

# Shared Science

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# Shared Success

Harvard Stem Cell Institute  
Annual Report 2011

The Harvard Stem Cell Institute  
is a scientific collaborative  
established to fulfill the  
promise of stem cell biology.

# 2011 in Review

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## From the Directors



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

As the title of this year's Harvard Stem Cell Institute annual report suggests, our seventh year has been one of shared successes growing out of shared science. Much of that shared science has been made possible by new technologies, new collaborations, and an understanding that 21<sup>st</sup> century science not only benefits from, but requires collaboration and cooperation in all aspects.

The changing scientific scene is reflected not only in the content of the 2011 HSCI annual report, but also in its form. Those of you who are used to receiving a traditional, 30-page bound report may wonder where the rest of your pages are. In fact, you will find the entirety of the report on the Internet at <http://www.hsci.harvard.edu/2011AR>. But even there, you will experience major changes, for rather than providing you with

written descriptions of our accomplishments, we asked HSCI scientists, from our senior Program Leaders to our most junior new faculty members, to tell you in videos about their research. By doing this, not only will you hear directly from the people doing the work but we are able to spend more money on our research programs and less on the production and printing of the annual report—the videos you will see were, in fact, filmed by a staff member using an iPhone, rather than professionally produced by a video company.

As you will learn, this has been a year in which HSCI has expanded its technological capabilities, from shared core facilities including a scale-up of the iPS Core, to launching a bioinformatics core based on successful pilot projects, including a shared Fellow with the Broad Institute.

And this has been a year in which we have continued the focus of last year's annual retreat—translational research—with an educational workshop, interdisciplinary meetings focused on applications of stem cells, a new pre-clinical project in Parkinson's disease, a study that gave a rat a functioning lung, a clinical trial in enhancing cord blood stem cells, and two junior faculty collaborative projects specifically focused on translational research, to name a few. We continue to reach out to external experts—for example, a representative from the FDA spoke at our Salon on tissue engineering and scientists from several biopharmaceutical companies were a part of our session on the role of stem cells in drug discovery.

We have made enormous strides these past seven years in moving stem cell science closer to the clinic and the payoff we know is coming for the millions of patients suffering from intractable diseases. But our path has been littered with political, legal, and financial stumbling blocks. In fact, this past year saw a temporary halt on federal funding of embryonic stem cell research as a result of a court case. We had good news just a few months ago when that suit was dismissed but we are still facing major challenges.

This year's national budget debates reminded us all that we are living in difficult economic times. It is painfully clear that massive cuts in federal spending of all kinds—including funding for scientific and medical research—are inevitable. So once again, we remind you that you have made our successes possible with your incredible generosity and that we literally could not have done this without you. And without your continued support, we will not be able to continue to build in the future upon the discoveries, advances, and investments we have made to date.

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

# 2011 in Review

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## Highlights of the Year

### 1 Research Programs

- Continuing our commitment to innovative science, we instituted new pilot projects within our disease programs.
- Conducting think tanks in each disease area further broadened the HSCI community and fostered new collaborations.
- We established a new bioinformatics fellowship with the Broad Institute in recognition of the growing synergy between stem cell science and genomic research.

### 2 Centers & Core Facilities

- The creation of an iPS Core facility with a new fee-for-service model offers a powerful new capability and service to the entire research community.
- The addition of a new shared cell sorting and analysis facility is already reducing wait times and accelerating research.
- We launched a new bioinformatics core facility based on two successful pilot projects.

### 3 Science Updates

- HSCI researchers developed new approaches to cellular reprogramming that don't require genetic modification.
- Our scientists demonstrated the use of a specific cell population to repair damaged cardiac tissue through stimulating internal mechanisms.
- The use of zebrafish as a high-throughput platform for finding drugs enabled HSCI scientists to bring a potential melanoma treatment to the clinic.
- Our cell-based drug screening program identified potential candidates for treating spinal muscular atrophy and ALS.

### 4 Investment in Future Leaders

- The ever-more-successful HSCI undergraduate internship program hosted a record 47 students.
- To enhance our goal of translating work from the lab to the clinic we developed a workshop for Junior Faculty with the latest information and methodologies.
- We tackled large interdisciplinary issues—New Technologies for Imaging Cells; Stem Cells and Drug Discovery; Tissue Engineering—through our Stem Cell Salons.
- We brought 23 experts from around the world to join our Seminar Series, Salons, and Annual Retreat to share their latest work.

# 4

VIEWS

## Shared Science at HSCI

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Working together we are shaping the future of health care.

# 1

RESEARCH PROGRAMS

## Fighting Diseases Requires Team Effort

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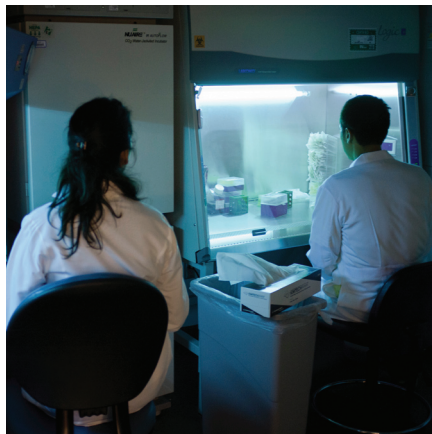
Think of them as Institutes within the Institute. Each of the Harvard Stem Cell Institute's Research Programs is itself a scientific institute, a collaborative made up of established world leaders and the brightest young stars, focused on the role of stem cell science in understanding and treating a particular group of diseases. With programs focused on diseases of the blood system, cardiovascular system, kidneys, diabetes, cancer, nervous system, and on translational research, our scientists are advancing the understanding and treatment of the most pressing human health problems. And like everything else at HSCI, each of the programs is interdisciplinary, with specialists in each area sharing insights and ideas with their colleagues in the other disease programs.

# 2

CENTERS & CORE FACILITIES

## Building Collaborative Tools & Places

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Nowhere is the HSCI philosophy that shared science contributes to shared success more apparent than in the establishment and growth of our multiple centers and core facilities. Whether it's the iPS Core, or the Therapeutic Screening Center, each of these facilities provides our scientists with access to invaluable technologies and shared expertise no one lab could afford to develop or acquire. And because these specialty facilities are available to our entire community, we are able to keep costs for individual users at a level that is affordable for even the most junior scientist with an initial grant.

For an insider's look at HSCI, we invite you to watch and listen to some of the key scientists and researchers on our team. This printed guide is a preview of their first-hand stories highlighting four key aspects of collaboration at HSCI.

# 3

## 2011 SCIENCE UPDATES

### Making Progress Through Basic Science

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If we had the time and space, and thought you had the inclination, we could fill an entire annual report with summaries of the scientific publications produced by HSCI scientists this past year; literally a week doesn't go by without one or two new studies by HSCI faculty appearing in prestigious publications such as *Cell*, *Cell Stem Cell*, *Nature*, *Science*, and others. Many of these findings are so basic as to seem arcane, but they are the building blocks upon which blockbusters are built. And each year produces its share of blockbuster ideas, from entirely new ways to create iPS cells, to using stem cells to find drugs, to new *in vitro* and *in vivo* disease models, or to new approaches to cell therapy.

# 4

## INVESTMENT IN FUTURE LEADERS

### Providing Encouragement & Funding

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From the very founding of the Harvard Stem Cell Institute, its senior scientists have believed that the Institute's most valuable capital is human. HSCI's leadership believed then, and continues to believe, that the way to achieve the highest return on investment in science is to invest directly in its future—the young women and men who enter this new field with superb training, but without preconceived notions about where their scientific inquiries may take them. That is why we invest in summer internships for brilliant college students from around the world, in our Junior Faculty programs, and in Seed Grants to advance science that is promising but so cutting-edge other funders are unwilling to underwrite it.



# Financial Highlights

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## Funding the team

Overall expenditures for Harvard Stem Cell Institute in fiscal year 2011 were \$17.7 million, \$1M less than last year, primarily due to accounting for sponsored research. Total spending on non-sponsored research and operations rose 1%, from \$12.3M in FY10 to \$12.5M in FY11.

FY11 targeted research spending increased 7% from FY10 to \$6.1M. Notable new programs included junior faculty projects focused on regenerative therapeutics, a joint research Fellow at the Broad Institute, and a new joint funding program at MGH.

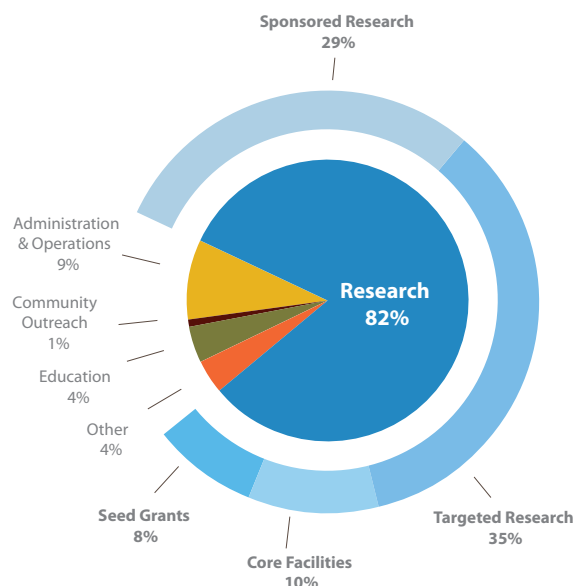
Support for Seed Grants in FY11 was \$460K lower this year due to timing of award payments, but overall program funding remains level and HSCI continues to budget \$1.8M annually to fund ten new grants per year.

Overall support of core facilities declined slightly due to timing of payments. Funding for the Therapeutic Screening Center as well as the flow cytometry cores stayed level, while significant funding was allocated to the iPS Core to become fully operational in its new location.

Education and training support of \$800,000 included graduate and postdoctoral training programs, as well as the undergraduate internship program which was able to accommodate a total of 47 students this summer thanks to directed philanthropic support.

HSCI managed sponsored research expenditures of \$5.2M in FY11, down from \$6.3M in FY10. This decline is due to the fact that HSCI has started to transfer projects to the Department of Stem Cell and Regenerative Biology at Harvard where many of its core faculty now reside. Several of the Therapeutic Screening Center grants will transition in FY12 so sponsored dollars and associated overhead recovery on HSCI's books will be significantly less going forward, although we expect the volume of sponsored activity in our faculty's labs to continue level if not increase.

2011 Expenditures as a % of Total Budget



## Supporters of HSCI

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### Thank you for sharing with us.

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/2011AR/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 insurmountable health problems.

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

