Division Introduction

Under the direction of Stephen X. Skapek, M.D., the faculty, fellows, and numerous support and administrative staff in the Division of Pediatric Hematology-Oncology continue to be 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 at Children’s Health Children’s Medical Center in Dallas and Plano. Each year, the team treats hundreds of children with cancer and blood diseases. We also offer outreach that includes educational and consultative resources for primary and referring physicians in the region.

Faculty in the Division of Pediatric Hematology and Oncology are conducting clinical as well as laboratory-based research in cancer and blood disease. Laboratory research efforts include both basic and translational studies that help to bridge the lab and clinical venues. The clinical research efforts include a portfolio of 50 or more clinical research studies extending from clinical trials sponsored by the National Cancer Institute through the Children’s Oncology Group; clinical research studies supported by other grant funding agencies, including the Cancer Prevention and Research Institute of Texas (CPRIT); and research studies carried out with industry partners. The research is carried out in laboratories at UT Southwestern, Children's Medical Center Research Institute and Children's Health℠.

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, and seven fellows. Drs. Kathryn Dickerson and Samuel John joined the faculty in 2017.

Kathryn E. Dickerson, M.D.
Assistant Instructor

B.S., cum laude
Indiana University, Bloomington, IN, 2006

M.D.
Indiana University School of Medicine, Indianapolis, IN, 2011

Postdoctoral Training
Residency, Pediatrics
Nationwide Children’s Hospital/Ohio State University, Columbus, OH, 2011-2014
Fellowship, Pediatric Hematology-Oncology
UT Southwestern Medical Center/Children’s Health, Dallas, TX, 2014-2017

Interests
Pediatric Myelodysplastic Syndrome (MDS), inherited acquired bone marrow failure syndromes, myeloproliferative disorders, acute myelogenous leukemia, and epigenetics
Samuel John, M.D.
Assistant Instructor
B.S.
Rensselaer Polytechnic Institute, Troy, NY, 2008
M.D.
Albany Medical College, Albany, NY, 2011
Postdoctoral Training
Residency, Pediatrics
UT Southwestern Medical Center/Children’s Health, Dallas, TX, 2011-2014
Fellowship, Pediatric Hematology-Oncology
UT Southwestern Medical Center/Children’s Health, Dallas, TX, 2014-2017
Interests
Childhood leukemia, including acute myelogenous leukemia (AML); experimental therapeutics of childhood cancer; cell-based immunotherapy of cancer using chimeric antigen receptor (CAR) T-cells
Honors / Awards
Promotions
• Laura Klesse, Associate Professor
• Andrew Koh, Associate Professor
• An Pham, Assistant Professor
• Tiffany Simms-Waldrip, Associate Professor
• Tamara Slone, Associate Professor
Best Pediatric Specialists in Dallas, D Magazine
• Daniel Bowers
• Janna Journeycake
• Laura Klesse
• Zora Rogers
• Stephen Skapek
• Naomi Winick
Texas Super Doctors, Texas Monthly Magazine
• Naomi Winick
Laura Klesse
• Dr. George Buchanan Fellow Teaching Award, Pediatric Hematology-Oncology Fellowship Training Program
Invited Lectures
Janna Journeycake
• Hemostasis and Thrombosis Research Society Scientific Symposium, Scottsdale, AZ, April 2017
  o “Catheter-related Thrombosis”
  o Trainee workshop, “DOACs”
Andrew Koh
• Immunology of Fungal Infections, Gordon Research Conference, Galveston, TX, January 2017
  o “Candida Albicans Gastrointestinal Colonization Resistance in the Mammalian Host”
• Antimicrobial Peptides, Gordon Research Conference, Ventura, CA, February 2017
  o “Activation of IL-37 by Commensal Bacteria Inhibits Candida Albicans Gastrointestinal Colonization and Dissemination”
- 7th FEBS Advanced Lecture Course on Human Fungal Pathogens, La Colle-sur-Loup, France, May 2017
  - “Commensal Anaerobic Gut Microbiota Promote Candida Albicans Colonization Resistance”
- Biology Department Seminar, Texas A&M University, College Station, TX, March 2017
  - “Bacterial-fungal Interactions in the Gastrointestinal Tract Modulate the Development of Invasive Microbial Infections in the Mammalian Host”
- University of Rochester Medical Center, Rochester, NY, March 2017
  - “Role of the Gut Microbiota in the Health of Cancer and Stem Cell Transplant Patients”
- Immunology Course, University of Maine, Bangor, ME, April 2017
  - “Hematopoietic Stem Cell Transplantation”

Theodore Laetsch

- Children’s Oncology Group Meeting, St. Louis, MO, March 2017
  - “Novel NTRK-fused Sarcomas: Therapeutic Implications”
- DHART SPORE Retreat, Indiana University, Indianapolis, IN, January 2017
  - “Targeted Therapies for Malignant Peripheral Nerve Sheath Tumors”
- FDA Mini-symposium, Washington, DC, April 2017
  - “TRK Inhibitors: New treatment, Potential New Toxicities – What We Would Like to Know”
- Texas Tech University, Lubbock, TX, June 2017
  - “Targeting Pediatric Cancer”

Patrick Leavey

- FACTOR Osteosarcoma Conference, Miami, FL, February 2017
  - “Development of a Computational tool to Interpret Osteosarcoma response to Chemotherapy”
- American Society of Pediatric Hematology/Oncology Annual Meeting, Montréal, Canada, April 2017
  - “Changing Workforce of Pediatric Hematology/Oncology”

Zora Rogers

- American Society of Pediatric Hematology/Oncology Board Review Course, Dallas, TX, February 2017
  - “Bone Marrow Failure”
- American Society of Pediatric Hematology/Oncology Annual Meeting, Montréal, Canada, April 2017
  - “Maintenance of Certification CME Course: TWITCH study”

Conference Presentations


  Poster, Pacific Symposium on Biocomputing, Hawaii, January 2017
  “Computer Aided Image Segmentation and Classification for Viable and Non-viable Tumor Identification for Osteosarcoma”


  Poster, American Society of Pediatric Hematology/Oncology Annual Meeting, Montréal, Canada, April 2017
  “Lymph Node Metastases in Pediatric and Young Adult Non-Rhabdomyosarcoma Soft Tissue Sarcoma: Findings from Children’s Oncology Group Study”

Poster, American Society of Pediatric Hematology/Oncology Annual Meeting, Montréal, Canada, April 2017
“Implementation of Computer-Based Image Pattern Recognition Algorithms to Interpret Tumor Necrosis: A First Step in Development of a Novel Biomarker in Osteosarcoma”

Neufeld E, Journeycake J, Aschman D, Cheng D, McCarthy E.

Poster, Hemostasis and Thrombosis Research Society Scientific Symposium, Scottsdale, AZ, April 2017
“A Real-World Assessment of New Coagulation Factors through the American Thrombosis and Hemostasis Network (ATHN)”

Salas N, Green-Henderson T, Journeycake J, Zia A.

Poster, Hemostasis and Thrombosis Research Society Scientific Symposium, Scottsdale, AZ, April 2017
“Developing a Multidisciplinary Young Women’s Blood Disorders Program: A Nursing Perspective”


Poster, 13th International Symposium on Bioinformatics Research and Applications, Honolulu, HI, May 2017
“Histopathological Diagnosis for Viable and Non-viable Tumor Prediction for Osteosarcoma Using Convolutional Neural Network”

Most Z, Meade J, Sue P, Winick N, Laetsch T.

Poster, Pediatric Academic Societies Meeting, San Francisco, CA, May 2017
“Cytomegalovirus Infection as a Cause of Fever and Morbidity among Pediatric Cancer Patients Receiving Chemotherapy without Hematopoietic Stem Cell Transplantation”


Oral, 34th Annual Society for Thermal Medicine Meeting, Cancun, Mexico, May 2017
“Effect of hyperthermia duration on MR-HIFU mediated doxorubicin delivery from thermosensitive liposomes: In vivo biodistribution and comparison with numerical models”


Poster, European Hematology Association Congress, Madrid, Spain, June 2017
“Global Registration Trial of Efficacy and Safety of CTL019 in Pediatric and Young Adult Patients with Relapsed/Refractory (R/R) Acute Lymphoblastic Leukemia (ALL): Update To the Interim Analysis”


Poster, ASCO Annual Meeting, Chicago, IL, June 2017
“Patient-reported Quality of Life (QOL) Following CTL019 in Pediatric and Young Adult Patients with Relapsed/refractory b-cell Acute Lymphoblastic Leukemia”

**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 continues to embrace its education of medical students at UT Southwestern.

**Third-Year Medical Students**
During their third year, medical students from UT Southwestern spend eight weeks in pediatrics training at Children's Medical Center Dallas, located on the UTSW campus. 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 tumor, bone tumor, 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 in 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 new patients referred to the Gill Center for further evaluation. This outpatient rotation allows the students to see these children in the clinic setting to complement learning 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 allows residents to learn to take care of what can be very complicated and sick patients with life threatening disease. Residents often look back on this time as a very rewarding experience.

Division faculty are consistently praised by the residents for their devotion to education. 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. 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, including 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, hematological malignancy and solid tumor patient care conferences, a weekly research seminar, and tumor board.

Fellows

The Division provides an excellent 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, this hospital is consistently ranked by US News and World Report as one of the nation’s finest children’s hospitals. Importantly, its proximity to UT Southwestern allows clinical fellows to easily move between clinical and research training venues during their fellowship.

2017 Incoming Fellows
Rida Abid, Michael Mitchell, Kendra Johnston
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 and clinical researchers 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. Nearly all of our fellows secure funding to support or, in some cases, to extend their research training. The numerous institutional resources enabling their ability to secure finding include a Physician Scientist Oncology T32 grant through the UTSW Simmons Cancer Center, an NCI designated Comprehensive Cancer Center.

**Research Activities**

Many faculty members of the Division of Pediatric Hematology/Oncology carry out laboratory and clinical research that is helping to reveal fundamental aspects of disease biology and beginning to lead to new, better therapies. Examples below represent just a sampling of that 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. Laboratory research projects are funded by a variety of mechanisms including research grants from the NIH National Cancer Institute and the Cancer Prevention and Research Institute of Texas.

**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, Ewing sarcoma, and other soft tissue sarcomas
- 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 the 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 in our Division 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 comprehensive 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. Clinical research projects are funded by a variety of mechanisms including funding from the NIH National Cancer Institute and the Cancer Prevention and Research Institute of Texas as well as other local and regional grant funding agencies and industry partners.

Particularly notable clinical research accomplishments in this past year focus on cancer. Our center was among only approximately 12 sites in the US that helped to demonstrate the usefulness of Kymriah, a CAR T-cell therapy targeting CD19-positive relapsed B-cell leukemia in children. Kymriah is now approved for this indication by the US Food and Drug Administration (FDA). In addition, Dr. Ted Laetsch, Assistant Professor of Pediatrics, was a lead investigator helping to develop LOXO-101 (larotrectinib) for children with cancer driven by \( TRK \) gene fusions. This drug was recently granted “Breakthrough Therapy” designation by the US FDA. Also notable this year is the fact that our site is selected to join the Neurofibromatosis (NF) Clinical Trials Consortium, a nationwide consortium of approximately 20 sites funded by the US Army Medical Research and Materiel Command. This selection enables Dr. Laura Klesse, Assistant Professor of Pediatrics, to provide the newest therapies in the form of clinical trials to children with neurofibromatosis, a neurodevelopmental and cancer predisposition syndrome. Finally, this year marked the initiation of a nascent Precision Medicine Program, co-led by Drs. Klesse and Kathleen Ludwig, Assistant Professor of Pediatrics, as a multifaceted program to identify causes of cancer in individual children and use that information to provide more “precise” therapies.

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 hematologic disorders
- Early phase clinical trials of immunotherapeutics for childhood cancer, including the use of CAR T-cells for childhood leukemia
- Prospective and translational research trials in children with venous thrombosis
- Clinical research in neurofibromatosis conducted as part of the national NF Clinical Trials Consortium
- Robust institutional Experimental Therapeutics and Precision Medicine Programs for children with cancer

The following list contains clinical studies approved by the Institutional Review Board (IRB) at UT Southwestern as of December 31, 2017, 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: Open
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- 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
- 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: Open
- 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
- BP-U-004, Phase I/II study of CaspaCide T cells from an HLA-partially matched family donor after negative selection of TCR αβ+ 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
- 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
- Risk-Adapted Therapy for Young Children with Embryonal Brain Tumors, High-Grade Glioma, Choroid Plexus Carcinoma or Ependymoma (SJYC07)
- After the Cancer Experience (ACE) Database
- SJMB12, A Clinical and Molecular Risk-Directed Therapy for Newly Diagnosed Medulloblastoma
- CRAD001CUS224T, Phase II Study of Everolimus (RAD001, AFINITOR®) for Children with Recurrent or Progressive Ependymoma

**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**

- 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
- 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
• Cardiovascular abnormalities in pediatric patients with neurofibromatosis type 1

Andrew Koh

• 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 Sucrosofate Liposome Injection) plus Intravenous Cyclophosphamide in Recurrent or Refractory Pediatric Solid Tumors
• Using Imaging and Computational Tools to Improve Risk Stratification in Children with Bone Cancer
• Programmed death ligand 1 (PD-L1) expression in pediatric sarcoma

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

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
- Compassionate Use for the CliniMACS CD34 Reagent System

Stephen Skapek
- Establishment of a biorepository at the University of Texas Southwestern for the Study of PHPV and PVR
- COG D9902 Soft Tissue Sarcoma Biospecimen Bank Study
- Establishment of Biospecimen Banking Study for Soft Tissue Sarcoma

Tamra Slone
- 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

Tanya Watt

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 NIH National Cancer Institute, National Heart, Lung, and Blood Institute, and National Eye Institute; the Cancer Research and Protection Institute of Texas; St. Baldrick’s Foundation, Children’s Cancer Fund of Dallas, Children’s Medical Center Foundation, Wipe-Out Kids’ Cancer, 1 Million for Anna Foundation, the Haggerty Family Foundation, Hyundai Hope on Wheels Foundation, and the Barrett Family Center for Pediatric Cancer.

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 the region, our program 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.

Core Clinical Programs in Hematology and Oncology

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

Additional Clinical/Research Programs

• After the Cancer Experience (ACE) Childhood Cancer Survivor Program
• Cancer Genetic Susceptibility Program
• Neurofibromatosis
• Adolescent and Young Adult Oncology
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 social work, child psychology/psychiatry, nutritional support, pastoral care, physical and occupational therapy, prosthetics services, and palliative care, where appropriate.

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.
Current Grant Support

James Amatruda

**Grantor:** 5 R01 CA151284-05 - University of Minnesota-NIH/NCI Principal Investigator  
**Title of Project:** Molecular Epidemiology of Pediatric Germ Cell Tumors  
**Role:** Principal Investigator  
**Dates:** 08/2011 – 05/2016

**Grantor:** CPRIT RP120685-C1  
**Title of Project:** C1: Central Sarcoma Processing Core  
**Role:** Principal Investigator  
**Dates:** 09/2012 – 08/2018

**Grantor:** CPRIT RP120685-P3  
**Title of Project:** P3: Functional Validation of Actionable Mutations in Sarcoma Genetic Model Systems  
**Role:** Principal Investigator  
**Dates:** 09/2012 – 08/2018

**Grantor:** Dana-Farber Institute/St. Baldrick's Subcontract #358099  
**Title of Project:** Malignant Germ Cell Tumors International Consortium  
**Role:** Principal Investigator  
**Dates:** 07/2015 – 06/2020

**Grantor:** RP160249 CPRIT  
**Title of Project:** DIS3L2 in Childhood Wilms Tumor: Mechanism to Medicines  
**Role:** Collaborator (Mendell)  
**Dates:** 03/2016 – 02/2020

**Grantor:** NIH 1 P50 CA196516-01-A1 (Project 4)  
**Title of Project:** Prognostic Significance and Therapeutic Potential of DROSHA Mutations in Wilms Tumor  
**Role:** Leader  
**Dates:** 08/2016 – 07/2021

**Grantor:** Alex’s Lemonade Stand Foundation  
**Title of Project:** DICER1-driven Cancers: Models, Mechanisms and Therapies  
**Role:** Principal Investigator  
**Dates:** 09/2016 – 08/2018

**Grantor:** 5 U10 CA 180884-03 / Children’s Hospital of Philadelphia  
**Title of Project:** Texas Pediatric Patient Derived Xenograft Facility  
**Role:** Co-Investigator (Adamson)  
**Dates:** 03/2017 – 02/2018

**Grantor:** 5 U10 CA 180884-03 / Children’s Hospital of Philadelphia  
**Title of Project:** NIH National Clinical Trials Network (NCTN)  
**Role:** Subcommittee Vice-Chair  
**Dates:** 03/2017 – 02/2018

**Grantor:** 1 Million for Anna Foundation
Title of Project: Determining the cell of origin of Ewing Sarcoma in a zebrafish genetic model  
Role: Principal Investigator  
Dates: 12/2015 – 11/2017

Grantor: Baylor/CPRIT, RFA R016-CFSA-2  
Title of Project: The Adolescent and Childhood Cancer Epidemiology and Susceptibility Service  
Role: Principal Investigator  
Dates: 06/2016 – 05/2021

Grantor: CPRIT RP170152  
Title of Project: Targeting the HNF4A and WNT/Beta-catenin pathways in childhood malignant  
Role: Co-Principal Investigator  
Dates: 12/2016 – 11/2020

Grantor: Baylor College of Medicine RP170071  
Title of Project: Genetic Epidemiology and Molecular Basis of Cancer Predisposition  
Role: Collaborator  
Dates: 12/2016 – 11/2019

Grantor: NIH/NCI 1P50CA196516-01-A1  
Title of Project: UTSW SPORE in Kidney Cancer Career Enhancement Program  
Role: Program Director  
Dates: 08/2016 – 07/2021

Grantor: Alex’s Lemonade Stand Foundation / Young Investigator  
Title of Project: The role of miRNA impairment in Wilms tumor formation  
Role: Mentor (Chen, Fellow)  
Dates: 06/2016 – 06/2019

Grantor: NIH/NCI 1K08 CA207849-01  
Title of Project: The role of 5p miRNA loss in Wilms tumor formation  
Role: Mentor (Chen, Fellow)  
Dates: 07/2016 – 06/2021

Victor Aquino

Grantor: Aquino - PIDTC #6902 / Children’s Hospital Los Angeles  
Title of Project: Per Case Reimbursement  
Role: Principal Investigator  
Dates: 04/2017 – 04/2018

Kenneth Chen

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
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: UT Health Science Center at Houston
Title of Project: HTC- Great Plains Regional Hemophilia Network
Role: Principal Investigator
Dates: 06/2017 – 05/2018

Grantor: UT Health Science Center at Houston
Title of Project: HTC- Great Plains Regional Hemophilia Network
Role: Principal Investigator
Dates: 06/2017 – 05/2018

Grantor: All Children’s Hospital
Title of Project: Kids-DOTT
Role: Principal Investigator
Dates: 04/2017 – 04/2018

Grantor: American Thrombosis & Hemostasis Network
Title of Project: A Longitudinal Observational Study of Previously Treated Hemophilia Patients
Role: Principal Investigator
Dates: 07/2017 – 06/2018

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

Grantor: Children’s Tumor Foundation
Title of Project: Developing Evidence-Based Criteria for Initiating Treatment for NF1-OPG
Role: Principal Investigator
Dates: 12/2017 – 11/2018

Grantor: Texas Neurofibromatosis Foundation
Title of Project: Cardiovascular abnormalities in pediatric patients with neurofibromatosis type 1
Role: Principal Investigator
Dates: 06/2016 – 05/2018
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: 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: NIH 1 R01 CA199937-01  
**Title of Project:** Image-guided doxorubicin delivery for pediatric sarcomas (MPI)  
**Role:** Co-Investigator (Chopra)  
**Dates:** 07/2015 – 06/2020

Grantor: University of Colorado  
**Title of Project:** A Phase I/Ib Study of Eribulin in Combination with Oral Irinotecan for Adolescent and Young Adult Patients with Relapsed or Refractory Solid Tumors  
**Role:** Principal Investigator  
**Dates:** 09/2017 – 08/2018

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:** 01/2015 – 12/2018

Grantor: US Department of Defense  
**Title of Project:** Eliminating ex-Vivo Manipulation and Viral Transfection of T-Cells in CAR-T Cell Immunotherapy of B-Cell Malignancies  
**Role:** Co-Investigator (Lux)  
**Dates:** 07/2017 – 06/2019

Grantor: NIH / Children’s Hospital of Philadelphia  
**Title of Project:** Match Committee-NIH National Clinical Trials Network (NCTN) Grant  
**Role:** Principal Investigator  
**Dates:** 03/2018 – 02/2019

Patrick Leavey

Grantor: Cancer Prevention Research Institute of Texas (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:** Cancer Prevention Research Institute of Texas (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

**Grantor:** Cancer Prevention Research Institute of Texas (CPRIT)  
**Title of Project:** Leavey - CPRIT IIRACCA  
**Role:** Principal Investigator  
**Dates:** 4/2014 – 2/2019

**Zora Rogers**

**Grantor:** NIH/St. Jude Children’s Research Hospital  
**Title of Project:** Hydroxyurea Management in Kids: Intensive versus Stable Dosage Strategies” (HUG KISS)  
**Role:** Principal Investigator  
**Dates:** 04/2016 – 03/2019

**Grantor:** MEDICAL UNIVERSITY OF SOUTH CAROLINA  
**Title of Project:** DISPLACE: Dissemination and Implementation of Stroke Prevention: Looking at the Care Environment  
**Role:** PI  
**Dates:** 07/01/2017- 06/30/2018

**Grantor:** NIH-NATIONAL HEART, LUNG AND BLOOD INST  
**Title of Project:** Baby Hug Follow-up Study II-Clinical Site  
**Role:** PI  
**Dates:** 08/01/2017- 04/30/2018

**Stephen Skapek**

**Grantor:** NIH/NEI  
**Title of Project:** Tgfβ2 Controls p19Arf During Eye Development  
**Role:** Principal Investigator  
**Dates:** 4/2014 –3/2018

**Grantor:** NIH/NCI  
**Title of Project:** Cancer Center Support Grant  
**Role:** Co-Investigator  
**Dates:** 8/2015 –7/2020

**Grantor:** Cancer Prevention Research Institute of Texas (CPRIT)  
**Title of Project:** Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas  
**Role:** Principal Investigator  
**Dates:** 9/2012 –8/2018

**Grantor:** Cancer Prevention Research Institute of Texas (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
**Grantor:** St. Baldrick’s Foundation  
**Title of Project:** Targeting LILRB4 by CAR-T cells for the treatment of pediatric AML  
**Role:** Mentor  
**Dates:** 07/2016 – 06/2018

**Grantor:** Cancer Prevention Research Institute of Texas (CPRIT)/UTHSCSA  
**Title of Project:** Texas Pediatric Patient Derived Xenograft Facility  
**Role:** Co-Investigator  
**Dates:** 06/2016 – 05/2021

**Grantor:** NIH  
**Title of Project:** Physician Scientist Oncology Training Program  
**Role:** PI  
**Dates:** 09/2009 – 08/2019

**Grantor:** NIH/NCI P30 CA142543  
**Title of Project:** Cancer Center Support Grant  
**Role:** Co-Leader of Development and Cancer Scientific Program  
**Dates:** 08/2010 – 07/2020

**Grantor:** Indiana University  
**Title of Project:** Developmental and Hyperactive RAS Tumor SPORE; Project 2: Targeted Therapies for Malignant Peripheral Nerve Sheath Tumor  
**Role:** Co-Leader Project 2  
**Dates:** 09/2015 – 08/30/2020

**Grantor:** CHOP/NIH (NCI) U10CA180884  
**Title of Project:** COG NCTN Solid Malignancy Integrated Translational Science Center  
**Role:** PI/MPI  
**Dates:** 03/2016 – 02/2018

**Grantor:** NIH/NHLBI  
**Title of Project:** Predicting and Preventing Poor Outcomes of Venous Thromboembolism in Children  
**Role:** Co-PI  
**Dates:** 05/2016 – 04/2021

**Grantor:** CHOP/NIH (NCI) UM1CA097452  
**Title of Project:** COG Phase 1 Pilot Consortium  
**Role:** PI  
**Dates:** 05/2017 – 03/2018

**Grantor:** CHOP/NIH  U10CA180886  
**Title of Project:** NIH NCTN Scientific Council  
**Role:** PI  
**Dates:** 03/2017 – 02/2019

**Grantor:** CPRIT RP120685-AC  
**Title of Project:** AC: Molecularly Targeted Therapy for Soft Tissue Sarcoma  
**Role:** PI  
**Dates:** 09/2012 – 08/2018
**Grantor:** CPRIT RP120685-P2  
**Title of Project:** P2: High Throughput Screening for Sarcoma Cell Proliferation and Survival Factors  
**Role:** PI  
**Dates:** 09/2012 – 08/2018

**Tanya Watt**

**Grantor:** SOUTH PLAINS ONCOLOGY CONSORTIUM  
**Title of Project:** SPOC 2014-001 Expanded Access Study of Fenretinide  
**Role:** PI  
**Dates:** 03/01/2015- 02/28/2018

**Ayesha Zia**

**Grantor:** NIH/NHLBI  
**Title of Project:** Predicting and Preventing Poor Outcomes of Venous Thromboembolism in Children  
**Role:** Principal Investigator  
**Dates:** 05/2016 – 04/2021

**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**


34. Powers JM, Buchanan GR. Potential for Improved Screening, Diagnosis, and Treatment for Iron Deficiency and Iron Deficiency Anemia in Young Children. *J Pediatr* 2017 Sep; 188:8-10. PMID: 28549635


