

## 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 and 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



**Stephen X. Skapek, M.D.**  
**Division Chief**

As the largest cancer and blood disease program in North Texas and one of the largest in the United States, each year physicians in the Division provide care for more than 300 children with newly diagnosed cancer and close to 1,000 children with newly diagnosed blood disease. Care is primarily provided in the Pauline Allen Gill Center for Cancer and Blood Disorders at Children’s Health Children’s Medical Center in Dallas and Plano. We continue to work toward increasing our geographic footprint by increasing the scope and scale of clinical care we can provide in Plano and other suburban sites. This includes a new outpatient hematology clinic at UT Southwestern Frisco and plans for a similar clinic at the RedBird facility. We also continue to look for opportunities to increase regional outreach by providing 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 diseases. Laboratory research efforts include both basic and translational studies that help 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 National Institutes of Health and 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 and the Children’s Medical Center Research Institute at UT Southwestern, as well as clinical sites within the Children’s Health system.

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 30 faculty and nine fellows.

### **Chelsee Greer, D.O.**

Assistant Professor



#### **D.O.**

University of North Texas Health Science Center, Fort Worth, TX, 2016

#### **Postdoctoral Training**

Residency, Pediatrics

University of Texas at Austin/Dell Children's Medical Center, Austin, TX, 2016-2019

Clinical Fellowship, Pediatric Hematology-Oncology

UT Southwestern, 2019-2022

Research Fellowship, Pediatrics

UT Southwestern, 2020-2022

#### **Interests:**

### **Margaret Nagel, M.D.**

Assistant Professor



#### **M.D.**

Chicago Medical School at Rosalind Franklin University, Chicago, IL, 2015

#### **Postdoctoral Training**

Residency, Pediatrics

University of Tennessee Health Sciences, Memphis, TN, 2015-2018

Fellowship, Pediatric Hematology-Oncology

St. Jude Children's Research Hospital, Memphis, TN, 2018-2021

Fellowship, Pediatric Solid Tumor

St. Jude Children's Research Hospital, Memphis, TN 2021-2022

**Interests:** Soft tissue sarcomas, rhabdomyosarcoma, AYA

### **Arhanti Sadanand, M.D.**

Assistant Professor



#### **M.D.**

Virginia Commonwealth University School of Medicine, Richmond, VA, 2016

#### **Postdoctoral Training**

Residency, Pediatrics

St. Louis Children's Hospital/Washington University, Saint Louis, MO, 2016-2019

Fellowship, Pediatric Hematology-Oncology

Emory University School of Medicine/Children's Healthcare of Atlanta, Atlanta, GA, 2019-2022

**Interests:** Neuroblastoma, pediatric solid tumors

**Ruchika Sharma, M.D.**  
Associate Professor



**M.D.**

Maulana Azad Medical College, New Delhi, India, 2008

**Postdoctoral Training**

Residency, Pediatrics

University of Toledo, Toledo, OH, 2009-2012

Fellowship, Pediatric Hematology-Oncology/BMT

Nationwide Children's Hospital/Ohio State University, Columbus, OH, 2012-2015

Fellowship, Pediatric Hemostasis-Thrombosis

Nationwide Children's Hospital/Ohio State University, Columbus, OH, 2015-2016

**Interests:** Bleeding disorders, thrombosis, platelet disorders, immune hematology

## Honors / Awards

### **Best Pediatric Specialists in Dallas, *D Magazine***

- Laura Klesse
- Patrick Leavey
- Kathleen Ludwig
- Tamra Slone
- Tanya Watt
- Jonathan Wickiser

### **Texas Super Doctors, *Texas Monthly Magazine***

- Kenneth Chen
- Kathryn Dickerson, 2022 Texas Rising Star
- Samuel John, 2022 Texas Rising Star
- Patrick Leavey
- Kathleen Ludwig, 2022 Texas Rising Star
- Naomi Winick

### **Patrick Leavey**

- Professor (Tenured)

### **Ayesha Zia**

- George R. Buchanan Teaching Award for Excellence in Fellow Education – UT Southwestern/Hematology-Oncology

## Invited Lectures

### **Victor Aquino**

- MD-2 Lions of Texas Diabetes, Wellness & Beyond Webinar Series, Dallas, TX, January 2022
  - "Beating the Odds with Childhood Cancer"

### **Daniel Bowers**

- St. Jude / VIVA Foundation, Childhood Cancer Survivorship Care Webinar Series, Singapore (virtual webinar), May 2022
  - "Late Occurring Stroke Among Childhood Cancer Survivors"

### **Kenneth Chen**

- DICER1 Symposium, Children’s Minnesota, Virtual, May 2022
  - *“Pineoblastoma: miRed in Development”*

### **Kathleen Ludwig**

- Pediatric Grand Rounds, Keck School of Medicine of University Southern California, Los Angeles, CA, May 2022
  - *“Making Sense of Vascular Anomalies, a pediatric overview”*

### **Ksenya Shliakhtsitsava**

- Children’s Oncology Group (COG) Annual Meeting, New Orleans, LA, September 2022
  - *“AMH as a Screening Tool to Assess for Diminished Ovarian Reserve”*

### **Ayesha Zia**

- International Society on Thrombosis and Haemostasis (ISTH) Congress Annual Meeting, London, England, July 2022
  - *“Heavy menstrual Bleeding: Which subgroup should be labeled as having Bleeding of Unknown Cause?”*

## **Conferences**

### **The American Society of Pediatric Hematology/Oncology (ASPHO) Conference, Pittsburgh, PA, May 2022**

Coleman M, Rachfal M, Williamson T, **John S**

Poster Presentation, *“Use of Crizotinib in a Neonate with a Life-Threatening, Alk-Positive, Low Grade Mesenchymal Neoplasm.”*

**Shliakhtsitsava K**, Baez Hernandez N, Butts R, Cochran C, Bonifacio J, McCaskill T, Bowers D

Poster Presentation, *“Building a Comprehensive Pediatric Cardio-Oncology Program at UT Southwestern in Dallas, Texas”*

Smith C, **Ludwig K**

Presentation, *“A case of familial hemophagocytic lymph histiocytosis presenting with coronary artery dilation”*

Stall M, **Watt T**

Abstract Presentation, *“Rapid Onset Bilateral Vision Loss as a Presenting Symptom of Burkitt Leukemia”*

### **Other Conferences**

Arnold DE, Parikh S, Logan B, Marsh RA, Griffith LM, Wu R, Mallhi K, Chellapandian D, Si S, Grunebaum E, Broglie LA, Prasad V, Heimall JR, Bunin NJ, Teira P, Touzot F, Burroughs LM, Petrovic A, Bleesing J, Chandra S, Kapoor N, Dara J, Dorsey MJ, Williams O, Kapadia M, Bednarski JJ, Bohnsack J, Chong H, Cuvelier G, Forbes LR, Vander Lugt MT, Yu Y, Chandrakasan S, Joshi A, Prockop S, Davila Saldana BJ, **Aquino V**, Ebens CL, Madden L, DeSantes K, Milner J, Rangarajan HG, Shah A, Gillio A, Knutsen A, Miller H, Moore T, Wright N, Cowan MJ, Dvorak CC, Haddad E, Kohn DB, Notarangelo LD, Pai SY, Puck JM, Pulsipher MA, Torgerson T, Malech HL, Kang EM, Leiding JW

TCT/Transplantation and Cellular Therapy Meetings, Salt Lake City, UT, April 2022

Abstract Presentation, *“Hematopoietic Cell Transplantation in 240 Patients with Chronic Granulomatous Disease: a PIDTC Report”*

**Bui A**

Neurofibromatosis Virtual Case Conference, Children's Tumor Foundation, New York, NY, January 2022  
Presentation, *"Management of Plexiform Neurofibromas, Malignant Peripheral Nerve Sheath Tumor, and Associated Treatment Side Effects"*

Leiding JW, Arnold DE, Parikh S, Logan B, Marsh RA, Griffith LM, Wu R, Mallhi K, Chellapandian D, Lim SS, Grunebaum E, Broglie LA, Prasad V, Heimall JR, Bunin NJ, Teira P, Touzot F, Burroughs LM, Petrovic A, Blessing J, Chandra S, Kapoor N, Dara J, Dorsey MJ, Williams O, Kapadia M, Bednarski JJ, Rayes A, Chen K, Chong H, Cuvelier GDE, Forbes LR, Vander Lugt MT, Yu L, Chandrakasan S, Joshi A, Prockop S, Blachy J, Davila Saldana BJ, **Aquino V**, Ebens CL, Madden L, DeSantes K, Milner J, Rangarajan HG, Shah A, Gillio A, Knutsen A, Miller H, Moore T, Wright N, Cowan MJ, Dvorak CC, Haddad E, Kohn DB, Notarangelo LD, Pai SY, Puck JM, Pulsipher MA, Torgerson T, Malech HL, Kang EM  
Clinical Immunology Society Annual Meeting, Charlotte NC, March 2022  
Abstract Presentation, *"Transplanted CGD Patients have Higher Burden of Disease than Conventionally Treated Patients that Improves with Transplantation – A PIDTC Report."*

Mascarenhas L, van Tilburg C, Doz F, Zwaan M, Albert C, Blattman C, Geoerger B, Nagasubramanian R, Pappo A, Singh A, **Watt T**, Norenberg R, Fellous M, De La Cuesta E, Laetsch T, Xu R  
American Society of Clinical Oncology (ASCO) Annual Meeting, Chicago, IL, June 2022  
Abstract Presentation, *"Efficacy and Safety of Larotrectinib in Pediatric Patients with Tropomyosin Receptor Kinase (TRK) Fusion-Positive Cancer: An Expanded Dataset"*

Sok P, Brown AL, Taylor OA, Bernhardt MB, Bernini JC, Erana RA, Griffin T, Heym K, Huynh VT, Klesse L, **Ludwig K**, Pruitt SL, Rabin KR, Scheurer ME, Lup PJ  
American Association for Cancer Research, New Orleans, LA, April 2022  
Presentation, *"Disparities in relapse among a large multi-ethnic population of children diagnosed with acute lymphoblastic leukemia (ALL): A report from the Reducing Ethnic Disparities in Acute Leukemia (REDIAL) Consortium."*

**Truscott J**

International Society for Pediatric Oncology Annual Congress, Barcelona, Spain, September 2022  
Oral Abstract, *"Outcomes Following CD19 CART Reinfusion in Children and Young Adults with B-ALL"*

**Watt T**

Oak Cliff Lion's Club, Dallas, Texas, October 2022  
Presentation, *"The Children's Cancer Journey"*

## Education and Training

The Division of Pediatric Hematology and Oncology continues to provide educational opportunities for medical students and pediatric residents, in addition to our Accreditation Council for Graduate Medical Education-accredited fellowship program in Pediatric Hematology/Oncology. Our goal is to impart knowledge, instill excitement for learning, and translate important clinical 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 through inpatient and outpatient experiences.

### 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 and Oncology is one of the core subspecialties for pediatric residents at UT Southwestern. All PL-1s 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 diseases. 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 the clinical training of our fellows. Directly adjacent to the UT Southwestern Medical Center, this hospital is consistently ranked by *U.S. 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.

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 the Howard Hughes Medical Institute. Nearly all of our fellows secure funding to support or, in some cases, extend their research training.

## **Research Activities**

Many faculty members of the Division of Pediatric Hematology and 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. The 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 across the entire UT Southwestern Medical Center campus, including the National Cancer Institute (NCI)-designated Simmons Comprehensive 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 National Institutes of Health (NIH) NCI and the Cancer Prevention and Research Institute of Texas.

#### **Active areas of basic research in the Division include:**

- Using zebrafish models to help understand metastasis
- Using complementary pre-clinical models to dissect the key “vulnerabilities” in rhabdomyosarcoma, Ewing sarcoma, and neuroblastoma
- Using complementary models to understand how defects in microRNA processing machinery, such as proteins encoded by the *DROSHA* and *DICER* genes, cause cancer in children
- Using novel computational approaches to nominate proteins that can be “targeted” as cancer therapeutics and gene-editing approaches, like CRISPR/Cas9, to validate their importance in pre-clinical models.
- Uncovering how hematopoietic and embryonic stem cells are controlled and how the control mechanisms can go awry in cancer and blood diseases, including bone marrow failure syndromes
- 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
- Developing new integrated computational analysis pipelines and applying artificial intelligence tools to interrogate molecular genomics and transcriptomics data as well as whole-slide digital pathology images
- Applying laboratory-based research tools to define prognostic determinants for outcomes in children with hemophilia and venous thromboembolism
- Elucidating the molecular mechanisms by which gut microbiota enhance anti-tumor immune responses
- Developing a novel gut microbiota-derived therapy to enhance cancer immunotherapy responses
- Investigating microbial drivers of autoimmunity to develop novel therapies for autoimmune complications in cancer and stem cell transplant patients

## 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 this point, there are 145 oncology trials and 31 hematology trials that 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.



Notable advances in our clinical research programs over the past year include the opening of a multicenter trial initiated by investigator Dr. Matt Campbell, Assistant Professor of Pediatrics, focused on the use of immunotherapy in combination with chemotherapy for pediatric patients with refractory solid tumors. On the hematology side, Dr. Ayesha Zia, Associate Professor of Pediatrics, is leading a multicenter study evaluating the effect of deep venous thrombosis and pulmonary embolism on exercise tolerance in patients. Our fertility preservation program for children with cancer, under the leadership of Dr. Ksenya Shliakhtsitsava, Assistant Professor of Pediatrics, has implemented an ongoing trial aimed at young male patients who currently have no clinical options.

The **Cell and Immunotherapy Program (CITP)** for childhood cancer and blood disease continues to grow its portfolio of therapy studies focused on cellular and immunotherapies, including a focus on genetic blood disorders such as hemophilia. Drs. Andrew Koh and Samuel John, seasoned faculty members in the Department of Pediatrics, are leading this multifaceted effort. Our **Experimental Therapies Program**, led by Dr. Campbell, continues to identify and develop new early-phase clinical trials for children with cancer and blood disease. Meanwhile, our Precision Medicine Program, co-led by Drs. Laura Klesse and Kathleen Ludwig, both Associate Professors of Pediatrics, has provided clinical tumor sequencing to over 400 pediatric patients with high-risk cancer diagnoses and has identified potential targetable variants in a third of patients, providing alternative 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 hematology 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 Neurofibromatosis (NF) Clinical Trials Consortium
- Robust institutional **cellular and immunotherapeutics, experimental therapeutics, and precision medicine programs** for children with cancer

The following list contains clinical studies and a sampling of the Institutional Review Board trials for children with cancer or blood disease at UT Southwestern:

#### Victor Aquino

- PIDTC 6908, Analysis of Autoinflammation in Chronic Granulomatous Disease Patients Undergoing Hematopoietic Cell Transplantation or Gene Therapy
- PIDTC 6907, Severe Combined Immune Deficiency: Prospective and Longitudinal Study of Genotypes, Management and Outcomes
- Global Registry for Long-Term Follow-up of Patients Participating in Clinical Trials with ALVR105 (Viralyim-M)
- TEAM Me (Totally Excited About Moving, Mobility, and Exercise) Clinical Research Protocol
- KD025-213, Phase 2, Randomized, Multicenter Study to Evaluate the Efficacy and Safety of KD025 in Subjects with Chronic Graft Versus Host Disease (cGVHD) After At Least 2 Prior Lines of Systemic Therapy (The ROCKstar Study)
- P-105-202, Phase 2, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Assess the Safety and Efficacy of Viralyim-M Compared to Placebo for the Prevention of AdV, BKV, CMV, EBV, HHV-6, and JCV Infection and/or Disease, in High-Risk Patients
- ALXN1210-TMA-314, A Phase 3, Open-label, Single Arm, Multicenter Study of Ravulizumab in Addition to Best Supportive Care in Pediatric Participants (from 1 month to < 18 years of age) with Thrombotic Microangiopathy (TMA) after Hematopoietic Stem Cell Tra
- ATA129-EBV-205, An Open-label, Single-arm, Multicohort, Phase 2 Study to Assess the Efficacy and Safety of Tabelecleucel in Subjects with Epstein-Barr Virus-associated Diseases
- BMT CTN 1702, Clinical Transplant-Related Long-term Outcomes of Alternative Donor Allogeneic Transplantation (CTRL-ALT-D)
- 17-SIBS, SUP1801, Identifying Predictors of Poor Health-Related Quality-of-Life among Pediatric Hematopoietic Stem Cell Donors
- PBMTN NMD 1801 (CSIDE), A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCR $\alpha\beta$ + /CD19+ depleted transplantation: A Phase II study by the Primary Immune Deficiency Treatment Conso
- GC P#05.01.020, A Multicenter, Randomized, Phase III Registration Trial of Transplantation of NiCord<sup>®</sup>, Ex Vivo Expanded, Umbilical Cord Blood-derived, Stem and Progenitor Cells, versus Unmanipulated Umbilical Cord Blood for Patients with Hematological Mal
- PBMTN SUP1701, Antiviral Cellular Therapy for Enhancing T-cell Reconstitution Before or After Hematopoietic Stem Cell Transplantation (ACES)
- Safety and Efficacy of Basiliximab and Infliximab in Children and Adolescents Undergoing Hematopoietic Stem Cell Transplantation
- PIDTC Protocol # 6903, Analysis of Patients Treated for Chronic Granulomatous Disease Since January 1, 1995
- PIDTC Protocol # 6904, Analysis of Patients Treated for Wiskott-Aldrich Syndrome Since January 1, 1990
- 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
- 10-CBA, 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
- PIDTC 6902, A Retrospective and Cross-Sectional Analysis of Patients Treated for SCID Since January 1, 1968
- PIDTC 6901, A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders
- Center for International Blood and Marrow Transplant Research (CIBMTR) - Consent for Participation and Donation of Blood Samples

## Daniel Bowers

- ACNS2031: A Phase 3 Study of Sodium Thiosulfate for the Reduction of Cisplatin-Induced Ototoxicity in Children with Average-Risk Medulloblastoma and Reduced Therapy for Children with Medulloblastoma with Low-Risk Features
- SJMB21: Phase 2 Study of Molecular and Clinical Risk-Directed Therapy for Infants and Young Children with Newly Diagnosed Medulloblastoma
- ONC201-108, ONC201 for the Treatment of Newly Diagnosed H3 K27M-mutant Diffuse Glioma Following Completion of Radiotherapy: A Randomized, Double-Blind, Placebo-Controlled, Multicenter Study
- DAY101-002, LOGGIC/FIREFLY-2: A Phase 3, Randomized, International Multicenter Trial Of DAY101 Monotherapy Versus Standard Of Care Chemotherapy In Patients With Pediatric Low-Grade Glioma Harboring An Activating RAF Alteration Requiring First-Line Systemic
- ACCL2031, A Phase 3 Randomized, Placebo-Controlled Trial Evaluating Memantine (IND# 149832) for Neurocognitive Protection in Children Undergoing Cranial Radiotherapy as Part of Treatment for Primary Central Nervous System Tumors
- Effect of Mineralocorticoid Receptor Antagonists Upon Anthracycline-Induced Cardiomyopathy
- ACNS2021, A Phase 2 Trial of Chemotherapy followed by Response-Based Whole Ventricular & Spinal Canal Irradiation (WVSCI) for Patients with Localized Non-Germinomatous Central Nervous System Germ Cell Tumor
- ACNS1931, A Phase 3 Study of Selumetinib (NSC# 748727, IND# 77782) or Selumetinib in Combination with Vinblastine for non-NF1, non-TSC Patients with Recurrent or Progressive Low-Grade Gliomas (LGGs) Lacking BRAFV600E or IDH1 Mutations
- ONC028: Intermediate-size Expanded Access to ONC201 for Patients with H3 K27M-mutant and/or Midline Gliomas
- ACNS1833, A Phase 3 Randomized Non-Inferiority Study of Carboplatin and Vincristine versus Selumetinib (NSC# 748727, IND# 77782) in Newly Diagnosed or Previously Untreated Low-Grade Glioma (LGG) not associated with BRAFV600E Mutations or Systemic Neurofib
- ONC018, Expanded Access to ONC201 for Patients with H3 K27M-mutant and/or Midline High Grade Gliomas
- ACNS1723, A Phase 2 Study of Dabrafenib (NSC# 763760) with Trametinib (NSC# 763093) after Local Irradiation in Newly-Diagnosed BRAFV600-Mutant High-Grade Glioma (HGG) (IND# 145355)
- Pilot Study of a Strict Classic Ketogenic Diet as a Therapy for Recurrent or Progressive and Refractory Brain Tumors in Children
- ACNS1422, A Phase 2 Study of Reduced Therapy for Newly Diagnosed Average-Risk WNT-Driven Medulloblastoma Patients
- MEK162, Phase I-II Study of MEK 162 for Children with Low-Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors
- CRAD001CUS224T, Phase II Study of Everolimus (RAD001, AFINITOR®) for Children with Recurrent or Progressive Ependymoma
- SJMB12, A Clinical and Molecular Risk-Directed Therapy for Newly Diagnosed Medulloblastoma

- ALTE11C2, Health Effects after Anthracycline and Radiation Therapy (HEART): Dexrazoxane and Prevention of Anthracycline-related Cardiomyopathy
- ACNS1123, Phase 2 Trial of Response-Based Radiation Therapy for Patients with Localized Central Nervous System Germ Cell Tumors
- Risk-Adapted Therapy for Young Children with Embryonal Brain Tumors, High-Grade Glioma, Choroid Plexus Carcinoma or Ependymoma (SJYC07)
- Evaluation of Radiation-Induced Vasculopathy by Transcranial Doppler (TCD) Among Survivors of Childhood Medulloblastoma Treated with Cranial Radiation Therapy
- ACNS0332, Efficacy of Carboplatin administered Concomitantly with Radiation and Isotretinoin as a Prop-Apoptotic Agent in Other Than Average Risk Medulloblastoma/PNET Patients.
- Childhood Cancer Survivor Study Expansion: Long-Term Follow-up Study
- Childhood Cancer Survivor Study

#### **Ashley Bui**

- ACNS1821, A Phase 1/2 Trial of Selinexor (KPT-330) and Radiation Therapy in Newly-Diagnosed Pediatric Diffuse Intrinsic Pontine Glioma (DIPG) and High-Grade Glioma (HGG)
- BLU-285-3101, A Phase 1/2, Single-arm Study to Evaluate the Safety, Pharmacokinetics, and Antitumor Activity of Avapritinib in Pediatric Patients with Solid Tumors Dependent on KIT or PDGFRA Signaling

#### **Erin Butler**

- HLH-RUXO: Use of a Response-Adapted Ruxolitinib-Containing Regimen for the Treatment of Hemophagocytic Lymphohistiocytosis.
- NACHO COBI, North American Consortium for Histiocytosis Cobimetinib Phase 2 Trial
- A Retrospective Review of our Institutional Use of Methotrexate in Langerhans Cell Histiocytosis
- LCH-IV: (NACHO) International Collaborative Treatment Protocol for Children and Adolescents with Langerhans Cell Histiocytosis

#### **Matthew Campbell**

- AOST2032, A Feasibility and Randomized Phase 2/3 Study of the VEGFR2/MET Inhibitor Cabozantinib in Combination with Cytotoxic Chemotherapy for Newly Diagnosed Osteosarcoma
- ARST2032, A Prospective Phase 3 Study of Patients with Newly Diagnosed Very Low-risk and Low-risk Fusion Negative Rhabdomyosarcoma
- AOST2031, A Phase 3 Randomized Controlled Trial Comparing Open vs Thoracoscopic Management of Pulmonary Metastases in Patients with Osteosarcoma
- ARST2031, A Randomized Phase 3 Trial of Vinorelbine, Dactinomycin, and Cyclophosphamide (VINO-AC) Plus Maintenance Chemotherapy with Vinorelbine and Oral Cyclophosphamide (VINO-CPO) vs Vincristine, Dactinomycin and Cyclophosphamide (VAC) plus VINO-CPO
- AOST2121: OST31-164-01 An Open-Label Phase 2 Study of Maintenance Therapy with OST31-164 After Resection of Recurrent Osteosarcoma
- VITAS: Atezolizumab in combination with chemotherapy for pediatric relapsed/refractory solid tumors: An open-label, phase I/II, single-arm, multi-center trial

- ARST1921, A Safety, Pharmacokinetic and Efficacy Study of a  $\gamma$ -Secretase Inhibitor, Nirogacestat (PF-03084014; IND# 146375), in Children and Adolescents with Progressive, Surgically Unresectable Desmoid Tumors
- ADVL2021, J1S-MC-JV01, A Randomized, Open-Label Phase 1/2 Study Evaluating Ramucirumab in Pediatric Patients and Young Adults with Relapsed, Recurrent, or Refractory Desmoplastic Small Round Cell Tumor
- MCC19487, A Phase Ib/II Study to Evaluate the Safety, Feasibility and Efficacy of Nivolumab or Nivolumab in Combination with Azacitidine in Patients with Recurrent, Resectable Osteosarcoma
- MCC20339, Evolutionary inspired therapy for newly diagnosed, metastatic, Fusion Positive Rhabdomyosarcoma
- ARST1431, A Randomized Phase 3 Study of Vincristine, Dactinomycin, Cyclophosphamide (VAC) Alternating with Vincristine and Irinotecan (VI) Versus VAC/VI Plus Temozolomide (TORI, Torisel, NSC# 683864, IND# 122782) in Patients with Intermediate Risk (IR) R

#### **Kathryn Dickerson**

- AG348-C-023: A Phase 3, Multicenter, Randomized, Double-blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of Mitapivat in Pediatric Subjects With Pyruvate Kinase Deficiency Who Are Not Regularly Transfused, Followed by a 5-Year Open-label
- NAPAAC Registry Study of Bone Marrow Failure and Myelodysplastic Syndromes
- AG348-C-022, A Phase 3, Multicenter, Randomized, Double-blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of Mitapivat in Pediatric Subjects With Pyruvate Kinase Deficiency Who Are Regularly Transfused, Followed by a 5-Year Open-label
- X4P-001-104, A Phase 1b, Open-Label, Multicenter Study of Mavorixafor in Patients with Severe Congenital Neutropenia and Chronic Neutropenia Disorders
- Defining Clonal Hematopoiesis in Childhood Cancer Survivors: A Novel Approach to Predicting Therapy-Related Myeloid Neoplasms and Other Late Effects [A prospective, single center study exploring clonal hematopoiesis in childhood cancer survivors]
- TransIT, Unrelated Donor Transplant Versus Immune Therapy in Pediatric Severe Aplastic Anemia PBMTC NMD1601
- Severe Chronic Neutropenia International Registry

#### **Jessica Garcia**

- ATLAS-NEO (EFC17574): A Phase 3, Single-arm, Multicenter, Multinational, Open-Label, One-Way Crossover Study to Investigate the Efficacy and Safety of Fitursiran Prophylaxis in Male Participants aged  $\geq$  12 Years with Severe Hemophilia A or B, With or Without
- The hemostatic potential and clot kinetics in persons with hemophilia who are on demand treatment and prophylactic treatment with novel therapies
- PRIORITY (Preventing Inhibitor Recurrence Indefinitely), A Multi-center, Prospective Study Evaluating the Rate of Inhibitor Recurrence Following Successful ITI in Patients Receiving Ongoing Once Per Weekly Factor VIII Therapy Along with Efficizumab and in
- POCUS: Hemostatic Potential and Joint Health in Patients with Severe Hemophilia A on Novel Replacement Therapies
- Hemophilia Efficizumab and EHL Factor Products Chart Review

- ATHN 10: Leveraging the ATHNdataset to Document the State of Rare Coagulation Disorders in the United States

### Samuel John

- JCAR017-BCM-004 | Transcend Pedall | A Phase 1/2, Open-Label, Single Arm, Multicohort, Multicenter Trial to Evaluate the Safety and Efficacy of JCAR017 in Pediatric Subjects with Relapsed/Refractory B-All and B-NHL
- Retrospective Study to Evaluate the Impact of Prior Blinatumomab on Subsequent CD19 CAR Outcomes
- UTSW/Children's Medical Center Outcomes Data of Patients with Relapsed/Refractory Acute Lymphoblastic Leukemia and Non-Hodgkin Lymphoma Evaluated for or treated with CART Therapy
- 208750, Long-Term Follow-Up (LTFU) of Participants Treated with GSK Adoptive Cell Therapies
- Outcomes in Pediatric and Young Adult B-Cell Acute Lymphoblastic Leukemia after Tisagenlecleucel
- 208467, Master Protocol to Assess the Safety and Antitumor Activity of Genetically Engineered NY-ESO-1-Specific (c259) T Cells, alone or in combination with other agents, in HLA-A2+ Participants with NY-ESO-1 and/or LAGE-1a Positive Solid Tumors
- AALL1721 CTL019G2201J: A phase II trial of tisagenlecleucel in first-line high-risk (HR) pediatric and young adult patients with B-cell acute lymphoblastic leukemia (B-ALL) who are minimal residual disease (MRD) positive at the end of consolidation (EOC)
- CCTL019B2003I: Managed Access Program (MAP) to provide access to CTL019, for acute lymphoblastic leukemia (ALL) or large B-cell lymphoma patients with out of specification leukapheresis product and/or manufactured tisagenlecleucel out of specification for
- CCTL019C2202, (BIANCA) A Phase II, single arm, multicenter open label trial to determine the safety and efficacy of tisagenlecleucel in pediatric patients with relapsed or refractory mature B-cell non-Hodgkin lymphoma (NHL)
- CCTL019A2205B: Long Term Follow-Up of Patients Exposed to Lentiviral-Based CAR T-Cell Therapy

### Laura Klesse

- Assessment of magnetic resonance imaging as a tool for measuring scoliosis in patients with neurofibromatosis type 1
- REC-2282-201, A Parallel-group, Two-staged, Phase 2/3, Randomized, Multicenter Study to Evaluate the Efficacy and Safety of REC-2282 in Participants with Progressive NF2 Mutated Meningiomas
- ANHL1931, A Randomized Phase 3 trial of Nivolumab(NSC# 748726 IND# 125462) in Combination with Chemo-immunotherapy for the Treatment of Newly Diagnosed Primary Mediastinal B-cell Lymphoma
- NF111 , A Phase II Trial of Poly-ICLC for Progressive, Previously Treated Low-Grade Gliomas in Children and Young Adults with Neurofibromatosis Type 1
- NF110, Open-label, Phase 2 Clinical Trial of Crizotinib for Children and Adults with Neurofibromatosis Type 2 and Progressive Vestibular Schwannomas
- ACNS1831, A Phase 3 Randomized Study of Selumetinib (IND # 77782) versus Carboplatin/Vincristine in Newly Diagnosed or Previously Untreated Neurofibromatosis Type 1 (NF1) Associated Low-Grade Glioma (LGG)

- MEK-NF-201, A Phase 2b Trial of the MEK 1/2 Inhibitor (MEKi) PD-0325901 in Adult and Pediatric Patients with Neurofibromatosis Type 1 (NF1)-Associated Inoperable Plexiform Neurofibromas (PNs) that are Causing Significant Morbidity
- Evaluation of [18F]FLT PET/CT as an early predictor of outcome in pediatric solid tumors
- Frameshift Peptides of Children with Neurofibromatosis Type 1 (NF1) and either Low-Grade Gliomas or Plexiform Neurofibromas
- NF PROTOCOL 105, A Phase II Study of Cabozantinib (XL184) for Plexiform Neurofibromas in Subjects with Neurofibromatosis Type 1 in Children and Adults
- ACCESS/REDIAL/SALUD, H-29892, Case Ascertainment for Epidemiologic Studies of Childhood Cancers and Hematological Conditions
- APEC14B1, Project: EveryChild- A Registry, Eligibility Screening, Biology and Outcome Study
- Developing Evidence-Based Criteria for Initiating Treatment for Neurofibromatosis type 1 Associated Optic Pathway Gliomas
- 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 Soli
- AHOD1331, A Randomized Phase III Study of Brentuximab Vedotin (SGN-35, IND #117117) for Newly Diagnosed High-Risk Classical Hodgkin Lymphoma (cHL) in Children and Adolescents
- Panel Based Next Generation Sequencing for High Risk Pediatric Oncology Patients
- Study of Pediatric Tumors and Cancer Predispositions
- ANHL12P1, A Randomized Phase II study of Brentuximab Vedotin (NSC# 749710) and Crizotinib (NSC# 749005) in Patients with Newly Diagnosed Anaplastic Large Cell Lymphoma (ALCL) IND #117117
- ALTE11C1, Longitudinal Assessment of Ovarian Reserve in Adolescents with Lymphoma
- ALTE05N1, Umbrella Long-Term Follow-up Protocol
- ALTE07C1, Neuropsychological, Social, Emotional, and Behavioral Outcomes in Children with Cancer
- ALTE03N1: Key Adverse Events Following Childhood Cancer
- Long-term Follow-up of Patients Enrolled on Children's Oncology Group Sponsored Research

#### **Andrew Koh**

- Isolation of peripheral blood mononuclear cells from healthy humans
- CCTL019BUS02T, Role of Gut Microbiota in Modulating CAR-T Efficacy and Adverse Effects
- Isolation of commensal gut microbiota from healthy humans

#### **Patrick Leavey**

- PEPN22P1, A Pharmacokinetic Study of VinCRISTine in Infants Dosed According to BSA-Banded Infant Dosing Tables and Older Children Dosed by Traditional BSA Methods
- PEPN2121, A Phase 1/2 Study of Tiragolumab (NSC# 827799, IND# 161266) and Atezolizumab (NSC# 783608, IND# 161266) in Patients with Relapsed or Refractory SMARCB1 or SMARCA4 Deficient Tumors
- PEPN1924, A Phase 2 Study of DS-8201a (NSC# 807708, IND#153036) in Adolescents, or Young Adults with Recurrent HER2+ Osteosarcoma
- E7080-G000-230, A Multicenter, Open-label, Randomized Phase 2 Study to Compare the Efficacy and Safety of Lenvatinib in Combination with Ifosfamide and Etoposide versus Ifosfamide and Etoposide in Children, Adolescents and Young Adults with Relapsed or Re

- The American Society of Hematology (ASH) Research Registry: A Multicenter Research Registry of Patients with Hematologic Disease
- ALTE16C1, Effects of Modern Chemotherapy Regimens on Spermatogenesis and Steroidogenesis in Adolescent and Young Adult (AYA) Survivors of Osteosarcoma
- Prospective evaluation of the use of imaging and computational tools to improve risk stratification in children with bone cancer
- AOST1421, A Phase II Study of Human-Mouse Chimeric Anti-Disialoganglioside Monoclonal Antibody ch14.18 (Dinutuximab, NSC# 764038, IND# 4308) in Combination with Sargramostim (GM-CSF) in Patients with Recurrent Osteosarcoma
- AOST1321, Phase 2 Study of Denosumab (IND#127430, NSC# 744010), a RANK Ligand Antibody, for Recurrent or Refractory Osteosarcoma
- AEWS1221, Randomized Phase 3 Trial Evaluating the Addition of the IGF-1R Monoclonal Antibody Ganitumab (AMG 479, NSC# 750008, IND# 120449) to Multiagent Chemotherapy for Patients with Newly Diagnosed Metastatic Ewing Sarcoma
- Pediatric Hematology/Oncology Workforce Evaluation in Texas
- 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
- AEWS1031, A Phase III Randomized Trial of Adding Vincristine-Topotecan-Cyclophosphamide to Standard Chemotherapy in Initial Treatment of Non-metastatic Ewing Sarcoma
- Silent Infarct Transfusion Trial (SITT)

#### **Kathleen Ludwig**

- APAL2020D, A randomized phase 3 trial of fludarabine/cytarabine/gemtuzumab ozogamicin with or without venetoclax in children with relapsed AML
- APAL2020SC, Pediatric Acute Leukemia (PedAL) Screening Trial – Developing New Therapies for Relapsed Leukemias
- Intermediate Size Expanded Access Program of Venetoclax (ABT-199) in combination with Navitoclax (ABT-263) for Pediatric Patients with Relapsed or Refractory Acute Lymphocytic Leukemia (ALL) or Lymphoblastic Lymphoma (LL)
- Selclax, a Phase I and Expansion Cohort Study of Selinexor and Venetoclax in Combination with Chemotherapy in Pediatric and Young Adult Patients with Refractory or Relapsed Acute Myeloid Leukemia
- EPIK-P2: A Phase II double-blind study with an upfront, 16-week randomized, placebo-controlled period, to assess the efficacy, safety and pharmacokinetics of alpelisib (BYL719) in pediatric and adult patients with PIK3CA-related overgrowth spectrum (PROS)
- ADVL18P1, An Open-Label Feasibility Study to Assess the Safety and Pharmacokinetics of Enasidenib in Pediatric Patients with Relapsed/Refractory Acute Myeloid Leukemia (R/R-Aml) with an Isocitrate Dehydrogenase-2 (IDH2) Mutation
- ACT15378, Open-label, Single-arm Trial to Evaluate Antitumor Activity, Safety, and Pharmacokinetics of Isatuximab Used in Combination With Chemotherapy in Pediatric Patients From 28 Days to Less Than 18 Years of Age With Relapsed/Refractory B or T Acute L
- ADVL1822 AC220-A-U202 A Phase 1/2, Multicenter, Dose-Escalating Study To Evaluate the Safety, Pharmacokinetics, Pharmacodynamics, and Efficacy Of Quizartinib Administered in Combination with Re-Induction Chemotherapy, and as a Single-Agent Continuation T

- Reducing Ethnic Disparities in Acute Leukemia (REDIAL) Consortium Retrospective Chart Review
- Pediatric Hematology and Oncology Bio-Specimen Repository

#### **An Pham**

- Secure-SCD Registry, Surveillance Epidemiology of Coronavirus (COVID-19) Under Research Exclusion

#### **Avanthi Shah**

- Evaluation of ctDNA as a prognostic biomarker for patients with newly diagnosed localized Ewing sarcoma or osteosarcoma
- APEC1621: NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)

#### **Ksenya Shliakhtsitsava**

- Assessment of Reproductive Risk Knowledge Among Childhood Cancer Survivors and Their Parents
- Testicular tissue cryopreservation for fertility preservation in male patients facing infertility-causing diseases or treatment regimens
- ALTE2031, StepByStep: A Randomized Trial of a Mobile Health and Social Media Physical Activity Intervention among Adolescent and Young Adult Childhood Cancer Survivors
- Retrospective Study Evaluating Our Institutional Experience With The Euronet Protocol in Hodgkin's Lymphoma Patients
- Primary Ovarian Insufficiency Among Childhood and Adolescent Cancer Survivors at Children's Medical Center and University of Texas Southwestern

#### **Tiffany Simms-Waldrup**

- TransIT Protocol: A Phase III Randomized Trial Comparing Unrelated Donor Bone Marrow Transplantation with Immune Suppressive Therapy for Newly Diagnosed Pediatric and Young Adult Patients with Severe Aplastic Anemia
- The Role of the Host Microbiome in the Health of Cancer and Stem Cell Transplant Patients
- Treatment Use of the CliniMACS® CD34 Reagent System to Prepare Cells for an Unlabeled Indication Using an HLA-Compatible Related or Unrelated Donor for Allogenic Transplant

#### **Stephen Skapek**

- Molecular characterization of childhood cancer specimens

#### **Tamra Slone**

- T2020-006: A Phase I Study of Tagraxofusp with or Without Chemotherapy in Pediatric Patients with Relapsed or Refractory CD123 Expressing Hematologic Malignancies
- M20-429, A Single Arm, Open-Label, Phase 1b Trial of Epcoritamab in Pediatric Patients with Relapsed/Refractory Aggressive Mature B-cell Neoplasms



- T2020-003: A Phase 2 Randomized Trial of Caloric Restriction and Activity to Reduce Chemoresistance In B-Cell Acute Lymphoblastic Leukemia (IDEAL2)
- AVM0703-001, An Open-Label, Phase 1/2 Study Evaluating AVM0703 in Patients With Lymphoid Malignancies (WWRD Study)
- AALL1821, A Phase 2 Study of Blinatumomab (NSC# 765986, IND# 147294) in Combination with Nivolumab (NSC # 748726, IND# 147294), a Checkpoint Inhibitor of PD-1, in B-ALL Patients Aged  $\geq 1$  to  $< 31$  Years Old with First Relapse
- AALL1922, A Pivotal Phase 1/2, Single-Arm, Open-label Study to Evaluate the Safety and Efficacy of Ponatinib With Chemotherapy in Pediatric Patients With Philadelphia Chromosome-Positive (Ph+) Acute Lymphoblastic Leukemia (ALL) Who Have Relapsed or Are Re
- T2016-005, Biology of Relapsed and Refractory Leukemias; TAEL Central Biological Repository (CBR)
- AAML1831, A Phase 3 Randomized Trial for Patients with de novo AML Comparing Standard Therapy Including Gemtuzumab Ozogamicin (GO) to CPX-351 with GO, and the Addition of the FLT3 Inhibitor Gilteritinib for Patients with FLT3 Mutations
- T2018-001, A retrospective cohort study of response and survival in pediatric patients with relapsed and refractory acute lymphoblastic leukemia (ALL) and lymphoblastic lymphoma (LLy) treated with contemporary salvage therapy
- AAML1921, A Phase I/II study of Bosutinib in pediatric patients with newly diagnosed chronic phase or resistant/intolerant Ph+ Chronic Myeloid Leukemia, study ITCC-054/COG AAML1921
- AALL1732, A Phase 3 Randomized Trial of Inotuzumab Ozogamicin (IND#:133494, NSC#: 772518) for Newly Diagnosed High-Risk B-ALL; Risk-Adapted Post-Induction Therapy for High-Risk B-ALL, Mixed Phenotype Acute Leukemia, and Disseminated B-LLy
- AAML18P1, Stopping Tyrosine Kinase Inhibitors (TKI) to Assess Treatment-Free Remission (TFR) in Pediatric Chronic Myeloid Leukemia - Chronic Phase (CML-CP)
- AALL1731, A Phase 3 Trial Investigating Blinatumomab (IND# 117467, NSC# 765986) in Combination with Chemotherapy in Patients with Newly Diagnosed Standard Risk or Down syndrome B-Lymphoblastic Leukemia (B-ALL) and the Treatment of Patients with Localized
- T2016-002, A Phase I/II Study of Nivolumab in Combination with 5-azacytidine in pediatric patients with relapsed/refractory acute myeloid leukemia (BMS reference CA209-9JY)
- T2017-002, A TAEL Phase 1/2 Study of PO Ixazomib in Combination with Chemotherapy for Childhood Relapsed or Refractory Acute Lymphoblastic Leukemia and Lymphoblastic Lymphoma
- ATA129-EBV-302, Multicenter, Open Label, Phase 3 Study of Tabelecleucel for Solid Organ or Allogeneic Hematopoietic Cell Transplant Subjects with Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease after Failure of Rituximab or Ritux
- Hypogammaglobulinemia and the impact of IVIG administration during treatment for childhood acute lymphoblastic leukemia, a retrospective review
- AALL15P1, A Groupwide Pilot Study to Test the Tolerability and Biologic Activity of the Addition of Azacitidine (IND# 133688, NSC# 102816) to Chemotherapy in Infants with Acute Lymphoblastic Leukemia (ALL) and KMT2A (MLL) Gene Rearrangement
- AALL1631: International Phase 3 trial in Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ ALL) testing imatinib in combination with two different cytotoxic chemotherapy backbones
- AALL1621: A Phase 2 Study of Inotuzumab Ozogamicin (NSC# 772518, IND#133494) in Children and Young Adults with Relapsed or Refractory CD22+ B-Acute Lymphoblastic Leukemia (B-ALL)

- T2012-002, A Pilot Study of Vincristine Sulfate Liposome Injection (Marqibo®) in Combination with Chemotherapy for Children, Adolescents, and Young Adults with Relapse of Acute Lymphoblastic Leukemia
- AALL1521 (INCB 18424-269), A Phase 2 Study of the JAK1 JAK2 Inhibitor Ruxolitinib With Chemotherapy in Children With De Novo High-Risk CRLF2-Rearranged and or JAK Pathway-Mutant Acute Lymphoblastic Leukemia
- AAML1421, A Phase 1/2 Study of CPX-351 (NSC# 775341; IND #129443) Alone Followed by Fludarabine, Cytarabine and G-CSF (FLAG) for Children with Relapsed Acute Myeloid Leukemia (AML)
- AAML1531, Risk-stratified Therapy for Acute Myeloid Leukemia in Down Syndrome
- Liver toxicity secondary to PEG-asparaginase during treatment for childhood acute lymphoblastic leukemia.
- AALL1331, Risk-Stratified Randomized Phase III Testing of Blinatumomab (IND#117467, NSC#765986) in First Relapse of Childhood B-Lymphoblastic Leukemia (B-ALL)
- AALL1231, A Phase III Randomized Trial Investigating Bortezomib (NSC# 681239; IND# 58443) on a Modified Augmented BFM (ABFM) Backbone in Newly Diagnosed T-Lymphoblastic Leukemia (T-ALL) and T-Lymphoblastic Lymphoma (T-LLy)
- 20140106 (former CFZ008), Phase 1b Study of Carfilzomib in Combination with Induction Chemotherapy in Children with Relapsed or Refractory Acute Lymphoblastic Leukemia
- AALL1131, A Phase 3 Randomized Trial for Newly Diagnosed High Risk B- Lymphoblastic Leukemia (B-ALL) Including a Stratum Evaluating Dasatinib (IND#73789, NSC#732517) in Patients with Ph-like Tyrosine Kinase Inhibitor (TKI) Sensitive Mutations
- Evaluation of Port Complications During Treatment of Pediatric Acute Lymphoblastic Leukemia.
- AAML1031, A Phase III Randomized Trial for Patients with de novo AML using Bortezomib and Sorafenib (IND#114480; NSC# 681239, NSC# 724772) for Patients with High Allelic Ratio FLT3/ITD
- AALL0932, Treatment of Patients with Newly Diagnosed Standard Risk B-Lymphoblastic Leukemia (B-ALL) or Localized B-lineage Lymphoblastic Lymphoma (B-LLy)
- AALL08B1, Classification of Newly Diagnosed Acute Lymphoblastic Leukemia
- AALL0434: Intensified Methotrexate, Nelarabine (Compound 506U78; IND# 52611) and Augmented BFM Therapy for Children and Young Adults with Newly Diagnosed T-cell Acute Lymphoblastic Leukemia
- Evaluation of the Risk Factors for Fungal Disease during Induction Therapy in Children with Acute Lymphoblastic Leukemia
- Secondary Acute Myeloid Leukemia and Myleodysplastic Syndrome after Treatment for Acute Lymphoblastic Leukemia
- Evaluation of the Influence of Abnormal Glucose Metabolism on the Risk of Infection in Children with Acute Lymphoblastic Leukemia and Lymphoblastic Lymphoma

### Tanya Watt

- I3Y-MC-JPCS: A Phase 1b/2 Study of Abemaciclib in Combination with Irinotecan and Temozolomide (Part A) and Abemaciclib in Combination with Temozolomide (Part B) in Pediatric and Young Adult Patients with Relapsed/Refractory Solid Tumors and Abemaciclib
- A8081056, Crizotinib (Xalkori) Expanded Access Protocol for the Treatment of Adult or Pediatric Patients with Solid or Hematologic Malignancies that Harbor a Crizotinib-Sensitive Molecular Alteration But Who Are Unable to Swallow Crizotinib Capsules

- NMTRC003B Chart Review
- ADVL1721, A non-randomized, open-label, multi-center, Phase I/II study of PI3K inhibitor copanlisib in pediatric patients with relapsed/refractory solid tumors or lymphoma
- ANBL19P1, A Pilot Study of Dinutuximab, Sargramostim (GM-CSF), and Isotretinoin in Combination with Irinotecan and Temozolomide in the Post-Consolidation Setting for High-Risk Neuroblastoma
- BCC015, Phase II Trial of Eflornithine (DFMO) and Etoposide for Relapsed/Refractory Neuroblastoma
- CL04, <sup>67</sup>Cu-SARTATE™ Peptide Receptor Radionuclide Therapy Administered to Pediatric Patients with High-Risk Neuroblastoma: A Multi-center, Dose-escalation, Open-label, Non-randomized, Phase 1/2a Theranostic Clinical Trial
- ADVL1823, Larotrectinib (LOXO-101, NSC# 788607, IND# 141824) for Previously Untreated TRK Fusion Pediatric Solid Tumors and TRK Fusion Relapsed Pediatric Acute Leukemias
- TPX-0005-07, A Phase 1/2, Open-Label, Safety, Tolerability, Pharmacokinetics, & Anti-Tumor Activity Study of Repotrectinib in Pediatric & Young Adult Subjects With Advanced or Metastatic Malignancies Harboring ALK, ROS1, or NTRK1-3 Alterations
- ANBL1821, A Phase 2 Randomized Study of Irinotecan/Temozolomide/Dinutuximab with or without Eflornithine (DFMO) (IND# 141913) in Children with Relapsed, Refractory or Progressive Neuroblastoma
- ON-TRK: PrOspective Non-interventional study in patients with locally advanced or metastatic TRK fusion cancer treated with larotrectinib
- <sup>131</sup>I-Metaiodobenzylguanidine (<sup>131</sup>I-MIBG) Therapy for Relapsed/Refractory Neuroblastoma
- ADVL1921, Phase 1/2 Study to Evaluate Palbociclib (Ibrance®) in Combination with Irinotecan and Temozolomide and/or in Combination with Topotecan and Cyclophosphamide in Pediatric Patients with Recurrent or Refractory Solid Tumors (A5481092)
- LOXO-RET-18036: A Phase 1/2 Study of the Oral RET Inhibitor LOXO-292 in Pediatric Patients with Advanced RET-Altered Solid or Primary Central Nervous System Tumors
- Pediatric Solid Tumor Metabolism [A prospective, single center study exploring solid tumor metabolism of extra-cranial tumors in the pediatric population]
- ANBL1531, A Phase 3 Study of <sup>131</sup>I-Metaiodobenzylguanidine (<sup>131</sup>I-MIBG) or ALK Inhibitor Therapy Added to Intensive Therapy for Children with Newly Diagnosed High-Risk Neuroblastoma (NBL) (IND# 134379)
- ADVL1711, A Phase 1/2 Study of Lenvatinib in Combination With Everolimus in Recurrent and Refractory Pediatric Solid Tumors, Including CNS Tumors
- ALTE15N2, LEAHRN (Late Effects After High-Risk Neuroblastoma) Study
- ADVL1622 Phase 2 Trial of XL184 (Cabozantinib) an Oral Small-Molecule Inhibitor of Multiple Kinases, in Children and Young Adults with Refractory Sarcomas, Wilms Tumor, and Other Rare Tumors
- An Open Label, Expanded Access Protocol Using <sup>131</sup>I-Metaiodobenzylguanidine (<sup>131</sup>I-MIBG) Therapy In Patients With Refractory, High-Risk Neuroblastoma and Pheochromocytoma, Or Paraganglioma (Not Eligible for Approved Treatment)
- ADVL1621, MK-3475-051, A Phase I/II Study of Pembrolizumab (MK-3475) in Children with advanced melanoma or a PD-L1 positive advanced, relapsed or refractory solid tumor or lymphoma (KEYNOTE-051)
- MIBG 2014-01, A Phase II, Open Label, Two Arm Study of Therapeutic Iobenguane (<sup>131</sup>I) as single agent or in combination with Vorinostat for Recurrent or Progressive High-Risk Neuroblastoma Subjects (OPTIMUM Trial)

- CDRB436G2201, Phase II open-label global study to evaluate the effect of dabrafenib in combination with trametinib in children and adolescent patients with BRAF V600 mutation positive Low Grade Glioma (LGG) or relapsed or refractory High Grade Glioma (HGG)
- NMTRC 014, NMTRC- Neuroblastoma Maintenance Therapy Trial Using Difluoromethylornithine (DFMO)
- Bayer 20290, A Phase 1/2 Study of the Oral TRK Inhibitor Larotrectinib in Pediatric Patients with Advanced Solid or Primary Central Nervous System Tumors
- ANBL1232, Utilizing Response- and Biology-Based Risk Factors to Guide Therapy in Patients with Non-High-Risk Neuroblastoma
- NMTRC V0706, A Phase II Trial of Nifurtimox for Refractory or Relapsed Neuroblastoma or Medulloblastoma
- ANBL12P1, Pilot Study Using Myeloablative Busulfan/Melphalan (BuMel) Consolidation Following Induction Chemotherapy for Patients with Newly Diagnosed High-Risk Neuroblastoma
- ANBL09P1, A COG Pilot Study of Intensive Induction Chemotherapy and 131I-MIBG Followed by Myeloablative Busulfan/Melphalan (Bu/Mel) for Newly Diagnosed High-Risk Neuroblastoma
- ANBL00B1, Neuroblastoma Biology Studies

#### **Jonathan Wickiser**

- AREN1921, Treatment of Newly Diagnosed Diffuse Anaplastic Wilms Tumors (DAWT) and Relapsed Favorable Histology Wilms Tumors (FHWT)
- AGCT1532, A Randomized Phase 3 Trial of Accelerated versus Standard BEP Chemotherapy for Patients with Intermediate and Poor-risk Metastatic Germ Cell Tumors
- AHEP1531, Pediatric Hepatic Malignancy International Therapeutic Trial (PHITT)
- AGCT1531 A Phase 3 Study of Active Surveillance for Low Risk and a Randomized Trial of Carboplatin vs. Cisplatin for Standard Risk Pediatric and Adult Patients with Germ Cell Tumors
- AHEP0731, Treatment of Children with All Stages of Hepatoblastoma with Temeirolimus (IND#122782, NSC#683864) Added to High Risk Stratum Treatment
- 9442: National Wilms Tumor Late Effects Study
- AREN03B2: Renal Tumors Classification, Biology and Banking

#### **Naomi Winick**

- ALTE1631, A Randomized Web-based Physical Activity Intervention among Children and Adolescents with Acute Lymphoblastic Leukemia
- Assessing neurocognitive functioning over time in pediatric patients with non-CNS solid tumors and nonlymphoblastic leukemia/lymphomas
- The feasibility of delivering a motivational interviewing session to primary caretakers of children with acute lymphoblastic leukemia (ALL) and adolescents with ALL: Comparison to an education-only control.
- Aim 3, Home or Away From Home: Comparing patient and caregiver reported quality of life and other patient-centered outcomes for inpatient versus outpatient management of neutropenia in children with AML
- Aim 1, Home or away from home: comparing clinical outcomes relevant to the care of pediatric acute myeloid leukemia during periods of neutropenia
- ACCL10P1, Computerized Cognitive Training for Pediatric Brain Tumor Patients: A Pilot Study

- ACCL1033, A Comprehensive Approach to Improve Medication Adherence in Pediatric ALL
- ACCL0922, (SCUSF 0901) A Phase II Placebo-Controlled Trial of Modafinil to Improve Neurocognitive Deficits in Children Treated for a Primary Brain Tumor

### Ayesha Zia

- Pediatric Antiphospholipid Syndrome: A National Registry Study
- Pulmonary Embolism Response Team (PERT) Consortium Registry
- Hemodynamic and Ventilatory Responses During Exercise in Pediatric Patients with Pulmonary Embolism
- ATHN Transcends: A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment in People with Non-Neoplastic Hematologic Disorders
- Thrombosis in Hospitalized Pediatric Patients with COVID
- FUVID Study: Functional Characterization of Children with Chronic Venous Thromboembolic Disease  
A multi-center, prospective study evaluating exercise intolerance and dyspnea on exertion in patients following first-episode pulmonary embolism with or without
- Examining the Experiences of Children with Blood Disorders
- Exercise Intolerance and Skeletal Muscle Bioenergetics in Children with Deep Venous Thrombosis.
- ATHN 7, A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment for People with Hemophilia (ATHN 7: Hemophilia Natural History Study)
- Venous Thromboembolism in Pediatric Patients: The role of gut microbiome
- Physical Activity in Children at Risk of Post-thrombotic Syndrome: A Pilot Randomized Controlled Trial
- Outcomes after pediatric venous thromboembolism
- Comprehensive and Multidisciplinary Approach to Evaluation of Young Women with Heavy Menstrual Bleeding (HMB): Impact on Diagnosis, Management and Outcomes
- CDC Public Health Surveillance for Bleeding Disorders - Registry for Bleeding Disorders Surveillance
- Evaluation of Thrombin Generation in Children with Venous Thromboembolism
- NN7999-3774 Safety, Efficacy and Pharmacokinetics of N9-GP in Previously Treated Children with Hemophilia B
- The American Thrombosis and Hemostasis Network (ATHN)
- Zimmerman Program for the Molecular and Clinical Biology of VWD

### 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, the National Heart, Lung, and Blood 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, the 1 Million for Anna Foundation, the Haggerty Family Foundation, the 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. Our team is developing new opportunities to augment the scope and scale of hematology and oncology services in Plano and at a new Frisco site, anticipated for the coming year.

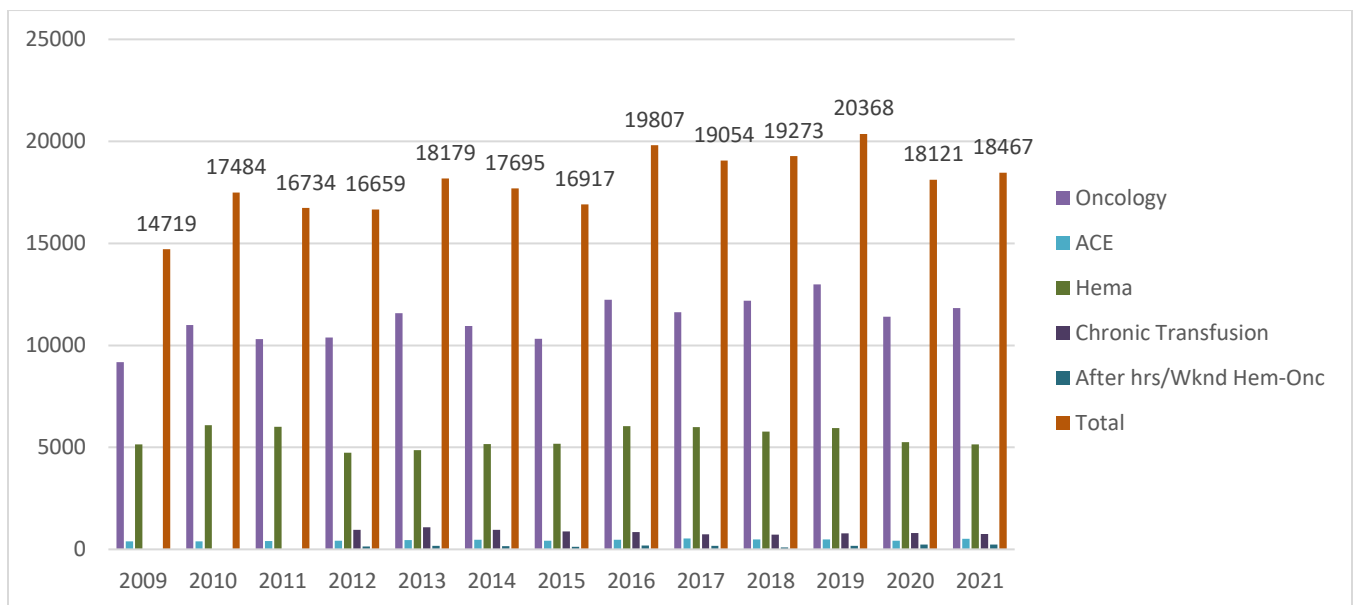
**Core Clinical Programs in Hematology and Oncology**

- 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
  - Transplant for Malignancy
  - Transplant for Non-Malignant Disease
- Young Women’s Blood Disorders

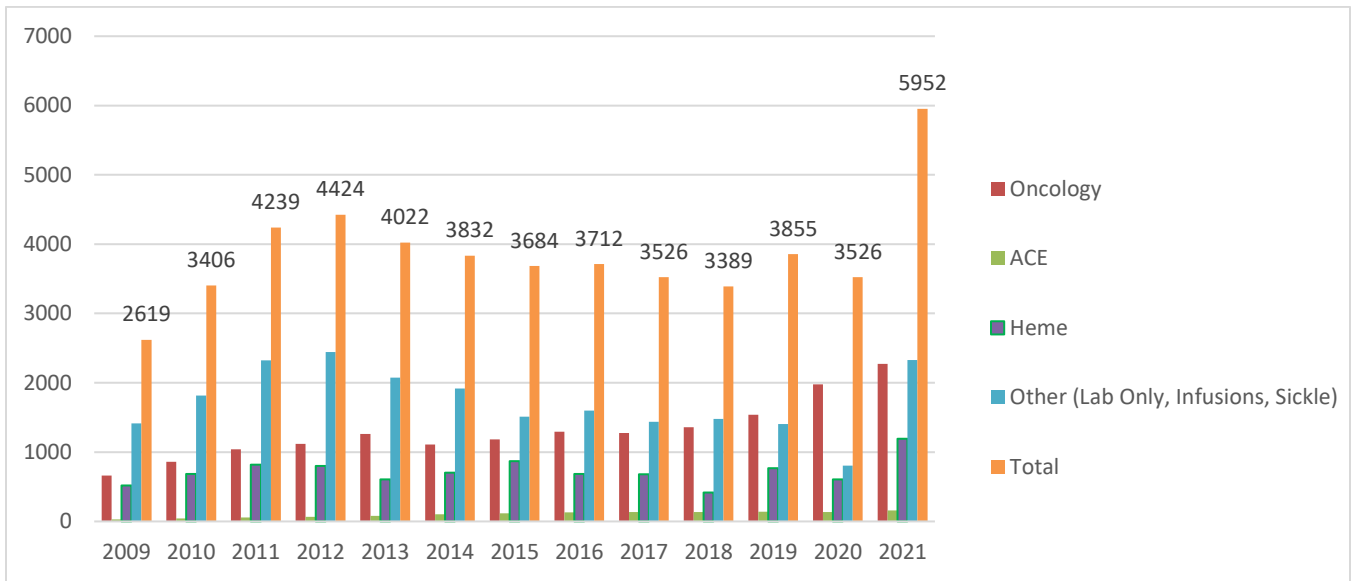
**Additional Clinical/Research Programs**

- After the Cancer Experience Childhood Cancer Survivor Program
- Cancer Genetic Susceptibility Program
- Neurofibromatosis
- Adolescent and Young Adult Oncology
- Experimental Therapeutics Program
- Precision Medicine Program
- 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 social work, child psychology/psychiatry, nutritional support, pastoral care, physical and occupational therapy, prosthetics services, and palliative care, where appropriate.

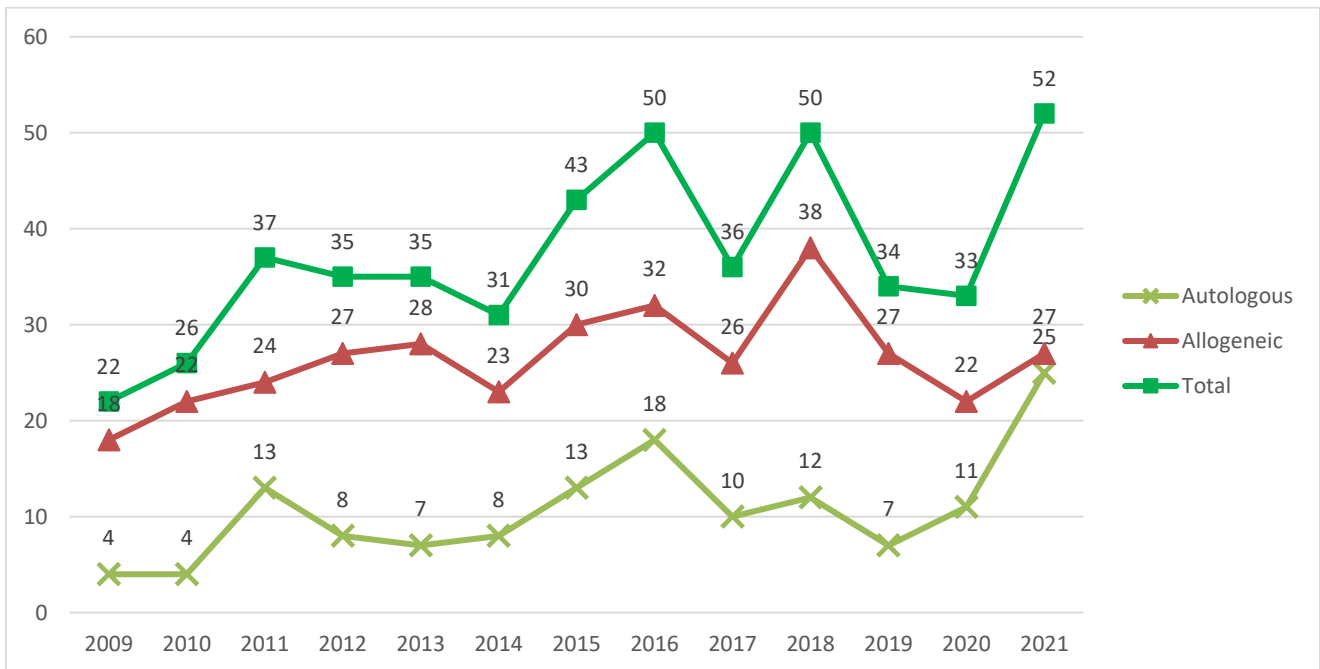


Dallas Clinic Visits



Plano Clinic Visits

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.



Stem Cell Transplants

## Current Grant Support

### Victor Aquino

**Grantor:** NIH-National Heart, Lung and Blood Inst/Center for International Blood & Marrow  
**Title of Project:** CIBMTR Study 17-SIBS, SUP1801, Identifying Predictors of Poor Health-Related Quality-of-Life among Pediatric Hematopoietic Stem Cell Donors  
**Role:** Principal Investigator  
**Dates:** 04/2019 – 04/2022

**Grantor:** Health Resources and Services Admin/University of California, San Francisco  
**Title of Project:** Severe Combined Immunodeficiency (SCID) Screening and Education  
**Role:** Principal Investigator  
**Dates:** 08/2021 – 07/2022

**Grantor:** NIH-National Institutes of Health/University of California, San Francisco  
**Title of Project:** Primary Immune Deficiency Treatment Consortium  
**Role:** Principal Investigator  
**Dates:** 09/2021 – 08/2024

**Grantor:** NIH-National Institutes of Allrgy Infect Dis/Pediatric Blood & Marrow Transplant Cons  
**Title of Project:** PBMTTC Study NMD 1801  
**Role:** Principal Investigator  
**Dates:** 05/2019 – 05/2022

**Grantor:** National Marrow Donor Program  
**Title of Project:** Center for International Blood and Marrow Transplant Research (CIBMTR) - Consent for Participation and Donation of Blood Samples  
**Role:** Principal Investigator  
**Dates:** 04/2015 – Current

**Grantor:** Pediatric Blood & Marrow Transplant Cons  
**Title of Project:** PBMTTC NMD 1801 (CSIDE), A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCR $\alpha\beta$ + /CD19+ depleted transplantation: A Phase II study by the Primary Immune Deficiency Treatment Consortium (PIDTC) and Pediatric Blood and Marrow Transplant Consortium (PBMTTC) PIDTC "CSIDE" Protocol (Conditioning SCID Infants Diagnosed Early)  
**Role:** Principal Investigator  
**Dates:** 05/2019 – Current

### Daniel Bowers

**Grantor:** US Department of Defense/University of Alabama at Birmingham  
**Title of Project:** NF Clinical Trials Consortium  
**Role:** Co-Principal Investigator  
**Dates:** 08/2017 – 08/2022

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** Leavey - NCTN Workload Intensity Year 3 PHI new fiscal sponsor (Previously FP00018512, FP00018512\_YR2)  
**Role:** Co-Investigator  
**Dates:** 03/2021 – 02/2025



**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** Bowers - COG NCTN Study Chair ACNS1931  
**Role:** Principal Investigator  
**Dates:** 03/2021 – 02/2022

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** COG\_Subaward AR60039\_study ACNS1833  
**Role:** Principal Investigator  
**Dates:** 07/2022 – 06/2025

**Grantor:** Array Biopharma Inc/Children's Hospital Los Angeles  
**Title of Project:** MEK162, Phase I-II Study of MEK 162 for Children with Low-Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors  
**Role:** Principal Investigator  
**Dates:** 08/2016 – 07/2024

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia  
**Title of Project:** NCTN-Workload Intensity Year 2  
**Role:** Co-Principal Investigator  
**Dates:** 03/2020 – 02/2025

#### **Erin Butler**

**Grantor:** St. Jude Children's Research Hospital/North American Consortium Histiocytosis  
**Title of Project:** Martin - North American Consortium for Histiocytosis LCH-IV Study  
**Role:** Principal Investigator  
**Dates:** 06/2019 – 06/2025

#### **Kenneth Chen**

**Grantor:** NIH - National Cancer Institute  
**Title of Project:** The Role of PLAG1 In Wilms Tumor Formation  
**Role:** Principal Investigator  
**Dates:** 07/2017 – 06/2023

**Grantor:** NIH - National Cancer Institute/Public Health Institute  
**Title of Project:** Chen\_COG\_NCTN - Rare Tumor Committee Chair  
**Role:** Principal Investigator  
**Dates:** 03/2021 – 02/2022

**Grantor:** NIH - National Cancer Institute/Public Health Institute  
**Title of Project:** PHI U10CA180886 Rare Tumor Committee 03.01.22-02.28.23  
**Role:** Principal Investigator  
**Dates:** 03/2022 – 02/2023

**Grantor:** Cancer Prevention & Research Institute Of Texas  
**Title of Project:** Nomination of Kenneth Sung-Man Chen, M.D. for CPRIT First-Time Tenure-Track Faculty Member Award  
**Role:** Principal Investigator  
**Dates:** 08/2018 – 08/2023

**Grantor:** NIH - National Cancer Institute/Public Health Institute  
**Title of Project:** PHI U10CA180886 Rare Tumor Committee 03.01.22-02.28.23\_COG order (AR13088)  
**Role:** Principal Investigator  
**Dates:** 03/2022 – 02/2023

**Grantor:** V Foundation for Cancer Research  
**Title of Project:** Drosha regulation of Igf2 in pineoblastoma  
**Role:** Principal Investigator  
**Dates:** 07/2022 – 07/2024

#### **Kathryn Dickerson**

**Grantor:** Hyundai Hope on Wheels  
**Title of Project:** Defining Clonal Hematopoiesis in Childhood Cancer Survivors: A Novel Approach to Predicting Treatment-Associated Myeloid Neoplasms  
**Role:** Principal Investigator  
**Dates:** 1/2020 – 12/2022

**Grantor:** Wipe Out Kids Cancer  
**Title of Project:** Defining clonal hematopoiesis and cytokine signatures in childhood cancer survivors: Looking toward inflammatory mechanisms in late effects  
**Role:** Principal Investigator  
**Dates:** 01/2022 – present

#### **Jessica Garcia**

**Grantor:** American Thrombosis & Hemostasis Network  
**Title of Project:** ATHN 10 – Leveraging the ATHNdataset to Document the State of Rare Coagulation Disorders in the United States  
**Role:** Principal Investigator  
**Dates:** 01/2020 – 12/2023

#### **Samuel John**

**Grantor:** Hyundai Hope on Wheels  
**Title of Project:** Targeting MDSCs in pediatric solid tumors by anti- LILRB4 CAR-T cells  
**Role:** Principal Investigator  
**Dates:** 02/2021 – 12/2023

**Grantor:** Gold Foundation of East Texas  
**Title of Project:** A novel anti-LILRB4 CAR-T cell for the treatment of pediatric leukemia  
**Role:** Principal Investigator  
**Dates:** 12/2020 - 12/2027

**Grantor:** Cary Council- Doc Stars  
**Title of Project:** Novel cell therapy discovery program  
**Role:** Principal Investigator  
**Dates:** 05/2021 - 12/2023

**Grantor:** Wipe out Kids Cancer  
**Title of Project:** Anti-LILRB4 CAR-T cells for the treatment of t(1;19) Pre-B ALL  
**Role:** Principal Investigator  
**Dates:** 12/2022- 12/2024

**Laura Klesse**

**Grantor:** US Department of Defense/University of Alabama At Birmingham

**Title of Project:** NF Clinical Trials Consortium

**Role:** Principal Investigator

**Dates:** 08/2017 – 08/2022

**Grantor:** NIH-National Cancer Institute/Baylor College of Medicine -

**Title of Project:** Survivorship and Access to care for Latinos to Understand and address Disparities (SALUD)

**Role:** Principal Investigator

**Dates:** 05/2021 – 04/2023

**Grantor:** NIH - National Cancer Institute/Public Health Institute

**Title of Project:** NCTN Work Order \_AR61630 - Cederberg

**Role:** Principal Investigator

**Dates:** 03/2022 – 02/2023

**Grantor:** NIH - National Cancer Institute/Public Health Institute

**Title of Project:** NCTN Work Order \_AHEP1531 - AR61631 - Schooler

**Role:** Principal Investigator

**Dates:** 03/2022 – 02/2023

**Grantor:** NIH - National Cancer Institute/Public Health Institute

**Title of Project:** NCTN Work Order \_AREN03B2\_AR61629 - Artunduaga

**Role:** Principal Investigator

**Dates:** 03/2022 – 02/2023

**Grantor:** Cancer Prevention & Research Inst of TX/Baylor College of Medicine

**Title of Project:** The Adolescent and Childhood Cancer Epidemiology and Susceptibility Service (ACCESS) for Texas

**Role:** Principal Investigator

**Dates:** 08/2020 – 08/2026

**Grantor:** NIH - National Cancer Institute/Public Health Institute

**Title of Project:** COG\_ALTE 2031 PCR\_AR60532: 09/01/2021 - 08/31/2022

**Role:** Principal -Investigator

**Dates:** 09/2021 – 08/2022

**Grantor:** Children's Tumor Foundation

**Title of Project:** Developing Evidence-Based Criteria for Initiating Treatment for NF1-OPG (“Main Study”)

**Role:** Principal Investigator

**Dates:** 12/2015 – 11/2023

**Grantor:** UT Health Science Center at San Antonio/Cancer Prevention & Research Inst of TX

**Title of Project:** Texas Pediatric Patient Derived Xenograft Facility-Year 5 6/1/20-5/53/21

**Role:** Co-Investigator

**Dates:** 07/2020 – 11/2022

**Grantor:** US Department of Defense/University of Alabama at Birmingham

**Title of Project:** NF111: A Phase II Trial of Poly-ICLC for Progressive, Previously Treated Low-Grade Gliomas in Children and Young Adults with Neurofibromatosis Type 1

**Role:** Principal Investigator

**Dates:** 08/2022 – 08/2026

**Grantor:** Dana-Farber Cancer Institute

**Title of Project:** Multicenter Cohort Study to Evaluate Outcomes after Receipt of Targeted Therapy Matched to an Individualized Cancer Therapy (iCat) Recommendation in C

**Role:** Principal Investigator

**Dates:** 11/2016 – 11/2023

**Grantor:** Cancer Prevention Research Institute Texas and St Baldrick's (ACCESS)

**Title of Project:** Case Ascertainment for Epidemiologic Studies of Childhood Cancers and Hematological Conditions.

**Role:** Principal Investigator

**Dates:**

**Grantor:** Fichtenbaum Charitable Trust

**Title of Project:** Identification of Novel Cancer Predispositions in Pediatrics

**Role:** Principal Investigator

**Dates:**

#### Andrew Koh

**Grantor:** NIH-NCI Center for Cancer Research

**Title:** Role of Gut Microbiota in Modulating Immune Checkpoint Inhibitory Therapy for Cancer

**Role:** Principal Investigator

**Dates:** 09/2019 – 08/2024

**Grantor:** Cancer Prevention & Research Inst of TX

**Title:** Pediatric Cancer Data Core

**Role:** Co-Investigator

**Dates:** 08/2018 – 08/2023

**Grantor:** NIH-National Institute of DDK Diseases

**Title:** Origin of Excess Acid in Uric Acid Urolithiasis

**Role:** Co-investigator

**Dates:** 09/2019 – 06/2024

**Grantor:** NIH-NCI Center For Cancer Research

**Title:** R01 Role of Gut Microbiota in Modulating Immune Checkpoint Inhibitory Therapy for Cancer - Diversity Supplement

**Role:** Principal Investigator

**Dates:** 09/2022 – 08/2024

**Grantor:** NIH-National Inst of Allrgy Infect Dis

**Title:** A Novel Bacterially-derived Product to Enhance Immunity and Response to Immune Checkpoint Therapy

**Role:** Principal Investigator

**Dates:** 05/2022 – 04/2023

#### Patrick Leavey

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia

**Title of Project:** Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)

**Role:** Principal Investigator

**Dates:** 05/2019 – 02/2025

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** CHILDREN'S ONCOLOGY GROUP-NCI Community Oncology Research Program (NCORP) Grant 2UG1CA189955-06  
**Role:** Principal Investigator  
**Dates:** 09/2019 – 07/2025

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** Health Effect after Anthracycline and Radiation Therapy (HEART) - Dexazoxane and Prevention of Anthracycline-related Cardiomyopathy (ALTE11C2)  
**Role:** Principal Investigator  
**Dates:** 10/2017 – 04/2022

**Grantor:** Pediatric Brain Tumor Fnd of The US/Children's Hospital of Philadelphia  
**Title of Project:** Leavey-COG ACNS1833 Supplemental PCR Visual Acuity  
**Role:** Principal Investigator  
**Dates:** 09/2021 – 09/2022

**Grantor:** Public Health Institute/Children's Hospital Of Philadelphia  
**Title of Project:** COG APEC1621SC  
**Role:** Principal Investigator  
**Dates:** 05/2022 – 06/2023

**Grantor:** Public Health Institute  
**Title of Project:** COG APEC1621SC - Children's Oncology Group Foundation Supplemental Funding  
**Role:** Principal Investigator  
**Dates:** 07/2017 – 06/2022

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia  
**Title of Project:** PER CASE REIMBURSEMENT and PATIENT STUDIES FUNDS:NIH COG Pediatric Early Phase Clinical Trial Network Grant  
**Role:** Principal Investigator  
**Dates:** 08/2021 – 07/2023

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** Leavey - NCTN Workload Intensity Year 3 PHI new fiscal sponsor (Previously FP00018512, FP00018512\_YR2)  
**Role:** Principal Investigator  
**Dates:** 03/2021 – 02/2025

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** Leavey - PHI COG NCTN Per Case Reimbursement 03.2021 to 02.2025  
**Role:** Principal Investigator  
**Dates:** 03/2021 – 02/2025

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** Leavey - PHI COG NCORP Per Case Reimbursement 8.1.2021 to 7.31.2025  
**Role:** Principal Investigator  
**Dates:** 08/2021 – 07/2025

**Grantor:** Public Health Institute/Children's Hospital of Philadelphia  
**Title of Project:** APEC14B1 - Project: Everychild PCR-COG Foundation, A Registry, Eligibility Screening, Biology and Outcome Study  
**Role:** Principal Investigator  
**Dates:** 10/2015 – 06/2022

**Grantor:** NIH-National Cancer Institute/ Children's Hospital of Philadelphia  
**Title of Project:** Leavey - COG ALTE07C1 NIH R01 Supplemental Funding  
**Role:** Principal Investigator  
**Dates:** 07/2021 – 07/2026

**Grantor:** Cancer Prevention & Research Inst Of TX  
**Title of Project:** Enhancing access to and diversity in cancer clinical trials through a financial reimbursement and outreach program  
**Role:** Principal Investigator  
**Dates:** 08/2021 – 08/2024

**Grantor:** NIH-National Cancer Institute/Children's Hospital Of Philadelphia  
**Title of Project:** NCTN-Workload Intensity Year 2  
**Role:** Principal Investigator  
**Dates:** 03/2020 – 02/2025

#### **Kathleen Ludwig**

**Grantor:** NIH-National Cancer Institute/Baylor College of Medicine  
**Title of Project:** Improving outcome disparities for Hispanic children and adolescents with acute lymphoblastic leukemia  
**Role:** Co-Investigator  
**Dates:** 08/2021 – 07/2022

**Grantor:** NIH-National Cancer Institute/Baylor College of Medicine  
**Title of Project:** Survivorship and Access to care for Latinos to Understand and address Disparities (SALUD)  
**Role:** Co- Principal Investigator  
**Dates:** 05/2021 – 04/2022

**Grantor:** St. Baldrick's Foundation/Baylor College of Medicine  
**Title of Project:** Reducing Ethnic Disparities in Acute Leukemia (REDIAL) Consortium - Year 5  
**Role:** Co-Investigator  
**Dates:** 07/2021 – 06/2022

**Grantor:** NIH-National Cancer Institute/Baylor College of Medicine  
**Title of Project:** Survivorship and Access to care for Latinos to Understand and address Disparities (SALUD)  
**Role:** Co- Principal Investigator  
**Dates:** 05/2022 – 04/2023

**Grantor:** Cancer Prevention & Research Inst of TX/Baylor College of Medicine  
**Title of Project:** The Adolescent and Childhood Cancer Epidemiology and Susceptibility Service (ACCESS) for Texas  
**Role:** Co-Investigator  
**Dates:** 08/2020 – 02/2022

## Stephen Skapek

**Grantor:** NIH-National Cancer Institute

**Title of Project:** Identifying neuroblastoma drivers and bringing them to the clinic

**Role:** Principal Investigator

**Dates:** 04/2021 – 03/2023

**Grantor:** NIH-National Institutes of Health/University of Chicago

**Title of Project:** Liquid biopsy approaches to inform neuroblastoma prognosis and disease monitoring

**Role:** Principal Investigator

**Dates:** 04/2022 – 03/2027

**Grantor:** NIH-NCI Center For Cancer Research

**Title of Project:** Targeting EWSR1-FLI1 through Functional, Structural and Chemical Approaches

**Role:** Principal Investigator

**Dates:** 09/2019 – 8/2024

**Grantor:** NIH-National Cancer Institute

**Title of Project:** UT Southwestern Simmons Comprehensive Cancer Center

**Role:** Principal Investigator

**Dates:** 08/2021 – 07/2026

**Grantor:** NIH-National Cancer Institute/Public Health Institute

**Title of Project:** COG PHI Committee Leadership - Developmental Therapeutics Committee 3.1.22\_2.28.23

**Role:** Principal Investigator

**Dates:** 03/2022 – 02/2023

**Grantor:** NIH-National Cancer Institute/Public Health Institute

**Title of Project:** Skapek - PHI/COG Scientific Council 3.1.22\_2.28.23

**Role:** Principal Investigator

**Dates:** 03/2022 – 02/2023

**Grantor:** NIH-National Cancer Institute/Nationwide Children's Hospital

**Title of Project:** COG Biospecimen Bank

**Role:** Principal Investigator

**Dates:** 04/2021 – 03/2022

**Grantor:** NIH-National Cancer Institute/Nationwide Children's Hospital

**Title of Project:** COG Biospecimen Bank

**Role:** Principal Investigator

**Dates:** 04/2022 – 03/2023

**Grantor:** NIH-National Cancer Institute/Public Health Institute

**Title of Project:** Skapek COG/PHI - Developmental Therapeutics Committee 3.1.21\_2.28.22

**Role:** Principal Investigator

**Dates:** 03/2021 – 02/2022

**Grantor:** NIH-National Cancer Institute/Public Health Institute

**Title of Project:** Skapek COG/PHI - ITSC Solid Chair 3.1.21\_2.28.22

**Role:** Principal Investigator

**Dates:** 03/2021 – 02/2022

**Grantor:** NIH-NCI Center for Cancer Research/Public Health Institute  
**Title of Project:** Skapek COG/PHI Scientific Council 3.1.21\_2.28.22  
**Role:** Principal Investigator  
**Dates:** 03/2021 – 02/2022

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** COG PHI PEP-CTN Agent Prioritization Committee 8.1.21\_7.31.22  
**Role:** Principal Investigator  
**Dates:** 08/2021 – 07/2022

**Grantor:** NIH-National Cancer Institute/Public Health Institute  
**Title of Project:** COG-PHI PEP-CTN Steering Committee Sci. Leadership - Biology Leader Solid Tumor 8.1.2021-7.31.22  
**Role:** Principal Investigator  
**Dates:** 08/2021 – 07/2022

**Grantor:** Andrew McDonough B+ Foundation  
**Title of Project:** Developing a novel therapeutic strategy for rhabdomyosarcoma  
**Role:** Principal Investigator  
**Dates:** 01/2019 – 12/2022

**Grantor:** Cancer Prevention & Research Inst of TX/UT Health Science Center at San Antonio  
**Title of Project:** Texas Pediatric Patient Derived Xenograft Facility  
**Role:** Principal Investigator  
**Dates:** 06/2020 – 11/2022

**Grantor:** Cancer Prevention & Research Institute of Texas  
**Title of Project:** Rhabdomyosarcoma vulnerabilities: Prioritizing and extending to the clinic  
**Role:** Principal Investigator  
**Dates:** 03/2018 – 02/2023

**Grantor:** Cancer Prevention & Research Institute of Texas  
**Title of Project:** Pediatric Cancer Data Core  
**Role:** Co-Investigator  
**Dates:** 08/2018 – 08/2023

**Grantor:** V Foundation for Cancer Research  
**Title of Project:** Drosha regulation of Igf2 in pineoblastoma  
**Role:** Principal Investigator  
**Dates:** 07/2022 – 07/2024

#### **Tamra Slone**

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Los Angeles  
**Title of Project:** Slone IDEAL-2 TACL T2020-003  
**Role:** Principal Investigator  
**Dates:** 11/2022 – 08/2026

**Grantor:** NIH-National Cancer Institute/ Public Health Institute  
**Title of Project:** Leavey - NCTN Workload Intensity Year 3 PHI new fiscal sponsor (Previously FP00018512, FP00018512\_YR2)  
**Role:** Co-Investigator  
**Dates:** 03/2021 – 02/2025



**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia  
**Title of Project:** NCTN-Workload Intensity Year 2  
**Role:** Co-Investigator  
**Dates:** 03/2020 – 02/2025

#### Tanya Watt

**Grantor:** NIH-National Cancer Institute/ Public Health Institute  
**Title of Project:** Leavey - NCTN Workload Intensity Year 3 PHI new fiscal sponsor (Previously FP00018512, FP00018512\_YR2)  
**Role:** Co-Investigator  
**Dates:** 03/2021 – 02/2025

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia  
**Title of Project:** NCTN-Workload Intensity Year 2  
**Role:** Co-Investigator  
**Dates:** 03/2020 – 02/2025

#### Jonathan Wickiser

**Grantor:** NIH-National Cancer Institute/ Public Health Institute  
**Title of Project:** Leavey - NCTN Workload Intensity Year 3 PHI new fiscal sponsor (Previously FP00018512, FP00018512\_YR2)  
**Role:** Co-Investigator  
**Dates:** 03/2021 – 02/2025

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia  
**Title of Project:** NCTN-Workload Intensity Year 2  
**Role:** Co-Investigator  
**Dates:** 03/2020 – 02/2025

#### Naomi Winick

**Grantor:** NIH-National Cancer Institute/Children's National Medical Center  
**Title of Project:** Longitudinal, Multimodal Assessment of Neuropsychological Functioning in Children Diagnosed with High-Risk Acute Lymphoblastic Leukemia (HR-ALL); Using Early Changes to Predict Later Impairment  
**Role:** Principal Investigator  
**Dates:** 06/2018 – 05/2022

**Grantor:** NIH-National Cancer Institute/Children's Hospital of Philadelphia  
**Title of Project:** PHI-COG Committee Leadership WO - AR10387  
**Role:** Principal Investigator  
**Dates:** 09/2022 – 02/2023

**Grantor:** American Association for Cancer Research  
**Title of Project:** Perceptions of LEP Parents of Children with Cancer on Care and Support  
**Role:** Principal Investigator  
**Dates:** 07/2022 – 06/2024

**Grantor:** Children's Hospital of Philadelphia  
**Title of Project:** Cancer Trials Support Unit (CTSU) Phase II Supplemental Payments (PCR) N02-CM-62212  
**Role:** Principal Investigator  
**Dates:** 01/2012 – 02/2022

## Yanbin Zheng

**Grantor:** Rally Foundation for Childhood Cancer Research  
**Title of Project:** Developing new therapeutic targets and diagnostic biomarkers for RMS  
**Role:** Co-Investigator  
**Dates:** 07/2021 – 6/2022

**Grantor:** Rally Foundation for Childhood Cancer Research  
**Title of Project:** Developing Synergistic Drug Combinations as New Therapies To Treat RMS  
**Role:** Co-Investigator  
**Dates:** 07/2020 – 02/2022

**Grantor:** Andrew McDonough B+ Foundation  
**Title of Project:** Developing a Novel Therapeutic Strategy for Rhabdomyosarcoma  
**Role:** Principal Investigator  
**Dates:** 01/2019 – 12/2022

## Ayesha Zia

**Grantor:** NIH-National Heart, Lung and Blood Inst  
**Title of Project:** Functional Characterization of Children with Chronic Venous Thromboembolic Disease  
**Role:** Principal Investigator  
**Dates:** 09/2021 – 06/2025

**Grantor:** American Heart Association  
**Title of Project:** Functional Characterization of Children with Chronic Thromboembolic Disease  
**Role:** Principal Investigator  
**Dates:** 05/2021 – 04/2023

**Grantor:** Health Resources and Services Admin/University of Tx Hlth Sci Ctr At Houston  
**Title of Project:** MCHB/HRSA Great Plains Hemophilia Network  
**Role:** Principal Investigator  
**Dates:** 06/2022 – 05/2027

**Grantor:** Health Resources and Services Admin/University of Tx Hlth Sci Ctr At Houston  
**Title of Project:** MCHB/HRSA Great Plains Regional Hemophilia Network 6.1.21-5.31.22  
**Role:** Principal Investigator  
**Dates:** 06/2021 – 05/2022

**Grantor:** NIH-National Heart, Lung and Blood Inst/Versiti Wisconsin, Inc.  
**Title of Project:** Zimmerman Program on the Biology of VWD P01 3/1/21-2/28/22  
**Role:** Principal Investigator  
**Dates:** 03/2021 – 02/2022

**Grantor:** Rheumatology Research Foundation  
**Title of Project:** The Impact of Antiphospholipid Antibodies on Hypercoagulability in Children with Systemic Lupus Erythematosus  
**Role:** Principal Investigator  
**Dates:** 07/2022 – 06/2025

**Grantor:** American Thrombosis & Hemostasis Network  
**Title of Project:** AZ - ATHN7 A Natural History Cohort Study  
**Role:** Principal Investigator  
**Dates:** 07/2019 – 03/2024

**Grantor:** Extracorporeal Life Support Organization

**Title of Project:** Defects of Primary Hemostasis during Extracorporeal Life Support and the Impact on Bleeding Complications and Blood Product Transfusions

**Role:** Co-Investigator

**Dates:** 01/2020 – 12/2022

**Grantor:** Centers for Disease Control & Prevention/UT Health Science Center at Houston

**Title of Project:** ATHN/CDC: Public Health Surveillance for the Prevention of Complications of Bleeding Disorders

**Role:** Principal Investigator

**Dates:** 09/2021 – 09/2025

## Peer-Reviewed Publications

1. Anderst J, Carpenter SL, Abshire TC, Killough E, AAP SECTION ON HEMATOLOGY/ONCOLOGY, THE AMERICAN SOCIETY OF PEDIATRIC HEMATOLOGY/ONCOLOGY, THE AAP COUNCIL ON CHILD ABUSE AND NEGLECT, Consultants, Mendonca EA, Downs SM, Section on Hematology/Oncology executive committee, 2020–2021, Wetmore C, Allen C, Dickens D, Harper J, Rogers ZR, Jain J, Warwick A, Yates A, past executive committee members, Hord J, Lipton J, Wilson H, staff, Kirkwood S, Council on Child Abuse and Neglect, 2020–2021, Haney SB, Asnes AG, Gavril AR, Girardet RG, Heavilin N, Gilmartin ABH, Laskey A, Messner SA, Mohr BA, Nienow SM, Rosado N, cast Council on Child Abuse and Neglect executive committee members, Idzerda SM, Legano LA, Raj A, Sirotnak AP, Liaisons, Forkey HC, Council on Foster Care, Adoption and Kinship Care, Keeshin B, American Academy of Child and Adolescent Psychiatry, Matjasko J, Centers for Disease Control and Prevention, Edward H, Section on Pediatric Trainees, staff, Chavdar M, American Society of Pediatric Hematology/Oncology Board of Trustees, 2020–2021, Di Paola J, **Leavey P**, Graham D, Hastings C, Hijiya N, Hord J, Matthews D, Pace B, Velez MC, Wechsler D, past board members, Billett A, Stork L, staff, Hooker R. [Evaluation for Bleeding Disorders in Suspected Child Abuse](#). *Pediatrics*. 2022 Oct 1;150(4):. PMID:36180615
2. Brady SW, Roberts KG, Gu Z, Shi L, Pounds S, Pei D, Cheng C, Dai Y, Devidas M, Qu C, Hill AN, Payne-Turner D, Ma X, Iacobucci I, Baviskar P, Wei L, Arunachalam S, Hagiwara K, Liu Y, Flasch DA, Liu Y, Parker M, Chen X, Elsayed AH, Pathak O, Li Y, Fan Y, Michael JR, Rusch M, Wilkinson MR, Foy S, Hedges DJ, Newman S, Zhou X, Wang J, Reilly C, Sioson E, Rice SV, Pastor Loyola V, Wu G, Rampersaud E, Reshmi SC, Gastier-Foster J, Guidry Auvil JM, Gesuwan P, Smith MA, **Winick N**, Carroll AJ, Heerema NA, Harvey RC, Willman CL, Larsen E, Raetz EA, Borowitz MJ, Wood BL, Carroll WL, Zweidler-McKay PA, Rabin KR, Mattano LA, Maloney KW, Winter SS, Burke MJ, Salzer W, Dunsmore KP, Angiolillo AL, Crews KR, Downing JR, Jeha S, Pui CH, Evans WE, Yang JJ, Relling MV, Gerhard DS, Loh ML, Hunger SP, Zhang J, Mullighan CG. [The genomic landscape of pediatric acute lymphoblastic leukemia](#). *Nat Genet*. 2022 Sep;54(9):1376-1389. PMID:36050548
3. Burke MJ, Devidas M, Chen Z, Salzer WL, Raetz EA, Rabin KR, Heerema NA, Carroll AJ, Gastier-Foster JM, Borowitz MJ, Wood BL, **Winick NJ**, Carroll WL, Hunger SP, Loh ML, Larsen EC. [Outcomes in adolescent and young adult patients \(16 to 30 years\) compared to younger patients treated for high-risk B-lymphoblastic leukemia: report from Children's Oncology Group Study AALL0232](#). *Leukemia*. 2022 Mar;36(3):648-655. PMID:34725453
4. Button JE, Autran CA, Reens AL, Cosetta CM, Smriga S, Ericson M, Pierce JV, Cook DN, Lee ML, Sun AK, Alousi AM, **Koh AY**, Rechtman DJ, Jenq RR, McKenzie GJ. [Dosing a synbiotic of human milk oligosaccharides and B. infantis leads to reversible engraftment in healthy adult microbiomes without antibiotics](#). *Cell Host Microbe*. 2022 May 11;30(5):712-725.e7. PMID:35504279

5. Das A, Sudhaman S, Morgenstern D, Coblentz A, Chung J, Stone SC, Alsafwani N, Liu ZA, Karsaneh OAA, Soleimani S, Ladany H, Chen D, Zatzman M, Cabric V, Nobre L, Bianchi V, Edwards M, Sambira Nahum LC, Ercan AB, Nabbi A, Constantini S, Dvir R, Yalon-Oren M, Campino GA, Caspi S, Larouche V, Reddy A, Osborn M, Mason G, Lindhorst S, Bronsema A, Magimairajan V, Opocher E, De Mola RL, Sabel M, Frojd C, Sumerauer D, Samuel D, Cole K, Chiaravalli S, Massimino M, Tomboc P, Ziegler DS, George B, Van Damme A, Hijiya N, Gass D, McGee RB, Mordechai O, **Bowers DC**, Laetsch TW, Lossos A, Blumenthal DT, Sarosiek T, Yen LY, Knipstein J, Bendel A, Hoffman LM, Luna-Fineman S, Zimmermann S, Scheers I, Nichols KE, Zapotocky M, Hansford JR, Maris JM, Dirks P, Taylor MD, Kulkarni AV, Shroff M, Tsang DS, Villani A, Xu W, Aronson M, Durno C, Shlien A, Malkin D, Getz G, Maruvka YE, Ohashi PS, Hawkins C, Pugh TJ, Bouffet E, Tabori U. [Genomic predictors of response to PD-1 inhibition in children with germline DNA replication repair deficiency](#). *Nat Med*. 2022 Jan;28(1):125-135. PMID:34992263
  
6. de Blank PMK, Gross AM, Akshintala S, Blakeley JO, Bollag G, Cannon A, Dombi E, Fangusaro J, Gelb BD, Hargrave D, Kim A, **Klesse LJ**, Loh M, Martin S, Moertel C, Packer R, Payne JM, Rauen KA, Rios JJ, Robison N, Schorry EK, Shannon K, Stevenson DA, Stieglitz E, Ullrich NJ, Walsh KS, Weiss BD, Wolters PL, Yohay K, Yohe ME, Widemann BC, Fisher MJ. [MEK inhibitors for neurofibromatosis type 1 manifestations: Clinical evidence and consensus](#). *Neuro Oncol*. 2022 Nov 2;24(11):1845-1856. PMID:35788692
  
7. Gajjar A, Mahajan A, Abdelbaki M, Anderson C, Antony R, Bale T, Bindra R, **Bowers DC**, Cohen K, Cole B, Dorris K, Ermoian R, Franson A, Helgager J, Landi D, Lin C, Metrock L, Nanda R, Palmer J, Partap S, Plant A, Pruthi S, Reynolds R, Ruggieri P, Stearns D, Storm P, Wang A, Warren K, Whipple N, Zaky W, McMillian NR, Pluchino LA. [Pediatric Central Nervous System Cancers, Version 2.2023, NCCN Clinical Practice Guidelines in Oncology](#). *J Natl Compr Canc Netw*. 2022 Dec;20(12):1339-1362. PMID:36509072
  
8. Gerber DE, Tiro JA, McNeill LH, Williams EL, Zhu H, Lee SJC, **Leavey PJ**, Sadeghi N, Kapinos KA, Dornsife DL, Nguyen V, Wileyto EP, Guerra CE. [Enhancing access to and diversity in cancer clinical trials through a financial reimbursement program: Protocol to evaluate a novel program](#). *Contemp Clin Trials*. 2022 Oct;121():106922. PMID:36096281
  
9. Goldenberg NA, Kittelson JM, Abshire TC, Bonaca M, Casella JF, Dale RA, Halperin JL, Hamblin F, Kessler CM, Manco-Johnson MJ, Sidonio RF, Spyropoulos AC, Steg PG, Turpie AGG, Schulman S, Kids-DOTT Trial Investigators and the ATLAS Group. [Effect of Anticoagulant Therapy for 6 Weeks vs 3 Months on Recurrence and Bleeding Events in Patients Younger Than 21 Years of Age With Provoked Venous Thromboembolism: The Kids-DOTT Randomized Clinical Trial](#). *JAMA*. 2022 Jan 11;327(2):129-137. PMID:35015038
  
10. Guest EM, Kairalla JA, Hilden JM, Dreyer ZE, Carroll AJ, Heerema NA, Wang CY, Devidas M, Gore L, Salzer WL, **Winick NJ**, Carroll WL, Raetz EA, Borowitz M, Loh ML, Hunger SP, Brown PA. [Outstanding outcomes in infants with KMT2A-germline acute lymphoblastic leukemia treated with chemotherapy alone: results of the Children's Oncology Group AALL0631 trial](#). *Haematologica*. 2022 May 1;107(5):1205-1208. PMID:35172563
  
11. Gupta S, Teachey DT, Chen Z, Rabin KR, Dunsmore KP, Larsen EC, Maloney KW, Mattano LA Jr, Winter SS, Carroll AJ, Heerema NA, Borowitz MJ, Wood BL, Carroll WL, Raetz EA, **Winick NJ**, Loh ML, Hunger SP, Devidas M. [Sex-based disparities in outcome in pediatric acute lymphoblastic leukemia: a Children's Oncology Group report](#). *Cancer*. 2022 May 1;128(9):1863-1870. PMID:35201611

12. Kendel NE, **Zia A**, Rosenbaum LE, Stanek JR, Haamid FW, Christian-Rancy M, O'Brien SH. [Intrauterine Device Outcomes in Young Women with Heavy Menstrual Bleeding: Comparing Patients with and without Inherited Bleeding Disorders](#). *J Pediatr Adolesc Gynecol*. 2022 Dec;35(6):653-658. PMID:35820606
13. Kenney LB, Ames BL, Huang MS, Yock T, **Bowers DC**, Nekhlyudov L, Williams D, Hudson MM, Ullrich NJ. [Consensus Recommendations for Managing Childhood Cancer Survivors at Risk for Stroke After Cranial Irradiation: A Delphi Study](#). *Neurology*. 2022 Oct 18;99(16):e1755-e1766. PMID:35977833
14. Kolitz E, Fernandes NJ, Agim NG, **Ludwig K**. [Response to Alpelisib in Clinically Distinct Pediatric Patients With PIK3CA-related Disorders](#). *J Pediatr Hematol Oncol*. 2022 Nov 1;44(8):482-485. PMID:35129141
15. **Leavey PJ**, Krailo MD, DuBois SG, Reed DR, Janeway KA, Mascarenhas L. [Reply to J.-G. Wang et al](#). *J Clin Oncol*. 2022 May 1;40(13):1507-1508. PMID:35235382
16. Liu APY, Dhanda SK, Lin T, Sioson E, Vasilyeva A, Gudenas B, Tatevossian RG, Jia S, Neale G, **Bowers DC**, Hassall T, Partap S, Crawford JR, Chintagumpala M, Bouffet E, McCowage G, Broniscer A, Qaddoumi I, Armstrong G, Wright KD, Upadhyaya SA, Vinitsky A, Tinkle CL, Lucas J, Chiang J, Indelicato DJ, Sanders R, Klimo P Jr, Boop FA, Merchant TE, Ellison DW, Northcott PA, Orr BA, Zhou X, Onar-Thomas A, Gajjar A, Robinson GW. [Molecular classification and outcome of children with rare CNS embryonal tumors: results from St. Jude Children's Research Hospital including the multi-center SJYC07 and SJMB03 clinical trials](#). *Acta Neuropathol*. 2022 Oct;144(4):733-746. PMID:35982322
17. Marsh RA, Hebert K, Kim S, Dvorak CC, **Aquino VM**, Baker KS, Chellapandian D, Dávila Saldaña B, Duncan CN, Eckrich MJ, Georges GE, Olson TS, Pulsipher MA, Shenoy S, Stenger E, Lugt MV, Yu LC, Gennery AR, Eapen M. [Comparison of hematopoietic cell transplant conditioning regimens for hemophagocytic lymphohistiocytosis disorders](#). *J Allergy Clin Immunol*. 2022 Mar;149(3):1097-1104.e2. PMID:34375618
18. Medvedev KE, Savelyeva AV, **Chen KS**, Bagrodia A, Jia L, Grishin NV. [Integrated Molecular Analysis Reveals 2 Distinct Subtypes of Pure Seminoma of the Testis](#). *Cancer Inform*. 2022;21():1.17693512211326E+16. PMID:36330202
19. Myers RM, Taraseviciute A, Steinberg SM, Lambie AJ, Sheppard J, Yates B, Kovach AE, Wood B, Borowitz MJ, Stetler-Stevenson M, Yuan CM, Pillai V, Foley T, Chung P, Chen L, Lee DW, Annesley C, DiNofia A, Grupp SA, **John S**, Bhojwani D, Brown PA, Laetsch TW, Gore L, Gardner RA, Rheingold SR, Pulsipher MA, Shah NN. [Blinatumomab Nonresponse and High-Disease Burden Are Associated With Inferior Outcomes After CD19-CAR for B-ALL](#). *J Clin Oncol*. 2022 Mar 20;40(9):932-944. PMID:34767461
20. O'Brien SH, **Zia A**. [Hemostatic and thrombotic disorders in the pediatric patient](#). *Blood*. 2022 Aug 11;140(6):533-541. PMID:34724564
21. Ortigoza EB, Cagle J, Brown LS, Mansi S, Gosser SP, Montgomery AD, Foresman Z, Boren ML, Pettit PS, Thompson TD, Vasil DM, Chien JH, Neu J, **Koh AY**, Sanghavi R, Mirpuri J. [Tachygastria in Preterm Infants: A Longitudinal Cohort Study](#). *J Pediatr Gastroenterol Nutr*. 2022 Nov 1;75(5):564-571. PMID:36305880
22. Parker M, Hannah M, **Zia A**. ["If I wasn't a girl!": Experiences of adolescent girls with heavy menstrual bleeding and inherited bleeding disorders](#). *Res Pract Thromb Haemost*. 2022 May;6(4):e12727. PMID:35664533

23. Rakheja D, Park JY, Fernandes NJ, **Watt TC**, Laetsch TW, Collins RRJ. [Pediatric Non-Myofibroblastic Primitive Spindle Cell Tumors with ALK Gene Rearrangements and Response to Crizotinib](#). *Int J Surg Pathol*. 2022 Sep;30(6):706-715. PMID:35164578
24. Rodwin RL, Kairalla JA, Hibbitts E, Devidas M, Whitley MK, Mohrmann CE, Schore RJ, Raetz E, **Winick NJ**, Hunger SP, Loh ML, Hockenberry MJ, Angiolillo AL, Ness KK, Kadan-Lottick NS. [Persistence of Chemotherapy-Induced Peripheral Neuropathy Despite Vincristine Reduction in Childhood B-Acute Lymphoblastic Leukemia](#). *J Natl Cancer Inst*. 2022 Aug 8;114(8):1167-1175. PMID:35552709
25. Sadler B, Minard CG, Haller G, Gurnett CA, O'Brien SH, Wheeler A, Jain S, Sharma M, **Zia A**, Kulkarni R, Mullins E, Ragni MV, Sidonio R, Dietrich JE, Kouides PA, Di Paola J, Srivaths L. [Whole-exome analysis of adolescents with low VWF and heavy menstrual bleeding identifies novel genetic associations](#). *Blood Adv*. 2022 Jan 25;6(2):420-428. PMID:34807970
26. Shulman DS, Whittle SB, Surdez D, Bailey KM, de Álava E, Yustein JT, Shlien A, Hayashi M, Bishop AJR, Crompton BD, DuBois SG, Shukla N, **Leavey PJ**, Lessnick SL, Kovar H, Delattre O, Grünewald TGP, Antonescu CR, Roberts RD, Toretzky JA, Tirode F, Gorlick R, Janeway KA, Reed D, Lawlor ER, Grohar PJ. [An international working group consensus report for the prioritization of molecular biomarkers for Ewing sarcoma](#). *NPJ Precis Oncol*. 2022 Sep 17;6(1):65. PMID:36115869
27. Sochet AA, Morrison JM, Jaffray J, Godiwala N, Wilson HP, Thornburg CD, Bhat RV, **Zia A**, Lawrence C, Kudchadkar SR, Hamblin F, Russell CJ, Streiff MB, Spyropoulos AC, Amankwah EK, Goldenberg NA, COVID-19 Anticoagulation in Children – Thromboprophylaxis (COVAC-TP) Trial Investigators. [Enoxaparin Thromboprophylaxis in Children Hospitalized for COVID-19: A Phase 2 Trial](#). *Pediatrics*. 2022 Jul 1;150(1):. PMID:35484817
28. Solmonson A, Faubert B, Gu W, Rao A, Cowdin MA, Menendez-Montes I, Kelekar S, Rogers TJ, Pan C, Guevara G, Tarangelo A, Zacharias LG, Martin-Sandoval MS, Do D, Pachnis P, Dumesnil D, Mathews TP, Tasdogan A, **Pham A**, Cai L, Zhao Z, Ni M, Cleaver O, Sadek HA, Morrison SJ, DeBerardinis RJ. [Compartmentalized metabolism supports midgestation mammalian development](#). *Nature*. 2022 Apr;604(7905):349-353. PMID:35388219
29. Teo KY, Daescu O, Cederberg K, Sengupta A, **Leavey PJ**. [Correlation of histopathology and multi-modal magnetic resonance imaging in childhood osteosarcoma: Predicting tumor response to chemotherapy](#). *PLoS One*. 2022;17(2):e0259564. PMID:35157711
30. von Itzstein MS, Gonugunta AS, Sheffield T, Homsy J, Dowell JE, **Koh AY**, Raj P, Fattah F, Wang Y, Basava VS, Khan S, Park JY, Popat V, Saltarski JM, Gloria-McCutchen Y, Hsiehchen D, Ostmeyer J, Xie Y, Li QZ, Wakeland EK, Gerber DE. [Association between Antibiotic Exposure and Systemic Immune Parameters in Cancer Patients Receiving Checkpoint Inhibitor Therapy](#). *Cancers (Basel)*. 2022 Mar 4;14(5):. PMID:35267634
31. Ware ME, Kadan-Lottick NS, Devidas M, Terrell S, Chow EJ, Ehrhardt MJ, Hardy KK, Chemaitilly W, Hein W, **Winick N**, Teachey D, Esbenshade A, Armenian SH, Partin RE, Ness KK. [Design and methods of a randomized web-based physical activity intervention among children with cancer: A report from the Children's Oncology Group](#). *Contemp Clin Trials*. 2022 Nov;122():106961. PMID:36228982

32. Willis KR, Sathe AA, Xing C, Koduru P, Artunduaga M, **Butler EB**, Park JY, Kurmasheva RT, Houghton PJ, **Chen KS**, Rakheja D. [Extrarenal Anaplastic Wilms Tumor: A Case Report With Genomic Analysis and Tumor Models](#). *J Pediatr Hematol Oncol*. 2022 May 1;44(4):147-154. PMID:35129140
33. Woods GM, Raffini L, Brandão LR, Jaffray J, Branchford BR, Ng CJ, Sartain SE, Pak J, Male C, **Zia A**, Rizzi M, Sirachainan N, Faustino EVS, Carpenter SL, Goldenberg NA. [Practical considerations and consensus opinion for children's hospital-based inpatient hemostasis and thrombosis \(HAT\) consultative services: Communication from the ISTH SSC Subcommittee on Pediatric/Neonatal Thrombosis and Hemostasis](#). *J Thromb Haemost*. 2022 Sep;20(9):2151-2158. PMID:35748322
34. Yang A, Patterson A, Pavlock T, **Chen KS**, Gagan J, Hatley ME, Frazier AL, Amatruda JF, Laetsch TW, Rakheja D. [Pitfalls in the diagnosis of yolk sac tumor: Lessons from a clinical trial](#). *Pediatr Blood Cancer*. 2022 Feb;69(2):e29451. PMID:34866303
35. Yang AT, Smith C, Callan A, Liu C, Stewart R, Sengupta A, **Leavey PJ**. [Metachronous diagnosis of synovial sarcoma and papillary thyroid carcinoma in a child](#). *Pediatr Blood Cancer*. 2022 Jan;69(1):e29310. PMID:34453481
36. Yang S, Wang S, Wang Y, Rong R, Kim J, Li B, **Koh AY**, Xiao G, Li Q, Liu DJ, Zhan X. [MB-SupCon: Microbiome-based Predictive Models via Supervised Contrastive Learning](#). *J Mol Biol*. 2022 Aug 15;434(15):167693. PMID:35777465
37. Yang W, Karol SE, Hoshitsuki K, Lee S, Larsen EC, **Winick N**, Carroll WL, Loh ML, Raetz EA, Hunger SP, Winter SS, Dunsmore KP, Devidas M, Relling MV, Yang JJ. [Association of Inherited Genetic Factors With Drug-Induced Hepatic Damage Among Children With Acute Lymphoblastic Leukemia](#). *JAMA Netw Open*. 2022 Dec 1;5(12):e2248803. PMID:36580335
38. Zhang X, Wang S, Rudzinski ER, Agarwal S, Rong R, Barkauskas DA, Daescu O, Furman Cline L, Venkatramani R, Xie Y, Xiao G, **Leavey P**. [Deep Learning of Rhabdomyosarcoma Pathology Images for Classification and Survival Outcome Prediction](#). *Am J Pathol*. 2022 Jun;192(6):917-925. PMID:35390316
39. Zhang-Velten ER, Parsons D, Lee P, Chambers E, Abdulrahman R, Desai NB, Dan T, Wardak Z, Timmerman R, Vusirikala M, Patel P, **Simms-Waldrip T**, **Aquino V**, **Koh A**, Tan J, Iqbal Z, Zhang Y, Reynolds R, Chiu T, Joo M, Hrycushko B, Ouyang L, Lamphier R, Yan Y, Jiang SB, Kumar KA, Gu X. [Volumetric Modulated Arc Therapy Enabled Total Body Irradiation \(VMAT-TBI\): Six-year Clinical Experience and Treatment Outcomes](#). *Transplant Cell Ther*. 2022 Feb;28(2):113.e1-113.e8. PMID:34775145
40. **Zia A**, Stanek J, Christian-Rancy M, Savelli S, O'Brien SH. [Iron deficiency and fatigue among adolescents with bleeding disorders](#). *Am J Hematol*. 2022 Jan 1;97(1):60-67. PMID:34710246