Division Introduction

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

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

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 1000 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 planning for a similar clinic at 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 disease. Laboratory research efforts include both basic and translational studies that help to bridge the lab and clinical venues. The clinical research efforts include a portfolio of 50 or more clinical research studies extending from clinical trials sponsored by the National Cancer Institute through the Children's Oncology Group; clinical research studies supported by other grant funding agencies, including the 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, and 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 29 faculty, and nine fellows.

Ashley P. Bui, M.D.
Assistant Professor

B.A., Medical Humanities, *summa cum laude*
Baylor University, Waco, TX, 2011
M.D.
Texas A&M University College of Medicine, Temple, TX, 2015
Postdoctoral Training
Residency, Pediatrics
University of Texas at Austin/Dell Children's Medical Center, Austin, TX (2015-2018)
Fellowship, Pediatric Hematology-Oncology
UT Southwestern, (2018-2021)
Interests: Management of children with cancer and blood disease, especially children with neurofibromatosis or brain tumor

Sisi Zheng, M.D.
Instructor

B.S.
University of Pennsylvania, Philadelphia, PA, 2010
M.D.
Weill Cornell Medical College, New York, NY, 2014
Postdoctoral Training
Residency, Pediatrics
Children's Hospital of Philadelphia, Philadelphia, PA, 2014-2017
Fellowship, Pediatric Hematology-Oncology
Children's Hospital of Philadelphia, Philadelphia, PA, 2014-2017
Fellowship, Postdoctoral – Division of Cancer Pathobiology
Children's Hospital of Philadelphia, Philadelphia, PA 2014-2017
Interests: Inpatient care of Hematopoietic Stem Cell Transplant patients and patients with non-malignant hematology conditions

Honors / Awards

Best Pediatric Specialists in Dallas, *D Magazine*

- Laura Klesse
- Patrick Leavey
- Kathleen Ludwig
- Martha Pacheco
- Stephen Skapek
- Tamra Slone
- Tanya Watt
- Jonathan Wickiser
- Naomi Winick

Texas Super Doctors, *Texas Monthly Magazine*

- Kathryn Dickerson, 2021 Texas Rising Star
- Samuel John, 2021 Texas Rising Star
- Naomi Winick

Erin Butler

- Call for Cure

Matt Campbell

- SCCC Early-Career Investigator Award

Kathryn Dickerson

- Dr. George Buchanan Teaching Award for Excellence in Fellow Education

Jessica Garcia

- 2021 HTRS Research Colloquium

Andrew Koh

- Texas Medical Center, CPRIT

Sisi Zheng

- Hyundai Young Investigator Grant from Hyundai Hope on Wheels

Yanbin Zheng

- Children's Cancer Fund Research Grant Award

Ayesha Zia

- NHLBI Bridge Award

Invited Lectures

Victor Aquino

- State of Texas Lions Club's Monthly Diabetes, Wellness and Beyond Webinar Series (Virtual), TX, February 2021
    - *"Blood Disorder & Blood Related Issues in Pediatric Cancer"*

Daniel Bowers

- Carson Leslie Foundation Researchers Roundup, Dallas, TX, March 2021
    - *"Second Malignancies Among Survivors of Childhood Brain Tumors"*

Erin Butler

- Pediatric Hematology Oncology Grand Rounds – UT Southwestern Medical Center, Dallas, TX January 2021
    - *"Functional imaging of RAS pathway targeting in malignant peripheral nerve sheath tumor cells and xenografts"*

Kenneth Chen

- DICER1 Symposium, Children's Minnesota (Virtual), 2021
    - *"Pineoblastoma: miRed in Development"*

John Samuel

- Pediatric Grand Rounds, UT Austin (Virtual), June 2021
    - *"Immune Effector Cell Therapy in Pediatrics: Updates and novel research"*

Andrew Koh

- Fungal Host Microbiota Program Seminar Series (Virtual), September 2021
    - *"Candida albicans Gastrointestinal Colonization and Dissemination"*
- The Scientist Webinar Series (Virtual), June 2021
    - *"Gut Microbiota and Cancer Immunotherapy"*
- Simmons Comprehensive Cancer Center, Cellular Networks and Cancer Program (Virtual), April 2021
    - *"Gut Microbiota and Cancer Immunotherapy"*

Patrick Leavey

- American Society of Pediatric Hematology/Oncology, Summer Symposium 2021, UT Austin (Virtual), June 2021
    - *"Artificial Intelligence (AI) in Pediatric Hematology Oncology – Not Just a Neural Net!"*

Tamra Slone

- DFW APHON (Virtual), September 2021
    - *"Artificial Intelligence (AI) in Pediatric Hematology Oncology – Not Just a Neural Net!"*

Conferences

American Society of Pediatric Hematology/Oncology Conference (Virtual), May 2021

Yang A, Smith C, Callan A, Liu C, Stewart R, Sengupta A, **Leavey P**
"Synovial sarcoma and subsequent papillary thyroid carcinoma in a pediatric patient."

Hanna M, **Pacheco M**, **Leavey P**
"Maria Hanna, Martha Pacheco and Patrick Leavey"

American Psychosocial Oncology Society, Annual Conference (Virtual), March 2021

Pennant S, Oppenheim J, Germann J**, Shliakhtsitsava K**, Cochran C, Bowers D
"Preliminary Findings from Implementing Routine Psychosocial Screening in a Pediatric Cancer Survivorship Clinic."
"Virtual Psychosocial Screening in a Multidisciplinary Pediatric Oncology Survivorship Clinic After the Onset of COVID-19."

Other Conferences

Bui A, John S
ASPHO Conference (Virtual), April 2021
Poster Presentation, *"Acute lymphoblastic leukemia presenting with hypereosinophilic syndrome."*

Klesse L
CTF Virtual Case Conference (Virtual), February and May 2021
Panel Speaker, *"MPNST and Pain"*

Klesse L

Children's Tumor Foundation National Neurofibromatosis Annual Meeting (Virtual), June 2021
Panel Speaker, *"MPNST"*
Presentation*, "Patient Access to Novel NF Treatments: Current Clinical Practices and Identification of Barriers"*
Session Chair*, "Clinical Science Platform"*

Koh A

World Microbe Forum (Virtual), June 2021
Session Presentation, "*Manipulating the Microbiome to Prevent Infection."*

Leavey P

American Society of Pediatric Hematology/Oncology, Summer Symposium 2021, UT Austin (Virtual), June 2021
Oral Presentation, *"Artificial Intelligence (AI) in Pediatric Hematology Oncology – Not Just a Neural Net!"*

Pappo A, **Watt T**, Petrilli S, et al.
International Society of Pediatric Oncology (Virtual), Honolulu, HI, October 2021
Poster Presentat*ion: "Pembrolizumab in Pediatric Patients with Relapsed or Refractory Classical Hodgkin Lymphoma"*

Odonohue T, **Watt T**, Mascarenhas, L, et al.
International Society of Pediatric Oncology (Virtual), Honolulu, HI, October 2021
Oral Presentation: *"A Phase ½, Open-label Study of Repotrectinib in Pediatric and Young Adult Patients with Advanced or Metastatic Malignancies Harboring ALK, ROS1, or NTRK1-3 Alterations."*

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 ACGME-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-Oncology is one of the core subspecialties for pediatric residents at UT Southwestern. All PL-1's spend four weeks covering the Inpatient Hematology/Oncology Service at Children's. Each month a PL-2 or PL-3 supervising resident and two or three PL-1s are assigned to the service. The month spent on the rotation allows residents to learn to take care of what can be very complicated and sick patients with life threatening disease. Residents often look back on this time as a very rewarding experience.

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

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

Fellows

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

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, to extend their research training.

Research Activities

Many faculty members of the Division of Pediatric Hematology/Oncology carry out laboratory and clinical research that is helping to reveal fundamental aspects of disease biology and beginning to lead to new, better therapies. Examples below represent just a sampling of that research.

Laboratory Research

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

Clinical Research

Physicians in our Division are engaged in a wide range of clinical research efforts spanning the cancer and blood disease programs. Clinical research efforts are supported by robust infrastructure provided by the Clinical Research Office (CRO) within the Gill Center and the Simmons Comprehensive Cancer Center at UT Southwestern, the only NCI-designated comprehensive cancer center in North Texas. At any point, 50 to 75 oncology trials and 20 to 30 hematology trials are open for enrollment for Gill Center patients. Clinical research projects are funded by a variety of mechanisms including funding from the NIH National Cancer Institute and the Cancer Prevention and Research Institute of Texas as well as other local and regional grant funding agencies and industry partners.

Particularly notable clinical research accomplishments over the past two years has been the focus on cancer. Our center was among only approximately 12 sites in the US that helped to demonstrate the usefulness of Kymriah, a CAR T-cell therapy targeting CD19-positive relapsed B-cell leukemia in children. Kymriah is now approved for this indication by the US Food and Drug Administration (FDA).

We are taking advantage of a period of transition to strategically launch a new **Cell and Immunotherapy Program (CITP)** for childhood cancer and blood disease. This clinical, research, and educational initiative reflects the emerging importance and, in some cases, routine use of cellular and immunotherapies in the clinical arena. 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. Patrick Leavey, Professor of Pediatrics, to identify and develop new early-phase clinical trials for children with cancer and blood disease.

Also notable, our site has joined the Neurofibromatosis (NF) Clinical Trials Consortium, a nationwide consortium of approximately 20 sites funded by the US Army Medical Research and Materiel Command. This selection enables Dr. Laura Klesse, Associate Professor of Pediatrics, to provide the newest therapies in the form of clinical trials to children with neurofibromatosis, a neurodevelopmental and cancer predisposition syndrome.

Finally, this year marked the continued growth of our still-nascent **Precision Medicine Program**, co-led by Drs. Klesse and Kathleen Ludwig, Assistant Professor of Pediatrics, as a multifaceted program to identify causes of cancer in individual children and use that information to provides more "precise" therapies.

Active areas of CLINICAL research include:

- Prospective clinical trials for children with cancer, conducted under the umbrella of the NCI-sponsored Children's Oncology Group
- Prospective, early-phase clinical trials for children with hematological malignancies, conducted as part of the Therapeutic Advances in Childhood Leukemia and Lymphoma (TACL) consortium and other academic and industry partners
- Prospective therapeutic trials for children with sickle cell disease, iron deficiency anemia and hemophilia
- Investigator-initiated and industry-sponsored therapeutic studies of children with cancer and blood disease
- Retrospective research studies investigating molecular and clinical factors influencing late effects in childhood cancer survivors
- Prospective and retrospective studies assessing a variety of quality measures of children with chronic 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 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 a sampling of the Institutional Review Board (IRB) trials for children with cancer or blood disease at UT Southwestern:

Victor Aquino

- Center for International Blood and Marrow Transplant Research (CIBMTR) - Consent for Participation and Donation of Blood Samples
- 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
- 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
- BMT CTN #1204, Reduced-Intensity Conditioning for Children and Adults with Hemophagocytic Syndromes or Selected Primary Immune Deficiencies (RICHI)
- 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
- BMT CTN 1202, Prospective Multi-Center Cohort for the Evaluation of Biomarkers Predicting Risk of Complications and Mortality Following Allogeneic HCT
- BP-U-004, Phase I/II study of CaspaCide T Cells from an HLA-partially Matched Family Donor After Negative Selection of TCR αβ+T Cells in Pediatric Patients Affected by Hematological Disorders
- CMX001-351, An Intermediate-size, Expanded Access Protocol to Provide Brincidofovir for the Treatment of Serious Adenovirus Infection or Disease

- Transition from Hospital to Home Following Hematopoietic Stem Cell Transplantation: A Feasibility Study for "Rooming In"
- GC P#05.01.020, A Multicenter, Randomized, Phase III Registration Trial of Transplantation of NiCord®, Ex Vivo Expanded, Umbilical Cord Blood-derived, Stem and Progenitor Cells, versus Unmanipulated Umbilical Cord Blood for Patients with Hematological Malignancies
- 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

Daniel Bowers

- ACNS0332, Efficacy of Carboplatin Administered Concomitantly with Radiation and Isotretinoin as a Prop-Apoptotic Agent in Other Than Average Risk Medulloblastoma/PNET Patients.
- 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
- "ACNS0821, Temozolomide with Irinotecan Versus Temozolomide, Irinotecan Plus Bevacizumab for Recurrent/Refractory Medulloblastoma/CNS PNET of Childhood, A COG Randomized Phase II Screening Trial
- Phase II Trial of Molecularly Determined Treatment of Children and Young Adults with Newly Diagnosed Diffuse Intrinsic Pontine Gliomas
- ACNS1123, Phase 2 Trial of Response-Based Radiation Therapy for Patients with Localized Central Nervous System Germ Cell Tumors
- SJMB12, A Clinical and Molecular Risk-Directed Therapy for Newly Diagnosed Medulloblastoma
- H-29892, Case Ascertainment for Epidemiologic Studies of Childhood Cancers and Hematological Conditions
- ACNS1422, A Phase 2 Study of Reduced Therapy for Newly Diagnosed Average-RiskWNT-Driven Medulloblastoma Patients
- CRAD001CUS224T, Phase II Study of Everolimus (RAD001, AFINITOR®) for Children with Recurrent or Progressive Ependymoma
- MEK162, Phase I-II Study of MEK 162 for Children with Low-Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors
- Evaluation of the Efficacy of Re-irradiation for Locally Recurrent Ependymoma [A Multi-Institutional Retrospective Chart Review]
- Childhood Cancer Survivor Study Expansion: Long-Term Follow-up Study
- Childhood Cancer Survivor Study
- ALTE11C2, Health Effects after Anthracycline and Radiation Therapy (HEART): Dexrazoxane and Prevention of Anthracycline-related Cardiomyopathy
- ALTE1621, Pharmacologic Reversal of Ventricular Remodeling in Childhood Cancer Survivors at Risk for Heart Failure (PREVENT-HF): A Phase 2b Randomized Placebo-Controlled (Carvedilol) Trial

Kathryn Dickerson

- TransIT, Unrelated Donor Transplant Versus Immune Therapy in Pediatric Severe Aplastic Anemia PBMTC NMD1601
- ETB115E2201: A Phase II, Open-label, Non-controlled, Intra-patient Dose-escalation Study to Characterize the Pharmacokinetics After Oral Administration of Eltrombopag in Pediatric Patients with Refractory, Relapsed or Treatment Naïve Severe Aplastic Anemia (SAA) or Recurrent Aplastic Anemia (AA)

Laura Klesse

- Bio-specimen Bank for Pediatric Tumors and Cancer Predispositions

- ACCESS/REDIAL, Case Ascertainment for Epidemiologic Studies of Childhood Cancers and Hematological Conditions Used by Adolescent and Childhood Cancer Epidemiology and Susceptibility Service (ACCESS) for Texas and Reducing Ethnic Disparities in Acute Leukemias (REDIAL) Consortium
- Developing Evidence-Based Criteria for Initiating Treatment for Neurofibromatosis type 1 Associated Optic Pathway Gliomas
- Cardiovascular Abnormalities in Pediatric Patients with Neurofibromatosis Type 1
- Compassionate Use of Trametinib in Low Grade Glioma
- NF1 LGG Synodos: Target Identification of Neurofibromatosis Type 1 Associated Low Grade Glioma

Andrew Koh

- Role of Commensal Flora in the Development of Bacteremia and Fungemia in Cancer and Stem Cell Transplant Patients

Patrick Leavey

- 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
- ALTE05N1, Umbrella Long-Term Follow-up Protocol
- ALTE16C1,Effects of Modern Chemotherapy Regimens on Spermatogenesis and Steroidogenesis in Adolescent and Young Adult (AYA) Survivors of Osteosarcoma
- SPOC-2012-001, Phase 1 Dose-escalating Study of MM-398 (Irinotecan Sucrosofate Liposome Injection) Plus Intravenous Cyclophosphamide in Recurrent or Refractory Pediatric Solid Tumors
- Using Imaging and Computational Tools to Improve Risk Stratification in Children with Bone Cancer
- Prospective Evaluation of the Use of Imaging and Computational Tools to Improve Risk Stratification in Children with Bone Cancer
- AEWS1031, A Phase III Randomized Trial of Adding Vincristine-Topotecan-Cyclophosphamide to Standard Chemotherapy in Initial Treatment of Non-metastatic Ewing Sarcoma
- 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
- Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas - Biospecimen Banking Protocol
- Evaluation of ctDNA as a Prognostic Biomarker for Patients with Newly Diagnosed Localized Ewing Sarcoma or Osteosarcoma
- Identification of Anxiety and Depression in Children with Cancer
- APEC14B1, Project: EveryChild- A Registry, Eligibility Screening, Biology and Outcome Study

Kathleen Ludwig

- Pediatric Hematology and Oncology Bio-Specimen Repository
- "ADVL1521, A Phase 2 Study of the MEK inhibitor Trametinib (IND #119346, NSC# 763093) in Children with Relapsed or Refractory Juvenile Myelomonocytic Leukemia"

Martha Pacheco

- ALTE11C1, Longitudinal Assessment of Ovarian Reserve in Adolescents with Lymphoma
- AHOD04B1, Hodgkin Disease (HD) Banking Study
- ANHL1131, Intergroup Trial for Children or Adolescents with B-cell Non-Hodgkin Lymphoma (NHL) or Mature B-cell Leukemia (B-AL): Evaluation of Rituximab Efficacy and Safety in High Risk Patients
- 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

- 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
- AHOD1721 (CA209744), Risk-based, Response-adapted, Phase II Open-label Trial of Nivolumab + Brentuximab Vedotin (N + Bv) for Children, Adolescents, and Young Adults with Relapsed/refractory (R/R) CD30 + Classic Hodgkin lymphoma (cHL) After Failure of First-line Therapy, Followed by Brentuximab + Bendamustine (Bv + B) for Participants with a Suboptimal Response
- 54179060LYM3003, A Randomized, Open-label, Safety and Efficacy Study of Ibrutinib in Pediatric and Young Adult Patients with Relapsed or Refractory Mature B-cell Non-Hodgkin Lymphoma
- Once-Weekly Intravenous Liposomal Amphotericin B (AmBisome) for Fungal Prophylaxis in Pediatric High-risk Hematologic Malignancy: A Retrospective Evaluation of Safety and Tolerability
- ICON 1: Physician Treatment Decisions and Patient-Reported Outcomes in Pediatric Refractory Immune Thrombocytopenia

An Pham

- B5201002: A Phase 3, Multicenter, Randomized, Double Blind, Placebo Controlled, Parallel Group Study to Evaluate the Efficacy and Safety of Rivipansel (GMI 1070) in the Treatment of Vaso-occlusive Crisis in Hospitalized Subjects with Sickle Cell Disease
- B5201003: An Open-label Extension Study to Evaluate the Safety of Rivipansel (GMI-1070) in the Treatment of One or More Vaso-occlusive crises (VOC) in Hospitalized Subjects with Sickle Cell Disease

Tiffany Simms-Waldrip

- 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
- 15-007, A Phase 3, Randomized, Adaptive Study Comparing the Efficacy and Safety of Defibrotide Versus Best Supportive Care in the Prevention of Hepatic Veno-occlusive Disease in Adult and Pediatric Patients Undergoing Hematopoietic Stem Cell Transplant
- The Role of the Host Microbiome in the Health of Cancer and Stem Cell Transplant Patients
- Identifying Risk Factors Associated with Supra-Therapeutic Levels Following Initial Tacrolimus Dosing

Stephen Skapek

- Molecular Characterization of Childhood Cancer Specimens

Tamra Slone

- ANHL1522,A Pilot Study of Rituximab (RTX) and Third Party Latent Membrane Protein (LMP)-specific Cytotoxic T-Lymphocytes (LMP-TC, IND # 17068) in Pediatric Solid Organ Recipients (SOT) with EBV-Positive CD20-Positive Post-Transplant Lymphoproliferative Disease (PTLD)
- 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
- 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)
- AAML1331, A Phase III Study for Patients with Newly Diagnosed Acute Promyelocytic Leukemia (APL) using Arsenic Trioxide and All-Trans Retinoic Acid
- 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
- AALL15P1, A Group-wide 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

- 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
- AALL05B1: A Children's Oncology Group Protocol for Collecting and Banking Relapsed Acute Lymphoblastic Leukemia Research Specimens
- AAML1531, Risk-stratified Therapy for Acute Myeloid Leukemia in Down Syndrome
- AALL0932, Treatment of Patients with Newly Diagnosed Standard Risk B-Lymphoblastic Leukemia (B-ALL) or Localized B-lineage Lymphoblastic Lymphoma (B-LLy)
- 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
- 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)
- AALL1631: International Phase 3 Trial in Philadelphia Chromosome-positive Acute Lymphoblastic Leukemia (Ph+ ALL) Testing Imatinib in Combination with Two Different Cytotoxic Chemotherapy Backbones
- Evaluation of the Influence of Abnormal Glucose Metabolism on the Risk of Infection in Children with Acute Lymphoblastic Leukemia and Lymphoblastic Lymphoma
- AALL08B1, Classification of Newly Diagnosed 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)

Tanya Watt

- ANBL12P1, Pilot Study Using Myeloablative Busulfan/Melphalan (BuMel) Consolidation Following Induction Chemotherapy for Patients with Newly Diagnosed High-Risk Neuroblastoma
- ANBL00B1, Neuroblastoma Biology Studies
- ANBL1221, A Phase II Randomized Trial of Irinotecan/Temozolomide with Temsirolimus (NSC# 683864, IND# 61010) or Chimeric 14.18 Antibody (ch14.18) (NSC# 623408, IND# 4308) in Children with Refractory, Relapsed or Progressive 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
- ANBL1232, Utilizing Response- and Biology-Based Risk Factors to Guide Therapy in Patients with Non-High-Risk Neuroblastoma
- An Open Label, Expanded Access Protocol Using 131I-Metaiodobenzylguanidine (131I-MIBG) Therapy in Patients with Refractory Neuroblastoma, Pheochromocytoma, or Paraganglioma
- MIBG 2014-01, A Phase II Single-Arm Study of Therapeutic Iobenguane (131) for High-Risk Neuroblastoma Subjects at the Time of First Relapse
- ALTE15N2, LEAHRN (Late Effects After High-Risk Neuroblastoma) Study
- ANBL1531, A Phase 3 Study of 131I-Metaiodobenzylguanidine (131I-MIBG) or Crizotinib Added to Intensive Therapy for Children with Newly Diagnosed High-Risk Neuroblastoma (NBL) (IND# 134379)
- SPOC-2013-001, Phase I Study of Fenretinide (4-HPR, NSC 374551) Lym-X-Sorb (LXS) Oral Powder Plus Ketoconazole Plus Vincristine in Patients with Recurrent or Resistant Neuroblastoma (IND #: 68,254)
- SPOC-2014-001, Expanded Access Study of Fenretinide (4-HPR, NSC 374551) Lym-X-Sorb (LXS) Oral Powder Plus Ketoconazole in Patients with Recurrent or Resistant Neuroblastoma (IND #68,254)
- "Pediatric Solid Tumor Metabolism
- A Prospective, Single-center Study Exploring Solid Tumor Metabolism of Extra-Cranial Tumors in the pediatric Population

Jonathan Wickiser

- AREN03B2: Renal Tumors Classification, Biology and Banking
- 9442: National Wilms Tumor Late Effects Study
- AHEP0731, Treatment of Children with All Stages of Hepatoblastoma with Temsirolimus (IND#122782, NSC#683864) Added to High Risk Stratum Treatment

- 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
- AHEP1531, Pediatric Hepatic Malignancy International Therapeutic Trial (PHITT)
- AGCT1532, A Randomized Phase 3 Trial of Accelerated Versus Standard BEP Chemotherapy for Patients with Intermediate and Poor-risk Metastatic Germ Cell Tumors

Naomi Winick

- The Feasibility and Acceptability of Incorporating Electronic Assessment Tools during Outpatient Visits for Patients in the Maintenance Phase of Therapy for Acute Lymphoblastic Leukemia
- ACCL0922, (SCUSF 0901) A Phase II Placebo-Controlled Trial of Modafinil to Improve Neurocognitive Deficits in Children Treated for a Primary Brain Tumor
- ACCL10P1, Computerized Cognitive Training for Pediatric Brain Tumor Patients: A Pilot Study
- AALL1122, A Phase 2 Multi-Center Historically - Controlled Study of Dasatinib Added to Standard Chemotherapy in Pediatric Patients with Newly Diagnosed Philadelphia Chromosome Positive Acute Lymphoblastic Leukemia (Ph+ ALL)
- Evaluation of MicroRNAs as Novel Markers of Cardiotoxicity in Children Undergoing Anthracycline Therapy for Pediatric Cancer
- Inpatient Outcomes and Chemotherapy Related Toxicities Among a National Cohort of Children with Acute Leukemia
- ACCL1033, A Comprehensive Approach to Improve Medication Adherence in Pediatric ALL
- Aim 1, Home or Away from Home: Comparing Clinical Outcomes Relevant to the Care of Pediatric Acute Myeloid Leukemia During Periods of Neutropenia
- 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
- 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.
- Assessing Neurocognitive Functioning Over Time in Pediatric Patients with Non-CNS Solid Tumors and Nonlymphoblastic Leukemia/Lymphomas
- Feasibility of Home-Based Computerized Cognitive Training during Maintenance Therapy for ALL
- ALTE1631, A Randomized Web-based Physical Activity Intervention among Children and Adolescents with Acute Lymphoblastic Leukemia
- A Randomized Evaluation of a Six-Week Grief Curriculum for Bereaved Parents
- Risk Factors for Toxicity During the Induction and Delayed Intensification Phases of Treatment for Acute Lymphoblastic Leukemia (ALL)

Ayesha Zia

- Multicenter, Open-Label, Active-controlled, Randomized Study to Evaluate the Efficacy and Safety of an age-and-body Weight-adjusted Rivaroxaban Regimen Compared to Standard of Care in Children with Acute Venous Thromboembolism
- Evaluation of Thrombin Generation in Children with Venous Thromboembolism
- Physical Activity in Children at Risk of Post-thrombotic Syndrome: A Pilot Randomized Controlled Trial
- Outcomes after pediatric venous thromboembolism
- Thrombin Generation in Children with Sickle Cell Anemia Using Platelet-Rich Plasma and Platelet-Poor Plasma
- Comprehensive and Multidisciplinary Approach to Evaluation of Young Women with Heavy Menstrual Bleeding (HMB): Impact on Diagnosis, Management and Outcomes
- Kids-DOTT: Prospective Multi-Center Evaluation of the Duration of Therapy for Thrombosis in Children (Protocol # 03-585)
- Zimmerman Program for the Molecular and Clinical Biology of VWD
- The American Thrombosis and Hemostasis Network (ATHN)
- NN7999-3774 Safety, Efficacy and Pharmacokinetics of N9-GP in Previously Treated Children with Hemophilia B

- CDC Public Health Surveillance for Bleeding Disorders - Registry for Bleeding Disorders Surveillance
- "My Life Our Future: A Hemophilia Genotyping Initiative
- Data and Sample Research Repository"
- A Longitudinal, Observational Study of Previously Treated Hemophilia Patients (PTPs) Switching Coagulation Replacement Factor Products (ATHN-2: Switching Study)
- ATHN 4: VTE Project - Transition of Care for patients with Venous Thromboembolism (VTE) at ATHN (American Thrombosis and Hemostasis Network) Affiliated Sites
- An Open-Label, Multicenter Evaluation of the Safety and Efficacy of Recombinant Coagulation Factor VIII Fc Fusion Protein (rFVIIIFc; BIIB031) in the Prevention and Treatment of Bleeding in Previously Untreated Patients with Severe Hemophilia A
- Genotype and Phenotype Analysis of Adolescents with Heavy Menstrual Bleeding and Low von Willebrand Activity

Research Funding

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

Clinical Activities

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

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

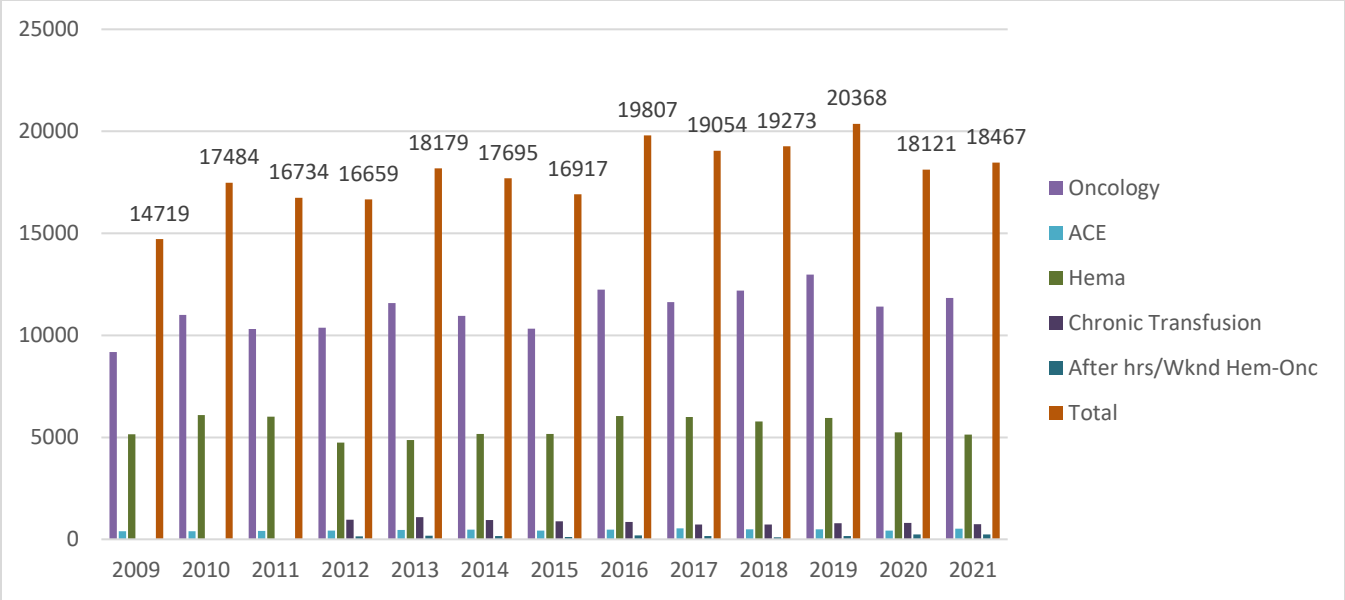
Core Clinical Programs in Hematology and Oncology

- 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

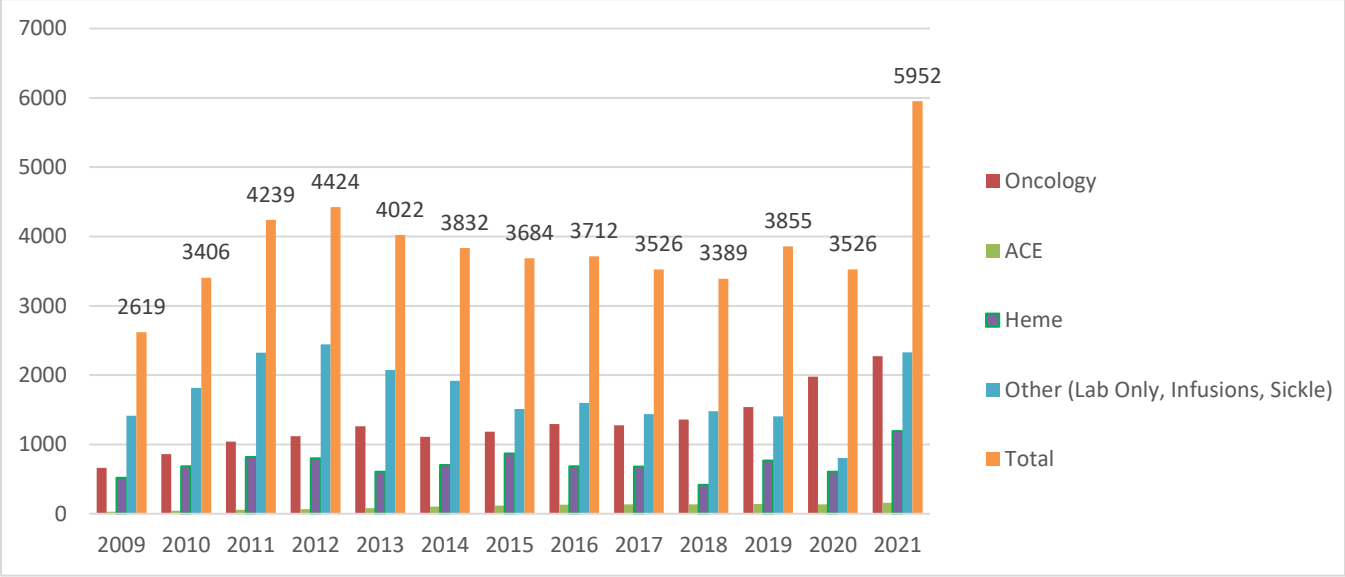
Additional Clinical/Research Programs

- After the Cancer Experience (ACE) 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.

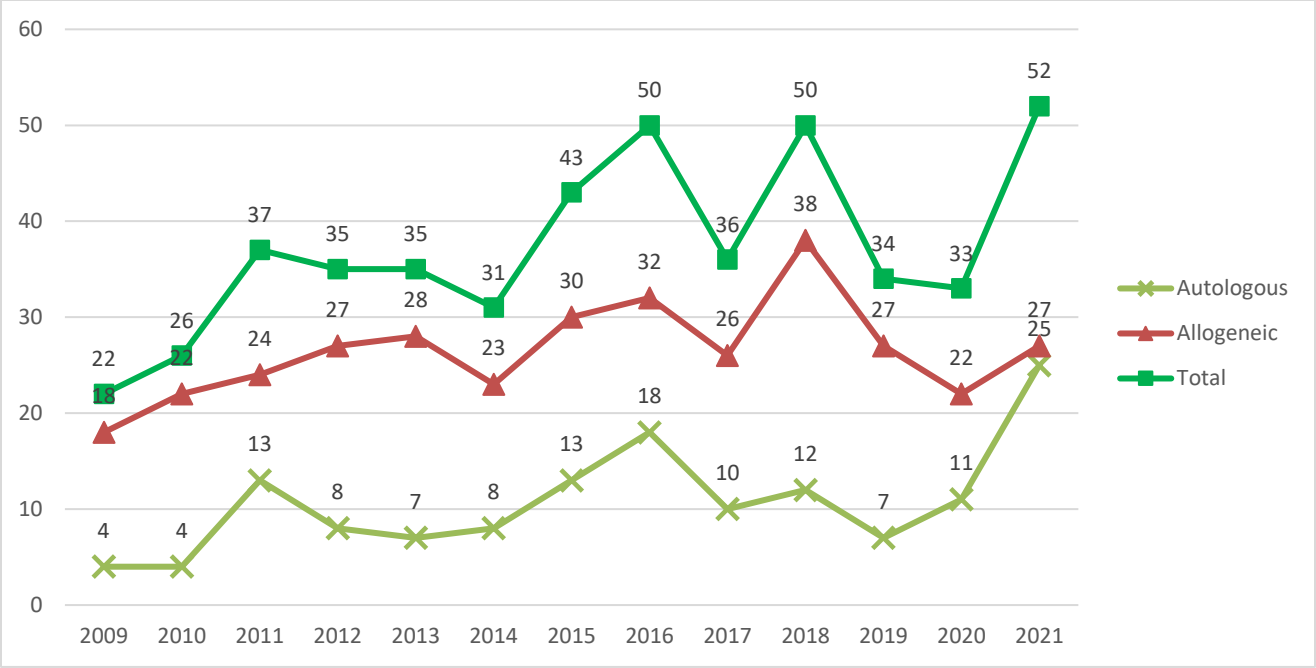


Dallas Clinic Visits



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: 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 – 04/2021

Grantor: Children's Hospital Los Angeles
Title of Project: A Retrospective and Cross-Sectional Analysis of Patients Treated for SCID since January 1, 1968
Role: Principal Investigator
Dates: 09/2020 – 08/2021

Grantor: Children's Hospital Los Angeles
Title of Project: A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders
Role: Principal Investigator
Dates: 09/2020 – 08/2021

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 – 04/2025

Grantor: Pediatric Blood & Marrow Transplant Cons
Title of Project: PBMTC NMD 1801 (CSIDE), A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCRαβ+/CD19+ depleted transplantation: A Phase II study by the Primary Immune Deficiency Treatment Consortium (PIDTC) and Pediatric Blood and Marrow Transplant Consortium (PBMTC) PIDTC "CSIDE" Protocol (Conditioning SCID Infants Diagnosed Early)
Role: Principal Investigator
Dates: 05/2019 – 04/2022

Grantor: University Of California, San Francisco
Title of Project: Primary Immune Deficiency Treatment Consortium
Role: Principal Investigator
Dates: 09/2020 – 08/2021

Daniel Bowers

Grantor: 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: Children's Hospital Of Philadelphia
Title of Project: Health Effects after Anthracycline and Radiation Therapy - Leukemia & Lymphoma Society
Role: Principal Investigator
Dates: 02/2014 – 02/2024

Grantor: Children's Hospital Of Philadelphia
Title of Project: Health Effects after Anthracycline and Radiation Therapy - St. Baldrick's Foundation
Role: Principal Investigator
Dates: 02/2014 – 02/2024

Grantor: Children's Hospital of Philadelphia
Title of Project: Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)
Role: Co-Investigator
Dates: 03/2016 – 02/2025

Grantor: NIH/ Children's Oncology Group Operations Ctr
Title of Project: COG NCTN Study Chair ACNS1931
Role: Principal Investigator
Dates: 02/2021 – 02/2022

Erin Butler

Grantor: North American Consortium Histiocytosis - St. Jude Children's Research Hospital
Title of Project: Martin - North American Consortium for Histiocytosis LCH-IV Study
Role: Principal Investigator
Dates: 06/2019 – 06/2022

Kenneth Chen

Grantor: Alex's Lemonade Stand
Title of Project: Bortezomib as a novel therapeutic for Wilms Tumor
Role: Principal Investigator
Dates: 11/2019 – 10/2021

Grantor: Cancer Prevention & Research Institute Of Texas
Title of Project: Recruitment of First-Time, Tenure-Track Faculty
Role: Principal Investigator
Dates: 08/2018 – 08/2023

Grantor: NIH - National Cancer Institute
Title of Project: The Role of PLAG1 In Wilms Tumor Formation
Role: Principal Investigator
Dates: 07/2017 – 06/2022

Grantor: NIH - National Cancer Institute
Title of Project: NCTN - Rare Tumor Committee Chair
Role: Principal Investigator
Dates: 03/2020 – 02/2022

Kathryn Dickerson

Grantor: Boston Children's Hospital
Title of Project: Feasibility Study Randomizing IST Vs URD BMT for Children with Aplastic Anemia
Role: Principal Investigator
Dates: 04/2018 – 03/2021

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 – 6/2022

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 – 01/2022

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 – 01/2023

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:

Grantor: Cary Council- Doc Stars
Title of Project: Novel cell therapy discovery program
Role: Principal Investigator
Dates:

Laura Klesse

Grantor: Baylor College Of Medicine - Cancer Prevention & Research Inst Of Tx
Title of Project: The Adolescent and Childhood Cancer Epidemiology and Susceptibility Service (ACCESS) for Texas
Role: Principal Investigator
Dates: 08/2017 – 08/2022

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

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: University Of Alabama At Birmingham
Title of Project: NF Clinical Trials Consortium
Role: Principal Investigator
Dates: 08/2017 – 08/2022

Grantor: UT Health Science Center at San Antonio
Title of Project: Texas Pediatric Patient Derived Xenograft Facility
Role: Co-Investigator
Dates: 06/2018 – 05/2021

Andrew Koh

Grantor: Cancer Prevention & Research Inst of TX RP180805 (Xie)
Title: Pediatric Cancer Data Core
Role: Co-Investigator
Dates: 08/31/2018 – 08/30/2023

Grantor: Children's Cancer Fund, Call For Cure 2021
Title: Role of Gut Microbiota-Derived Metabolites in Modulating Cancer Immunotherapy
Role: Principal Investigator
Dates: 7/1/21 – 6/30/22

Grantor: Curing Kids Cancer
Title: Identifying Microbial Biomarkers to Predict CAR-T Therapy Outcomes
Role: Principal Investigator
Dates: 1/15/21 – 1/14/22

Grantor: H Lee Moffitt Cancer Center & Rsrch Inst/ Pediatric Cancer Research Foundation
Title of Project: The Sunshine Project -- Microbiome Study
Role: Principal Investigator
Dates: 07/2019 – 06/2021

Grantor: NIH/NCI
Title of Project: Role of the Gut Microbiota in Modulating Immune Checkpoint Inhibitor Therapy for Cancer
Role: Principal Investigator
Dates: 09/2019 – 08/2024

Grantor: NIH - NIAID
Title of Project: Candida Albicans Gastrointestinal Colonization and Dissemination
Role: Principal Investigator
Dates: 09/2016 – 08/2021

Grantor: NIH - NIAID
Title of Project: Mentoring Patient Oriented Research in the Microbiome of Cancer and Stem Cell Transplant Patients
Role: Principal Investigator
Dates: 02/2014 – 03/2025

Grantor: NIH/NIDDK, 1R01DK115703-01 (Moe, Browning, Sakhaee)
Title: Origin of Excess Acid in Uric Acid Urolithiasis
Role: Co-investigator
Dates: 9/5/19 – 6/30/24

Grantor: Merck
Title of Project: SRA201912-0003, To test the efficacy of Bacteroides thetaiotamicron and Faecalibacterium prausnitzii in reversing antibiotic-induced hyporesponsiveness to anti-PD-1 therapy in syngeneic murine tumor models, and to investigate the mechanisms underlying the synergistic effect
Role: Principal Investigator
Dates: 7/1/20 – 6/30/22

Grantor: Novartis, (Koh)
Title: CTL019BUS02T, Role of Gut Microbiota in Modulating CAR-T Efficacy and Adverse Effects
Role: Principal Investigator
Dates: 8/1/20 – 7/30/22

Grantor: UTSW Kidney Cancer SPORE Pilot Grant Award
Title: Role of the Microbiome in Modulating Immune Checkpoint Inhibitor Therapy in Kidney Cancer Patients
Role: Principal Investigator
Dates: 5/1/2019 – 4/30/21

Patrick Leavey

Grantor: Children's Hospital of Philadelphia
Title of Project: NIH National Clinical Trials Network (NCTN) Biomarker, Imaging and Quality of Life Studies Funding Program (BIQSFP) PCR via U10CA180886-02S7
Role: Principal Investigator
Dates: 03/2016 – 02/2021

Grantor: Children's Hospital of Philadelphia
Title of Project: Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)
Role: Principal Investigator
Dates: 03/2016 – 02/2025

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: 12/2012 – 1/4/2022

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

Grantor: Children's Hospital Of Philadelphia/ NIH-National Cancer Institute
Title of Project: Specimen Banking Supplements: NCTN Match, Autopsy, Relapse, and Rare Tumor Supplement
Role: Principal Investigator
Dates: 03/2020 – 02/2021

Grantor: Children's Oncology Group Operations Center
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 – 04/2021

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

Grantor: Children's Oncology Group Operations Center
Title of Project: NCI Community Oncology Research Program (NCORP) Grant 2UG1CA189955-06
Role: Principal Investigator
Dates: 09/2019 – 07/2025

Martha Pacheco

Grantor: Children's Hospital of Philadelphia
Title of Project: Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)
Role: Co-Investigator
Dates: 03/2016 – 02/2025

Stephen Skapek

Grantor: Baylor College Of Medicine/ Cancer Prevention & Research Institute of Texas
Title of Project: Genetic Epidemiology and Molecular Basis of Cancer Predisposition in Pediatric Rhabdomyosarcoma
Role: Principal Investigator
Dates: 01/2018 – 11/2021

Grantor: Cancer Prevention & Research Institute of Texas
Title of Project: Pediatric Cancer Data Core
Role: Co-Investigator
Dates: 08/2018 – 8/2023

Grantor: Cancer Prevention & Research Institute of Texas
Title of Project: Rhabdomyosarcoma vulnerabilities: Prioritizing and extending to the clinic
Role: Principal Investigator
Dates: 03/2018 – 02/2022

Grantor: Children's Hospital of Philadelphia
Title of Project: Leadership of Solid Malignancy Integrated Translational Science Center
Role: Principal Investigator
Dates: 03/2016 – 02/2022

Grantor: Children's Hospital of Philadelphia
Title of Project: Contribution to clinical research design and implementation
Role: Principal Investigator
Dates: 03/2017 – 02/2022

Grantor: Children's Hospital of Philadelphia
Title of Project: Contribution to clinical research design and implementation, Soft Tissue Sarcoma
Role: Principal Investigator
Dates: 03/2018 – 02/2022

Grantor: Children's Hospital of Philadelphia
Title of Project: COG PEP-CTN Agent Prioritization Committee -UM1CA228823-01
Role: Principal Investigator
Dates: 09/2019 – 07/2023

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: Nationwide Children's Hospital
Title of Project: COG Biospecimen Bank
Role: Principal Investigator
Dates: 04/2021 – 03/2023

Grantor: NIH-NCI Center For Cancer Research
Title of Project: Targeting EWSR1-FLI1 through Functional, Structural and Chemical Approaches
Role: Co-Investigator
Dates: 09/2019 – 8/2024

Grantor: St. Baldrick's Foundation
Title of Project: Targeting LILRB4 by CAR-T Cells for the Treatment of Pediatric AML
Role: Principal Investigator
Dates: 07/2018 – 06/2021

Grantor: UT Health Science Center at San Antonio
Title of Project: Texas Pediatric Patient Derived Xenograft Facility
Role: Principal Investigator
Dates: 06/2018 – 05/2021

Tamra Slone

Grantor: Children's Hospital of Philadelphia
Title of Project: Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)
Role: Co-Investigator
Dates: 03/2016 – 02/2025

Tanya Watt

Grantor: Children's Hospital of Philadelphia
Title of Project: Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)
Role: Co-Investigator
Dates: 03/2016 – 02/2025

Jonathan Wickiser

Grantor: Children's Hospital of Philadelphia
Title of Project: Children's Oncology Group - National Clinical Trials Network (NCTN) Grant (2U10CA180886)
Role: Co-Investigator
Dates: 03/2016 – 02/2025

Naomi Winick

Grantor: Children's Hospital of Philadelphia
Title of Project: COG NCTN HemOnc Committee
Role: Principal Investigator
Dates: 03/2017 – 02/2025

Grantor: 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 – 05/2021

Grantor: Children's Oncology Group Operations Center
Title of Project: A Randomized Web-based Physical Activity Intervention among Children and Adolescents with Acute Lymphoblastic Leukemia
Role: Principal Investigator
Dates: 07/2018 – 06/2021

Grantor: St. Jude Children's Research Hospital
Title of Project: Web-based physical activity intervention for children with ALL
Role: Principal Investigator
Dates: 07/2018 – 06/2021

Yanbin Zheng

Grantor: Andrew McDonough B+ Foundation
Title of Project: Developing a Novel Therapeutic Strategy for Rhabdomyosarcoma
Role: Principal Investigator
Dates: 01/2019 – 12/2020

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

Ayesha Zia

Grantor: American Thrombosis & Hemostasis Network
Title of Project: A Longitudinal Observational Study of Previously Treated Hemophilia Patients
Role: Principal Investigator
Dates: 07/2015 – 06/2021

Grantor: American Heart Association Innovator Award.
Title of Project Functional Characterization of Children with Chronic Thromboembolic Disease
Role: Principal Investigator
Dates: 5/2021 – 02/2023

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

Grantor: NIH - National Heart, Lung, and Blood Institute
Title of Project: Predicting and Preventing Poor Outcomes of Venous Thromboembolism in Children
Role: Principal Investigator
Dates: 05/2016 – 04/2021

Grantor: NHLBI RO1 HL153963
Title of Project Functional Characterization of Children with Chronic Venous Thromboembolic Disease
Role: Principal Investigator
Dates: 9/2020 – 06/2025

Grantor: UT Health Science Center at Houston
Title of Project: Great Plains Hemophilia Network Hemophilia Treatment Centers
Role: Principal Investigator
Dates: 06/2018 – 05/2020

Grantor: UT Health Science Center at Houston
Title of Project: Public Health Surveillance for Bleeding Disorders
Role: Principal Investigator
Dates: 09/30/2020 – 09/29/2025

Grantor: Versiti Wisconsin, Inc.
Title of Project Zimmerman Program on the Biology of VWD P01
Role: Principal Investigator
Dates: 3/2019 – 02/2024

Peer-Reviewed Publications

1. Advani AS, Larsen E, Laumann K, Luger SM, Liedtke M, Devidas M, Chen Z, Yin J, Foster MC, Claxton D, Coffan K, Tallman MS, Appelbaum FR, Erba H, Stone RM, Hunger SP, McNeer JL, Loh ML, Raetz E, **Winick N**, Carroll W, Larson RA, Stock W. Comparison of CALGB 10403 (Alliance) and COG AALL0232 toxicity results in young adults with acute lymphoblastic leukemia. *Blood Adv.* 2021 Jan 26;5(2):504-512. PMID:33496745

2. Andrés-Jensen L, Attarbaschi A, Bardi E, Barzilai-Birenboim S, Bhojwani D, Hagleitner MM, Halsey C, Harila-Saari A, van Litsenburg RRL, Hudson MM, Jeha S, Kato M, Kremer L, Mlynarski W, Möricke A, Pieters R, Piette C, Raetz E, Ronceray L, Toro C, Grazia Valsecchi M, Vrooman LM, Weinreb S, **Winick N**, Schmiegelow K, Ponte di Legno Severe Toxicity Working Group.. Severe toxicity free survival: physician-derived definitions of unacceptable long-term toxicities following acute lymphocytic leukaemia. *Lancet Haematol.* 2021 Jul;8(7):e513-e523. PMID:34171282

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