Human Stem Cells Policy
MUSC Research Using Human Stem Cells Policy

- Review and approval of human pluripotent stem cell research by the Stem Cell Research Oversight (SCRO) committee
- Policy becomes effective **July 1, 2017**
- Requires:
  - Registration by **July 1, 2017**
  - Training completed: CITI Human Stem Cell Research modules
MUSC Research Using Human Stem Cells Policy

**WHAT?**
Examples of types of activities requiring registration:
• Storing, purchasing, making, using, receiving from a collaborator etc. of:
  o Human embryonic stem cells
  o Human induced pluripotent stem cells (iPSC)
  o Human adult stem cells with pluripotent capabilities
  o Human gametes
  o Human embryos

**HOW?**
SCRO registration form in RedCap:
[https://is.gd/MUSC_SCRO_form](https://is.gd/MUSC_SCRO_form)
MUSC Research Using Human Stem Cells Policy

For more information:

Yashmin Karten, PhD
karteny@musc.edu
792-6521

research.musc.edu/ori/scro
College of Medicine
Research Strategic Plan Overview

Changing What’s Possible | MUSC.edu
2011-2015 College of Medicine Research Strategic Plan

Areas identified for investment: Genetics, Public Health Science, Infrastructure, Translational Research

Outcomes: Center for Genomic Medicine, Department of Public Health Sciences, Infrastructure Committees (Space, Cores….) Bridge Funding and COMETS Programs
College of Medicine 2016-2017
Research Strategic Plan Committee

Steve Carroll
Craig Crosson
Michael de Arellano
Steve Duncan
Gary Gilkeson
Phil Howe
Donna Johnson
Peter Kalivas
Donna Kern

Zihai Li
Jimmy McElligott
Jennifer Nall
Sunil Patel
Don Rockey
Lynn Schnapp
Ken Tew
Tom Uhde
John Vena
2017 College of Medicine Research Strategic Plan

Goal:
Develop an integrated program across the College around the theme of **Healthy Aging/Longevity**.

New Program Areas:
Age-Related Neurodegeneration
Aging and Metabolic Disease (Digestive/Endocrine)

Infrastructure Development:
Genomic and Precision Medicine
Microbiome
Age-Related Neurodegeneration

Goals:
Development of three thematic areas:
• Building toward an Alzheimer's Disease Research Center (ADRC)
• Stress and Aging
• Sensory Physiology and Aging

Areas of investment:
• Recruitment of senior faculty to lead programs
• Development of neuroimaging technologies
• Developing community outreach
Aging and Metabolic Disease

**Goals:**

Development of thematic areas:
- Building toward Digestive Disease Center
- Building toward of a Diabetes/Obesity Center
- Redox Homeostasis

**Areas of Investment:**
- Recruitment of senior faculty to lead programs
- Development of core resources
- Increased collaborative initiatives
Genomic and Precision Medicine

**Goal:**
To expand and develop genomics and precision medicine across the College and overall health care enterprise.

**Areas of Investment:**
- Continued recruitment of individuals employing genomics in their research
- Promoting genomics and precision medicine across the College
- Increased informatics infrastructure
Microbiome

Goal:
To transforming medicine and healthcare by personalizing treatments through understanding of host-microbiome interactions and integration of health data on individuals and communities.

Areas of Investment:
• Build critical mass of microbiome researchers
• Establish Microbiome Core
  • High-throughput microbiome assays
  • Computational infrastructure for screening of clinical specimens
• Develop informatics resources
DDBS Faculty

Change in Department Leadership (Interim chair August 2015, Permanent chair January 2017)
6 Professor, 6 Associate Professor, 1 Assistant Professor, 1 Research Associate Professor
3 of the 6 full professors are SmartState® Endowed Chairs

DDBS Facilities

2\textsuperscript{nd} and 3\textsuperscript{rd} floor of the QE building, 2\textsuperscript{nd}, 3\textsuperscript{rd} and 4\textsuperscript{th} floor of the QF building, 4\textsuperscript{th} and 5\textsuperscript{th} floor of the Discovery Building

Drug

SmartState® Center for Cancer Drug Discovery
Drug Design and Synthesis Core (hit-to-lead and lead optimization services)
COBRE Targeted Cancer Therapeutics synthetic core (synthesis support for COBRE projects)
MUSC Cell and Molecular Imaging Shared Resource

DDBS Graduate Students

Ph.D. in one of 2 tracks: \textit{Bioorganic and Medicinal Chemistry} or \textit{Cell Death, Injury and Regeneration}
15 current students (2 M.D./Ph.D., 1 D.M.D./Ph.D., 12 Ph.D.)
T32 training program: Training in Bioenergetics, Oxidative Stress and Metabolic Syndrome

2017 Graduates

Jessie McClure, Ph.D.
Development of Class I and sub-Class I Selective Inhibitors of Lysine Deacylases: Implications for Inflammation and Hematologic Malignancies

Craig Kutz, M.D./Ph.D.
Design of Novel Histone Demethylase Inhibitors as Drug Candidates to Prevent Cardiac Ischemia-Reperfusion Injury

Ryan Whitaker, M.D./Ph.D.
Stimulation of Mitochondrial Biogenesis Through Induction of cGMP Promotes Recovery of Mitochondrial and Renal Function Following Acute Kidney Injury
<table>
<thead>
<tr>
<th>Principal Investigator</th>
<th>Title</th>
<th>Funding Agency</th>
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<tr>
<td>Eduardo Maldonado</td>
<td>VDAC Regulation of Warburg Metabolism in Hepatocarcinoma</td>
<td>NIH/NCI (R01)</td>
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<tr>
<td>Hereward Wimborne</td>
<td>Aldehyde Dehydrogenase 2 as a Drug Target for Cholestatic Liver Fibrosis</td>
<td>NIH (F31)</td>
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<tr>
<td>James Chou</td>
<td>Novel Lysine Deacyetylase 6 HSP Domain Inhibitors</td>
<td>NIH/NCI (R01)</td>
</tr>
<tr>
<td>John Lemasters</td>
<td>Mechanisms of I/R Injury to Hepatocytes</td>
<td>NIH/NIDDK (R01)</td>
</tr>
<tr>
<td>John Lemasters</td>
<td>Training in Bioenergetics, Oxidative Stress &amp; Metabolic Syndrome</td>
<td>NIH/NIDDK (T32)</td>
</tr>
<tr>
<td>John Lemasters</td>
<td>Small Molecule Screening Against VDAC</td>
<td>NIH/NIAAA (R21)</td>
</tr>
<tr>
<td>John Lemasters</td>
<td>VDAC in Ethanol and Aldehyde-Induced Hepatotoxicity</td>
<td>NIH/NIAAA (R01)</td>
</tr>
<tr>
<td>John Lemasters</td>
<td>Liver Preservation for Transplantation</td>
<td>NIH/NIAAA (R56)</td>
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<tr>
<td>John Lemasters</td>
<td>Confocal/Multiphoton Microscope Upgrade</td>
<td>NIH/NIGMS (S10DO1)</td>
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<tr>
<td>Patrick M. Woster</td>
<td>Identification of novel spermatozoan oxidase (SMOX) inhibitors as probes for an emerging chemoprevention target</td>
<td>NIH/NCI (R01)</td>
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<tr>
<td>Patrick M. Woster</td>
<td>Center for Targeted Therapeutics Drug Design and Synthesis Core</td>
<td>NIH/NCI (P20)</td>
</tr>
<tr>
<td>Patrick M. Woster</td>
<td>Novel Epigenetic Reprogramming To Inhibit or Reverse EMT in Lung Cancer</td>
<td>DOD Concept</td>
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<tr>
<td>Rick G. Schnellmann</td>
<td>Mitochondrial Biogenesis Promotes Recovery from Oxidant Injury</td>
<td>NIH/NIGMS (R01)</td>
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<tr>
<td>Robert Cameron</td>
<td>Elucidation of Beta-2 Adrenergic Receptor Pathways Leading to Mitochondrial Biogenesis</td>
<td>NIH/NIDDK (F30)</td>
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<tr>
<td>Ryan Whitaker</td>
<td>Stimulation of Mitochondrial Biogenesis with PDE5 Inhibitors</td>
<td>NIH/NIDDK (F30)</td>
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<tr>
<td>Sherine Chan</td>
<td>A Genomic and Biochemical Analysis of the Causes of Mitochondrial Disease</td>
<td>NIH/NIGMS (R01)</td>
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<tr>
<td>Sherine Chan</td>
<td>Adverse Reactions of Stimulants on Embryonic Development and Energetics</td>
<td>NIH/NIDDK (R21)</td>
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<tr>
<td>Sherine Chan</td>
<td>A model of Alpers Syndrome</td>
<td>NIH/HD (R21)</td>
</tr>
<tr>
<td>Mark Hamann</td>
<td>HCV Leads from Endangered Plant Endophytes</td>
<td>NIH/NIAID (R01)</td>
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<td>2016 Foundation/Industry Awards ($337,023)</td>
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<tr>
<td>Rick G. Schnellmann</td>
<td>Urinary Biomarkers of Renal Mitochondrial Dysfunction</td>
<td>Mitogen, Inc.</td>
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<tr>
<td>Rick G. Schnellmann</td>
<td>Formoterol, An FDA-Approved Drug, Stimulates Mitochondrial Biogenesis as a Novel Therapeutic</td>
<td>SC Spinal Cord Injury Fund</td>
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<tr>
<td>Mark Hamann</td>
<td>Natural Product Replacements to Current Food Preservatives</td>
<td>Kraft Foods</td>
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<tr>
<td></td>
<td>2016 SmartState Funding ($322,389)</td>
<td></td>
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<tr>
<td>Patrick M. Woster</td>
<td>Smart State Center for Cancer Drug Discovery</td>
<td>SC Centers for Economic Excellence</td>
</tr>
<tr>
<td>John Lemasters</td>
<td>Glaxo Smith Kline Smart State Endowed Chair</td>
<td>SC Centers for Economic Excellence/MUSC Foundation</td>
</tr>
<tr>
<td>Mark Hamann</td>
<td>Charles &amp; Carol Cooper Smart State Endowed Chair</td>
<td>SC Centers for Economic Excellence/MUSC Foundation</td>
</tr>
<tr>
<td>Patrick M. Woster</td>
<td>Smart State Endowed Chair in Medicinal Chemistry</td>
<td>SC Centers for Economic Excellence</td>
</tr>
<tr>
<td>Patrick M. Woster</td>
<td>SmartState Center for Medication Safety</td>
<td>SC Centers for Economic Excellence</td>
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<tr>
<td></td>
<td>2016 MUSC Internal Funding ($95,618)</td>
<td></td>
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<tr>
<td>Monika Goos</td>
<td>Modifiers of Cyst Progression</td>
<td>SCIR/MUSC</td>
</tr>
<tr>
<td>Eduardo Maldonado</td>
<td>VDAC Opening Drugs to Induce Mitochondrial Dysfunction and Cancer Cell Death</td>
<td>SCIR/MUSC</td>
</tr>
<tr>
<td>James Chou/Sherine Chan</td>
<td>The development of vitamin K analog for the treatment of Parkinson’s disease</td>
<td>SCIR/MUSC</td>
</tr>
<tr>
<td>James Chou</td>
<td>Redox-targeted compound, E61, overcomes proteasome inhibitor resistance in Multiple Myeloma</td>
<td>SCIR/MUSC</td>
</tr>
<tr>
<td>Patrick M. Woster</td>
<td>Epigenetic Modulators for the Treatment of Sickle Cell Disease</td>
<td>SCIR UL1R00008</td>
</tr>
</tbody>
</table>

**DDBS Factoids**

- **14 full-time faculty**
- **3 SmartState® Endowed Chairs**
  - (4 of 15 supported by NIH F30/F31 awards)

$4,558,983 in external grant funds (an increase of $1,079,061 over 2015 (31.0% increase) ($4,219,983 post RGS – 21.3% increase)

$301,427.00 per FTE

- **40 peer-reviewed publications in 2015**
- **39 peer-reviewed publications in 2016**

2014-present: 24 disclosures of new intellectual property, 36 patent applications and 6 issued patents

- **5 start-up companies** (MitoChem, MitoHealth, Schnellgen, Inquisatex Epitherapeutics, Neuroene Therapeutics)
The “Demerger” and DDBS Strategic Planning

**Preamble**
The intent of this strategic plan is to capture the current position of the Department of Drug Discovery and Biomedical Sciences (DDBS) in the broad areas of research, education and department life, and to create specifically designed goals with sufficient relevance, insight and power to propel DDBS, its programs, and most importantly, its people to the next level of excellence. DDBS has enjoyed impressive successes over the past decade in the scope and the peer standing of its basic research programs, in interdisciplinary teaching and learning, and in the visibility and value of its programs inside and outside the university. **Through a strategic plan that sets achievable goals and ensures effectiveness by defining strategic priorities for quality improvement, the Department will be recognized as one of the finest within the Medical University of South Carolina, and among its peer departments at other universities.** We strive to realize these objectives through a plan that is focused and feasible, with explicit goals, prioritized actions, productive metrics, and enabling benchmarks and milestones.

**DDBS Mission Statement:**

The faculty of the Department of Drug Discovery and Biomedical Sciences shares a commitment to research and teaching excellence in drug discovery and the biomedical sciences that incorporates cutting edge scientific advances and teaching methodologies.

**DDBS Vision Statement:**

To lead innovation in drug discovery and biomedical sciences through creation, dissemination and translation of new knowledge.
Challenges 2017

1. NIH funding rates will decrease and most funds will shift to clinical projects; student stipends will increase while funding agencies will stop allowing stipends in grants.
2. Public interest in health research topics will change, as will topics that are the focus of NIH; student expectations are changing and the jobs they are applying for will require additional skill sets.
3. The visibility of the department and college should be improved at the university, national and international level. Despite significant successes, DDBS does not get the attention or visibility it deserves because we are not telling our story well enough.
4. DDBS has no stated clear goals to pursue from an institutional perspective. Goals for growth, funding and enhanced intra- and inter-institutional collaboration need to be set.

Strategic Initiatives 2017-2022

1. Increase research funding in the department by 15%.
2. Hire 3 young faculty members, first in pharmaceutics/drug delivery/nanotechnology, then in research areas that complement existing strengths.
3. Upgrade and expand research infrastructure (new building) and make drug discovery resources available to MUSC faculty.
4. Work with the Department of Clinical Pharmacy and Outcomes Sciences to develop an integrated Pharm.D. curriculum.
5. Major revision of the DDBS graduate curriculum and submission of a successful NIH T32 training grant in drug discovery.
The Department of Drug Discovery and Biomedical Sciences at the Medical University of South Carolina (MUSC) invites applications for a 12-month, tenure track position at the Assistant or Associate Professor level. Applicants should possess a Ph.D. degree in medicinal or bioorganic chemistry, pharmaceutics, drug delivery or a related discipline, as well as relevant postdoctoral experience. Preference will be given to applicants with expertise in pharmaceutics, nanotechnology or gene/drug delivery, but outstanding candidates from all areas are encouraged to apply. The successful applicant is expected to develop a vigorous, externally funded research program, and to participate in professional and graduate education. The position includes excellent compensation, a competitive start-up package and comprehensive benefits. MUSC is a research-intensive university with more than $250 million in external funding, and the College of Pharmacy ranks in the top 20 in NIH funding among US schools of pharmacy. The MUSC campus provides an outstanding environment for collaboration, and is home to the NCI-designated Hollings Cancer Center. MUSC also features a number of shared resource centers, including drug design and synthesis, NMR, biomedical imaging, genomics, mass spectroscopy and structural biology. Application review will begin June 1, 2017 and will continue until the position is filled. Applicants should send a CV and research plan, and arrange to have letters from 3 professional references sent in PDF format to Marianne Rogers at rogersmr@musc.edu.

The Medical University of South Carolina is an Affirmative Action/Equal Opportunity Employer. Candidates from underrepresented groups are encouraged to apply.
Timeline

• Federal Policy for the Protection of Human Subjects Originally promulgated as a Common Rule in 1991
• Advanced Notice of Proposed Rulemaking (ANPRM) in July 2011
• The agency published a more modest set of proposed changes via the Notice of Proposed Rulemaking (NPRM) in September 2015
• Final Rule Publication Date: January 19, 2017
• Final Rule Effective Date: January 19, 2018
• Compliance Date for most provisions: January 19, 2018
• Compliance Date for single IRB: January 20, 2020
Some Major Provisions

- Informed Consent
- Single IRB for Domestic Cooperative Research (with exceptions)
- Broad Consent as an option for Secondary Research
- Wider Portfolio of Exemptions
- Reduction of Continuing Review
- Posting of Consent Forms
Interagency Consultation for harmonization of guidance 101j

- Transition Provisions [Secretary’s Advisory Committee on Human Research Protections (SACHRP)]
- Guidance is provided regarding the Newborn Screening Saves Lives Reauthorization Act of 2014
- 102(e) The definition of identifiability could be modified through consultation process and guidance
- 102(j) (minimal risk) guidance may be issued on the issue and list of minimal risk activities
- 102(l)(4) public health surveillance guidance will be useful
- Program improvement’s scope could be better addressed through other means [than regulation]
- The federalwide assurance will be modified to eliminate the checked boxes, remove the submission of the IRB roster, and remove the listing of an IRB
- An Exemption Tool could be developed
- 104(d)(3) The exemption for Benign Behavioral Interventions: guidance would be helpful [SACHRP]
- A Resource for listing exempt studies under 104(d)(5) will be created
- Guidance is provided in the preamble on clinical data registries in response to section 511 of MACRA
- The Expedited Review List will be revised [SACHRP]
- 111(a)(7) HHS Secretary will issue guidance on protecting subject privacy and confidentiality
- 114 Cooperative research: guidance will need to be developed
- 116(a)(5)—Informed Consent: “Key information” and how it will operate may be discussed in guidance
- 116(d)—Broad consent template and guidance may be developed [SACHRP]
- Site for posting consent forms will be created
Have we changed our policies?

• Not yet!
• Holding Pattern
  • Currently being reviewed by Administration
• Possible new effective date?
NIH Policy on Reporting Preprints & Other Interim Research Products

Goal: Speed dissemination and enhance scientific rigor

Effective Date: May 25, 2017
Definition of Interim Research Products

Complete, public research products that are not final:

1) Preprints: a complete and public draft of a scientific document; typically *unreviewed manuscripts* written in the style of a peer-reviewed journal article

2) Preregistered Protocols: Publicly-available research protocols or methods

• Uses: References, Biosketches, Progress Report Publications, Research Performance Progress Reports (RPPRs)
How to Cite Interim Research Products

• Obtain a Digital Object Identifier (DOI) by depositing your preprint in a “best practice, ethical, secure” server
• Ensure that your work will be protected by copyright law
• The NIH “does not want people to cite documents posted to personal or laboratory webpages”
• In the text of the document:
  • Acknowledge NIH funding (NIH Grants Policy Statement Chapter 8.2.1)
  • Clearly state that the work is not peer-reviewed
  • Declare any competing interests
Before You Proceed, Consider Consequences...

- NIH awardees are NOT required to create or cite interim products.
- Upon acceptance for publication, many journals require that authors remove preprints from electronic servers:
  - NONE of the free archiving preprint servers allow for removal/take down of deposited preprints.
- Guidelines and requirements from journals are likely to continue changing as this policy is implemented.
- Ensure all your collaborators agree to submitting preprint.
- The peer reviewed system remains the best practice.
Intellectual Property Ramifications

Remember: Before Public Disclosure

- Submit a Record of Invention form to the MUSC Foundation for Research Development (FRD)
- MUSC Intellectual Property (IP) Policy: Compliance with IP policy is condition of employment and/or resource utilization

http://academicdepartments.musc.edu/frd/inventors/policy
Questions

General:
Carla Stipe Frichtel, Director, Office of Research Development
  • 2-0869; frichte@musc.edu

Disclosure and Intellectual Property:
Michael Rusnak, Executive Director, Foundation for Research Development
  • 2-1900; rusnak@musc.edu

Citations:
Teri Lynn Herbert, Associate Professor, MUSC Library
  • 2-1370; herbertl@musc.edu
Recent Successes with Technology Advancement

MUSC Foundation for Research Development

MUSC Town Hall

June 7, 2017
Technology Maturation

Existing Programs

- SCRA
- CMDi
- FRD Prototyping Fund
- TAC
- PriMed
- Technology Development Fund
- SBIR-STTR
- IAN
Granting Results

**FY16 FRD Value Added**

- $1.87 MM Total
  - $600K Licensing
  - $696K Tech Development
  - $575K Other
  - $456K to MUSC
  - $144K to FRD
  - $423K to MUSC
  - 273K to Startups
  - $575K Other

- $1.45 MM to MUSC

**YTD FY17 FRD Value Added**

- $2.66 MM Total
  - $305K Licensing
  - $1.41 MM Tech Development
  - $943K Other
  - $191K to MUSC
  - $114K to FRD
  - $443K to MUSC
  - 967K to Startups
  - $943K Other

- $1.58 MM to MUSC
FRD Value Added History 2010-2017

Notes:
• Tech Development split out beginning 2016, in previous years Tech Development and Other is combined
• 2012 includes $2.32MM licensing from Immunologix exit
FRD Cumulative Economic Impact

- $8.0MM Licensing
- $20.5MM SBIR/STTR
- >$700MM Exits

- $109 Other
- $111MM Taligen
- $480MM Micrus

>$728MM Economic Impact

$15.4MM 1995–2016 MUSC Funding to FRD
Office of Clinical Research (OCR)

• The OCR partners with study teams, research administration offices, Epic Research, and MUSC Health to ensure research billing compliance and support clinical research operations and financial performance.

• **All** studies involving human subjects must be submitted to the OCR
  
  • Studies with billable services will require Prospective Reimbursement Analysis (PRA)
  
  • Parallel processing – submit early via SPARCRequest

• Visit [horseshoe.musc.edu/research/ocr](https://horseshoe.musc.edu/research/ocr) for more information

• Faculty Presentations and Study Team Trainings
OCR PRA Team: 84 Studies Received to Date

PRA Services by Status (March-June 2017)

<table>
<thead>
<tr>
<th>Service</th>
<th># of Services</th>
</tr>
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<tr>
<td>Active</td>
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<tr>
<td>Awaiting Requestor Response</td>
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<td>Complete</td>
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<td>On Hold</td>
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<tr>
<td>Withdrawn</td>
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</table>
External Affiliate NetID Metrics

• For Non-MUSC research personnel who, for study purposes, needs access to REDCap, eIRB, SPARCRequest, and CITI training systems only.
  • Visit SCTR Research Toolkit “Study Conduct” tab for info
  • [http://academicdepartments.musc.edu/sctr/tools_links/toolkit_conduct.html](http://academicdepartments.musc.edu/sctr/tools_links/toolkit_conduct.html)

• 1st year Implementation (6/1/16 – 6/1/17)
  • 526 External Affiliate NetIDs created for 93 different protocols
  • Saving researchers $34,190
    • $65 background check avoided
Association of Clinical Research Professionals (ACRP) Training Metrics

• Free, online eLearning library for any MUSC employee provided by SCTR
  • Many courses offer CEUs
  • Visit SCTR Education and Training website for info
    • [http://academicdepartments.musc.edu/sctr/education_training/acrp](http://academicdepartments.musc.edu/sctr/education_training/acrp)

• SCTR Site License Implemented 2/1/16
  • 220 registered users
  • 318 courses completed
  • Approximate $47,770 value
    • $150 each course if purchased individually