investment community to help turn them into long-lasting entities.

HSCI**Influence**



Influencing Science and Careers

Leading scientists, and aspiring leaders, want to be in an environment surrounded by colleagues whose work and opinions they value, can draw on and to which they can contribute. The HSCI network of people, labs and institutions, all within close proximity of each other, not only enables collaboration and influences the work that people do here, but influences them to come and to stay because this is where they feel they can do their best work.

HSCI**Inspire**



Inspiring the Next Generation

The future of science depends on future scientists. In addition to focusing on junior faculty and graduate students, HSCI has created a summer internship program where undergraduates from around the world come to engage in hands-on projects in the labs of the world's best scientists. In the words of a recent intern, "This was the best summer of my life."

HSCI**Impact**



Impacting the Future of Medical Care

HSCI scientists made many discoveries this year in multiple disease categories, organ systems, and technologies. These have ranged from advancement of our understanding of the fundamentals of disease and the body's ability to regenerate and repair to identifying new cellular therapies and new drugs. Whether understanding, for example, new approaches to heart repair or how to transplant functional nerve cells, our ultimate goal is to have an impact on patients and medical care.

Words in Action

The idea that started HSCI is impacting people, professions and stem cell science.

- HSCIdea** Create and nurture a collaboration of more than 1000 scientists all focused on reinventing the way biological problems are approached, scientific research is undertaken, and cures and treatments for diseases are discovered and developed.
- HSCIinnovate** Develop new scientific solutions through innovative approaches by bringing multiple scientific disciplines to bear on a problem and develop a new business model that accelerates scientific discovery across the value chain through efficient use of intellectual, physical and financial capital.
- HSCIinvest** Put money into the best ideas, not the biggest names; provide risk takers with the capital they need to produce enough data to get them the large federal grants and industry collaborations that will carry their ideas to the next level, and ultimately to the clinic.
- HSCIinteract** Create an interdisciplinary community of innovative scientists, at all career levels, who share three things: a passion to cure diseases; a belief that stem cell biology is key to understanding development and disease, and; an eagerness to work together and learn from one another.
- HSCIincubate** Provide the critical support, from scientific advice, to access to specialized research cores, to financial underwriting, to business development that is needed to grow the ideas that our talent produces into services, products and companies that make a difference to patients.
- HSCIinfluence** Create an environment and network of intellectual cross pollination that changes career trajectories, that helps scientists realize the potential of their expertise and apply that expertise across boundaries they thought were insurmountable.
- HSCIinspire** Open the door for the next generation of scientists by giving them the opportunity to work side-by-side with today's leaders in tackling questions that will drive their imagination and inspiration to new heights they could not otherwise imagine.
- HSCIimpact** On a daily basis, reshape the future of biological discovery and medical science, making today's diseases tomorrow's history.

Finances in Brief

During fiscal year 2012 Harvard Stem Cell Institute's expenditures totaled \$16.9M, 85% of which was invested in direct research.

Targeted Research (39% of expenditures) in our disease-focused programs increased from the prior year reaching \$6.7M. This was boosted during the year through new support such as a five-year gift that is enabling a multi-lab, consortium project in a specific nervous system disease. We continue successfully to leverage our donor supported investments through seeking additional funding from federal, non-profit and commercial sources.

Non-sponsored research increased by approximately 30% from the prior year while sponsored funds were a smaller percent of our expenditure as we continued to transition certain projects to the Harvard Department of Stem Cell and Regenerative Biology where many of our core faculty members now reside.

The Seed Grant Program for early stage, innovative projects continued to support 20 faculty members within Harvard University and our affiliated hospitals in multiple therapeutic and technical areas. Our annual project survey shows that a high percentage of the seed grants go on to secure longer term funding from federal and other sources.

Support of core facilities (13% of expenditures)—the Therapeutic Screening Center, the Flow Cytometry Cores, the iPS Core Facility and the new Center for Stem Cell Bioinformatics—increased slightly over last year. This was largely attributable to the significant funding we allocated to the expansion of the iPS Core which is now fully operational. In the coming year, we expect the investment required there will be less as the iPS Core becomes more financially self-sustaining through external grants and its fee-for-service operations. We also invested additional money to expand the bioinformatics program projects into the Center for Stem Cell Bioinformatics, housed at the Harvard School of Public Health and supporting all HSCI affiliates.

Training included graduate and postdoctoral support training programs, with a focus on clinician scientists, as well as HSCI's flagship undergraduate internship program which funded 42 students this year from around the world to work in the laboratories of HSCI faculty members on intensive summer projects.

FY 2012 Expenditure Allocation



To Our Supporters

We extend our sincere thanks to all our donors, large and small. A complete list of our donors is available online at <http://www.hsci.harvard.edu/2012AR/supporters.php>

Our deepest gratitude goes to those individuals and organizations whose major gifts and pledges not only evince their profound commitment to stem cell science at Harvard but will also touch the lives of future patients suffering from currently intractable health problems.

Financial support is crucial to the success of HSCI. We believe that our success is crucial to the future of health care.

