Division Details

Division Data Summary

Research and Training Details

<table>
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<td>Number of Faculty</td>
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<td>Number of Research Fellows</td>
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<td>Direct Annual Industry Support</td>
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<td>Peer Reviewed Publications</td>
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Clinical Activities and Training

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<tr>
<td>Number of Clinical Staff</td>
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<td>Inpatient Encounters</td>
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<tr>
<td>Outpatient Encounters</td>
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Significant Publications


This paper describes an important mechanism of leukemogenesis, relevant to etiology and treatment of leukemia in very young children.


This paper describes for the first time the clinical importance of BK viremia in children after transplant. These findings have changed screening and therapy for this virus at our own and at other institutions.


This paper is the first systematic description of immune system defects in fanconi Anemia.

Shin, C.R., J. Nathan, M. Alonso, N. Yazigi, S. Kocoshis, G. Tiao, and S.M. Davies, **Incidence of acute and

This paper, written in collaboration with the department of surgery, is the largest description of GVHD after solid organ transplant in the literature and has lead to changes in screening and treatment at Cincinnati Children's Hospital Medical Center.


This paper, written in collaboration with the division of human genetics, describes the previously overlooked phenomenon of hemophagocytic lymphohistiocytosis in adults, and provides a mechanistic explanation for the clinical findings.

Division Publications


Faculty, Staff, and Trainees

**Faculty Members**

**Stella Davies, MBBS, PhD, MRCP, Professor**
- **Leadership** Jacob G. Schmidlapp Endowed Chair; Director, Bone Marrow Transplantation and Immune Deficiency; Executive Co-Director, Cancer and Blood Diseases Institute; Director, Division of Bone Marrow Transplantation and Immune Deficiency
- **Research Interests** Blood and Marrow Transplant, Fanconi anemia and other marrow failure disorders, Pharmacogenetics and Pharmacokinetics, Survivorship Research, and BMT for Children with Leukemia.

**Jacob Bleesing, MD, PhD, Associate Professor**
- **Research Interests** Clinical Investigation of Primary Immunodeficiency Disorders, with emphasis on disorders of immunodysregulation and B-cell disorders

**Alexandra Filipovich, MD, Professor**
- **Leadership** Ralph J. Stolle Chair in Clinical Immunology; Director, Immunodeficiency and; Medical Director, Diagnostic Laboratory
- **Research Interests** Histiocytic disorders, primary immune deficiency disorders and Immunoreconstitution Following Pediatric Stem Cell Transplantation

**Michael Grimley, MD, Associate Professor**
- **Research Interests** Bone marrow transplantation for children with malignant and non-malignant disorders and treatment of chronic GVHD.

**Richard Harris, MD, Professor**
- **Research Interests** Transplantation for children with bone marrow failure syndromes and aplastic anemia

**Sonata Jodele, MD, Assistant Professor**
- **Research Interests** Phase I clinical trials; stem cell transplantation; high risk pediatric malignancies; childhood neuroblastoma, Ewing's sarcoma, renal complications of transplantation.

**Sarita Joshi, MD, Assistant Professor**
- **Research Interests** Management of Diamond-Blackfan anemia, transplantation for hemoglobinopathies, and autologous transplantation for malignancy.

**Ashish Kumar, MD, PhD, Assistant Professor**
- **Research Interests** Etiology of childhood leukemia, transplantation of children with malignancy and primary immune deficiencies.

**Rebecca Marsh, MD, Assistant Professor**
- **Research Interests** Pathogenesis of HLH with XIAP deficiency, diagnostic laboratory assays, improving
outcomes of allogeneic BMT in primary immune deficiencies, use of reduced intensity conditioning regimens.

**Parinda Mehta, MD**, Associate Professor  
**Research Interests** Blood and Marrow Transplant, Fanconi anemia and other failure disorders, Pharmacogenetics and Pharmacokinetics

**Kasiani Myers, MD**, Instructor  
**Research Interests** Blood and Marrow Transplant, Fanconi anemia and other failure disorders, longitudinal studies of hematopoiesis

**Janos Sumegi, MD, PhD**, Professor  
**Research Interests** Lymphoproliferative disease, Hemophagocytic Lymphohistiocytosis, Usher syndrome, etiology of childhood sarcomas.

**Joint Appointment Faculty Members**

**Mark Johnson, MD**, Assistant Professor (Child Psychiatry)  
**Research Interests** Psychiatric support of bone marrow transplant patients

**Michael Jordan, MD**, Associate Professor (Immunobiology)  
**Research Interests** Regulation of the immune response; immunotherapy of cancer, animal models of hemophagocytic disorders.

**Mi-Ok Kim, PhD**, Associate Professor (Biostatistics & Epidemiology)

**Ahna Pai, PhD**, Assistant Professor (Adherence Psychology in children receiving bone marrow transplantation)

**Clinical Staff Members**

- Teresa Finke, MD
- Zahida Khan, MD
- Ernest Lawhorn, MD
- Gregory Wallace, DO
- Paula Cuthrell, RN, MSN, CFNP
- Jennifer Detzel, RN, MSN, CFNP
- Laura Diggs, RN, MSN, CNP
- Mary Ann Michael, RN, MSN, CPNP
- Gretchen Vaughn, RN, MSN, CPNP

**Trainees**

- Shan Chandrakasan, MD, PL-IV, Children’s Hospital of Michigan
- Anne Hladik, MD, PL-IV, Baylor College of Medicine
- Pooja Khandelwal, MD, PL-V, University of Arizona College of Medicine
- Omar Niss, MD, PL-V, University of Nebraska Medical Center/Creighton University

**Division Collaboration**

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

**Experimental Hematology and Cancer Biology » 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)
Experimental Hematology and Cancer Biology » J. Mulloy and J. Cancelas
The molecular origin of monosomy 7 in very young children. Translational research project exploring the causes of monosomy 7. (P. Mehta, SM Davies)

Experimental Hematology and Cancer Biology » N. Ratner
MEIS1 and MPNSTs: Research project aimed at understanding the role of MEIS1 in malignant peripheral nerve sheath tumors (MPNST). (A. Kumar)

Nephrology; Biostatistics & Epidemiology » J. Goebel, B. Dixon, P. Devarajan, and J. Khoury
A Prospective Analysis of Clinical and Biochemical Markers for Pediatric Stem Cell Transplant-Associated Thrombotic Microangiopathy. Study open and more than half the needed patients enrolled. Preliminary data generated and grant applications submitted. (J. Bleesing, SM. Davies, A. Filipovich, R. Harris, S. Jodele, M. Jordan, S. Joshi, A. Kumar, R. Marsh, P. Mehta)

Critical Care; Radiology; Nephrology » H. Wong, S. Poynter, W. Ball, J. Leach, J. Goebel, and M. Mitsnefes
PRES working group
To identify patients at risk for PRES, outline uniform evaluation and therapy guidelines. (S. Jodele, SM Davies)

Nephrology » J. Goebel and BL Laskin

Nephrology » J. Goebel and BL Laskin
Small vessels, big trouble in the kidneys and beyond: hematopoietic stem cell transplantation-associated thrombotic microangiopathy. (S Jodele, SM Davies).

Critical Care » R. Chima and D. Wheeler
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. (S Jodele, SM Davies)

Critical Care » R. Chima and D. Wheeler
Paper describing excellent outcomes for children admitted to PICU post-transplant admitted to PICU. (S Jodele, SM Davies)

Surgery; Gastroenterology » J. Nathan, G.Tiao, M. Alonzo, N. Yazigi, and S. Kocoschis

Surgery; Dermatology » R. Azizkhan and A. Lucky
Preparation of a stem cell transplant protocol for children with epidermolysis bullosa. (S Jodele and SM Davies).

Genetics; Pathology » T. Smoralek, S. Zimmerman, and R. McMasters

Experimental Hematology and Cancer Biology » K. Komurov
Investigating the role of MEIS1 in gliomas (Kumar A)

Oncology » J. Perentesis
Novel therapeutic approaches for EVI1+ leukemias(Kumar A)

Genetics » K. Zhang
Genotyping for PID and HLH (Filipovich, Bleesing)
The Fanconi Anemia Pathway Limits Human Papillomavirus Replication. (P. Mehta, Davies, S.)

Fanconi Anemia Links Reactive Oxygen Species to Insulin Resistance and Obesity. (P. Mehta, Davies, S.)

The FA pathway counteracts oxidative stress through selective protection of antioxidant defense gene promoters. (P. Mehta, Davies, S., Myers, K.)

Etanercept Treatment in Fanconi Anaemia; Combined US and Italian Experience. (P. Mehta, Davies, S., Harris, R)

Ongoing collaboration on a translational study: Quercetin in patients with Fanconi anemia, a Pilot study (P. Mehta)

Identify the cell of origin of the monosomy 7 clone in children with MDS arising in children with and without known genetic predisposition syndromes, and establish timing of onset in very young children (in utero vs ex utero). (P.Mehta)

Endocrine phenotype of children and adults with Fanconi anemia. (P. Mehta, S. Davies, Myers, K., Harris, R.)

HPV and Immune Function in Fanconi anemia: Research project investigating the epidemiology of HPV infection and the role of immune function in Fanconi Anemia. (P. Mehta)

Determination of antibody responses to natural infection with HPV and HPV vaccination in individuals with Fanconi anemia and to evaluate the predictors of effective or ineffective immune responses to HPV in individuals with FA. (P. Mehta)

Therapeutic Drug Monitoring of Voriconazole in pediatric BMT patients. (P. Mehta)

Pharmacokinetics of every 4th day Micafungin in high risk pediatric BMT patients. (P. Mehta)

The Impact of Alemtuzumab Levels on Donor Chimerism and Graft Versus Host Disease Following Allogeneic Hematopoietic Cell Transplantation: A Prospective Study. (P. Mehta)

Severe Congenital Neutropenia: Research project investigating novel genetic etiologies and molecular and cellular phenotypes of severe congenital neutropenia (K.Myers)

HPV and Immune Function in Fanconi anemia: Research project investigating the epidemiology of HPV infection and the role of immune function in Fanconi Anemia. (K.Myers, P. Mehta)

Endocrine function after RIC: Research project investigating endocrine function in pediatric patients after
reduced intensity bone marrow transplantation. (K.Myers)

**Ophthalmology » T. Schwartz**
Ophthalmic GHVD: Research project investigating the incidence, severity and treatment of ophthalmic graft versus host disease in patients with systemic GVHD after bone marrow transplant. (K.Myers)

**Experimental Hematology and Cancer Biology » P. Malik**
Gene therapy for Hemophagocytic Lymphohistiocytosis (M. Jordan)

**Experimental Hematology and Cancer Biology » P. Malik**
Novel methods to promote stem cell engraftment (M. Jordan)

**Allergy and Immunology » K. Risma**
Gene therapy for Hemophagocytic Lymphohistiocytosis (M. Jordan)

**Allergy and Immunology » K. Risma**
The biology of cytotoxicity: Genotype/phenotype correlations (M. Jordan)

**Cellular and Molecular Immunology » D. Hildeman**
Selective ablation of undesirable T cell responses (M. Jordan)

**Allergy and Immunology » M. Jordan**
Immune profiling in Hemophagocytic Lymphohistiocytosis

**Nephrology; Biostatistics ; Epidemiology » Dr.Prasad, Devarajan, J. Goebel, and J. Khoury**
Project: “A Prospective Analysis of Clinical and Biochemical Markers for Pediatric Stem Cell Transplant-Associated Thrombotic Microangiopathy (TA-TMA)"
Project support:
1. P50 Application in response to RFA-DK-11-009 – Pediatric Centers of Excellence in Nephrology
2. Center for Clinical and Translational Science and Training (CCTST), University of Cincinnati Academic Health Center, T1 Pilot Grant

**Genetics ; Hematology » K. Zhang and R. Gruppo**
Project: “Complement gene testing in patients with hematopoietic stem cell transplant-associated thrombotic microangiopathy”
Project support: The Investigator Initiated Trial Grant from Alexion Therapeutics (S. Jodele, S. Davies)

**Cardiology ; Critical Care ; Pathology » R. Hirsch, R. Chima, and D. Witte**
Project: “Pulmonary Hypertension in Patients with Hematopoietic Stem Cell Transplant -Associated Thrombotic Microangiopathy”
Manuscript is accepted in Biology of Bone Marrow Transplantation journal (S. Jodele, S. Davies)

**Nephrology » J. Goebel**
Project: “Retrospective analysis of BK virus infections in an existing cohort of pediatric hematopoietic cell transplant recipients at Cincinnati Children’s Hospital Medical Center” (S. Jodele, S. Davies)

**Nephrology ; Pathology » J.Goebel , H. Yin, and J. Khoury**
Project: “Cystatin C to Estimate Renal Function in Bone Marrow Transplant and Oncology Patients”

**Critical Care » R. Chima**
Pediatric intensive care for patients undergoing hematopoietic stem cell transplantation. (S. Jodele, S. Davies)

**Nephrology; Hoxworth Cell Therapy Team; Biostatistics; Epidemiology » J. Goebel, J. Khoury, and P. Carey**
Project: “Does early initiation of therapeutic plasma exchange improve outcome in pediatric stem cell transplant-
associated thrombotic microangiopathy?" Publication: Transfusion. 2012 Jul 15. d (S. Jodele, S. Davies)

**Critical Care; Biostatistics; Epidemiology » R. Chima and D. Wheeler**
BMT-PICU risk score index working group.(S. Jodele, S. Davies)

**Critical Care » R. Chima and D. Wheeler**

**Rheumatology; Human Genetics; Allergy » A. Grom, Barnes, K. Zhang, and K. Risma**
Gene expression profiling of peripheral blood mononuclear cells from children with active hemophagocytic lymphohistiocytosis. (J. Sumegi, A. Filipovich)

**Oncology; Human Genetics » L. Wagner and T. Smolarek**
Assessment of minimal residual disease in ewing sarcoma. (J. Sumegi)

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**Grants, Contracts, and Industry Agreements**

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<th>Grant and Contract Awards</th>
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<td><strong>DAVIES, S</strong></td>
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<tr>
<td>Antileukemic Effect of NK Cells in HCT for Pediatric AML National Institutes of Health(St Jude's Children's Hospital) R01 CA 120583 08/01/07-06/30/12</td>
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<td>Childhood Cancer Survivor Study National Institutes of Health(St Jude's Children's Hospital) U24 CA 055727 12/01/11-11/30/16</td>
<td>$177,391</td>
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<td>Children's Oncology Chair Award National Institutes of Health(Children's Oncology Group) U10 CA 098543 03/11-02/28/14</td>
<td>$12,831</td>
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<td>Multicenter Pilot Trial of HSCT Lacking a Genotype Identical Donor Fanconi Anemia Research Fund</td>
<td>05/01/10-04/30/13 $1,160</td>
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<td>Molecular Epidemiology of Pediatric Germ Cell Tumors National Institutes of Health(University of Minnesota) R01 CA151284 08/10/11-05/31/16</td>
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<td><strong>FILIPOVICH, A</strong></td>
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<td>Gene Therapy for SCID-X1 Using Self-Inactivating (SIN) Gammaretroviral Vector National Institutes of Health(Children's Hospital Boston) U01 AI 087628 09/01/10-08/31/15</td>
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<td>Hypoxia and Potassium Channel Activity in T Lymphocytes National Institutes of Health(University of Cincinnati) R01 CA 095286 06/01/09-04/30/12</td>
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<td>Rare Diseases Clinical Consortia for the Rare Diseases - Per Patient National Institutes of Health(The Regents of the Univ of California) U54 AI 082973 09/12-09/08/31/14</td>
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<td><strong>KUMAR, A</strong></td>
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<td>Molecular Pathogenesis of MLL-Fusion Gene Leukemia National Institutes of Health K08 CA 122191 08/19/09-06/30/12</td>
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<td><strong>MARSH, R</strong></td>
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<td>Studies to Determine Why ZIAP Deficiency Leads to HLH</td>
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# Clinical Immunology Society

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<th>MEHTA, P</th>
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<tr>
<td><strong>Quercetin in Patients with Fanconi Anemia, a Pilot Study</strong></td>
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<td>Aplastic Anemia &amp; MDS International Fdn</td>
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<td>07/01/11-06/30/13</td>
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<th>SUMEGI, J</th>
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<td><strong>Biomarkers in Primary and Secondary Hemophagocytic Lymphohistiocytosis</strong></td>
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<td>Histiocytosis Association of America</td>
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<td>01/01/12-12/31/12</td>
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<td><strong>Identification of PAX3-NCOA1/NCOA2-Regulated Genes in Rhabdomyosarcoma</strong></td>
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<td>Joanna McAfee Childhood Cancer Fdn. Inc.</td>
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**Current Year Direct** $719,207

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## Industry Contracts

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<td>Chimerix, Inc</td>
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<td>HARRIS, R</td>
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<td>Alexion Pharmaceuticals, Inc.</td>
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**Current Year Direct Receipts** $88,225

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## Service Collaborations

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<td>Nat Marrow Donor Pro</td>
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**Current Year Direct** $1,500

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## Funded Collaborative Efforts

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<tr>
<td><strong>NIAMS Multidisciplinary Clinical Research Center</strong></td>
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<td>National Institutes of Health</td>
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<tr>
<td>Lovell, D</td>
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<tr>
<td>DAVIES, S</td>
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<tr>
<td><strong>Nonadherence: Undermining Health Outcomes in Pediatric HSCT</strong></td>
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<td>National Institutes of Health</td>
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<td>Pai</td>
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**Total** $808,932