Bone Marrow Transplant and Immune Deficiency Annual Report, 2009-10

Division Data Summary

<table>
<thead>
<tr>
<th>Research and Training Details</th>
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<tbody>
<tr>
<td>Number of Faculty</td>
<td>11</td>
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<tr>
<td>Number of Joint Appointment Faculty</td>
<td>4</td>
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<tr>
<td>Number of Research Fellows</td>
<td>2</td>
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<tr>
<td>Number of Support Personnel</td>
<td>46</td>
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<tr>
<td>Direct Annual Grant Support</td>
<td>$854,673</td>
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<tr>
<td>Direct Annual Industry Support</td>
<td>$44,970</td>
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<tr>
<td>Peer Reviewed Publications</td>
<td>17</td>
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<table>
<thead>
<tr>
<th>Clinical Activities and Training</th>
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<tbody>
<tr>
<td>Number of Clinical Staff</td>
<td>4</td>
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<tr>
<td>Number of Clinical Fellows</td>
<td>1</td>
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<tr>
<td>Inpatient Encounters</td>
<td>9,111</td>
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<tr>
<td>Outpatient Encounters</td>
<td>3,850</td>
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<tr>
<th>Significant Publications</th>
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**Division Collaboration**

**Collaboration with Immunobiology**

**Collaborating Faculty: L. Grimes**

MEIS1 and MLL-fusion leukemia: Research project investigating the role of MEIS1 in MLL-fusion leukemia using transgenic mice. (A. Kumar)

**Collaboration with Experimental Hematology and Cancer Biology**

**Collaborating Faculty: J. Mulloy**

Animal models of human leukemia: Research project aimed at developing xenograft mouse models of human leukemia using primary patient derived material. (A. Kumar)

**Collaboration with Experimental Hematology and Cancer Biology**

**Collaborating Faculty: N. Ratner**

MEIS1 and MPNSTs: Research project aimed at understanding the role of MEIS1 in malignant peripheral nerve sheath tumors (MPNST). (A. Kumar)

**Collaboration with Nephrology; Biostatistics & Epidemiology; Pathology**

**Collaborating Faculty: J. Goebel; J. Khoury; Hong J Yin**

Early Clinical Indicators of Transplant Associated-Thrombotic Microangiopathy in Pediatric Neuroblastoma Patients Undergoing Autologous Stem Cell Transplantation. *Bone Marrow Transplantation, in press* (J. Bleesing, S. Davies, A. Filipovich, S. Jodele, P. Mehta)

**Collaboration with Nephrology; Biostatistics & Epidemiology**

**Collaborating Faculty: J. Goebel; B. Dixon; P. Devarajan; J. Khoury**


**Collaboration with Hematology/Oncology; Biostatistics & Epidemiology**

**Collaborating Faculty: J. Perentesis; L. Wagner; R. Nagarajan; M. Kim**


**Collaboration with Critical Care; Radiology; Nephrology**

**Collaborating Faculty: H. Wong; S. Poynter; W. Ball; J. Leach; J. Goebel; M. Mitsnefes**

PRES working group: To identify patients at risk for PRES, outline uniform evaluation and therapy guidelines. (S. Jodele, S. Davies)

**Collaboration with Critical Care**

**Collaborating Faculty: R. Chima**

BMT-PICU risk score index working group: To determine outcome predictors for BMT patients that are treated in PICU. To validate BMT-PICU risk score at CCHMC and later at collaborating institutions.

**Collaboration with Hoxworth Blood Center**

**Collaborating Faculty: P. Carey**

Plasmapheresis – Early Intervention Produces Positive Outcome For Pediatric Patients with Transplant Associated Thrombotic Microangiopathy. Awarded Poster presentation at American Association of Blood Banks (AABB) Annual Meeting.

**Collaboration with Clinical Pharmacology**

**Collaborating Faculty: S. Vinks**

Alternate day Micafungin Anti-fungal Prophylaxis in Immunocompromised Pediatric Patients: A Pharmacokinetic Study.
Collaboration with Human Genetics; Biostatistics & Epidemiology
Collaborating Faculty: T. Smolarek; M. Kim
The Clinical significance of Gains in 1q and 3q and Deletions of 7 and 7q as Detected by FISH in Fanconi Anemia Patients followed longitudinally over Time. *Cancer, Genetics and Cytogenetics* In Press (P. Mehta, R. Harris, S. Davies)

Collaboration with Hematology/Oncology

Collaborating Faculty: F. Smith

Collaboration with Hematology/Oncology
Collaborating Faculty: C. Phillips
Computed tomography induced double-stranded DNA breaks as a potential marker of radiation sensitivity in Fanconi anemia. *Pediatric Blood and Cancer* Abstract for 23rd Annual meeting of ASPHO, April 7-10, 2010, Montreal, Quebec, Canada. (P. Mehta, S. Davies)

**Faculty Members**
Stella Davies, MBBS, PhD, MRCP, Professor; Division Head

**Significant Accomplishments**

**A record year**
The clinical program in bone marrow transplantation and immune deficiency performed a record 108 transplants in 2009. This busy program serves as the backbone of our clinical and research efforts as a Jeffrey Modell Foundation and FOCIS center of excellence in primary immune deficiencies. Our investigators also have obtained significant funding for basic science and translational efforts involving bone marrow failure.

**Graft versus host disease**
We are working with the Division of Gastroenterology, Hepatology and Nutrition and the Department of Surgical Services to identify important clinical phenomenon of graft versus host disease after transplant, and to prepare clinical guidelines for monitoring and treatment.

**Novel parameters for thrombotic microangiopathy**
We collaborated with the Division of Nephrology and Hypertension to describe the incidence of thrombotic microangiopathy after transplantation, and we have published novel clinical and biological diagnostic parameters. A prospective study, now IRB-approved, seeks to further clarify the pathophysiology of this disorder.

**Testing a severity index**
We also are testing a BMT-specific severity index we developed for post-transplant children admitted to the PICU, in collaboration with the Division of Critical Care Medicine. These data will be reported later in the year.

**Division Publications**
1. :

**Grants, Contracts, and Industry Agreements**

**Grant and Contract Awards**

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<tr>
<th>Grant and Contract Awards</th>
<th>Annual Direct / Project Period Direct</th>
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<tr>
<td>Multicenter Pilot Trial of HSCT Lacking a Genotype Identical Donor Fanconi Anemia Research Fund</td>
<td>05/01/2010 - 04/30/2013</td>
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<td>Antileukemic Effect of NK Cells in HCT for Pediatric AML St Jude's Children's Hospital (National Institutes of Health)</td>
<td>08/01/2007 - 06/30/2012</td>
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<td>Childhood Cancer Survivor Study St Jude's Children's Hospital (National Cancer Institute) U24 CA 055727</td>
<td>12/01/2005 - 11/30/2010</td>
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<td>Mechanisms of RET/PTC Rearrangement in Thyroid Cancer University of Pittsburgh (National Institutes of Health)</td>
<td>R01 CA 088041</td>
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<tr>
<td>Environmental Exposure: Susceptibility Alleles in a DNA Damage Response Pathway University of Cincinnati (National Institutes of Health)</td>
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Prediction Model for Radiation Sensitivity in Children with Cancer
National Cancer Institute
U01 CA 139275
07/16/2009 - 06/30/2011
$150,618 / $279,136

The Children's Oncology Group Chairs Grant
National Childhood Cancer Foundation (National Institutes of Health)
U10 CA 098543
03/01/2008 - 02/28/2011
$12,294 / $35,913

HSCT-CHESS to Enhance Hematopoietic Transplant Recovery
New England Medical Center Hospital, Inc. (National Institutes of Health)
S01 CA 120583
06/01/08 - 05/31/11
$12,321 / $20,568

Filipovich, A
Primary Immune Deficiency Treatment Consortium
The Regents of the Univ of California (National Institutes of Health)
U54 AI 082973
09/12/2009 - 08/31/2010
$25,000 / $25,000

Hypoxia and Potassium Channel Activity in T Lymphocytes
University of Cincinnati (National Institutes of Health)
R01 CA 095286
06/01/2009 - 04/30/2011
$19,766 / $38,956

Kumar, A
Molecular Pathogenesis of MLL-Fusion Gene Leukemia
National Institutes of Health
K08 CA 122191
08/19/2009 - 06/30/2012
$125,250 / $375,750

Marsh, R
Investigations into XIAP-deficient X-linked Lymphoproliferative Disease
National Institutes of Health
R03 AI 079797
09/15/2008 - 08/31/2010
$50,000 / $100,000

Myers, K
Leukemogenesis, Genomic Instability and Fanconi Anemia
Bear Necessities Pediatric Cancer Fdn
07/01/2009 - 06/30/2010
$40,000 / $40,000

Sumegi, J
Search for Growth Inhibitory Genes in Ewing's Sarcoma by Epigenetic Profiling
University of Nebraska Medical Center (La Fondation des Gouverneurs de l'espoir)
01/01/2008 - 12/31/2010
$49,500 / $125,793

Functional Identification of Genes Mutated in FHLH
National Institutes of Health
R21 AI 079759
07/15/2008 - 06/30/2011
$132,333 / $275,000

Studies of the t(2;2)(p23;q35)(PAX3-NCOA1) and t(2;8)(q35;q13)(PAX3-NCOA2) Translocations in Alveolar Rhabdomyosarcoma
Joanna McAfee Childhood Cancer Foundation, Inc.
01/01/2010 - 12/31/2010
$5,000 / $5,000

Identification of Genes Involved in FHLH
National Institutes of Health
R21 AI 076746
09/01/2009 - 08/31/2011
$150,000 / $275,000

Current Year Direct $854,673

Industry Contracts

Mehta, P
Astellas Pharma US, Inc
$ 44,970

Current Year Direct Receipts $44,970
|-funded-collaborative-efforts-
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<tr>
<td><strong>Bleesing, J</strong></td>
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<tr>
<td>Cincinnati Multidisciplinary Clinical Research Center</td>
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<td>National Institutes of Health</td>
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<td>Glass, D</td>
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<td>08/18/08 - 07/31/13</td>
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