2013
Annual Report
for the
Clinical Research Center

Stavroula Osganian, MD, ScD, MPH
Co-Chief

Ellis Neufeld, MD, PhD
Co-Chief

An interdisciplinary center fostering excellence in clinical research at Boston Children’s Hospital
I will support world class clinical research until every child is well.
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We are delighted to share with you the FY2013 annual report of The Clinical Research Center (CRC) at Boston Children’s Hospital. Over the past year, we have worked to effectively integrate our teams, work more efficiently and seamlessly with investigators, and successfully meet the goals of our Center and the mission of our Institution. The Center directly supports a core mission of the hospital, to be the leading source of research and discovery and make significant contributions to the improvement of children’s health through CRC’s four primary areas of focus:

1) clinical research methodology  
2) assistance on research project initiation and implementation  
3) resources for the conduct of clinical research visits and ancillary services and  
4) education on research methods and practice.

This annual report summarizes key accomplishments of the Center and its faculty and staff, who strive to support the mission of the Center and the Hospital. CRC faculty and center staff serve as institutional leaders, mentors, teachers and scientific collaborators. They provide critical expertise and support to junior faculty beginning their research careers, as well as to senior faculty more established in their fields of study. They work to translate innovative research on the diagnosis, prevention and treatment of childhood illnesses into practice with high standards of quality and scientific rigor. They also continue to build partnerships and collaborative relationships with departments throughout the hospital.

Our key accomplishments and activities for FY2013 include:

- Services provided for 243 new requests for assistance from 170 researchers broadly distributed across the various Departments and Divisions of the Hospital.
- Successful collaborations with several BCH investigators on 128 funded projects totaling $1,219,668 in direct support of CRC activities (representing a 27% increase in dollars of support compared to projects funded in FY2012), with a large proportion (82%) of this funding coming from extramural grants and contracts ($991,140).
- 72 peer-reviewed collaborative publications that were co-authored by CRC staff.
- Strong support from Departments and Divisions for shared faculty and staff with funding totaling $1,321,223 in FY2013, a 1% increase from FY2012.
- Continued demand for clinical research education, with 11 courses or seminar offerings drawing 1,388 registrants, resulting in a 14% increase from FY2012.
- The launch of the first Clinical Research Grand Rounds with 10 investigators presenting their clinical research on a variety of topics.
- Successful continuation of our triage and pilot navigation system with four Departments or Divisions and 85 project requests handled by this new mechanism promote strategic use of clinical research resources.
- Growing demand for biostatisticians, study coordinators and research specialists resulting in 16 departmental partnerships with CRC.
- Growth in Center faculty and staff with 5 new hires.
- Successful renewal and ongoing integration with the Harvard Catalyst Clinical and Translational Sciences Center, which is in its sixth year of funding and is now funded through 2018.
As the 2013 fiscal year closes, we would like to recognize the support from our collaborators and take pride in the work we have accomplished together. During the coming year, we look forward to achieving our FY14 strategic goals, strengthening our teams, and working together to meet the needs of the BCH research community in the context of the national fiscal challenges facing research and healthcare.

Ellis and Voula  
Co-Chiefs, Clinical Research Center  

Our Thanks: We wish to thank Dr. Mandell, CEO, Ms. Fenwick, COO and President, and the hospital leadership for the continued financial support provided to the Center. This support has made it possible for the Center to provide valuable assistance on hundreds of requests from the BCH research community and achieve many of its accomplishments.

“Coming together is a beginning; keeping together is progress; working together is success.”  
(Henry Ford)
Center Leadership and Administration

Stavroula Osganian, MD, ScD, MPH
Co-Chief, Clinical Research Center

Ellis Neufeld, MD, PhD
Co-Chief, Clinical Research Center

Andrew Dauber MD, MMSc
Assistant Medical Director, CTSU Core

Robert Fuhlbrigge MD, PhD
Medical Director, CTSU Core

Colette Hendricks, MSW, MBA
Operations Director, Clinical Research Center

Jenifer Lightdale MD, MPH
Director, Education Core
Michael Monuteaux, ScD
Scientific Director, Design & Analysis Core

Nicolle Quinn, MS, RD, LDN
Nutrition Manager, CTSU Core

Ellen Godfrey McCarthy, MPH
Manager, Development & Operations Core

Cindy Williams, MSN, RN, PNP, NE-BC
Nurse Director, CTSU Core

Affiliated Faculty

Jon Bickel, MD, MS
Director, Clinical Research Information Technology
Center Vision, Mission, and Goals

Vision
To be a state-of-the-art, dynamic, and integrated clinical research program that provides leadership, infrastructure, and resources to support patient-centered studies.

Mission
The mission of The Clinical Research Center is to promote excellence and quality in clinical research methods, to encourage best practices in clinical study implementation, and to empower investigators to conduct high quality independent research.

Goals

- **Scientific Leadership**: To provide scientific leadership and expertise on the design, conduct, analysis, and reporting of clinical research studies.
- **Education and Training**: To provide education, training and mentoring to the clinical researcher community on clinical research methods and study implementation best practices.
- **Scientific Collaboration and Consultation**: To serve as active scientific collaborators with hospital research initiatives and provide consultation services and/or collaboration in support of the design, conduct, analysis and reporting of clinical research studies.
- **Research**: To conduct independent research that serves to innovate and advance methodology in statistical design and analysis, survey and data collection methods, electronic data capture methods, and clinical research information technology.
- **Integration**: To provide a formal interdisciplinary home that links faculty members from the Center with various Departments and Divisions throughout the Hospital.
- **Growth**: To assist Departments and Divisions throughout the Hospital in recruiting and mentoring faculty members who are engaged in clinical research.
- **Professional Development**: To support the professional development of our faculty and staff so that they may develop their skills and advance their careers.

Values
The Clinical Research Center is committed to ensuring standardized and consistent best practices in clinical research methods, study conduct, data integrity, and the design of ethical protocols for all human subjects.

The Clinical Research Center is committed to providing a positive and motivational work environment that fosters teamwork, mutual respect, integrity, responsiveness and concern for each project, with creative interaction at the interface between areas of science.

The Clinical Research Center is committed to fostering a culture of collaborative, interdisciplinary research of the highest quality, respecting the priorities of each contributing discipline while placing priority on excellence in the joint effort.
Organizational Structure, Staffing and Space

The CRC is an institutional Center, reporting to the CEO and COO, and organized into four functional teams: the Design & Analysis Team (DAC), Education Team, Development & Operations Team (DOC), and the Clinical & Translational Study Unit (CTSU). The teams interact closely with the affiliated Clinical Research Information Technology (CRIT) Team located in the Information Systems Department. In FY2013, the CRC included 57 faculty and professional staff and occupied approximately 7,000 square feet of space located at 21 Autumn Street as well as Pavilion 6.
**Financial Resources and Expenditures**

Institutional, extramural and departmental sources of support for the Center are shown in **Table 1**. There continues to be a substantial institutional commitment, totaling $2,216,757 million in FY2013, which facilitates the Center’s growth and visibility. Equally important has been the funding from departments, totaling $1,312,223, with $1,200,635 supporting jointly hired faculty and professional staff and $120,588 supporting work on specific research projects.

**Table 1: CRC Funding Sources and Expenses in FY13**

<table>
<thead>
<tr>
<th>Source</th>
<th>Budget</th>
<th>Expenses</th>
<th>Unexpended</th>
</tr>
</thead>
<tbody>
<tr>
<td>Institutional</td>
<td>$ 2,216,757</td>
<td>$ 1,476,730</td>
<td>$ 740,027</td>
</tr>
<tr>
<td>Department (Total)</td>
<td>$ 1,321,223</td>
<td>$ 1,321,223</td>
<td>-</td>
</tr>
<tr>
<td>Faculty</td>
<td>$ 1,200,635</td>
<td>$ 1,200,635</td>
<td>-</td>
</tr>
<tr>
<td>Projects^</td>
<td>$ 120,588</td>
<td>$ 120,558</td>
<td>-</td>
</tr>
<tr>
<td>CGRE</td>
<td>$ 245,389</td>
<td>$ 245,389</td>
<td>-</td>
</tr>
<tr>
<td>Harvard Catalyst*</td>
<td>$ 2,111,392</td>
<td>$ 1,874,148</td>
<td>$ 237,244</td>
</tr>
<tr>
<td>Biostats</td>
<td>$ 213,789</td>
<td>$ 192,120</td>
<td>$ 21,669</td>
</tr>
<tr>
<td>CTSU</td>
<td>$ 1,897,603</td>
<td>$ 1,682,028</td>
<td>$ 215,575</td>
</tr>
<tr>
<td>Extramural Projects</td>
<td>$ 1,014,443</td>
<td>$ 1,014,443</td>
<td>-</td>
</tr>
<tr>
<td>Intramural Projects</td>
<td>$ 138,454</td>
<td>$ 138,454</td>
<td>-</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>$ 7,047,658</td>
<td>$ 6,070,387</td>
<td>$ 977,271</td>
</tr>
</tbody>
</table>

*Grant Year 5 is from May 1, 2012 - April 30, 2013; total amount does not include Education or Bioinformatics funds

^Includes all project management services (CTSU: $50,621; Other: $69,966)

One hundred twenty-eight (128) investigator projects provided financial support to CRC faculty and staff in FY2013 (**Table 2 and Figure 1**). The majority (n=88) were funded through collaborations with researchers in the Department of Medicine. NIH was the primary source of funding for these projects (48%), followed by funding from foundations (24%) and Internal Awards (11%). Among the 48 NIH funded projects, 12 were funded through the R01 mechanism.

**Table 2: Funding Sources for 128 Investigator Projects supporting CRC Faculty and Staff**

<table>
<thead>
<tr>
<th>Source</th>
<th>Number</th>
<th>Dollars</th>
<th>Percent</th>
</tr>
</thead>
<tbody>
<tr>
<td>NIH</td>
<td>48</td>
<td>$ 584,242</td>
<td>48%</td>
</tr>
<tr>
<td>Other Federal</td>
<td>11</td>
<td>$ 72,936</td>
<td>6%</td>
</tr>
<tr>
<td>Foundation/Association</td>
<td>32</td>
<td>$ 289,709</td>
<td>24%</td>
</tr>
<tr>
<td>Industry</td>
<td>4</td>
<td>$ 44,253</td>
<td>4%</td>
</tr>
<tr>
<td>Department Projects</td>
<td>13</td>
<td>$ 90,074</td>
<td>7%</td>
</tr>
<tr>
<td>Internal Awards</td>
<td>20</td>
<td>$ 138,454</td>
<td>11%</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>128</td>
<td>$ 1,219,668</td>
<td>100%</td>
</tr>
</tbody>
</table>
Figure 1: Distribution of Departments for the 128 Investigator Projects supporting CRC Faculty and Staff

- Medicine: 68%
- Surgery: 6%
- Cardiology: 1%
- Psychology: 2%
- CRC: 1%
- Ophthalmology: 2%
- Patient Services: 3%
- Anesthesiology: 2%
- Dentistry: 1%
- Urology: 1%
- Neurology: 9%
- Radiology: 4%

Hem/Onc, 7%
GI/Nutrition, 12%
Endocrinology, 14%
Adolescent, 13%
Newborn Medicine, 2%
Dev Med, 6%
Critical Care, 1%
General Peds, 13%
Infectious Disease, 1%
Genetics, 2%
General Medicine, 5%
Nephrology, 1%
ED, 11%
Pulmonary, 2%
Respiratory Medicine, 1%
Gynecology, 1%
Immunology, 8%
Utilization of Services

The Clinical Research Center (CRC) provides a range of services to assist investigators in the design, conduct and analysis of their clinical research studies. Institutional support is provided for limited guidance to investigators who have unfunded studies, while greater involvement on projects is provided for collaborative relationships with investigators on funded projects.

During FY2013, the CRC received 243 new requests for assistance from 170 Boston Children’s Hospital faculty or staff as shown in Figure 1 and Figure 2. The requests are broadly distributed across several hospital departments and divisions with the majority of requests from investigators with appointments in Medicine (n=145) and within the Divisions of General Medicine (29) and GI/Nutrition (25), followed by the Departments of Radiology (33) and Neurology (16).

Of these 243 requests, 24 (10%) were supported by Harvard Catalyst, 151 (62%) supported by Children’s Departments and Divisions and 37 (15%) supported by grants and contracts. The 31 (13%) that had other sources of funding are now closed.

Figure 1: Distribution of 243 New Requests for Assistance
As in past years, the 170 investigators requesting assistance were more likely to be junior faculty (Figure 2). A substantial number were also non-faculty, hospital staff and trainees (residents and fellows).

Figure 2: Distribution of Rank of 170 Investigators requesting CRC services
Assistance with grant applications and success in securing funding for research is central to the mission of the CRC (Figures 3 and 4). In 2012, the CRC assisted with 69 applications submitted to a funding agency and of those submitted, 22 were funded (32% overall success rate). Among these, 33 applications were to federal agencies of which 7 were funded (21% success rate). The total direct costs and indirect costs of these awards across all years to BCH were $7,911,125 and $3,009,694 respectively, with $639,026 allocated to support CRC faculty and staff efforts on these projects.

Figure 3: Follow up of Grant Application Funding Success Rates from 2012

Figure 4: Grant Dollars Awarded to BCH and Amount of Dollars Allocated to CRC Staff
CRC User Feedback Survey

Annually, we survey BCH investigators and staff who collaborated with the CRC, requested services, or participated in its courses to gather valuable feedback on the impact of our assistance and educational efforts (Table 1). The overall response rate was 20% this year (162 out of 819). Among the 121 respondents who used CRC services (15% of those surveyed), 27 (22%) had worked in the past year with CRC faculty and staff on research projects through the Design and Analysis Core, 36 (30%) worked with the Development and Operations Core, 33 (27%) worked with the CTSU, 2 (2%) worked with the Behavioral Science Core and 54 (44%) had attended an education course. Of the respondents who used services, 3 were professors, 4 were associate professors, 15 were assistant professors, 16 were instructors and 83 were non-faculty.

In general, respondents reported fairly high levels of knowledge of how to design, implement, and report results of their research study after receiving CRC assistance or taking educational courses. The respondents felt that the CRC faculty and staff was knowledgeable, professional and responsive, and stated that they were highly likely to request assistance from the CRC again. These results were consistent across faculty rank and Core and reinforce the valuable contributions of the Center to the education, functional mentoring, and collaborative assistance provided to the BCH clinical research community.

Table 1: Responses to CRC Survey

<table>
<thead>
<tr>
<th>Statement</th>
<th>N</th>
<th>Mean (SD)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>The assistance I received from the CRC was valuable in terms of enhancing the quality of the science and methods of my study.</td>
<td>106</td>
<td>6.01(1.72)</td>
</tr>
<tr>
<td>After receiving assistance from the CRC, I feel more confident in designing a research study.</td>
<td>105</td>
<td>6.08(1.93)</td>
</tr>
<tr>
<td>After receiving assistance from the CRC, I feel more confident in conducting a research study.</td>
<td>104</td>
<td>6.00(1.88)</td>
</tr>
<tr>
<td>After receiving assistance from the CRC, I feel more confident in reporting results from a research study.</td>
<td>107</td>
<td>6.05(2.02)</td>
</tr>
<tr>
<td>The education I received was valuable in terms of enhancing the quality of the science and methods of my research.</td>
<td>51</td>
<td>5.75(1.40)</td>
</tr>
<tr>
<td>The education I received helped me to feel more confident in designing research studies.</td>
<td>51</td>
<td>5.96(1.56)</td>
</tr>
<tr>
<td>The education I received helped me to feel more confident in implementing research studies.</td>
<td>51</td>
<td>5.75(1.56)</td>
</tr>
<tr>
<td>The education I received helped me to feel more confident in reporting results of research studies.</td>
<td>51</td>
<td>5.53(1.76)</td>
</tr>
<tr>
<td>The faculty and staff of the CRC worked with me in a responsive and professional manner.</td>
<td>107</td>
<td>6.02(1.57)</td>
</tr>
<tr>
<td>The majority of the CRC staff with whom I interacted was knowledgeable about the services that I requested.</td>
<td>107</td>
<td>6.10(1.48)</td>
</tr>
<tr>
<td>I received assistance in a timely manner.</td>
<td>107</td>
<td>5.78(1.70)</td>
</tr>
<tr>
<td>I am likely to request assistance from the CRC in the future.</td>
<td>106</td>
<td>5.90(1.44)</td>
</tr>
</tbody>
</table>

*1 =very strongly disagree to 7 = very strongly agree
CRC Hospital-Supported Consult Service

This past year, the CRC continued to offer hospital-supported one-hour consults for investigators. In FY13, our staff has performed 102 consults with the majority coming from the Department of Medicine (37). See Figure 1. Requests range from the simple to the complex, such as scientific questions: “Can someone advise on available tools for delivering online surveys?”; or “I need advice on how to plan data and project management for an upcoming clinical trial”; or “Is there an expert on cost effectiveness studies who can explain some basic concepts as they relate to our study?” Additionally, out of the 102 consults, 18 (18%) requests have resulted in a scope of work/funded project utilizing CRC services. See Figure 2.

Figure 1: Distribution of 102 New Consults

Figure 2: Distribution of a Consult Resulting in a Scope of Work
Department Triage/Navigation Consults

For the past two years, a department triage service/navigation pilot has been available in collaboration with four departments/divisions: Emergency Medicine, Developmental Medicine, Endocrinology and Otolaryngology in order to provide a point-of-entry process to help investigators more efficiently access clinical research support services. A key aspect of this service is that investigators must consult with their department/division chiefs (or designees) regarding their research project, level of support for their clinical research project, and priority use of available resources for support. They must have approval to obtain institutional resources.

Benefits of this model include:

- Promotes support of high-quality and high-impact clinical research at BCH
- Promotes strategic use of institutional resources
- Reduces investigator time to obtain all necessary support from Cores
- Promotes shorter completion times for clinical research projects
- Enhances investigators' ability to publish and gain extramural funding

In FY13, the Navigation Service had 85 investigator requests, with 65% located in the ED, and have been triaged by a team of staff located in each of the pilot departments. **See Figure 3.** The team consists of a CRC project manager, CRC biostatistician and department/division clinical research designee who sit down with the investigator and discuss his/her research project. The team looks not only at feasibility (funding, space, staff), but also assesses whether the aims and hypotheses are clearly stated, what services are needed and if the research project is aligned with department/division strategic initiatives.

**Figure 3: Distribution of Navigation Consults**

![Pie chart showing the distribution of Navigation Consults: Emergency Department, 55; Otolaryngology, 13; Endocrinology, 15; Developmental Medicine, 2]
In addition, of the 85 consults that occurred in the departments, 94% (or 80) met the division/department strategic initiatives and were given the “green light” to move forward. See Figure 4.

**Figure 4: Number of Navigation Consults Meeting Objectives**

![Bar chart showing number of consults and objectives met for Emergency Medicine, Endocrinology, Developmental Medicine, and Otolaryngology.]

Preliminary data of this service supports the feasibility of having a department triage/navigation team by promoting efficient use of institutional resources, reducing investigator time to obtain support from all CRC Cores, and promoting alignment with departmental strategic goals.

In the coming years, the Center will examine whether this system promotes securing of extramural funding, enabling shorter completion times for the clinical research projects, and enhancement of investigators’ ability to publish.
Harvard Catalyst- The Harvard Clinical and Translational Science Center

“Harvard Catalyst” (the Harvard Clinical and Translational Science Center, or CTSC) is the name given to Harvard’s Clinical and Translational Science Awards (CTSA) program, the largest recipient among almost 60 NIH-sponsored nationwide.

With the effects of sequestration on the NIH budget, and uncertainties about future NIH funding, the early part of 2013 was spent in a holding pattern for new initiatives, as we awaited news of federal funding to continue the Harvard Catalyst program after its first five year grant ended in the spring. As the annual report year drew to a close, we received the good news that the Harvard Catalyst CTSC received a five year renewal of its NIH funding from the National Center for Advancing Translational Sciences (NCATS), from 2013 to 2018. NCATS’ vision for the national consortium of CTSAs has evolved from the original plans before 2013. In the new grant cycle, three areas of focus will be the highest priorities. These include (1) the resources to do clinical research better, faster and safer; (2) educational opportunities in clinical and translational research, in the form of masters programs through Harvard Medical School, as well as numerous courses and mentoring opportunities for trainees and junior faculty, and (3) an academic home through Harvard Catalyst Central, to facilitate interchange and collaboration across the Harvard programs and across CTSCs nationwide.

The CRC at Boston Children’s receives more than $2 million annually from Harvard Catalyst to support patient-oriented research; the largest share of support goes toward clinical research in the CTSU, but many other CRC activities are supported as well, including biostatistics, informatics, education, consultations, research coordinators, and training of young physician-scientists.

During this past year, the Child Health Committee of Harvard Catalyst, co-led by Dr. Neufeld and Dr. Elizabeth Goodman of Massachusetts General Hospital, which fosters activities in pediatrics and related fields across the Harvard Community and its affiliated hospitals, planned the inaugural Child Health Symposium for Harvard Catalyst. Several senior members of the Boston Children’s Faculty are involved in the Child Health Committee, including Drs. Jane Newburger, Associate Cardiologist-in-Chief, Mark Schuster, Chief, Division of General Pediatrics; Scott Pomeroy, Neurologist-in-Chief; Ken Mandl, Director of Children’s Hospital Informatics Program, and Linda van Marter of Neonatology.

In addition, the CTSU team will be working with Harvard Catalyst to implement a study scheduling system that will enable staff across all sites to study subject visits.

Institutional Initiative: End- 2- End Task Force

In the Spring of 2013, the hospital enlisted Ms. Carol Weinrib to lead a group of institutional research leaders, the Clinical Research Coordinating Committee, to address issues related to clinical research, propose policy changes, and articulate the need for resources. The process included interviewing several clinical researchers to gain their perspective on needs and issues, conducting an assessment of the current policies, guidelines, tool, templates, training materials and educational offerings to identify gaps, highlighting the areas of importance that needed attention and identifying areas that require additional resources. Key areas that were considered and addressed included infrastructure needed to support FDA-regulated trials, the creation of a CTBO (Clinical and Trials Budgeting Office), Training and Education, and Website organization. The CRC was represented by our Co-Chiefs, Drs. Osganian and Neufeld and administrator, Colette Hendricks. The CRC leadership appreciates the opportunity to be an integral part of this important process and looks forward to continuing to support the hospital in the implementation of this important endeavor.
Collaborations, Consultation and Integration: Investigate the feasibility of expanding CRC services, including the Navigation/Department Triage Service

Education and Training: Standardize study coordinator education and training

Finance: Meet estimated cost recovery goals

Growth: Increase Center faculty/staff partnerships with Departments or Divisions

Research: Disseminate and publish our best practices and methods in academic journals.

Quality/Performance Improvement: Establish a Center quality assurance plan and performance metrics

Culture: Promote excellence, communication, and transparency
Design and Analysis Core

**Staffing**

- Michael Monuteaux, ScD, Scientific Director
- Emily Blood, PhD, Senior Biostatistician
- Henry Feldman, PhD, Principal Biostatistician
- Peter Forbes, MA, Senior Biostatistician
- Lin Huang, PhD, Senior Biostatistician
- Jisun Jang, MA, Biostatistician
- Leslie Kalish, ScD, Principal Biostatistician
- Kush Kapur, PhD, Senior Biostatistician
- Heather Litman, PhD, Senior Biostatistician
- Tanya Logvinenko, PhD, Senior Biostatistician
- Carly Milliren, MPH, Biostatistician
- Paul Mitchell, MS, Senior Biostatistician
- Mihail Samnaliev, PhD, Health Economist
- Vishnudas Sarda, MPH, Statistical Programmer
- Georgios Sideridis, PhD, Survey Methodologist
- Caterina Stamoulis, PhD, Senior Biostatistician
- Matthew White, PhD, Senior Biostatistician
- Kathryn Williams, MS, Senior Biostatistician

The DAC expanded its statistical staff during FY13 with the arrival of six new members. We welcomed Drs. Kush Kapur, Heather Litman, Tanya Logvinenko, Georgios Sideridis, and Matthew White, and staff Kathryn Williams and Vishnudas Sarda.

**DAC Role**

The DAC provides assistance throughout the lifetime of a research project from design through implementation to analysis and reporting. During design, Core members work with investigators to plan studies, prepare and document statistical analyses, calculate statistical power and sample size, and contribute to the writing of proposals and protocols. During study implementation, Core members supervise and participate in database construction, randomization, data cleaning, quality control, and data and safety monitoring. In the analysis and reporting phase, the Core members perform both routine and innovative statistical analyses, collaborate with investigators to interpret results, and participate as co-authors to prepare scientific abstracts, presentations and journal articles.

**FY13 Highlights**

FY13 was another successful and productive year for the DAC. The Core demonstrated its commitment to exceptional service by consulting and collaborating on its greatest number of research projects, study protocols and publications to date. In addition, the faculty has independent research activities and prominent collaborative roles in several research projects. Selected highlights from this past year include:

- **Dr. Blood** received an Aerosmith Endowment Foundation grant in 2013 to conduct methodological research, titled *Methods for Predicting Sexual Intercourse from Affective States using Ecological Momentary Assessment Time-to-Event Data*.
- **Dr. Blood** was also promoted to Assistant Professor in the Division of Adolescent and Young Adult Medicine.
- **Dr. Samnaliev** delivered a platform presentation at The International Society of Pharmacoeconomics and Outcomes Research Annual International Meeting in New Orleans, titled, *Does comparative effectiveness research increase economic efficiency?* He also, in collaboration with colleagues from BCH, was awarded research funding from...

- **Dr. Stamoulis** became the site PI for an R01 grant titled *Cortical Connectivity and Stimulation in Human Epilepsy* in collaboration with Beth Israel Deaconess Medical Center. She also delivered a platform presentation titled, *Dynamic Temporal Scales in Long-Term Scalp Electroencephalograms from Epilepsy Patients* and chaired a session titled *Time-Frequency Analysis of Biosignals* at the 35th International Conference of the IEEE Engineering in Medicine and Biology Society, in Osaka Japan.

- **Dr. Stamoulis** was also nominated for the 2013 Mentor of the Year Award by the Harvard Graduate Women in Science and Engineering (HGWISE) for excellence in mentoring graduate women at Harvard University.

- **Dr. White** delivered a platform presentation at the International Biometric Society Eastern North American Region Conference in Orlando, titled, *Optimal design for diagnostic accuracy studies when the biomarker is subject to measurement error.*

- **Dr. Monuteaux** was granted membership to the Society of Pediatric Research.

DAC faculty and staff were especially active in educational activities throughout the hospital, including both the continuation of longstanding courses as well as new offerings. They play a prominent role in the educational offerings of the CRC. The annual course, *Introduction to Clinical Research*, a two-day overview for fellows and junior faculty, featured four hours of statistical material taught by Core faculty (Drs. Feldman, Monuteaux, Samnaliev and Sideridis). The introductory course generates demand for more advanced short courses, some of which are well-established while others are in development.

Dr. Kapur and Ms. Milliren taught *Introduction to Biostatistics with SPSS*, which combines eight lectures on elementary descriptive and inferential statistics with companion computer laboratory sessions and is available via webcast to address high demand. In addition, our staff taught two advanced courses - Dr. Huang taught *Introduction to Regression* with support from Mr. Forbes, and Dr. Blood taught *Introduction to Longitudinal Analysis*, also with assistance from Mr. Forbes.

In addition to the CRC short-course program, members of the Biostatistics Core regularly deliver hospital seminars and conduct training at national meetings. Below is a selection of other educational activities from FY13:

- **Dr. Feldman** delivered an 8-lecture series titled *Study Design in Clinical and Translational Research* under auspices of CRC Education Core between February and April, 2013, with 150 BCH trainees and faculty enrolled, and participated as faculty in the American Heart Association Ten-Day Seminar on Epidemiology and Prevention of Cardiovascular Disease, for the sixteenth consecutive year.

- The faculty of the Design and Analysis Core provided its sixth year of statistical mentorship to the Harvard Pediatric Health Services Fellowship at their bimonthly Works in Progress sessions.

- Several faculty members provided instruction in biostatistics and research methodology to clinical fellows and other trainees throughout the hospital.

An important organizing principle of CRC is the integration of its faculty with BCH Departments and Divisions, which provide academic appointments and/or funding and foster creative research collaborations between department-based investigators and CRC-based biostatisticians. Typically, the department supports a substantial fraction of a biostatistician’s effort, drawing on departmental funds and/or research grants. The purpose is to provide the methodologist with scientific focus and continuity of collegial contact, leading to a facilitative
environment for informal consulting, generating new ideas and attracting new grant support. The following departmental academic affiliations and/or funding sources are in place.

- Dr. Blood, Assistant Professor, Division of Adolescent and Young Adult Medicine
- Dr. Feldman, Associate Professor in the Division of Endocrinology
- Mr. Forbes, Nursing and Department of Psychiatry
- Dr. Huang, Instructor, Department of Otolaryngology
- Dr. Kalish, Associate Professor, Division of Infectious Diseases and Department of Orthopedics
- Dr. Kapur, Instructor, Department of Neurology
- Dr. Litman, Instructor, Division of Gastroenterology and Nutrition
- Dr. Logvinenko, Instructor, Department of Urology
- Ms. Milliren, Department of Adolescent Medicine
- Mr. Mitchell, Division of Gastroenterology and Nutrition
- Dr. Monuteaux, Assistant Professor, Division of Emergency Medicine
- Mr. Petty, General Pediatrics
- Dr. Sideridis, Instructor, Division of Developmental Medicine
- Dr. Stamoulis, Assistant Professor, Department of Radiology
- Dr. White, Instructor, Departments of Psychiatry and Cardiology
- Mrs. Williams, General Pediatrics and Department of Cardiology
Development and Operations Core

Staffing
Ellen Godfrey McCarthy, MPH, Manager
Edwin Anderson, BA, CCRP, Senior Clinical Research Coordinator
Mark Berry, MA, CCRC, Senior Clinical Research Specialist
Kimberly Chin, BA, Clinical Data Manager
Erica Denhoff, MPH, Clinical Research Specialist
Gloria Faboyede, MPH Clinical Research Coordinator
Hana Gragg, MPH, CHRC Project Manager
Brenda MacKinnon, RN Nurse Project Manager
Prajakta Mangeshkar, MSc, MS, Senior Clinical Research Specialist
Molly McDonald, BA Clinical Research Coordinator
Kara McLaughlin, BA Clinical Research Coordinator
Emily Pariseau, BA Clinical Research Coordinator
Bethany Trainor, BSN, RN Nurse Project Manager
Grace Yoon, CNNP, MSN, RN Nurse Project Manager

The Core welcomed the hires of Emily Pariseau, Kara McLaughlin, Molly McDonald, Hana Gragg, Erica Denhoff, Gloria Faboyede and Ellen McCarthy during this past fiscal year.

DOC Role
The DOC offers a full range of staff, tools and services to complement the Design and Analysis Core (DAC) and CTSU for full clinical research support. Focusing on project management, DOC staff work with investigators to plan the research study to address all scientific, clinical, logistical, data management and regulatory issues. The project management service places heavy emphasis on managing study milestones and timelines with an eye towards the Investigators’ future funding submissions and publications. Importantly, the DOC works in close collaboration with the DAC, and so has the benefit of involving CRC statisticians for planning, development and interim and final reporting.

FY13 Highlights
Enhancing and refining the project management services of the Center continued as a top priority in 2013. Andrew Dauber and Ellen McCarthy comprise the Project Management Triage Team that meets with investigators following their request for project management services.

After the initial assessment, The Triage Team assembles a project team tailored to the particular study and investigator’s needs. The triage team aims to ensure study success by:
1) Offering an integrated, interdisciplinary approach to project management
2) Best utilizing CRC resources for each investigator and project by assigning trained staff with relevant expertise and therapeutic experience
3) Providing excellent and timely study planning, implementation and execution, accomplished through coordination of all CRC activities including but not limited to statistical and data management support
4) Providing general clinical research training for new investigators to ensure protocol and regulatory compliance.

Harvard Catalyst provides significant support to the CRC for infrastructure and support of junior Investigators. Offering a range of 150 to 350 grant-funded hours of interdisciplinary clinical research expertise/workforce creates a clinical research environment where investigators are guided through best practices in clinical research and will learn in context. Research activities
are staged in the proper sequence while integrating the appropriate clinical research resources/services of the institution and the Harvard Catalyst.

Supporting Catalyst-funded research continued in FY13 as a major goal for the DOC. As in years past, the DOC offered project management and/or coordinator staffing to support over 50 different studies across clinical departments and divisions including Cardiology, Surgery, Immunology, Cardiac Surgery, Developmental Medicine, GI/Nutrition, Hematology/Oncology and Patient Services.

This past year, DOC staff continued to support new and ongoing NIH, foundation and industry sponsored trials including:

- Several industry-funded trials for Autism spectrum disorders conducted with the Division of Developmental Medicine, managed by Prajakta Mangeshkar and coordinated by Lucy Abernethy.
- Erica Denhoff, Kimberly Chin and Emily Pariseau also supported project management of research projects coordinated by the New Balance Foundation Obesity Prevention Center such as the POOL registry with Dr. Stavroula Osganian from the Division of General Pediatrics.
- Erica Denhoff provided project management for Prototyping a Mobile, Web-based Integrated Care Plan Application for Children with Complex Medical needs. This project is led by Dr. Jay Berry from the Division of General Pediatrics.
- Grace Yoon supported the Translational Research Program by leading the development and implementation of two gene-therapy studies of Childhood Cerebral Adrenoleukodystrophy (CCALD), Wiskott-Aldrich Syndrome (WAS) and Severe combined Immunodeficiency Diseases (SCID). She also played a key role in the Pediatric Myelodysplastic Syndrome (MDS) and Bone Marrow Failure Registry and Tissue Depository, working closely with Dr. Inga Hofmann from the Division of Hematology/Oncology, accruing subjects to meet targets for enrollment. In May 2013, this study was featured in Vector, BCH’s science and clinical innovation blog, which reported the finding that a unique set of mutations in a single gene may play a larger-than-realized role in a group of rare blood diseases.
- Brenda MacKinnon assisted with project management, recruitment and follow-up for gene-therapy studies of Childhood Cerebral Adrenoleukodystrophy (CCALD), Wiskott-Aldrich Syndrome (WAS) and Severe combined Immunodeficiency Diseases (SCID). She also assisted with start-up and implementation of a study of Preterm Erythropoietin Neuroprotection Trial (PENUT) in a cross-institutional effort with the Beth Israel Deaconess Hospital.
- Hana Gragg provided project management for a multicenter, randomized, blinded study entitled ‘Peanut Reactivity Reduced by Oral Tolerance in an anti-IgE Clinical Trial (PRROTECT)’. Funded by the Food Allergy Initiative, BCH is the lead institution. This is an example of a CRC full-service project that is cutting-edge science, requiring senior project management skills, a high degree of organization, leadership, professionalism and collegiality.
- Bethany Trainor supported research being conducted by Cardiovascular Surgery where she screened neonatal surgical cases for eligibility into a number of ongoing studies, streamlining and providing ‘program’ level support - a highly effective approach that enhanced recruitment, project management and regulatory reporting.
- Kimberly Chin supported over 25 different investigators by providing data management services over a wide range of projects. She assisted in the design and development of CRFs, development, deployment and maintenance of multiple REDCap databases, and provided data cleaning, dataset exports and DSMB reporting.
Working closely with the staff from the Education Core, DOC staff also played significant leadership roles and participated in CRC educational offerings including the Orientation for Study Coordinators. Prajakta Mangeshkar served as course director for this monthly orientation available to all new Boston Children’s clinical research staff. The Orientation included presentations by DOC staff including Obtaining Informed Consent/Assent: A Practical Approach, and Study Implementation & Timeline Development, Principles of Data Management, and Case Report Forms & Manuals of Operations.

In addition, Ms. Mangeshkar played a pivotal role in organizing and conducting Study Coordinator Rounds, a monthly workshop-style series aimed at providing additional networking and educational opportunities for BCH Study Coordinators. Study Coordinator Rounds aim to provide practical advice for conducting research at BCH and importantly, provides an appropriate forum for study coordinators to raise questions and gain a wider understanding of research.
Clinical & Translational Study Unit Core : A Harvard Catalyst Clinical Research Center

Staffing
Robert Fuhlbrigge, MD, PhD, Medical Director
Andrew Dauber, MD, MMSc, Assistant Medical Director
Cindy Williams, MSN, RN, PNP, NE-BC, Nurse Director
Nicolle Quinn, MS, RD, LDN, Nutrition Manager
Kyla Almeida, Staff Nurse
Kayla Arouchon, Diet Technician
Nathalee Chambers, Clinical Assistant
Ivy Dang, Staff Nurse
Laura Feloney, Lab Technician
Tina Kim, Administrative Associate
Stephanie Patriarca, Lab Technician
Joanna Sullivan, Staff Nurse
Courtney Silver, Staff Nurse
Chelsea Tyler, Administrative Associate

The Core welcomed the hires of Joanna Sullivan, Kayla Arouchon, Chelsea Tyler and Stephanie Patriarca during this past fiscal year.

CTSU Role
The CTSU provides state-of-the-art clinical research infrastructure for investigators in the design, initiation, conduct and reporting of clinical research with the goal of translating scientific knowledge into new therapies for pediatric conditions. Funding for the CTSU services is from the Harvard Catalyst, which is an affiliate-wide effort to facilitate laboratory-to-bedside translational research and to encourage researcher collaboration.

FY13 Highlights
A varied portfolio of studies have been supported this past year. Several examples of studies are:

Wanda Phipatanakul, MD
The Mouse Allergen Asthma Intervention Study
School Inner-City Asthma Study

The MAAIT team (The Mouse Allergen Asthma Intervention Study) conducts research visits in homes of asthmatic children. The team is studying whether mouse-targeted integrated pest management intervention is helpful in reducing the effects of asthma and mouse allergy in children ages 6 to 17 years old. This NIH-funded study is a unique collaboration between Boston Children’s Hospital, Columbia University and Johns Hopkins University. Participants in this study receive asthma management education, pest extermination services, air purifiers, and allergen-proof mattress covers. Within the Boston Public Schools, working with elementary school students, the SICAS Team (School Inner-City Asthma Study) works to determine the role of the environment and allergens in schools and homes in order to further understand the relationship between allergens and asthma. If there is a meaningful relationship, interventions targeting school classrooms and home environments could help many students with asthma.
Charles Berde, MD, PhD
Joseph Cravero, MD

Neosaxitoxin (NeoSTX), alone and in combination with bupivacaine as a prolonged-duration local anesthetic: A Phase I Investigator Initiated Dose Escalation Study

A Phase I study performed under an Investigator-Initiated FDA IND to further establish the systemic and local safety of escalating doses of NeoSTX via sub-cutaneous infiltration in healthy and awake young adult male human volunteer subjects. The primary aim of this Phase 1 study is to evaluate the systemic safety of a novel prolonged-duration local anesthetic, neosaxitoxin (NeoSTX), given by subcutaneous injection, either alone or in combination with the commonly used local anesthetic, bupivacaine.

Lynda Schneider, MD

A Randomized, Open Label Mechanistic Study in Atopic Dermatitis (AD) to Assess the Immunogenicity of Fluzone Intradermal and Intramuscular Vaccines

This is a multi-site, randomized, open-label, mechanistic study designed to compare the immune response in non-atopic and AD participants receiving a single dose of the 2012-2013 seasonal Fluzone Intradermal vaccine administered per label. The primary objective is to compare the antibody response of non-atopic controls and participants with moderate to severe AD, following administration per label of a single dose of the seasonal 2012-2013 Fluzone Intradermal vaccine. A secondary objective is to compare the immune response of AD participants receiving intradermal influenza vaccination to the response of AD participants receiving intramuscular influenza vaccination.

Basil Darras, MD

An Open-Label, Dose Escalation Study to Assess the Safety, Tolerability and Dose-Range Finding of Multiple Doses of ISIS 396443 Delivered Intrathecally to Patients with Spinal Muscular Atrophy (Phase 1/2a)

Spinal muscular atrophy (SMA) is an autosomal recessive neuromuscular disease characterized by degeneration of the motor neurons in the anterior horn of the spinal cord, resulting in atrophy of the voluntary muscles of the limbs and trunk. With an incidence of 1:6000 to 1:10,000 live births, it is the most common genetic cause of infant mortality, and a major cause of childhood morbidity due to weakness, in the U.S. The primary objective is to examine the safety and tolerability of multiple doses of ISIS 396443 administered intrathecally to patients with SMA. This study will test the safety, tolerability, and dose-range finding of multiple doses of ISIS 396443 administered as IT injections by lumbar puncture (LP). Three dose levels (3 mg, 6 mg, 9 mg) will be evaluated sequentially. Each dose level will be studied in a cohort of 8 subjects where all subjects will receive ISIS 396443. Within each cohort, no more than 4 subjects >7 years of age at screening will be enrolled.

The CTSU Nutrition Team has been involved in many protocols over the past year providing a variety of services including preparing double-blind, placebo-controlled food challenges, measuring resting energy expenditure via indirect calorimetry, measuring body composition with BIA, BodPod and skinfold measurements. The team has also analyzed more than 450 food recalls for several nutrition-related protocols. Starting in September, Boston Children’s Hospital CTSU began a collaboration with MGH and BWH to create a combined Harvard Catalyst nutrition research rotation for Dietetic Interns enrolled in the internships at these two sites. Over the next year more than 30 Dietetic Interns will spend a day under the mentorship of the Nutrition Research Manager at Boston Children’s Hospital.
FY13 was one of the busiest since the Harvard Catalyst started in 2008:
- The number of outpatient visits in Boston was consistent with FY12, while Waltham and the Infusion Unit almost doubled their visits. (Figure 1).
- Inpatient days have decreased over the past few years, but this year stayed relatively in-line with FY12 numbers (Figure 2).
- More than half of the investigators receiving services in FY13 were Junior Investigators.
- The CTSU had over 129 active protocols and worked with 20 different departments and divisions.

Figure 1. CTSU Visits Fiscal Years (FY) 2010 – 2013

Figure 2. CTSU Admissions Fiscal Years (FY) 2010-2013
**Education Core**

**Staffing**  
Jenifer Lightdale, MD, MPH, Director  
Erin Deneen, Education Program Coordinator

**Education Role**  
The Clinical Research Education Core aims to provide an integrated, useful, scientifically accurate, and comprehensive educational curriculum with course offerings that primarily aim to enhance quality of clinical research at Boston Children’s Hospital.

**FY13 Highlights**  
The Education Core continues to deliver a range of services to the Boston Children’s Hospital clinical research community, including:

- Developing, coordinating, and implementing courses and seminar series on research methods and topics for faculty, fellows, residents, and clinical research staff
- Offering didactic series on other advanced topics of interest to the broad research community at Boston Children’s Hospital
- Serving on hospital and university research education-related committees
- Developing Web-accessible best practices and educational tools for researchers

Under the direction of Dr. Jenifer R. Lightdale, the Education Core has consistently provided considerable educational opportunities for BCH staff and faculty involved in clinical research. Core achievements over the past year include the support of regular instruction in NIH-funded comprehensive software packages (i.e., REDCap and i2B2) that are becoming essential tools for academic clinical investigation in the new era of healthcare informatics and electronic data capture.

Total CRC course enrollment from 2006 to 2013 continues to reflect strong demand for CRC educational offerings from clinical research faculty and staff across the entire hospital (Table 1 and Figure 1). The Education Core continues to build upon its curriculum by adding 1-2 new courses each year (Table 2).

Our annual introductory clinical research and biostatistics courses are designed to teach junior investigators and fellows the fundamentals of study organization, analysis, and data management. In 2013, we collaborated with the Clinical Research Information Technology (CRIT) core to offer an Introduction to i2b2, a scalable computational framework designed to help accelerate case finding and hypothesis testing across and beyond all of the Harvard hospitals. We were also successful in working with the Harvard Catalyst to promote instruction in REDCap, an NIH sponsored, freely-available, GCP-compliant database package.

The Education Core also supported the continued availability of many advanced biostatistics courses introduced in recent years, including Introduction to Regression, Study Design in Clinical and Translational Research, and Introduction to Longitudinal Analysis. In addition, as part of a multi-disciplinary effort, the Education Core co-sponsored an encore production of a seminar series with Brigham and Women’s Hospital, entitled The Art and Anatomy of Writing a Career Development Grant. Agendas for our longstanding and regularly featured Introduction to Clinical Research and Orientation for Study Coordinators sessions are shown in the Appendix.
As a final accomplishment in FY 2013, the Education Core was proud to design and sponsor the **Clinical Research Center Grand Rounds**, a monthly session featuring speakers from across the hospital. The primary goal of the CRC’s Grand Rounds is to provide a series of exciting, state of the art sessions at the cutting edge of clinical and translational research, with the goal of educating and inspiring the clinical research community at Children’s. Each month, the participants have the opportunity to gain insight into developing, evaluating, and conducting their own research studies from a broad range of topics. Each session features a 45-minute presentation and 15 minutes of Q & A and is held on the third Tuesday of every month at noon in the Folkman Auditorium.

**Speakers from FY13**

- **Claudia Ordonez, MD** - Challenges and Opportunities in Drug Development in Orphan Diseases
- **Kenneth Mandl, MD, MPH** - Building the “App Store” for Health and Discovery
- **Greg Koski, MD, MPH** - On Being a PI: Principal Investigator or Practically Invisible?
- **Catherine Gordon, MD, MSc** – Lessons Learned from Pediatric Models of Malnutrition
- **Deborah Waber, PhD** - The NIH Toolbox: A New Tool for Behavioral Science and Clinical Trials
- **Ellis Neufeld, MD, PhD** - Get Ready—The FDA is Coming to Audit: Lessons Learned
- **Cara B. Ebbeling, PhD** - Dietary Interventions for Obesity: Paying Attention to Treatment Fidelity in RCTs
- **Wanda Phipatanakul, MD, MS** - Urban Environmental Exposures and the Asthma/Allergy Epidemic: Working with the Community to Make a Difference
- **Susanna Y. Huh, MD, MPH** - Cesarean Delivery: A Risk Factor For Obesity?
- **Mark Schuster, MD, PhD and Laura Bogart, PhD** - The SNaX Study: A Community-Academic Partnership to Address Obesity Among Middle School Students

Looking ahead, the CRC is excited to announce the Advanced REDCap Workshop: Building a Database, a new monthly offering beginning January 2014 that aims to help with the initial programming of a REDCap database for a research study. Another new upcoming course is Economic Evaluation in Healthcare Research, a three-lecture course that will give participants an understanding of the principles of economic evaluation, as well as various data sources that can be used for economic evaluations and the basic principles of analysis of economic data.
**Table 1. CRC Course Registration, 2004-2013; Total: 1,388**

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**Statistical Mini Courses:**

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*Includes attendees from Brigham & Women’s Hospital

**Figure 1. CRC Course Attendees by Department 2013; Total: 818*`

*Includes only one count for each person; a person could attend more than one course
Table 2. CRC Course Listings 2004-2013 (offered***)

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<td>Introduction to Longitudinal Analysis</td>
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<td>Introduction to Regression Analysis</td>
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<td>Art and Anatomy of Writing a Career Development Grant</td>
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<td>Program for Research Assistant Development and Achievement</td>
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<td>Qualitative Research in Clinical Investigation</td>
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<td>Study Design in Clinical and Translational Research</td>
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<td>CRC Grand Rounds</td>
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Behavioral Science Core

Staffing
Deborah Waber, PhD, Co-Director
Michelle Bosquet Enlow, PhD, Co-Director
Jonathan Girard, Research Study Coordinator

This past year, the CRC partnered with the Department of Psychiatry to create a Behavioral Science Core. Housed within the Department of Psychiatry’s Program for Behavioral Science, this service is directed by Deborah Waber, Ph.D. and co-directed by Michelle Bosquet Enlow, Ph.D.

The Core provides consultation services for the preparation of grants, protocols and clinical trials as well as psychometrician services, including the new NIH Toolbox.

Five protocols have used these services. In addition, consultation services have been provided to a number of investigators to assist with protocol development. The Core has served PIs in various departments throughout the hospital, including Psychiatry, Neurology, Hematology, Infectious Disease, and GI/Nutrition.

Partial funding for this service is provided by Harvard Catalyst | The Harvard Clinical and Translational Science Center.
AFFILIATED CORES

CLINICAL RESEARCH INFORMATION TECHNOLOGY (CRIT)

Staffing
FY2013 Clinical Research Information Technology staff included the following:
Jonathan Bickel, Director of Clinical Research Informatics
Ramkrishna Chakrabarty
Mohamad Daniar
James Gregoric
Ying-Feng Hsu
Arunkumar Nedunseralathan
Nandan Patibandla
Elizabeth Phillips
Joseph Rezuke

Clinical Research IT Core Mission
The mission of the Clinical Research Information Technology group is to provide leadership and innovative information technology services to enable and support the BCH clinical research enterprise.

Clinical Research IT Core Services
The CRIT provides IT services to the clinical investigator, core program and administrative communities. The CRIT is responsible primarily for sharing technical skills and resources, enhancing collaboration and improving the efficiency of clinical research applications development. The unit serves to champion innovative informatics solutions and expand IT services to the entire clinical research community.

CRIT services include systems for clinical trials data management, subject randomization, Web-based survey research and study coordination and research data warehousing for enhanced analytics. Informatics consultation and custom software applications development are also available.

Clinical Research IT Progress Report
The CRIT continued to provide its core services and has continued to expand its focus to the development of innovative products to support clinical research.

EDC development and use continues to grow using Oracle InForm® and REDCap
FY 2013 continued the trend of the last several years. The use of EDC (electronic data capture) grows as the institution continues to advance its vision of gathering clinical research data into centralized, supported databases.

2013 saw the continued rise in the utilization of REDCap, an academically supported EDC tool grown out of the CTSA grant at Vanderbilt University. The use of this tool has seen tremendous growth. REDCap enables clinical researchers to build and deploy their own clinical research database with little IT intervention. Currently, there are two deployments of REDCap at BCH; one is an external instance which is accessible from the internet (currently has 1487 user accounts and 918 projects) and the other is an internal instance which can contain any PHI data (currently has 1062 user accounts and 636 projects)
The Phase Forward InForm EDC (electronic data capture) is in its sixth full year of use. The InForm system has continued to allow the enterprise to support major clinical trials.

We have also successfully deployed a new instance of InForm with the newer version (InForm 5.5) and already been used in production for clinical trials.

**Clinical Research Coordination System**

The CRIT has developed and deployed a new enterprise software application that is used by study teams to coordinate daily research activities. The product, titled the Clinical Research Coordination System, supports the following features:

- Centralized Patient Enrollment
- Study Scheduling
- Task and Patient Contact Management
- Patient Lab and Demographic Data Export from the Clinical data warehouse

**Deployed End-User Interface for Cohort Discovery - i2b2**

The CRIT team has continued its service offering of i2b2 (Informatics for Integrating Biology and the Bedside) as a clinical data warehouse research tool. i2b2’s comprehensive software and methodological framework enables the clinical research community to accelerate the translation of clinical findings into novel diagnostic, prognostic and therapeutic findings. It also provides a collaborative organizational and software infrastructure for basic and clinical researchers.

The CRIT team has greatly expanded the amount of data that is available to use in this data warehouse and is working with the i2b2 development team to expand the functionality of the web client used to access the warehouse.

Besides i2b2, CRIT also provides services for cohort discovery for clinical trials where data is in the data warehouse but not in i2b2.
Staff Publications - FY2013

2013 Published


40. Mangeshkar P; Hansen R; Huntington N. Integration of Clinical Research Activities in A Clinical Setting- Division of Developmental Medicine, Boston Childrens Hospital


60. Stamoulis C, Schomer DL, Chang BS, Information theoretic measures of network coordination in high-frequency scalp EEG reveal dynamic patterns associated with seizure termination, Epilepsy Res. April 2013 PMID: 23608198


2013 Epub


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# Course Agendas

## INTRODUCTION TO CLINICAL RESEARCH
### FALL 2013 COURSE AGENDA

<table>
<thead>
<tr>
<th>AM</th>
<th>Day One: Tuesday, September 10, 2013</th>
<th>Folkman Auditorium</th>
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<tbody>
<tr>
<td>8:30-8:45</td>
<td>Registration &amp; Check-in</td>
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<tr>
<td>8:45-9:00</td>
<td>Introduction and Overview</td>
<td>A. Dauber/D. Graham</td>
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<tr>
<td>9:00-9:45</td>
<td>Overview of Clinical Research at Children’s Hospital</td>
<td>V. Osganian</td>
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<tr>
<td>9:45-10:45</td>
<td>Statistics in Clinical Research: An Overview</td>
<td>H. Feldman</td>
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<tr>
<td>10:45-11:00</td>
<td>Break</td>
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<tr>
<td>11:00-11:45</td>
<td>The IRB Review Process: An Insider’s View</td>
<td>L. Shrier</td>
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<tr>
<td>11:45-12:30</td>
<td>Human Subjects and the IRB</td>
<td>S. Kornetsky</td>
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<tr>
<td>12:30-1:15</td>
<td>Lunch</td>
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<tr>
<th>PM</th>
<th>Day One: Tuesday, September 10, 2013</th>
<th>Folkman Auditorium</th>
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<tbody>
<tr>
<td>1:15-2:15</td>
<td>Observational Study Designs</td>
<td>M. Monuteaux</td>
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<tr>
<td>2:15-3:00</td>
<td>Clinical Trials: Design and Monitoring</td>
<td>E. Neufeld</td>
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<tr>
<td>3:00-3:15</td>
<td>Break</td>
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<tr>
<td>3:15-4:00</td>
<td>Designing Surveys</td>
<td>G. Sideridis</td>
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<tr>
<td>4:00-5:00</td>
<td>Writing for Scientific Publication</td>
<td>S. J. Emans</td>
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</table>
# INTRODUCTION TO CLINICAL RESEARCH

## FALL 2013 COURSE AGENDA

| PM | Day Two: Thursday, September 12, 2013  
Auditorium B, 1 Autumn St. |
<table>
<thead>
<tr>
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<tbody>
<tr>
<td>1:00-1:15</td>
<td>Arrival/ Check In</td>
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</table>
| 1:15-2:30 | Descriptive and Univariate Statistics  
D. Graham |
| 2:30-3:15 | Evaluating Measures in Clinical Research  
G. Sideridis |
| 3:15-3:30 | Break |
| 3:30-4:15 | TIDO  
M. Tessier/F. Valles |
| 4:15-5:00 | Industry and Philanthropy  
S. Nurko |

| AM | Day Three: Friday, September 13, 2013  
Folkman Auditorium |
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<tbody>
<tr>
<td>8:00-8:15</td>
<td>Arrival/ Check In</td>
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</table>
| 8:15-9:15 | Introduction to Regression Analysis  
H. Feldman |
| 9:15-9:45 | I2b2 and Current Technologies  
J. Bickel |
| 9:45-10:00 | Break |
| 10:00-11:00 | Collecting and Managing Clinical Research Data  
M. Berry |
| 11:00-11:30 | Common Study Documentation Errors & Best Practices  
E. Newbert |
| 11:30-12:30 | Lunch |

<table>
<thead>
<tr>
<th>PM</th>
<th>Day Three: Friday, September 13, 2013</th>
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</table>
| 12:30-1:15 | Grant Writing 101  
J. Lightdale |
| 1:15-2:00 | Introduction to Cost-Effectiveness Analysis  
M. Samnaliev |
| 2:00-2:15 | Break |
| 2:15-3:00 | Scientific Presentations  
J. Finkelstein |
| 3:00-3:30 | Wrap-Up  
A. Dauber |
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Presenter</th>
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<tbody>
<tr>
<td>9:00 - 9:25</td>
<td>Welcome and Overview</td>
<td>P. Mangeshkar</td>
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<tr>
<td>9:25 - 9:40</td>
<td>The Study Coordinator’s Central Role in Clinical Research</td>
<td>M. Stafford</td>
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<tr>
<td>9:40 - 9:45</td>
<td>Break</td>
<td>C. Bennett</td>
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<tr>
<td>9:45 - 10:05</td>
<td>Human Subject Protections IRB Issues: Before the Research Begins</td>
<td>E. Caglier</td>
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<tr>
<td>10:05 - 10:30</td>
<td>IRB Issues: During the Research</td>
<td>A. Mitchell</td>
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<td>10:30 - 10:40</td>
<td>Break</td>
<td>L. Abernethy</td>
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<tr>
<td>10:40 - 11:10</td>
<td>Protecting Patient/Subject Information</td>
<td>S. Corl</td>
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<td>11:10 - 11:25</td>
<td>Considering the Research Volunteer Perspective</td>
<td>M. Berry</td>
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<tr>
<td>11:25 - 11:35</td>
<td>Break</td>
<td>P. Mangeshkar</td>
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<tr>
<td>11:35 - 12:00</td>
<td>Informed Consent/Assent/Subject Recruitment</td>
<td>E. Caglier</td>
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<tr>
<td>12:00 - 12:30</td>
<td>Catered Lunch</td>
<td>L. Abernethy</td>
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<tr>
<td>12:30 - 1:30</td>
<td>Obtaining Informed Consent/Assent: A Practical Approach</td>
<td>S. Corl</td>
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<td>1:30 - 1:40</td>
<td>Break</td>
<td>M. Berry</td>
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<td>2:35 - 2:45</td>
<td>Break</td>
<td>P. Mangeshkar</td>
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<tr>
<td>2:45 - 3:30</td>
<td>Introduction to the Clinical Research Center (CRC)</td>
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<tr>
<td>3:30 - 3:50</td>
<td>Principles of Data Management</td>
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<td>Wrap-Up &amp; Review</td>
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