Division Summary

RESEARCH AND TRAINING DETAILS

- Number of Faculty: 16
- Number of Joint Appointment Faculty: 3
- Number of Research Fellows: 2
- Number of Support Personnel: 28
- Direct Annual Grant Support: $726,676
- Direct Annual Industry Support: $106,294
- Peer Reviewed Publications: 41

CLINICAL ACTIVITIES AND TRAINING

- Number of Clinical Staff: 7
- Number of Staff Physicians: 7
- Number of Clinical Fellows: 5
- Number of Other Students: 1
- Inpatient Encounters: 9,674
- Outpatient Encounters: 5,258

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.
Sharat Chandra, MD, Instructor
Research Interests Bone marrow transplantation for children with malignant and primary immune deficiency disorders.

Javier El-Bietar, MD, Instructor
Research Interests Bone marrow transplantation for malignant and nonmalignant diseases of childhood, transplant-associated complications and infections involving the gut.

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, Associate Professor
Research Interests Phase I clinical trials; stem cell transplantation; high risk pediatric malignancies; childhood neuroblastoma, Ewing’s sarcoma, renal complications of transplantation.

Pooja Khandelwal, MD, Instructor
Research Interests Acute Graft Versus Host Disease, Refractory autoimmune cytopenias post transplant, Biology of engraftment syndrome.

Zeynep Kucuk, MD, Instructor
Research Interests Characterization and clinical investigation of Primary Immunodeficiency Disorders including immune dysregulation disorders, autoimmune gastroenteropathy and epidemiology of HLH.

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

Adam Lane, PhD, Instructor
Research Interests Adaptive designs for phase I/II clinical trials, survival analysis and structural equation modeling.

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, Assistant Professor
Research Interests Blood and Marrow Transplant, Fanconi anemia and other marrow 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.

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

Clinical Staff Members

- Stephanie Benoit, MD
- Paulina Daniels, MD
- Michael Huang, MD
- Ernest Lawhorn, MD
- Lisa Petiniot, MD
- Kristen Stefanski, MD
- Gregory Wallace, DO
- Paula Cuthrell, RN, MSN, CFNP
- Jennifer Detzel, RN, MSN, CFNP
- Laura Diggs, RN, MSN, CNP
- Emily Herman, RN, CNP
- Mary Ann Michael, RN, MSN, CPNP
- Katie Scholle, RN, CNP
- Gretchen Vaughn, RN, MSN, CNP

Trainees

- **Christopher Dandoy, MD**, PL-VII, Miami Children's Hospital
- **Aharon Gefen, MD**, PL-VII, Rambam Medical Center, Israel
- **Adam Nelson, MD**, PL-VII, Centre for Children's Cacer & Blood Disorders, Sydney Children's Hospital
- **Ahmad Rayes, MD**, PL-V, SUNY Upstate Medical University
- **Beverly Schaefer, MD**, PL-IV, Thomas Jefferson University/Dupont Hospital for Children

Division Collaboration

Gene therapy for Hemophagocytic Lymphohistiocytosis (Michael Jordan, MD)

**Allergy and Immunology** » Kimberly Risma, MD, PhD

The biology of cytotoxicity: Genotype/phenotype correlations (Michael Jordan, MD)

**Allergy and Immunology** » Kimberly Risma, MD, PhD

Immune profiling in Hemophagocytic Lymphohistiocytosis (Michael Jordan, MD)

**Allergy and Immunology** » Kimberly Risma, MD, PhD
HPV and Immune Function in Fanconi anemia: Research project investigating the epidemiology of HPV infection and the role of immune function in Fanconi Anemia. (Parinda Mehta, MD)

**Asthma Research** » Melinda Butsch-Kovacic, MD, MPH

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. (Parinda Mehta, MD)

**Asthma Research** » Melinda Butsch-Kovacic, MD, MPH

Invited Pulmonary Hypertension in HSCT patient’s manuscript is published in Biology of Bone Marrow Transplantation journal. First author, C. Dandoy, Hematology/Oncology Fellow and mentee to Sonata Jodele, MD for this project. *(Dandoy, et al, Bio. Blood Marrow Transplant 2013, Jul 23)* (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

**Cardiology** » John L. Jefferies, MD, MPH, FAAP, FACC and Russel Hirsch, MD

**Critical Care** » Ranjit Chima, MD and Hector Wong, MD

**Pathology** » David Witte, MD

**Hematology/Oncology** » Christopher Dandoy, MD

“Echocardiographic screening for pulmonary hypertension in HSCT patients”. I am mentoring Dr. Christopher Dandoy for this project. This project was completed, data analyzed and manuscript is in under review in BBMT journal. Project was awarded oral presentation at ASBMT 2013 and plenary session at ASPHO 2013 meetings. (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

**Cardiology** » John L. Jefferies, MD, MPH, FAAP, FACC and Russel Hirsch, MD

**Critical Care** » Ranjit Chima, MD and Hector Wong, MD

**Pathology** » David Witte, MD

**Hematology/Oncology** » Christopher Dandoy, MD

Pulmonary Hypertension in HSCT patients: biomarker pilot study”. I am mentoring Dr. Christopher Dandoy for this project. Study samples are being analyzed based on preliminary data.

(Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

**Cardiology** » John L. Jefferies, MD, MPH, FAAP, FACC and Russel Hirsch, MD

**Critical Care** » Ranjit Chima, MD and Hector Wong, MD

**Pathology** » David Witte, MD

**Hematology/Oncology** » Christopher Dandoy, MD

Selective ablation of undesirable T cell responses (Michael Jordan, MD)

**Immunobiology** » David Hildeman, PhD

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 Cincinnati Children’s and later at collaborating institutions.

(Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP, Adam Lane, PhD)

**Critical Care Medicine** » Ranjit Chima, MD and Derek Wheeler, MD

Book Chapter: "Pediatric intensive care for patients undergoing hematopoetic stem cell transplantation." Chapter
Now, to identify patients at risk for PRES, outline uniform evaluation and therapy guidelines. (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

**Endocrine phenotype of children and adults with Fanconi anemia.** (Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP, Kasiani Myers, MD, Richard Harris, MD)

**Endocrinology** » Deborah Elder, MD and Susan Rose, MD

Quercetin in patients with Fanconi Anemia: A pilot study. (Parinda Mehta, MD)

**Endocrinology** » Deborah Elder, MD and Susan Rose, MD

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

**Endocrinology** » Susan Rose, MD

Gene correction strategies for Hemophagocytic lymphohistiocytosis. (Michael Jordan, MD)

**Experimental Hematology and Cancer Biology** » Punam Malik, MD

**Allergy and Immunology** » Kimberly Risma, MD, PhD

Novel methods to promote stem cell engraftment. (Michael Jordan, MD)

**Experimental Hematology and Cancer Biology** » Punam Malik, MD

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

**Experimental Hematology and Cancer Biology** » James Mulloy, PhD

The molecular origin of monosomy 7 in very young children. Translational research project exploring the causes of monosomy 7. (Parinda Mehta, MD, S. Davies, MB BS, PhD, MRCP)

**Experimental Hematology and Cancer Biology** » Jose Cancelas, MD, PhD and James Mulloy, PhD

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

**Experimental Hematology and Cancer Biology** » Nancy Ratner, PhD

The Fanconi Anemia Pathway Limits Human Papillomavirus Replication. (Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP)

**Experimental Hematology and Cancer Biology** » Susanne Wells, PhD
Fanconi Anemia Links Reactive Oxygen Species to Insulin Resistance and Obesity. (Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP)

Experimental Hematology and Cancer Biology » Qishen Pang, PhD

The FA pathway counteracts oxidative stress through selective protection of antioxidant defense gene promoters.
(Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP)

Experimental Hematology and Cancer Biology » Qishen Pang, PhD

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

Experimental Hematology and Cancer Biology » Qishen Pang, PhD

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). (Parinda Mehta, MD)

Experimental Hematology and Cancer Biology » Jose Cancelas, MD, PhD and James Mulloy, PhD

Gene therapy for Hemophagocytic Lymphohistiocytosis. (Michael Jordan, MD)

Experimental Hematology and Cancer Biology » Punam Malik, MD

Novel methods to promote stem cell engraftment. (Michael Jordan, MD)

Experimental Hematology and Cancer Biology » Punam Malik, MD

Biology of MAP2K1 mutations in histiocytoses. (Ashish Kumar, MD, PhD)

Experimental Hematology and Cancer Biology » Nicolas Nassar, PhD

Novel immune-dysregulatory diseases presenting as IBD. (Ashish Kumar, MD, PhD)

Gastroenterology, Hepatology and Nutrition » Lee Denson, MD

Discovering biomarkers and pathogenesis of hepatitis associated aplastic anemia. (Ashish Kumar, MD, PhD)

Gastroenterology, Hepatology and Nutrition » Alexander Miethke, MD

The clinical phenotype of children with Fanconi anemia caused by biallelic FANCD1/BRCA2 mutations. Pediatr Blood Cancer. 2011 (Kasiani Myers, MD, Stella Davies, MB BS, PhD, MRCP, Richard Harris, MD, Parinda Mehta, MD)

Human Genetics » Teresa Smolarek, PhD and S. Zimmerman, PhD

Pathology » Richard McMasters, MD

Project: Abnormalities in the alternative pathway of complement in children with hematopoietic stem cell transplant-associated thrombotic microangiopathy. Manuscript is published in Blood (Jodele et al, Blood. 2013 Jun 27. [Epub ahead of print]) (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

Human Genetics » Kejian Zhang, MD, MBA

Nephrology » Bradley Dixon, MD and Jens Goebel, MD

Project: “Complement gene testing in patients with hematopoietic stem cell transplant-associated thrombotic
microangiopathy"
Project support: The Investigator Initiated Trial Grant from Alexion Therapeutics. (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

Human Genetics » Kejian Zhang, MD, MBA
Hematology » Ralph Gruppo, MD

Genotyping for PID and HLH (Alexandra Filipovich, MD, Jacob Bleesing, MD, PhD)

Human Genetics » Kejian Zhang, MD, MBA

Genetics of hepatitis-associated aplastic anemia (Ashish Kumar, MD, PhD)

Human Genetics » Kejian Zhang, MD, MBA

Identification of novel mutations in CVID (Ashish Kumar, MD, PhD)

Human Genetics » Kejian Zhang, MD, MBA

Development of molecular diagnostic assays for LCH (Ashish Kumar, MD, PhD)

Human Genetics » Kejian Zhang, MD, MBA

Discovery and characterization of new primary immune deficiencies (Michael Jordan, MD, Rebecca Marsh, MD)

Human Genetics » Kejian Zhang, MD, MBA


Human Genetics » Kejian Zhang, MD, MBA
Nephrology » Bradley Dixon, MD and Jens Goebel, MD

Role of Pharmacogenetics and Therapeutic Drug Monitoring in BMT Patients (Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP)

Clinical Pharmacology » Sander Vinks, PharmD, PhD, FCP
Human Genetics » Kejian Zhang, MD, MBA

Assessment of minimal residual disease in ewing sarcoma. (Janos Sumegi, PhD)

Human Genetics » Teresa Smolarek, PhD

Exploiting the DNA damage response for selective immunomodulation (Michael Jordan, MD)

Immunobiology » David Hildeman, PhD and Jonathan Katz, PhD

Severe Congenital Neutropenia: Research project investigating novel genetic etiologies and molecular and cellular phenotypes of severe congenital neutropenia (Kasiani Myers, MD, Parinda Mehta, MD)

Immunobiology » H. Leighton Grimes, PhD

Incidence and Outcomes of Fungal Infections in Children after Bone Marrow Transplant: CCHMC Experience. (Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP)

Infectious Diseases » Beverly Connelly, MD
Incidence and outcomes of RSV infections in pediatric bone marrow transplant center. (Javier El-Bietar, MD, Stella Davies, MB BS, PhD, MRCP)

Infectious Diseases » Lara Danziger-Isakov, MD, MPH

“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 (Ongoing)
2. Center for Clinical and Translational Science and Training (CCTST), University of Cincinnati Academic Health Center, T1 Pilot Grant (Completed)

(Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP, Adam Lane, PhD, All BMT Faculty)

Biostatistics and Epidemiology » Jane Khoury, PhD

Nephrology and Hypertension » Prasad Devarajan, MD and Jens Goebel, MD

Project: “Retrospective analysis of BK virus infections in an existing cohort of pediatric hematopoietic cell transplant recipients at Cincinnati Children’s Hospital Medical Center” Manuscript published in Biology of Bone Marrow Transplantation journal. (Laskin, et al, Biol Blood Marrow Transplant. 2013 Aug;19(8):1175-82) (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

Nephrology and Hypertension » Jens Goebel, MD

Project: “Cystatin C to Estimate Renal Function in Bone Marrow Transplant and Oncology Patients” Publication: Biol Blood Marrow Transplantation (Laskin, et al, Biol Blood Marrow Transplant 2012 Nov; 18(11):1745-52) (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

Nephrology and Hypertension » Jens Goebel, MD

Biostatistics and Epidemiology » Jane Khoury, PhD

Project: " Complement C4d staining in tissue samples of patients with stem cell transplant associated thrombotic microangiopathy (SCT-TMA)" Manuscript is published in Transplantation journal (Laskin, et al, Transplantation. 2013 Jul 27;96(2):217-223) (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

Nephrology and Hypertension » Jens Goebel, MD

Project: Estimated versus measured glomerular filtration rate in children prior to hematopoietic cell transplantation. Manuscript is print in Biology of Bone Marrow Transplantation journal. (Laskin et al, Biol Blood Marrow Transplant. 2014) (Sonata Jodele, MD)

Nephrology and Hypertension » Jens Goebel, MD and Edward Nehus, MD, MS

Clinical assay for Eculizumab measurements in serum (Sonata Jodele, MD)

Nephrology and Hypertension » Bradley Dixon, MD

Novel therapeutic approaches for EV11+ leukemias (Ashish Kumar, MD, PhD)

Oncology » John Perentesis, MD, FAAP

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 (Kasiani Myers, MD)
Pharmacokinetics of Quercetin in patients with Fanconi anemia (Parinda Mehta, MD, Stella Davies, MB BS, PhD, MRCP)

**Pathology and Laboratory Medicine** » Kenneth Setchell, PhD

**Clinical Pharmacology** » Sander VInks, PharmD, PhD, FCP

Intestinal transplant-associated microangiopathy (iTMA) Project. Retrospective review of intestinal biopsy samples to explore the incidence, risk factors and outcomes of patients with iTMA. (Javier El-Bietar, MD, Stella Davies, MB BS, PhD, MRCP)

**Pathology and Laboratory Medicine** » Mikako Warren, MD

Therapeutic Drug Monitoring of Voriconazole in pediatric BMT patients. (Parinda Mehta, MD)

**Clinical Pharmacology** » Sander VInks, PharmD, PhD, FCP

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

**Clinical Pharmacology** » Sander VInks, PharmD, PhD, FCP

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

**Clinical Pharmacology** » Sander VInks, PharmD, PhD, FCP

Gene expression profiling of peripheral blood mononuclear cells from children with active hemophagocytic lymphohistiocytosis. (Janos Sumegi, PhD, Alexandra Filipovich, MD)

**Allergy and Immunology** » Kimberly Risma, MD, PhD

**Human Genetics** » Kejian Zhang, MD, MBA

**Rheumatology** » Alexi Grom, MD

Preparation of a stem cell transplant protocol for children with epidermolysis bullosa. (Sonata Jodele, MD, Stella Davies, MB BS, PhD, MRCP)

**Pediatric Dermatology** » Anne Lucky, MD

**Pediatric General and Thoracic Surgery** » Richard Azizkhan, MD

"Intersection of PI3K and DNA damage signaling for immune modulation" (Michael Jordan, MD)

**UC, Department of Cancer Biology** » D. Plas

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**Grants, Contracts, and Industry Agreements**

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<td><strong>Childhood Cancer Survivor Study</strong></td>
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<td>National Institutes of Health(St Jude's Children's Hospital)</td>
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<td><strong>COG Group Chair Award - Scientific Leadership</strong></td>
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<td>National Institutes of Health(Children's Hospital of Philadelphia)</td>
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Molecular Epidemiology of Pediatric Germ Cell Tumors
National Institutes of Health (University of Minnesota)
R01 CA 151284 08/10/11-05/31/16 $11,370

Children’s Oncology Group Chair - WorkLoad Intensity
National Institutes of Health (Children’s Hospital of Philadelphia)
U10 CA 098543 04/13/12-02/28/15 $5,775

FILIPOVICH, A

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 $54,140

Rare Diseases Clinical Consortia for the Rare Diseases
National Institutes of Health (The Regents of the University of California)
U54 AI 082973 09/12/09-08/31/14 $1,000

KUMAR, A

The Role of MEIS1 in Hematopoiesis and Hematopoietic Transformation
National Institutes of Health
R01 HL 111192 05/01/13-04/30/17 $271,032

MEHTA, P

Phase I Study of Quercetin for the Treatment of Fanconi Anemia
U.S. Food and Drug Administration
R01 FD 004383 09/16/13-07/31/16 $130,719

MYERS, K

Chemoprevention of Leukemia in a Genetically Susceptible Population
Conquer Cancer Foundation
07/01/12-06/30/15 $62,466

Current Year Direct $726,676

Industry Contracts

GRIMLEY, M
Baxter Healthcare Corporation $1,143
Chimerix Inc $58,769

HARRIS, R
Alexion Pharmaceuticals, Inc $1,155

JODELE, S
<table>
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<th>Company</th>
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<td>Alexion Pharmaceuticals, Inc</td>
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<td><strong>Current Year Direct Receipts</strong></td>
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