

Division Introduction

Under the direction of Stephen X. Skapek, M.D., the faculty, fellows, and more than 100 support and administrative staff in the Division of Pediatric Hematology-Oncology are dedicated to the fulfillment of a four-fold mission:

- The diagnosis and care of infants, children and adolescents with cancer and myriad hematologic disorders
- The education of medical students, residents, fellows, and other trainees, as well as provision of continuing education to practicing physicians
- Clinical, translational, and laboratory research aimed at improving and extending our knowledge about blood diseases and cancer
- Advocacy of our cause on behalf of the patients and families we serve

As the major program of its kind in North Texas and one of the largest in the United States each year, physicians in the Division primarily provide care in the [Pauline Allen Gill Center for Cancer and Blood Disorders](#) to hundreds of patients with uncommon and complex disorders. We also offer outreach that includes educational and consultative resources for primary and referring physicians in the region.



Stephen Skapek, M.D.

Faculty in the Division of Pediatric Hematology and Oncology are conducting molecular and cellular biology experiments in cancer and blood disease. Laboratory research efforts are both basic and translational studies that help to bridge the lab and clinical venues. Research is carried out in laboratories at UT Southwestern, the Children's Medical Center Research Institute and Children's HealthSM.

Our education mission includes medical students, pediatric residents, and hematology/oncology fellows. The Division also sponsors an innovative summer student internship program for outstanding premedical and medical students.

Faculty

The Division has a team of 24 faculty, nine fellows. Dr. Jacquelyn Powers joined the faculty in 2015, and she brings particular expertise in benign hematology, including iron deficiency anemia.

Jacquelyn M. Powers, M.D.

Assistant Instructor



B.A.

Rice University, Houston, TX, 2004

M.D.

UT Medical Branch at Galveston, Galveston, TX 2008

Postdoctoral Training

Residency, Pediatrics

UT Southwestern/Children's, 2008-2011

Chief Resident, Pediatrics

UT Southwestern/Children's, 2011-2012

Fellowship, Pediatric Hematology/Oncology

UT Southwestern, 2012-2015

Honors / Awards

Best Pediatric Specialists in Dallas, *D Magazine*

- George Buchanan
- Janna Journeycake
- Laura Klesse
- Patrick Leavey
- Tim McCavit
- Zora Rogers
- Stephen Skapek
- Naomi Winick

Texas Super Doctors, *Texas Monthly Magazine*

- George Buchanan
- Naomi Winick

Daniel Bowers

- Promotion to Professor of Pediatrics, September 2015

George Buchanan

- Named Annual Lectureship, "George Buchanan Lecture," American Society of Pediatrics Hematology/Oncology
- Lasting Impact Award, UT Southwestern Sickle Cell Disease Program
- Physician Champion Award, Children's Medical Center's Advanced Practice Program
- Wall of Fame Award, Webster Groves Providers (Missouri) High School

Jacquelyn Powers

- American Society of Hematology Clinical Research Training Institute (ASH CRTI)
- Burroughs-Wellcome Fund Trainee Travel Award, Association for Clinical and Translational Science

Zora Rogers

- Mom-Approved Doctor, June 2015, *Child Magazine*

Ayesha Zia

- CCRAC Research Award in Junior Investigator Category, UT Southwestern

Invited Lectures

Victor Aquino

- Pediatric Oncology Group, Texas Tech University, Lubbock, TX, May 2015
 - "Novel Cellular Therapies for Pediatric Leukemia"
- UT Health Science Center, San Antonio, TX, September 2015
 - "Novel Cellular Therapies for Pediatric Leukemia"

James Amatruda

- 2015 Malignant Germ Cell Tumor International Collaboration/Teenage Cancer Trust, University of Cambridge, Department of Pathology, Cambridge, UK, January 2015
 - "Integrating high-resolution genomics and genetic models to identify oncogenic drivers and novel targets for therapy of childhood germ cell tumors"

Janna Journeycake

- 2015 National Hemophilia Foundation Annual Meeting, Dallas, TX, August 2015
 - *"Hemophilia 101"*

Laura Klesse

- Texas Neurofibromatosis Education Day, Austin, TX, August 2015
 - *"Ongoing Clinical Trials for Neurofibromatosis"*
- Carol Zimmerman Lecture in Neuro-Ophthalmology, Dallas, TX, June 2015
 - *"Optic Gliomas in Neurofibromatosis"*
- UT Health Science Center, Internal Medicine Grand Rounds, Tyler, TX, December 2015

Andrew Koh

- Epiva Therapeutics, Cambridge, MA, July 2015
 - *"Role of the Gut Microbiota in the Development of GVHD"*
- Cincinnati Children's Hospital, Cincinnati, OH, September 2015
 - *"Role of the Gut Microbiota in the Health of Stem Cell Transplant Patients"*
- UT Southwestern, Department of Immunology Seminar, October 2015
 - *"Role of the Gut Microbiota in the Development of Infections and GVHD"*

Ted Laetsch

- 2015 Neuroblastoma and Medulloblastoma Translational Research Consortium (NMTRC) Symposium, Phoenix, AZ, May 2015
 - *"Panel Discussion: Moving Genomic Profiling to Clinical Care"*
- IGNITE Consortium Meeting, Cincinnati, OH, May 2015
 - *"Clinical Trials in MRI-guided HIFU for Pediatric Cancer"*
- Division of Hematology/Oncology, University of Oklahoma Health Sciences Center, Oklahoma City, OK, June 2015
 - *"Novel Cellular Therapies for Pediatric Leukemia"*
- Grand Rounds, Texas Tech University, Lubbock, TX, May 2015
 - *"Novel Cellular Therapies for Pediatric Leukemia"*
- Hematology/Oncology Grand Rounds, Cook Children's Hospital, Fort Worth, TX, July 2015
 - *"CART 19 Therapy for Pediatric ALL"*

Patrick Leavey

- American Society of Pediatric Hematology/Oncology Annual Meeting, Phoenix, AZ, May 2015
 - *"Pediatric Hematology/Oncology Workshop – Job Market and Trainees"*
- Pediatric Grand Rounds, Arkansas Children's Hospital, Little Rock, AR, May 2015
 - *"Pediatric Sarcoma – Advances in Treatment"*
- Taru Hays Hematology Symposium, Children's Hospital Colorado, Denver, CO, July 2015
 - *"Pediatric Sarcoma Advances"*

Jacquelyn Powers

- Texas Children's Hospital, Houston, TX, November 2015
 - *"Iron Replacement Strategies in Children with Iron Deficiency Anemia and the BESTIRON Clinical Trial"*
- St. Jude Children's Research Hospital, Memphis, TN, December 2015
 - *"Iron Replacement Strategies in Children with Iron Deficiency Anemia and the BESTIRON Clinical Trial"*

Naomi Winick

- National Children's Hospital, Pediatric Hematology-Oncology Grand Rounds, Washington, DC, March 2015
 - *"Developing an Adolescent and Young Adult Oncology Program"*
- 2018 COG Leukemia Planning Meeting, San Francisco, CA, July 2015
 - *"Recommendations for the Treatment of Children with Acute Lymphoblastic Leukemia and CNS Disease at Diagnosis"*

Zora Rogers

- ASPHO Review Course, Dallas, TX, February 2015
 - *"Bone Marrow Failure"*

Ayesha Zia

- The International Society of Haemostasis and Thrombosis, The Women's Health Subcommittee and Scientific Committee, Toronto, Canada, June 2015
 - *"Approach to Evaluation of Young Women with Heavy Menstrual Bleeding"*
- The International Society of Haemostasis and Thrombosis, The Neonatal and Pediatric Subcommittee and Scientific Committee, Toronto, Canada, June 2015
 - *"Adolescent Menorrhagia"*

Platform & Poster Presentations**James Amatruda and Ted Laetsch**

- *" β -lapachone: A Novel Targeted Therapy for Atypical Teratoid Rhabdoid Tumors (ATRTs)"*
Moore Z, Stuart S, Venkataraman A, Xu J, Erdreich-Epstein A, Rakheja D, Boothman DA, **Amatruda JF, Laetsch TW**
 - Poster Presentation: AACR Annual Meeting, Philadelphia, PA, April 2015
- *"EGFR and mTORC1 Are Novel Therapeutic Targets in Nonseminomatous Germ Cell Tumors"*
Budhipramono A, Chen C, Fustino N, Ateek C, Shukla A, Kapur P, Frazier AL, Lum L, Looijenga L, **Laetsch TW, Rakheja D, Amatruda JF**
 - Poster Presentation: AACR Advances in Pediatric Cancer Research Meeting, Ft. Lauderdale, FL, November 2015
- *"NQO1 as a Therapeutic Target for Atypical Teratoid/Rhabdoid Tumor"*
Meade JC, Moor JR, Stuart SH, Cholka A, Venkataraman A, Xu J, Erdreich-Epstein A, Rakheja D, Boothman DA, **Amatruda JF, Laetsch, TW**
 - Poster Presentation: AACR Advances in Pediatric Cancer Research Meeting, Ft. Lauderdale, FL, November 2015

Victor Aquino

- *"Nasal Endoscopy in the Evaluation of Prolonged Febrile Events in Children Undergoing Hematopoietic Stem Cell Transplantation"*
Aquino VM, Holmes S, Olivarez K, DeMasi J, Simms-Waldrup, Koh AY, and Siegel J
 - Poster Presentation: BMT Tandem Meetings, San Diego, CA, February 2015
- *"Increased Evidence of Fatigue in Pediatric Hematopoietic Stem Cell Transplantation Recipients"*
Sarkees J, Pavlock T, German J, Koh AY, and **Aquino VM**
 - Poster Presentation: BMT Tandem Meetings, San Diego, CA, February 2015
- *"Incidence and Outcome of Rhinovirus in Children Undergoing Hematopoietic Stem Cell Transplantation"*
DeMasi JM, Holmes S, Simms-Waldrup T, Koh AY and **Aquino VM**
 - Poster Presentation: BMT Tandem Meetings, San Diego, CA, February 2015

Daniel Bowers

- *“Morbidity and Mortality Associated with Subsequent Meningiomas Among Childhood Cancer Survivors Exposed to Cranial Radiotherapy: A Report from the Childhood Cancer Survivor Study (CCSS)”*
Bowers DC, Moskowitz CS, Chaya S, Chou JF, Mazewski CM, Neglia JP, Armstrong GT, Leisenring W, Oeffinger KC
 - Platform Presentation: American Society of Clinical Oncology (ASCO), Chicago, IL, June 2015
- *“Developmental Pharmacokinetics of Topotecan (TPT), a Renally Excreted Drug, in Infants and Young Children with Brain Tumors”*
Daryani VM, Harstead KE, Scott JF, Ward DA, Throm SL, **Bowers DC**, Bendel AE, Fisher PG, Wright KD, Broniscer A, Robinson GW, Gajjar AJ, Stewart CF
 - Poster Presentation: American Society of Clinical Oncology (ASCO), Chicago, IL, June 2015
- *“Morbidity and Mortality Associated with Subsequent Meningiomas Among Childhood Cancer Survivors Exposed to Cranial Radiotherapy: A Report from the Childhood Cancer Survivor Study (CCSS)”*
Bowers DC, Moskowitz CS, Chaya S, Chou JF, Mazewski CM, Neglia JP, Armstrong GT, Leisenring W, Oeffinger KC
 - Platform Presentation: 14th International Conference on Long-Term Complications of Treatment of Children and Adolescents for Cancer, Washington, DC, June 2015

George Buchanan, Tim McCavit and Jacquelyn Powers

- *“BESTIRON: A Single-center, Double-blind, Randomized, 12-week Superiority Study Comparing NovaFerrum Versus Ferrous Sulfate in the Treatment of Young Children with Nutritional Iron Deficiency Anemia”*
Powers JM, Shamoun M, **McCavit TL**, **Buchanan GR**
 - Poster Presentation: Translational Science 2015, Washington, DC, April 2015
- *“Efficacy and Safety of Ferric Carboxymaltose in Children with Iron Deficiency Anemia Not Responsive to Oral Iron Therapy”*
Powers JM, Shamoun M, **McCavit TL**, **Buchanan GR**
 - Poster Presentation: ASPHO 28th Annual Meeting, Phoenix, AZ, May 2015
- *“Low Dose Once Daily Oral Iron Treatment of Young Children with Nutritional Iron Deficiency Anemia”*
Powers JM, **McCavit TM**, Adix L, **Buchanan G**
 - Poster Presentation: ASH 57th Annual Meeting, Orlando, FL, December 2015

Janna Journeycake

- *“Pediatric Thrombophilia and Early Recurrence of Stroke”*
Jung J, **Journeycake J**, Dowling M
 - Poster Presentation: Pediatric Academic Society, San Diego, CA, May 2015
- *“Pediatric Traumatic Brain Injury on ECMO: Case Presentation”*
Anton-Martin P, Braga B, Megison S, **Journeycake J**, Taylor D, Potter D, Moreland J
 - Poster Presentation: 26th Annual ELSO Conference, Atlanta, GA, September 2015
- *“Thrombin Generation in Children after Acute Venous Thromboembolism”*
Zia A, **Journeycake J**, Sarode R
 - Poster Presentation: 58th Annual American Society of Hematology Meeting, San Diego, CA, December 2015
- *“Immune Tolerance Induction Using rFVIII-Fc (Eloctate)”*
Malac L, Ragni M, **Journeycake J**, Alabek M
 - Poster Presentation: 58th Annual American Society of Hematology Meeting, San Diego, CA, December 2015

Ted Laetsch

- *“MR-HIFU Mild Hyperthermia at an Ex Vivo Bone Interface Using Multi-directional Thermometry”*
Staruch R, Bing C, Nofiele J, Chopra R, **Laetsch TW**
 - Poster Presentation: International Society for Thermal Ultrasound Conference, Utrecht, Netherlands, April 2015

- *“MR-HIFU Hyperthermia for Drug Delivery: Heating Quality in Rabbit VX2 Tumors”*
Staruch R, Bing C, Ladouceur-Wodzak M, Nofiele J, Futch C, Chopra R, **Laetsch TW**
 - Poster Presentation: International Society for Thermal Ultrasound Conference, Utrecht, Netherlands, April 2015

Patrick Leavey and Stephen Skapek

- *“Whole Slide Images Stitching for Osteosarcoma Detection”*
Armaselu B, Arunachalam HB, Daescu O, Bach JP, Cederberg K, Rakheja D, Sengupta A, **Skapek S, Leavey P**
 - Poster Presentation: 5th IEEE International Conference on Computational Advances in Bio and Medical Sciences (ICCABS), Miami, FL, October 2015

Zora Rogers

- *“TCD with Transfusions Changing to Hydroxyurea (TWITCH): Hydroxyurea Therapy as an Alternative to Transfusions for Primary Stroke Prevention in Children with Sickle Cell Anemia”*
Russell E, Ware B, Davis R, Schultz WH, Brown C, Aygun B, Sarnaik SA, Odame I, Fuh B, George A, Owen W, Luchtman-Jones L, **Rogers ZR**, Hilliard L, Gauger Cy, Piccone CM, Lee MT, Kwiatkowski J, Jackson S, Miller ST, Roberts CW, Heeney MM, Kalfa TA, Nelson SC, Imran H, Nottage KA, Alvarez OA, Rhodes M, Thompson AA, Rothman J, Helton KJ, Roberts D, Coleman J, Bonner MJ, Kutlar A, Patel N, Wood JC, Piller L, Wei P, Luden J, Mortier NA, Stuber S, Luban NLC, Cohen AR, Pressel SL, Adams RJ
 - Poster Presentation: ASH Scientific Plenary Session, Orlando, FL, December 2015

Naomi Winick

- *“Neurocognitive Function of Children Treated for High-risk B-acute Lymphoblastic Leukemia (HR_ALL) Randomized to Capizzi (CMTX) Versus High-dose Methotrexate (HDMTX): A Report from the Children’s Oncology Group (COG)”*
 - Platform Presentation: ASCO, Chicago, IL, June 2015
- *“The Impact of Initial Cerebrospinal Fluid (CSF) Finding on Outcome Among Patients with NCI Standard (SR) and High-risk (HR) B-lymphoblastic Leukemia (B-ALL): A Report from the Children’s Oncology Group (COG) Studies AALL0331 and AALL0232”*
 - Platform Presentation: B-Lineage ALL Therapy 2018 Retreat, Chicago, IL, July 2015
- *“Neurocognitive Aims for the Coming 2018 ALL Trial”*
 - Platform Presentation: B-Lineage ALL Therapy 2018 Retreat, San Francisco, CA, July 2015

Education and Training

The Division of Pediatric Hematology and Oncology provides educational opportunities for medical students and pediatric residents, in addition to our fully accredited fellowship program. Our goal is to impart knowledge, instill excitement for learning, and translate questions into focused areas of research.

Medical Students

The Division of Pediatric Hematology-Oncology has taken a major role in the education of medical students at UT Southwestern.

Third-Year Medical Students

During their third year, medical students from UT Southwestern spend eight weeks at Children's Medical Center Dallas learning pediatrics. Approximately one-fourth of these students will spend two weeks on the Inpatient Hematology/Oncology Service. During this time, the students learn about and participate in the care of children with a wide

range of hematologic and oncologic disorders, including sickle cell disease, hemophilia, aplastic anemia, leukemia, lymphoma, brain tumors, bone tumors, and other childhood cancers.

Fourth-Year Medical Students

Fourth-year medical students have the option to participate in a four-week elective in the outpatient hematology/oncology clinics and the Pauline Allen Gill Center for Cancer and Blood Disorders, at Children's. During this elective, the students see children with cancer and blood disorders, as well as children who are referred to the Gill Center for further evaluation. This outpatient rotation allows the students to see these children in a more relaxed, "normal" setting than is possible in the inpatient area, where our children are often more acutely ill. With prior approval, this elective is also available for a limited number of fourth-year students from other medical schools.

Residents

Pediatric Hematology-Oncology is one of the core subspecialties for pediatric residents at UT Southwestern. All PL-1's spend four weeks covering the Inpatient Hematology/Oncology Service at Children's. Each month a PL-2 or PL-3 supervising resident and two or three PL-1s are assigned to the service.

The month spent on the rotation can be a tough time for residents, as they learn to take care of often very complicated and sick patients, some of whom may be dying. In retrospect, residents look back on this time as a very rewarding experience.

Division faculty are consistently praised by the residents for their devotion to education. With the institution of a "night-float" system, a new educational curriculum for the residents is being implemented. Over the course of the four-week rotation, several afternoons each week provide enhanced learning opportunities which may include lectures, pathology review, and bedside teaching, among others. The curriculum covers most, if not all, of the American Board of Pediatrics Content Specifications for "Disorders of the Blood and Neoplastic Disorders."

Pediatric residents may also elect to spend a month in the outpatient clinic at the Gill Center during their second or third year. This month allows the residents to learn about, and help care for, children with a wide range of hematologic or oncologic conditions to which they may never be exposed in the inpatient setting. Over the course of the month, the residents spend time in a number of clinics – general hematology, hemophilia, thrombosis, general oncology, neuro-oncology, and stem cell transplantation. They are also invited to attend the many educational programs offered by the Division, including weekly hemostasis and sickle cell team meetings, a weekly research seminar, and tumor board.

Fellows

The Division provides an unsurpassed opportunity for clinical fellowship training. Children's Medical Center Dallas, our primary pediatric teaching hospital, is the principal site for clinical training of our fellows. Directly adjacent to the UT Southwestern Medical Center campus, this hospital is consistently ranked as one of the nation's finest children's hospitals by US News and World Report. Importantly, its proximity to UT Southwestern allows clinical fellows to easily move between clinical and research training venues during their fellowship.

The Division prides itself on an atmosphere that welcomes new ideas, change, and creativity for fellowship education. The overall goals and objectives for pediatric hematology/oncology fellows are to gain extensive experience in the diagnosis and ongoing care of children with cancer and hematologic disorders, and to become researchers and teachers of pediatric hematology/oncology.

Fellow Research

Our Division includes physician scientists with funded and successful clinical and laboratory research programs. We provide the opportunity to obtain clinical, translational or basic laboratory research training at an institution that hosts a dazzling

array of world renowned investigators, including distinguished faculty who are Nobel laureates and many more who are members of the National Academy of Sciences, the Institute of Medicine, and Howard Hughes Medical Institute.

Research Activities

The Division of Pediatric Hematology and Oncology is nationally distinguished for its design and conduct of NIH-funded multi-center clinical trials involving childhood cancer, sickle cell disease, and hemorrhagic disorders. Division faculty are actively involved in numerous clinical research projects and laboratory research.

Laboratory Research

Faculty are conducting molecular and cellular biology experiments in cancer and blood disease. Laboratory research efforts are both basic and translational studies that help to bridge the lab and clinical venues. Research is carried out in laboratories in the Division of Hematology/Oncology and also across the entire UT Southwestern Medical Center campus, including the NCI-designated Simmons Cancer Center and the Children's Medical Center Research Institute at UT Southwestern.

Active areas of basic research in the Division include:

- Using fruit fly and zebrafish models to understand the genetic defects causing rhabdomyosarcoma, Ewing sarcoma and malignant germ cell tumor
- Using complementary pre-clinical models to dissect the key "vulnerabilities" in rhabdomyosarcoma
- Understanding the molecular machinery by which normal cells can undergo "senescence" as a tumor suppressor mechanism in the presence of a cancer-causing oncogene
- Identifying novel proteins that can be "targeted" as novel therapies in childhood cancer
- Understanding how certain cancer-causing mutations influence the metabolism in childhood brain tumors and certain types of sarcoma
- Uncovering how hematopoietic and embryonic stem cells are controlled and how these control mechanisms can go awry in cancer and blood disease
- Elucidating the molecular machinery that guides erythrocyte development
- Using novel model systems to elucidate the host and bacterial factors that cause invasive bacterial and fungal infections

Clinical Research

Physicians at the Gill Center are engaged in a wide range of clinical research efforts spanning the cancer and blood disease programs. Clinical research efforts are supported by robust infrastructure provided by the Clinical Research Office (CRO) within the Gill Center and the Simmons Comprehensive Cancer Center at UT Southwestern, the only NCI-designated cancer center in North Texas. At any point, 75 to 100 oncology trials and 20 to 30 hematology trials are open for enrollment for Gill Center patients.

Active areas of clinical research include:

- Prospective clinical trials for children with cancer, conducted under the umbrella of the NCI-sponsored Children's Oncology Group
- Prospective, early-phase clinical trials for children with hematological malignancies, conducted as part of the Therapeutic Advances in Childhood Leukemia and Lymphoma (TACL) consortium and other academic and industry partners
- Prospective therapeutic trials for children with sickle cell disease, iron deficiency anemia and hemophilia
- Investigator-initiated and industry-sponsored therapeutic studies of children with cancer and blood disease
- Retrospective research studies investigating molecular and clinical factors influencing late effects in childhood cancer survivors

- Prospective and retrospective studies assessing a variety of quality measures of children with chronic hematology disorders
- Early phase clinical trials of immunotherapeutics for childhood cancer, including the use of CAR T-cells for childhood leukemia.

The following list contains clinical studies approved by the Institutional Review Board (IRB) at UT Southwestern as of December 31, 2015 and excludes more than 70 Children's Oncology Group (COG) trials.

James Amatruda

- Archival Studies on Germ Cell Tumor Specimens

Victor Aquino

- Center for International Blood and Marrow Transplant Research (CIBMTR) - Consent for Participation and Donation of Blood Samples
- Evaluating Fatigue and Hope in Pediatric Hematopoietic Stem Cell Transplantation Recipients
- PIDTC 6901, A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders
- PIDTC 6902, A Retrospective and Cross-Sectional Analysis of Patients Treated for SCID Since January 1, 1968
- A Multicenter Access And Distribution Protocol For Unlicensed Cryopreserved Cord Blood Units (CBUs) For Transplantation In Pediatric And Adult Patients With Hematologic Malignancies And Other Indications
- Epidemiology and Outcome of BK-virus Associated Hemorrhagic Cystitis in Children After Hematopoietic Stem Cell Transplantation (HSCT)
- Outcomes of Hematopoietic Stem Cell Transplantation for the Treatment of Biphenotypic Leukemia: A Pediatric Blood and Marrow Transplant Consortium Study
- Outcome of haploidentical allogeneic peripheral blood stem transplantation in children with severe combined immunodeficiency
- A multicenter safety study of unlicensed investigational cryopreserved cord blood units (CBUs) manufactured by the National Cord Blood Program (NCBP) and provided for unrelated hematopoietic stem cell transplantation of pediatric and adult patients
- PIDTC Protocol # 6904, Analysis of Patients Treated for Wiskott-Aldrich Syndrome Since January 1, 1990
- PIDTC Protocol # 6903, Analysis of Patients Treated for Chronic Granulomatous Disease Since January 1, 1995
- Incidence and outcome of rhinovirus in children undergoing hematopoietic stem cell transplantation
- BP-U-004, Phase I/II study of CaspaCide T cells from an HLA-partially matched family donor after negative selection of TCR $\alpha\beta$ +T cells in pediatric patients affected by hematological disorders
- Use of Endoscopy for the Evaluation of Prolonged Fever and Neutropenia in Children Undergoing Hematopoietic Stem Cell Transplantation
- 13-TLEC, Natural History and Biology of Long-Term Late Effects Following Hematopoietic Cell Transplant for Childhood Hematologic Malignancies
- Hematopoietic Stem Cell Transplantation for CD40 Ligand Deficiency
- Multi-center study to Determine Risk Factors and Outcomes of Pediatric Hematology-Oncology/Bone Marrow Transplant Patients that Develop Central Line-Associated Bloodstream Infections (CLABSIs)
- A Prospective Study to Determine if "Rooming In" Improves Caregiver Satisfaction with Post Hematopoietic Stem Cell Transplant Discharge

Daniel Bowers

- Childhood Cancer Survivor Study
- Childhood Cancer Survivor Study Expansion: Long-Term Follow-up Study
- Evaluation of Radiation-Induced Vasculopathy by Transcranial Doppler (TCD) Among Survivors of Childhood Medulloblastoma Treated with Cranial Radiation Therapy

- Risk-Adapted Therapy for Young Children with Embryonal Brain Tumors, High-Grade Glioma, Choroid Plexus Carcinoma or Ependymoma (SJYC07)
- TOPNOC 001 (H-24549), A Phase II Study of Valproic Acid and Radiation, Followed by Maintenance Valproic Acid and Bevacizumab in Children with Newly Diagnosed High-Grade Gliomas or Brainstem Gliomas
- After the Cancer Experience (ACE) Database
- Metabolic Syndrome, Cardiovascular Risk and Insulin Resistance in Pediatric Survivors of Brain Tumors Treated with Radiation
- Phase II Trial of Molecularly Determined Treatment of Children and Young Adults with Newly Diagnosed Diffuse Intrinsic Pontine Gliomas
- SJMB12, A Clinical and Molecular Risk-Directed Therapy for Newly Diagnosed Medulloblastoma
- PBT0X1, Proton Beam Radiation Therapy vs. Conventional Beam Radiation Therapy: Toxicities During & After Craniospinal Radiation Therapy in Children
- CRAD001CUS224T, Phase II Study of Everolimus (RAD001, AFINITOR®) for Children with Recurrent or Progressive Ependymoma
- ACNS1221, A Phase II Study For The Treatment Of Non-Metastatic Nodular Desmoplastic Medulloblastoma In Children Less Than 4 Years Of Age

George Buchanan

- GFR Measurement and Urinary Substances in Children with Sickle Cell Anemia
- Severe Chronic Neutropenia International Registry
- Natural History of Immune Thrombocytopenic Purpura (ITP) during Childhood: The Dallas ITP Cohort Study
- Family Studies in Children with Unexplained Hematologic Disorders
- Amgen Protocol:20090340 An open label study evaluating the safety and efficacy of long-term dosing of Romiplostim in Thrombocytopenic pediatric subjects with immune (Idiopathic) Thrombocytopenia Purpura (ITP)
- Retrospective Review of Discontinuing Prophylactic Penicillin at 5 Years of Age in Children with Sickle Cell Anemia
- Jaundice in Hemolytic Anemia: Frequency, Severity, and Impact on Quality of Life
- ICON 1: Physician Treatment Decisions and Patient-Reported Outcomes in Pediatric Refractory Immune Thrombocytopenia

Janna Journeycake

- Glanzmann Thrombasthenia (GT) Human Research
- Zimmerman Program for the Molecular and Clinical Biology of VWD
- The American Thrombosis and Hemostasis Network (ATHN)
- Kids-DOTT: Prospective Multi-Center Evaluation of the Duration of Therapy for Thrombosis in Children (Protocol # 03-585)
- NN7999-3774 Safety, Efficacy and Pharmacokinetics of N9-GP in Previously Treated Children with Hemophilia B
- Hemophilia Inhibitor PUP Study (HIPS)
- CDC Public Health Surveillance for Bleeding Disorders - Registry for Bleeding Disorders Surveillance
- My Life Our Future: A Hemophilia Genotyping Initiative Data and Sample Research Repository
- A Phase III Open Label, Multicenter, Extension Study to Assess the Safety and Efficacy of Recombinant Coagulation Factor VIII (rVIII-SingleChain, CSL627) in Subjects with Severe Hemophilia A (CSL627_3001)
- A Longitudinal, Observational Study of Previously Treated Hemophilia Patients (PTPs) Switching Coagulation Replacement Factor Products (ATHN-2: Switching Study)

Laura Klesse

- Progression free survival and outcomes of primary spinal cord tumors in pediatrics
- Utility of Magnetic Resonance Imaging in Identifying Optic Gliomas in Children Less Than 3 Years of Age with Neurofibromatosis Type 1
- Progression Free Survival and Outcomes of Tectal Plate Lesions in Children
- The Role of Surgical Resection in Long Term Outcome for Children with Neuroblastoma

- Genomic Analysis of Recurrent Pediatric Medulloblastoma
- Neurofibromatosis associated plexiform neurofibromas analysis for novel therapeutic targets
- Bio-specimen Bank for Pediatric Tumors and Cancer Predispositions
- Developing Evidence-Based Criteria for Initiating Treatment for Neurofibromatosis type 1 Associated Optic Pathway Gliomas

Andrew Koh

- Role of Commensal Microbial Flora in Acute Intestinal Graft Versus Host Disease
- Role of Commensal Flora in the Development of Bacteremia and Fungemia in Cancer And Stem Cell Transplant Patients

Ted Laetsch

- T2009-012, A Phase I Dose Finding Study Of Panobinostat In Children With Refractory Hematologic Malignancies
- T2009-003, A Pilot Study of Decitabine and Vorinostat with Chemotherapy for Relapsed ALL
- A Phase II Study of Sirolimus and Erlotinib in Recurrent/Refractory Germ Cell Tumors
- Evaluation of NQO1 Expression in Pediatric Cancers
- A retrospective chart review to determine the time to and pattern of relapse in pediatric patients with recurrent sarcoma
- Radiological and Clinical Features of Pediatric Sarcoma and Neuroblastoma
- Assessing the precision of MR thermometry in Pediatric Solid Tumor Patients
- NMTRC 003B, A Phase II Preventative Trial of DFMO (eflornithine HCl) as a Single Agent in Patients with High Risk Neuroblastoma in Remission
- NMTRC V0706, A Phase II Trial of Nifurtimox for Refractory or Relapsed Neuroblastoma or Medulloblastoma
- CFZ008, Phase 1b/2 Study of Carfilzomib in Combination with Dexamethasone, Mitoxantrone, PEG-asparaginase, and Vincristine (UK R3 Induction Backbone) in Children with Relapsed or Refractory Acute Lymphoblastic Leukemia
- T2014-004, A retrospective cohort study of re-induction treatment outcome among pediatric patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)
- Panel Based Next Generation Sequencing for High Risk Pediatric Oncology Patients
- CCTL019B2202: A Phase II, single arm, multicenter trial to determine the efficacy and safety of CTL019 in pediatric patients with relapsed and refractory B-cell acute lymphoblastic leukemia
- CCTL019B2206: A multicenter study of apheresis collection of peripheral blood mononuclear cells (PBMC) in patients with CD19 expressing malignancies who could be eligible for a CTL019 clinical research trial
- CCTL019A2205B: Long Term Follow-Up of Patients Exposed to Lentiviral-Based CD19 directed CART Cell Therapy
- T2014-001, A Phase I Trial of Temsirolimus (CCI-779, Pfizer, Inc.) in Combination with Etoposide and Cyclophosphamide in Children with Relapsed Acute Lymphoblastic Leukemia and Non-Hodgkins Lymphoma
- The iCat2, GAIN Consortium Study, Multicenter Cohort Study To Evaluate Outcomes after Receipt of Targeted Therapy Matched to an Individualized Cancer Therapy (iCat) Recommendations in Children and Young Adults with Recurrent, Refractory, or High Risk Solid Tumors

Patrick Leavey

- Long-term Follow-up of Patients Enrolled on Children's Oncology Group Sponsored Research
- Identification of Anxiety and Depression in Children with Cancer
- Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas - Biospecimen Banking Protocol
- SPOC-2012-001, Phase 1 Dose-escalating Study of MM-398 (Irinotecan Sucrosfate Liposome Injection) plus Intravenous Cyclophosphamide in Recurrent or Refractory Pediatric Solid Tumors
- Pediatric Hematology/Oncology Workforce Evaluation in Texas
- Using Imaging and Computational Tools to Improve Risk Stratification in Children with Bone Cancer
- Programmed death ligand 1 (PD-L1) expression in pediatric sarcoma

Erika Lopez-Bertiery (Fellow)

- Ethnic Disparities in Pediatric Cancer Outcomes in Texas

Andrew Martin

- A retrospective review of toxicities in intermediate risk rhabdomyosarcoma patients treated on ARST0531

Tim McCavit

- Extended Survival Analysis of the Dallas Newborn Cohort
- Iron Deficiency Anemia and Menorrhagia in Adolescents
- Accuracy of Identification of Patients with Sickle Cell Disease and its Common Complications through ICD-9-CM Coding
- MST-188-01: Evaluation of Purified Poloxamer 188 in Vaso-occlusive Crisis of Sickle Cell Disease (EPIC): A Phase 3 Randomized, Double-blind, Placebo Controlled, Multicenter Clinical Trial of MST-188 (Purified Poloxamer 188) Injection in Subjects with Sickle Cell Disease Experiencing Vaso-occlusive Crisis

Jacquelyn Powers

- A single-center, double-blinded, randomized, 12 week, superiority study in infants and young children to compare the efficacy of NovaFerrum® versus Ferrous Sulfate in the treatment of nutritional iron deficiency anemia (BESTIRON)
- Efficacy and Safety of Low Molecular Weight Iron Dextran (LMWID) in Children with Iron Deficiency Anemia: Update of a single institution experience
- Efficacy and Safety of Ferric Carboxymaltose in Children with Iron Deficiency Anemia

Zora Rogers

- TWITCH - TCD With Transfusions Changing to Hydroxyurea: A Phase III randomized clinical trial to compare standard therapy (erythrocyte transfusions) with alternative therapy (hydroxyurea) for the maintenance of lowered TCD velocities in pediatric subjects with sickle cell anemia and abnormal pre-treatment TCD velocities
- Leucine in DBA: The Use of Novel Therapies to Reconstitute Blood Cell Production and Promote Organ Performance, using Bone Marrow Failure as a Model: A Pilot, Phase I/II Study of the Amino Acid Leucine in the Treatment of Patients with Transfusion-Dependent Diamond Blackfan Anemia
- Retrospective Study of Pediatric Aplastic Anemia

Tiffany Simms-Waldrip

- Defibrotide for Patients with Hepatic Venous-Occlusive Disease (VOD): A Treatment IND Study
- Compassionate Use for the CliniMACS CD34 Reagent System

Stephen Skapek

- Molecular characterization of childhood cancer specimens
- Establishment of a biorepository at the University of Texas Southwestern for the Study of PHPV and PVR

Tamra Slone

- Evaluation of the Influence of Abnormal Glucose Metabolism on the Risk of Infection in Children with Acute Lymphoblastic Leukemia and Lymphoblastic Lymphoma
- Secondary Acute Myeloid Leukemia and Myelodysplastic Syndrome after Treatment for Acute Lymphoblastic Leukemia
- Evaluation of the Risk Factors for Fungal Disease during Induction Therapy in Children with Acute Lymphoblastic Leukemia

- Longitudinal Assessment of Cardiotoxicity in long-term Cancer Survivors
- Evaluation of the Outcome of the Dallas Institutional Protocol for Treatment of Children with a Bone Marrow Relapse of Acute Lymphoblastic Leukemia
- Evaluation of the Safety of Discharge of Children with Acute Myeloid Leukemia at Completion of Chemotherapy and Prior to White Blood Cell Count Recovery
- Evaluation of Port Complications During Treatment of Pediatric Acute Lymphoblastic Leukemia
- Liver toxicity secondary to PEG-asparaginase during treatment for childhood acute lymphoblastic leukemia

Martha Stegner

- Once-Weekly intravenous liposomal amphotericin B (AmBisome) for fungal prophylaxis in pediatric high-risk hematologic malignancy: A retrospective evaluation of safety and tolerability
- Protocol#: Amgen20101221 A Single Arm, Open-label, Long-term Efficacy and Safety Study of Romiplostim in Thrombocytopenic Pediatric Subjects With Immune Thrombocytopenia (ITP)

Tanya Watt

- SPOC-2013-001, Phase I Study of Fenretinide (4-HPR, NSC 374551) Lym-X-Sorb(LXS) Oral Powder Plus Ketoconazole Plus Vincristine in Patients with Recurrent or Resistant Neuroblastoma (IND #: 68,254)
- SPOC-2014-001, Expanded Access Study of Fenretinide (4-HPR, NSC 374551) Lym-X-Sorb(LXS) Oral Powder Plus Ketoconazole in Patients with Recurrent or Resistant Neuroblastoma (IND #68,254)

Naomi Winick

- Evaluation of microRNAs as novel markers of cardiotoxicity in children undergoing anthracycline therapy for pediatric cancer
- Inpatient outcomes and chemotherapy related toxicities among a national cohort of children with Acute Leukemia
- A Randomized Evaluation of a Six-Week Grief Curriculum for Bereaved Parents
- The Feasibility and Acceptability of Incorporating Electronic Assessment Tools during Outpatient Visits for Patients in the Maintenance Phase of Therapy for Acute Lymphoblastic Leukemia
- Risk factors for toxicity during the induction and delayed intensification phases of treatment for acute lymphoblastic leukemia (ALL)
- A Family Bereavement Camp: Emerging Themes Regarding Its Impact on the Lives of Bereaved Parents and Siblings

Ayesha Zia

- Evaluation of Thrombin Generation in Children with Venous Thromboembolism
- Comprehensive and Multidisciplinary Approach to Evaluation of Young Women with Heavy Menstrual Bleeding (HMB): Impact on Diagnosis, Management and Outcomes
- Hemostatic Variables and Markers of Hypercoagulability in Adolescent Girls on Low Dose Estrogen Containing Oral Contraceptives (OCPs)

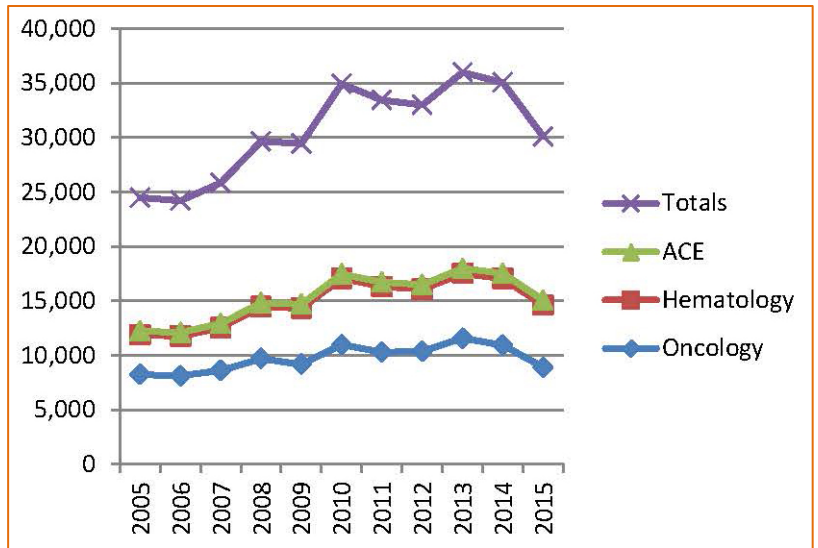
Research Funding

Clinical and laboratory research efforts are funded by a wide variety of national, regional and local organizations, such as the National Cancer Institute, the National Heart, Lung, and Blood Institute, National Eye Institute, Cancer Research and Protection Institute of Texas, American Cancer Society, St. Baldrick's Foundation, Children's Cancer Fund of Dallas, Children's Medical Center Foundation, Wipe-Out Kids' Cancer, the 1 Million for Anna Foundation, and Hyundai Hope on Wheels Foundation.

Clinical Activities

The Pauline Allen Gill Center for Cancer and Blood Disorders at Children’s Medical Center Dallas is the clinical site for most of the pediatric hematology and oncology care. The largest program of its kind in North Texas and across most of the middle United States, the Gill Center is internationally known for its excellence in patient care, education, clinical and laboratory research, and patient advocacy.

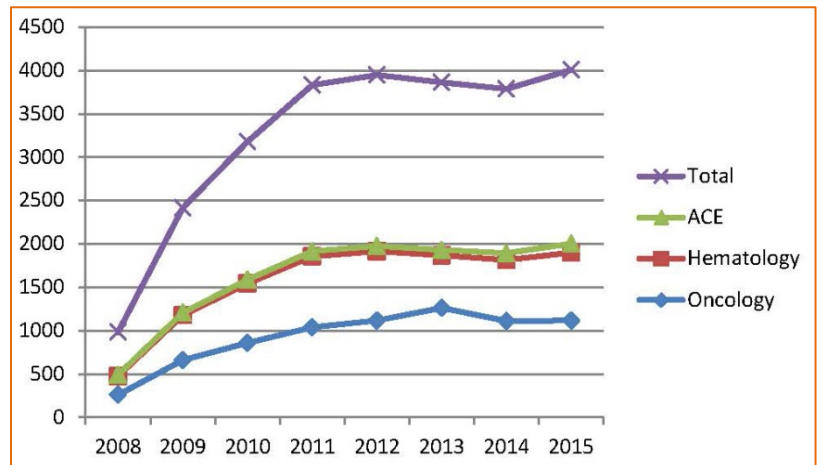
New sites for clinical care include the Children’s Medical Center Plano hospital for outpatient clinics and inpatient delivery of scheduled chemotherapy, and the Texas Health Resources Presbyterian Hospital for general hematology clinics.



Outpatient Visits - Dallas

Clinical Programs in Hematology and Oncology

- Brain Tumor
- Bone and Soft Tissue Sarcoma
- Bone Marrow Failure
- Genitourinary Neoplasms
- Hemophilia and Thrombosis
- Hemostasis and Thrombosis
- Hepatoblastoma
- Iron Deficiency and other General Hematology
- Leukemia/Lymphoma
- Neuroblastoma
- Rare Tumors
- Sickle Cell Disease/Hemoglobinopathies
- Stem Cell Transplant Programs
 - Transplant for Malignancy
 - Transplant for Non-malignant Disease
- Young Women’s Blood Disorders



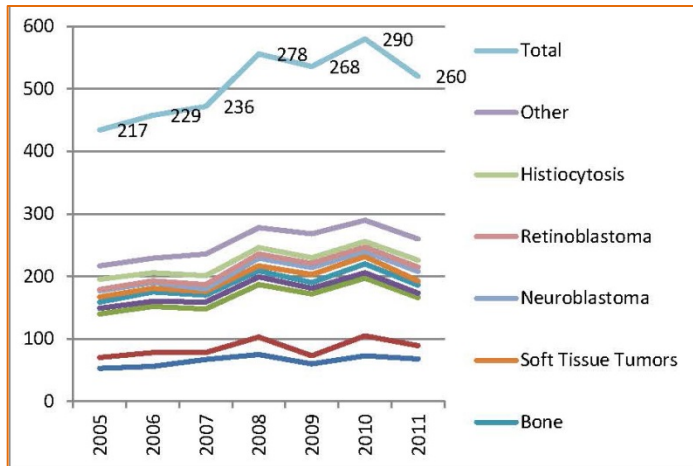
Outpatient Visits - Legacy

Additional Programs

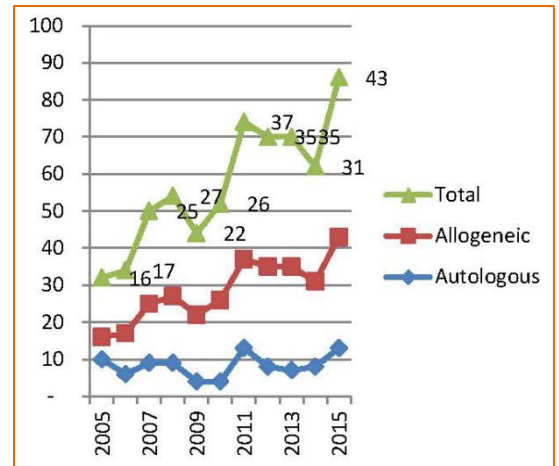
- After the Cancer Experience (ACE) Childhood Cancer Survivor Program
- Cancer Genetic Susceptibility Program
- Neurofibromatosis
- Adolescent and Young Adult Oncology
- Experimental Therapeutics for Cancer and Blood Disease
- Palliative Care Program

A multidisciplinary approach is used in the Gill Center to plan and deliver clinical care that is targeted to meet the needs of each child. Among the services offered are child life, social work, child psychiatry, nutritional support, pastoral care, physical and occupational therapy, and prosthetics services.

Faculty members also provide a consulting service for newborn patients with hematological conditions at Parkland Memorial Hospital, the 997-bed Dallas County hospital with approximately 16,000 newborn deliveries each year that is the site of the newborn nursery. New sites for hematology consultations include the newborn nursery at the Clements University Hospital and the Texas Health Resources Presbyterian Hospital.



New Oncology Patients



Stem Cell Transplants

Current Grant Support

James Amatruda

Grantor: CPRIT RP120685-C1
Title of Project: Central Sarcoma Processing Core
Role: Principal Investigator
Dates: 8/2012 – 8/2018

Grantor: CPRIT RP120685-P3
Title of Project: Functional Validation of Actionable Mutations in Sarcoma Genetic Model Systems
Role: Principal Investigator
Dates: 8/2012 – 8/2018

Grantor: NIH/NCI 1 R21 CA187516-01
Title of Project: A novel functional genomic pipeline for target identification in sarcoma
Role: Principal Investigator
Dates: 7/2014 – 6/2016

Grantor: Regents of the University of Minnesota
Title of Project: Molecular Epidemiology of Pediatric Germ Cell Tumors
Role: Principal Investigator
Dates: 8/2011 – 5/2016

Grantor: Alex's Lemonade Stand

Title of Project: REACH Award: B-lapachone as a novel targeted therapy for ATRT and other pediatric cancers

Role: Principal-Investigator

Dates: 1/2014 – 12/2016

Grantor: Alex's Lemonade Stand

Title of Project: Zebrafish modeling of PAX3-FOXO1 driven rhabdomyosarcoma

Role: Mentor

Dates: 7/2015 – 6/2017

Grantor: Catholic Foundation / Platt / Weir

Title of Project: Kevin's Ewing Sarcoma Fund

Role: Principal Investigator

Dates: 3/2015 – Current

Grantor: Curing Kids Cancer

Title of Project: Identification of targets for novel therapies of children's cancers

Role: Principal Investigator

Dates: 11/2013 – Current

Grantor: CCRAC

Title of Project: Identification of small-molecule agonists of bone morphogenetic protein signaling

Role: Principal Investigator

Dates: 7/2015 – 6/2016

Grantor: Children's Hospital of Philadelphia (on behalf of COG)

Title of Project: Amatruda – COG SMITSCS

Role: Principal Investigator

Dates: 3/2015 – 2/2016

Grantor: Children's Hospital of Philadelphia (on behalf of COG)

Title of Project: NIH National Clinical Trials Network Grant

Role: Principal Investigator

Dates: 3/2015 – 2/2016

Grantor: Dana-Farber Cancer Institute

Title of Project: Malignant Germ Cell Tumors International Consortium

Role: Principal Investigator

Dates: 7/2015 – 6/2020

Grantor: QuadW Foundation – American Association for Cancer Research

Title of Project: Zebrafish modeling of PAX3-FOXO1 driven Rhabdomyosarcoma

Role: Mentor

Dates: 7/2015 – 6/2016

Grantor: The 1 Million 4 Anna Foundation

Title of Project: Ewing Sarcoma Research Program

Role: Principal Investigator

Dates: 12/2015 – 11/2017

Daniel Bowers

Grantor: CPRIT

Title of Project: Genotype and Metabolic Phenotype in Pediatric Brain Cancer

Role: Co-Investigator

Dates: 6/2013 – 5/2016

Kenneth Chen

Grantor: Damon Runyon Cancer Research Foundation

Title of Project: Dysregulation of the MYCN/let-7 axis in Wilms tumor

Role: Fellow (J. Amatruda-Mentor)

Dates: 9/2013 – 8/2017

Grantor: CCRAC, W.W. Caruth Scholar

Title of Project: Dysregulation of the N-myc/Lin28/let-7 axis in childhood Wilms tumors

Role: Fellow (J. Amatruda-Mentor)

Dates: 6/2013 – Current

Grantor: CCRAC, Micaela's Army Foundation

Title of Project: Replacement therapy for miRNA-impaired Wilms tumors

Role: Principal Investigator

Dates: 11/2015 – Current

Grantor: Children's Cancer Foundation

Title of Project: A novel molecular prognostic approach to improve outcomes in childhood Wilms tumors

Role: Principal Investigator

Dates: 2/2015 – Current

Janna Journeycake

Grantor: Blood Center of Wisconsin, Inc. (NIH-NHLBI Flowthrough)

Title of Project: Comparative Effectiveness in the Diagnosis of VWD

Role: Principal Investigator

Dates: 12/2013 – 11/2018

Grantor: Blood Center of Wisconsin, Inc. (NIH-NHLBI Flowthrough)

Title of Project: Zimmerman Program for the Molecular and Clinical Biology of VWD

Role: Principal Investigator

Dates: 2/2015 – 1/2016

Laura Klesse

Grantor: Southwestern Medical Foundation / Dedman Family Scholarship Fund

Title of Project: Dedman Scholar Support

Role: Principal Investigator

Dates: 3/2009 – Current

Grantor: The Children's Tumor Foundation

Title of Project: Children's Tumor Foundation Support

Role: Principal Investigator

Dates: 1/2011 – Current

Ted Laetsch

Grantor: Micaela's Army Foundation

Title of Project: A Clinical Trial Combining Targeted Therapy for Pediatric and Young Adult Patients with Refractory Germ Cell Tumors

Role: Principal Investigator

Dates: 7/2014 – Current

Grantor: Hyundai Hope on Wheels

Title of Project: MR-guided High Intensity Focused Ultrasound (MR-HIFU) Hyperthermia for the Treatment of Pediatric Solid Tumors

Role: Principal Investigator

Dates: 1/2015 – 12/2016

Grantor: Alex Lemonade Stand Foundation

Title of Project: B-lapachone as a Novel Targeted Therapy for ATRTs and Other Pediatric Cancers

Role: Co-Principal Investigator

Dates: 1/2014 – 12/2016

Grantor: Children's Cancer Foundation

Title of Project: Experimental Therapeutics Program

Role: Principal Investigator

Dates: 12/2014 – Current

Grantor: Children's Cancer Foundation

Title of Project: Taking Advantage of Emerging Technologies to Bring Cancer Genetics to the Clinic

Role: Principal Investigator

Dates: 12/2014 – Current

Grantor: Rally Foundation for Childhood Cancer Research

Title of Project: Maximizing the Therapeutic Impact of CDK4/6 Inhibition in Rhabdomyosarcoma

Role: Principal Investigator

Dates: 4/2015 – 3/2016

Grantor: NCI

Title of Project: Image-guided Doxorubicin Delivery for Pediatric Sarcomas (MPI)

Role: Multi-Project Director/Principal Investigator

Dates: 7/2015 – 6/2020

Grantor: Trustees of Indiana University (NIH/NCI Flowthrough)

Title of Project: Development and Hyperactive RAS Tumor Spore

Role: Co-Investigator, Project 2

Dates: 9/2015 – 8/2020

Patrick Leavey

Grantor: CPRIT

Title of Project: Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas

Role: Co-Investigator and Principal Investigator Biospecimen Banking Study

Dates: 9/2012 – 8/2018

Grantor: CPRIT

Title of Project: Using Imaging and Computational Tools to Improve Risk Stratification in Children with Bone Cancer

Role: Principal Investigator

Dates: 3/2015 – 2/2019

Jacquelyn Powers

Grantor: NIH / National Center for Advancing Translational Sciences (NCATS)

Title of Project: UT Southwestern Center for Translational Medicine – KL2

Role: KL2 Scholar

Dates: 9/2013 –4/2018

Zora Rogers

Grantor: NIH / NHLBI

Title of Project: Pediatric Hydroxyurea Phase III Clinical Trial – Clinical Center (BABY HUG) Follow Up Study II

Role: Chair of Follow up Study II, Site Principal Investigator

Dates: 1/2012 –12/2016

Stephen Skapek

Grantor: NIH / NEI

Title of Project: Tgf β 2 Controls p19Arf During Eye Development

Role: Principal Investigator

Dates: 4/2014 –3/2018

Grantor: NIH

Title of Project: Cancer Center Support Grant

Role: Co-Investigator

Dates: 8/2015 –7/2020

Grantor: CPRIT

Title of Project: Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas

Role: Principal Investigator

Dates: 9/2012 –8/2018

Grantor: Indiana University / NIH

Title of Project: Developmental and Hyperactive Ras Tumor SPORE

Role: Clinical Co-Leader

Dates: 9/2015 – 8/2020

Grantor: Bear Necessities Pediatric Cancer Foundation

Title of Project: Maximizing the Therapeutic Impact of CDK/6 Inhibition in Rhabdomyosarcoma

Role: Co-Investigator

Dates: 4/2015 – 3/2016

Grantor: CPRIT

Title of Project: Using Imaging and Computational Tools to Improve Risk Stratification in Children with Bone Cancer

Role: Co-Investigator

Dates: 3/2015 – 2/2019

Tanya Watt

Grantor: Dedman Family Scholar
Title of Project: Neuroblastoma Program Project
Role: Principal Investigator
Dates: 10/2011 – Present

Yanbin Zheng

Grantor: NIH / NEI
Title of Project: Tgf Beta 2 controls p19Arf During Eye Development
Role: Co-Investigator
Dates: 4/2014 – 3/2018

Peer-Reviewed Publications

1. Aygun B, Mortier NA, Kesler K, Lockhart A, Schultz WH, Cohen AR, Alvarez O, **Rogers ZR**, et al. [Therapeutic phlebotomy is safe in children with sickle cell anaemia and can be effective treatment for transfusional iron overload.](#) *British Journal of Haematology* 2015;169:262-6.
2. **Aquino V**, Holmes S, Olivarez K, DeMasi J, **Simms-Waldrip TR**, **Koh AY**, **Siegel J**. [Nasal Endoscopy in the evaluation of prolonged febrile events in children undergoing hematopoietic stem cell transplantation.](#) *Biology of Blood and Marrow Transplantation* 2015;Feb; 21(2):S242-43.
3. Borowitz MJ, Wood BL, Devidas M, Loh ML, Raetz EA, Salzer WL, Nachman JB, Carroll AJ, Heerema NA, Gastier-Foster JM, Willman CL, Dai Y, **Winick NJ**, et al. [Prognostic significance of minimal residual disease in high risk B-ALL: a report from Children's Oncology Group study AALL0232.](#) *Blood* 2015;126:964-71.
4. Chen L, Shern JF, Wei JS, Yohe ME, Song YK, Hurd L, Liao H, Catchpoole D, **Skapek SX**, et al. [Clonality and evolutionary history of rhabdomyosarcoma.](#) *PLoS Genetics* 2015;11:e1005075.
5. DeMasi J, **Aquino V**, **Simms-Waldrip TR**, **Koh AY**, Holmes S. [Incidence and outcome of rhinovirus in children undergoing hematopoietic stem cell transplantation.](#) *Biology of Blood and Marrow Transplantation* 2015;Feb; 21(2):S248.
6. Diouf B, Crews KR, Lew G, Pei D, Cheng C, Bao J, Zheng JJ, Yang W, Fan Y, Wheeler HE, Wing C, Delaney SM, Komatsu M, Paugh SW, McCorkle JR, Lu X, **Winick NJ**, et al. [Association of an inherited genetic variant with vincristine-related peripheral neuropathy in children with acute lymphoblastic leukemia.](#) *Jama* 2015;313:815-23.
7. Dreyer ZE, Hilden JM, Jones TL, Devidas M, **Winick NJ**, et al. [Intensified chemotherapy without SCT in infant ALL: Results from COG P9407 \(Cohort 3\).](#) *Pediatric Blood & Cancer* 2015;62:419-26.
8. Estep JH, Melloni C, Thornburg CD, Wiczling P, **Rogers Z**, et al. [Pharmacokinetics and bioequivalence of a liquid formulation of hydroxyurea in children with sickle cell anemia.](#) *Journal of Clinical Pharmacology* 2016;56:298-306.
9. Fan D, Coughlin LA, Neubauer MM, Kim J, Kim MS, Zhan X, **Simms-Waldrip TR**, Xie Y, Hooper LV, **Koh AY**. [Activation of HIF-1 \$\alpha\$ and LL-37 by commensal bacteria inhibits *Candida albicans* colonization.](#) *Nature Medicine* 2015;21:808-14.
10. Fernandez CA, Smith C, Yang W, Mullighan CG, Qu C, Larsen E, Bowman WP, Liu C, Ramsey LB, Chang T, Karol SE, Loh ML, Raetz EA, **Winick NJ**, et al. [Genome-wide analysis links NFATC2 with asparaginase hypersensitivity.](#) *Blood* 2015;126:69-75.

11. Flood VH, Schlauderer AC, Haberichter SL, Slobodianuk TL, Jacobi PM, Bellissimo DB, Christopherson PA, Friedman KD, Gill JC, Hoffmann RG, Montgomery RR, Zimmerman Program Investigators (**Journeycake J**). [Crucial role for the VWF A1 domain in binding to type IV collagen](#). *Blood* 2015;125:2297-304.
12. Flores A, **Buchanan GR**. [Occult hemorrhage in children with severe immune thrombocytopenia](#). *American Journal of Hematology* 2015.
13. Frazier AL, Hale JP, Rodriguez-Galindo C, Dang H, Olson T, Murray MJ, **Amatruda JF**, et al. [Revised risk classification for pediatric extracranial germ cell tumors based on 25 years of clinical trial data from the United Kingdom and United States](#). *Journal of Clinical Oncology: Official Journal of the American Society of Clinical Oncology* 2015;33:195-201.
14. Gajjar A, **Bowers DC**, Karajannis MA, Leary S, Witt H, Gottardo NG. [Pediatric Brain Tumors: Innovative Genomic Information Is Transforming the Diagnostic and Clinical Landscape](#). *Journal of Clinical Oncology: Official Journal of the American Society of Clinical Oncology* 2015;33:2986-98.
15. Germann JN, Leonard D, Stuenzi TJ, Pop RB, Stewart SM, **Leavey PJ**. [Hoping Is Coping: A Guiding Theoretical Framework for Promoting Coping and Adjustment Following Pediatric Cancer Diagnosis](#). *Journal of Pediatric Psychology* 2015;40:846-55.
16. Goldenberg NA, Abshire T, Blatchford PJ, Fenton LZ, Halperin JL, Hiatt WR, Kessler CM, Kittelson JM, Manco-Johnson MJ, Spyropoulos AC, Steg PG, Stence NV, Turpie AG, Schulman S, Kids-DOTT Trial Investigators (**Journeycake J**). [Multicenter randomized controlled trial on Duration of Therapy for Thrombosis in Children and Young Adults \(the Kids-DOTT trial\): pilot/feasibility phase findings](#). *Journal of Thrombosis and Haemostasis: JTH* 2015;13:1597-605.
17. HaDuong JH, **Martin AA**, **Skapek SX**, Mascarenhas L. [Sarcomas](#). *Pediatric Clinics of North America* 2015;62:179-200.
18. Hingorani P, Missiaglia E, Shipley J, Anderson JR, Triche TJ, Delorenzi M, Gastier-Foster J, Wing M, Hawkins DS, **Skapek SX**. [Clinical Application of Prognostic Gene Expression Signature in Fusion Gene-Negative Rhabdomyosarcoma: A Report from the Children's Oncology Group](#). *Clinical Cancer Research: an Official journal of the American Association for Cancer Research* 2015;21:4733-9.
19. Holland AA, Hughes CW, Harder L, Silver C, **Bowers DC**, Stavinoha PL. [Effect of motivation on academic fluency performance in survivors of pediatric medulloblastoma](#). *Child Neuropsychology: a Journal on Normal and Abnormal Development in Childhood and Adolescence* 2015:1-17.
20. Karol SE, Yang W, Van Driest SL, Chang TY, Kaste S, Bowton E, Basford M, Bastarache L, Roden DM, Denny JC, Larsen E, **Winick N**, et al. [Genetics of glucocorticoid-associated osteonecrosis in children with acute lymphoblastic leukemia](#). *Blood* 2015;126:1770-6.
21. Kirane A, **Ludwig KF**, Sorrelle N, et al. [Warfarin Blocks Gas6-Mediated Axl Activation Required for Pancreatic Cancer Epithelial Plasticity and Metastasis](#). *Cancer Research* 2015;75:3699-705.
22. Kim J, Bu L, Koduru PR, Wilson KS, Fuda FS, Kumar KR, Timmons CF, **Slone TL**, Luu HS. [Novel r\(2\)\(p25q31\) cytogenetic abnormality in a pediatric patient with acute leukemia of ambiguous lineage](#). *Pediatric and Developmental Pathology: the Official Journal of the Society for Pediatric Pathology and the Paediatric Pathology Society* 2015;18:76-9.
23. Kumar M, Lambert MP, Breakey V, **Buchanan, GR**, et al. [Sports Participation in Children and Adolescents with Immune Thrombocytopenia \(ITP\)](#). *Pediatric Blood & Cancer* 2015;62:2223-5.
24. Leger K, **Slone T**, **Lemler M**, Leonard D, Cochran C, Bownan WP, Bashore L, **Winick N**. [Subclinical cardiotoxicity in childhood cancer survivors exposed to very low dose anthracycline therapy](#). *Pediatric Blood & Cancer* 2015;62:123-7.

25. Lopez-Medina E, Fan D, Coughlin LA, Ho EX, Lamont IL, Reimann C, Hooper LV, **Koh AY**. [Candida albicans Inhibits Pseudomonas aeruginosa Virulence through Suppression of Pyochelin and Pyoverdine Biosynthesis](#). *PLoS Pathogens* 2015;11:e1005129.
26. Lupo PJ, Danysh HE, Plon SE, Curtin K, Malkin D, Hettmer S, Hawkins DS, **Skapek SX**, et al. [Family history of cancer and childhood rhabdomyosarcoma: a report from the Children's Oncology Group and the Utah Population Database](#). *Cancer Medicine* 2015;4:781-90.
27. Mahajan P, **Leavey PJ**, Galindo RL. [PAX genes in childhood oncogenesis: developmental biology gone awry?](#) *Oncogene* 2015;34:2681-9.
28. Mathias MD, **McCavit TL**. [Timing of opioid administration as a quality indicator for pain crises in sickle cell disease](#). *Pediatrics* 2015;135:475-82.
29. Matloub Y, Stork L, Asselin B, Hunger SP, Borowitz M, Jones T, Bostrom B, Gastier-Foster JM, Carroll A, **Winick N**, et al. [Outcome of Children with Standard-Risk T-Lineage Acute Lymphoblastic Leukemia-Comparison among Different Treatment Strategies](#). *Pediatric Blood & Cancer* 2015.
30. Mir A, Frank S, **Journeycake J**, **Wolovits J**, Guleserian K, **Heistein L**, **Lemler M**. [Aspirin Resistance in Single-Ventricle Physiology: Aspirin Prophylaxis Is Not Adequate to Inhibit Platelets in the Immediate Postoperative Period](#). *The Annals of Thoracic Surgery* 2015;99:2158-64.
31. Mitchell HR, Lu X, Myers RM, Sung L, Balsamo LM, Carroll WL, Raetz E, Loh ML, Mattano LA Jr., **Winick NJ**, et al. [Prospective, longitudinal assessment of quality of life in children from diagnosis to 3 months off treatment for standard risk acute lymphoblastic leukemia: Results of Children's Oncology Group study AALL0331](#). *International Journal of Cancer Journal International du Cancer* 2015.
32. Moriyama T, Metzger ML, Wu G, Nishii R, Qian M, Devidas M, Yang W, Cheng C, Cao X, Quinn E, Raimondi S, Gastier-Foster JM, Raetz E, Larsen E, Martin PL, Bowman WP, **Winick N**, et al. [Germline genetic variation in ETV6 and risk of childhood acute lymphoblastic leukaemia: a systematic genetic study](#). *The Lancet Oncology* 2015;16:1659-66.
33. Neunert C, Noroozi N, Norman G, **Buchanan GR**, et al. [Severe bleeding events in adults and children with primary immune thrombocytopenia: a systematic review](#). *Journal of Thrombosis and Haemostasis: JTH* 2015;13:457-64.
34. Olson TA, Murray MJ, Rodriguez-Galindo C, Nicholson JF, Billmire DF, Krailo MD, Dang HM, **Amatruda JF**, et al. [Pediatric and Adolescent Extracranial Germ Cell Tumors: The Road to Collaboration](#). *Journal of Clinical Oncology: Official Journal of the American Society of Clinical Oncology* 2015;33:3018-28.
35. Pashankar F, Hale JP, Dang H, Krailo M, Brady WE, Rodriguez-Galindo C, Nicholson JC, Murray MJ, Bilmire DF, Stoneham S, Arul GS, Olson TA, Stark D, Shaikh F, **Amatruda JF**, et al. [Is adjuvant chemotherapy indicated in ovarian immature teratomas? A combined data analysis from the Malignant Germ Cell Tumor International Collaborative](#). *Cancer* 2016;122:230-7.
36. **Powers JM**, **McCavit TL**, **Buchanan GR**. [Management of iron deficiency anemia: a survey of pediatric hematology/oncology specialists](#). *Pediatric Blood & Cancer* 2015;62:842-6.
37. Ramsay E, Mougnot C, Kazem M, **Laetsch TW**, Chopra R. [Temperature-dependent MR signals in cortical bone: potential for monitoring temperature changes during high-intensity focused ultrasound treatment in bone](#). *Magnetic Resonance in Medicine: Official Journal of the Society of Magnetic Resonance in Medicine / Society of Magnetic Resonance in Medicine* 2015;74:1095-102.

38. Rescorla FJ, Ross JH, Billmire DF, Dicken BJ, Villaluna D, Davis MM, Krailo M, Cullen JW, Olson TA, Egler RA, **Amatruda JF**, Rodrigues-Galindo C, Frazier AL. [Surveillance after initial surgery for Stage I pediatric and adolescent boys with malignant testicular germ cell tumors: Report from the Children's Oncology Group](#). *Journal of Pediatric Surgery* 2015;50:1000-3.
39. Roy A, Kumar V, Zorman B, Fang E, Haines KM, Doddapaneni H, Hampton OA, White S, Bavle AA, Patel NR, Eldin KW, John Hicks M, Rakheja D, **Leavey PJ**, **Skapek SX**, **Amatruda JF**, et al. [Recurrent internal tandem duplications of BCOR in clear cell sarcoma of the kidney](#). *Nature Communications* 2015;6:8891.
40. Rudzinski ER, Anderson JR, Hawkins DS, **Skapek SX**, Parham DM, Teot LA. [The World Health Organization Classification of Skeletal Muscle Tumors in Pediatric Rhabdomyosarcoma: A Report from the Children's Oncology Group](#). *Archives of Pathology & Laboratory Medicine* 2015;139:1281-7.
41. Salzer WL, Jones TL, Devidas M, Dreyer ZE, Gore L, **Winick NJ**, et al. [Decreased induction morbidity and mortality following modification to induction therapy in infants with acute lymphoblastic leukemia enrolled on AALL0631: a report from the Children's Oncology Group](#). *Pediatric Blood & Cancer* 2015;62:414-8.
42. Savage WJ, **Buchanan GR**, Yawn BP, et al. [Evidence gaps in the management of sickle cell disease: A summary of needed research](#). *American Journal of Hematology* 2015;90:273-5.
43. Sectish TC, Hay WW, Jr., Mahan JD, Mendoza FS, Spector ND, Stanton B, Szilagyi PG, Turner TL, Walker LR, Slaw K, Members of the Federation of Pediatric Organizations' Visioning Summit Working, Groups Planning, Committee (**Leavey PJ**). [Blueprint for Action: Visioning Summit on the Future of the Workforce in Pediatrics](#). *Pediatrics* 2015;136:161-9.
44. **Simms-Waldrup T**, Rosen G, Nielsen-Saines K, Ikeda A, Brown B, Moore T. [Invasive fungal infections in pediatric hematopoietic stem cell transplant patients](#). *Infectious Diseases* 2015;47:218-24.
45. Tarlock K, Chang B, Cooper T, Gross T, Gupta S, Neudorf S, Adlard K, Ho PA, McGoldrick S, **Watt T**, et al. [Sorafenib treatment following hematopoietic stem cell transplant in pediatric FLT3/ITD acute myeloid leukemia](#). *Pediatric Blood & Cancer* 2015;62:1048-54.
46. Telen MJ, Wun T, **McCavit TL**, et al. [Randomized phase 2 study of GMI-1070 in SCD: reduction in time to resolution of vaso-occlusive events and decreased opioid use](#). *Blood* 2015;125:2656-64.
47. Thienprayoon R, Campbell R, **Winick N**. [Attitudes and Practices in the Bereavement Care Offered by Children's Hospitals: A Survey of the Pediatric Chaplains Network](#). *Omega* 2015;71:48-59.
48. Thienprayoon R, Lee SC, Leonard D, **Winick N**. [Hospice Care for Children With Cancer: Where Do These Children Die?](#) *Journal of Pediatric Hematology/Oncology* 2015;37:373-7.
49. Thienprayoon R, Marks E, Funes M, Martinez-Puente LM, **Winick N**, Lee SC. [Perceptions of the Pediatric Hospice Experience among English- and Spanish-Speaking Families](#). *Journal of Palliative Medicine* 2016;19:30-41.
50. Tubman VN, Fung EB, Vogiatzi M, Thompson AA, **Rogers ZR**, et al. [Guidelines for the Standard Monitoring of Patients With Thalassemia: Report of the Thalassemia Longitudinal Cohort](#). *Journal of Pediatric Hematology/Oncology* 2015;37:e162-9.
51. Ware RE, Davis BR, Schultz WH, Brown RC, Aygun B, Sarnaik S, Odame I, Fuh B, George A, Owen W, Luchtman-Jones L, **Rogers ZR**, et al. [Hydroxycarbamide versus chronic transfusion for maintenance of transcranial doppler flow velocities in children with sickle cell anaemia-TCD With Transfusions Changing to Hydroxyurea \(TWITCH\): a multicenter, open-label, phase 3, non-inferiority trial](#). *Lancet* 2016;387:661-70.47.

52. Ware RE, Davis BR, Schultz WH, Brown C, Aygun B, Sarnaik S, Odame I, Fuh B, George A, Owen W, Luchtman-Jones L, **Rogers ZR**, et al. [TCD with Transfusions Changing to Hydroxyurea \(TWITCH\): Hydroxyurea Therapy As an Alternative to Transfusions for Primary Stroke Prevention in Children with Sickle Cell Anemia](#). *Blood* 2015;126:3.
53. **Winick NJ**. [The evolution of central nervous system prophylaxis for patients with acute lymphoblastic leukemia](#). *Pediatric Blood & Cancer* 2015;62:1877-8.
54. Winter SS, Dunsmore KP, Devidas M, Eisenberg N, Asselin BL, Wood BL, Leonard Rn MS, Murphy J, Gastier-Foster JM, Carroll AJ, Heerema NA, Loh ML, Raetz EA, **Winick NJ**, Carroll WL, Hunger SP. [Safe integration of nelarabine into intensive chemotherapy in newly diagnosed T-cell acute lymphoblastic leukemia: Children's Oncology Group Study AALL0434](#). *Pediatric Blood & Cancer* 2015;62:1176-83.
55. Wood JC, Pressel S, **Rogers ZR**, et al. [Liver iron concentration measurements by MRI in chronically transfused children with sickle cell anemia: baseline results from the TWITCH trial](#). *American Journal of Hematology* 2015;90:806-10.
56. Wu SY, **McCavit TL**, Cederberg K, Galindo RL, **Leavey PJ**. [Chemotherapy for Generalized Infantile Myofibromatosis with Visceral Involvement](#). *Journal of Pediatric Hematology/Oncology* 2015;37:402-5.
57. Wylie A, Jones AE, D'Brot A, Lu WJ, Kurtz P, Moran JV, Rakheja D, **Chen KS**, Hammer RE, Comerford SA, **Amatruda JF**, Abrams JM. [p53 genes function to restrain mobile elements](#). *Genes & Development* 2016;30:64-77.
58. Xu H, Zhang H, Yang W, Yadav R, Morrison AC, Qian M, Devidas M, Liu Y, Perez-Andreu V, Zhao X, Gastier-Foster JM, Lupo PJ, Neale G, Raetz E, Larsen E, Bowman WP, Carroll WL, **Winick N**, et al. [Inherited coding variants at the CDKN2A locus influence susceptibility to acute lymphoblastic leukaemia in children](#). *Nature Communications* 2015;6:7553.
59. Yawn BP, **Buchanan G**, Hassell K. [Management of patients with sickle cell disease--reply](#). *Jama* 2015;313:91-2.
60. Yoon J, **Martin A**, Malicki D, Newbury R, Crawford J. [Unusual Spinal Cord Tumor in A 16-Month-Old Child](#). *Brain Pathology* 2015;25:784-5.
61. **Zia A**, Callaghan MU, Callaghan JH, et al. [Hypercoagulability in adolescent girls on oral contraceptives-global coagulation profile and estrogen receptor polymorphisms](#). *American Journal of Hematology* 2015;90:725-31.
62. **Zia AN**, Chitlur M, Rajpurkar M, et al. [Thromboelastography identifies children with rare bleeding disorders and predicts bleeding phenotype](#). *Haemophilia: the Official Journal of the World Federation of Hemophilia* 2015;21:124-32.
63. **Zia A**, O'Brien SH. [Global coagulation assays: A clinical perspective](#). *Journal of Thrombosis and Thrombolysis* 2015;39(1):89-94.

Book Sections

1. Frazier AL, Olson TA, Schneider DT, Perlman EJ, **Amatruda JF**. Germ Cell Tumors. In: Pizzo P, Poplack D, eds. *Principles and Practice of Pediatric Oncology*. 7th ed. Philadelphia: Wolts Kluwer; 2015.
2. Frazier AL, **Amatruda JF**. Germ Cell Tumors. In: Fisher DE, Nathan D, Look AT, eds. *Nathan and Oski's Textbook of Pediatric Hematology-Oncology*. 2nd ed. Philadelphia: Saunders; 2015.
3. **Martin AA**, **Leavey PJ**. Signs and Symptoms. In: Cripe TP, Yeager ND, eds. *Malignant Pediatric Bone Tumors - Treatment & Management*. 1st ed: Springer; 2015.