

## Division Introduction

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

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

As the 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 more than 600 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. 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.



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

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.

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## Faculty

The Division has a team of 27 faculty, and eight fellows. Four new faculty joined the team in 2018.

### **Erin Butler, M.D.**

Assistant Instructor



**B.M.**, *summa cum laude*

DePaul University, Chicago, IL, 2008

**M.D.**

UT Southwestern, 2012

#### **Postdoctoral Training**

Residency, Pediatrics

UT Southwestern/Children's, 2012-2015

Fellowship, Pediatric Hematology-Oncology

UT Southwestern/Children's, 2015-2018

#### **Interests**

Soft tissue sarcomas

### **Jessica Garcia, M.D.**

Instructor



**B.S.**

University of Illinois, Urbana-Champaign, IL, 2007

**M.D.**

University of Illinois College of Medicine, Peoria, IL, 2012

#### **Postdoctoral Training**

Residency, Pediatrics

University of Illinois College of Medicine, Peoria, IL, 2012-2015

Fellowship, Pediatric Hematology-Oncology

Medical College of Wisconsin Affiliated Hospitals, Milwaukee, WI, 2015-2018

#### **Interests**

Health related quality of life outcomes in patients with bleeding disorders, clinical/translational research

### **Holly Pacenta, M.D.**

Instructor



**B.A.**

Miami University, Oxford, OH, 2008

**M.D.**

University of Toledo College of Medicine, Toledo, OH, 2012

#### **Postdoctoral Training**

Residency, Pediatrics

Indiana University School of Medicine, Indianapolis, IN, 2012-2015

Fellowship, Pediatric Hematology-Oncology

University of Colorado School of Medicine, Aurora, CO, 2015-2018

#### **Interests**

Experimental therapeutics, CAR-T

**Ksenya Shliakhtsitsava, M.D., M.A.Sc.**

Assistant Professor



**M.D.**

Belarusian State Medical University, Belarus, 2006

**M.A.Sc.**

University of California, San Diego, CA, 2018

**Postdoctoral Training**

Residency, Pediatrics

Children’s Regional Hospital of Mogilev, Belarus, 2006-2007

University of San Francisco-Fresno, Fresno, CA, 2010-2014

Fellowship, Pediatric Hematology-Oncology

University of California, San Diego, CA, 2014-2017

4<sup>th</sup> Year Research Fellowship, Pediatric Hematology-Oncology

University of California, San Diego, CA, 2017-2018

**Interests**

Leukemia and Lymphoma, Fertility preservation, Cancer survivorship, Reproductive health after cancer treatment completion

**Honors / Awards**

**Promotions**

- Theodore Laetsch, Associate Professor
- Tanya Watt, Associate Professor
- Kathryn Dickerson, Instructor
- Samuel John, Instructor

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

- Daniel Bowers
- Laura Klesse
- Patrick Leavey
- Andrew Martin
- Stephen Skapek
- Tamra Slone
- Jonathan Wickiser
- Naomi Winick

**Texas Super Doctors, Texas Monthly Magazine**

- Kenneth Chen, 2018 Texas Rising Star
- Andrew Martin, 2018 Texas Rising Star
- Naomi Winick

**Theodore Laetsch**

- Rising Star Award - UT Southwestern Leaders in Clinical Excellence

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*Dr. Theodore Laetsch received one of the inaugural UT Southwestern Leaders in Clinical Excellence awards - the Rising Star Award. This award recognizes exceptional early career clinical faculty whose actions and activities consistently exemplify enthusiasm, commitment, professionalism, and leadership. Awardees demonstrate exceptional clinical care beyond the level expected of an early career clinician through a consistent pattern of high-quality care, clinical leadership and innovation.*

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**Invited Lectures**

**Victor Aquino**

- Department of Pediatrics Grand Rounds, Texas Tech University Health Sciences Center, Lubbock, TX, April 2018
  - *“Update of Childhood Low-grade Gliomas”*

**Theodore Laetsch**

- Cancer Drug Development Forum (CDDF) 10<sup>th</sup> Annual Alpine Conference, Innsbruck Austria, February, 2018
  - *“Clinical Development of Larotrectinib”*
- United States and Canadian Academy of Pathology 107<sup>th</sup> Annual Meeting, Vancouver, Canada, March, 2018
  - Session Moderator: *“Identifying the Rare: Evolving Diagnostics Standards in Characterizing Cancer”*
- Children’s Oncology Group Annual Meeting, St. Louis, MO, April, 2018
  - *“A Pediatric Phase I Study of Larotrectinib, a Highly Selective Inhibitor of the Tropomyosin Receptor Kinase (TRK) Family”*

**Ayesha Zia**

- 50<sup>th</sup> Annual Kenneth C. Haltalin Pediatrics for the Practitioner, Irving, TX, April 2018
  - *“Heavy Menstrual Bleeding in Young Women: Causes and Consequences”*
- The Neonatal & Pediatric SSC of the International Society of Haemostasis and Thrombosis, Dublin, Ireland, July 2018
  - *“Update on Appropriate and Necessary Care for Adolescents with Heavy Menstrual Bleeding and Bleeding Disorders”*

**Conference Presentations**

Chi S, Fouladi M, Shukla N, Bourdeaut F, Margo A, Makin G, McCowage G, Wetmore C, Macy M, **Laetsch TW**, et al.

Oral Presentation, ASPHO Annual Meeting, Pittsburgh, PA, May 2018  
*“Phase 1 Study of the EZH2 Inhibitor, Tazemetostat, in Children with Relapsed or Refractory INI1-Negative Tumors Including Rhabdoid Tumors, Epithelioid Sarcoma, Chordoma; and Synovial Sarcoma”*

**Laetsch TW**, Maude SL, Grupp SA, et al.

Oral Presentation, ASPHO Annual Meeting, Pittsburgh, PA, May 2018  
*“Tisagenlecleucel (CTLO19) Therapy Appears Safe and Effective in Pediatric Patients with Down Syndrome with Relapsed/Refractory (R/R) Acute Lymphoblastic Leukemia”*

Shweta Murthi S, Tweed J, Greenwell C, **Zia A**, Raman L.

Poster Presentation, Pediatric Academic Society, Toronto, Canada, May 2018  
*“Acute Traumatic Coagulopathy: Assessing Risk Factors Among Subgroups”*

**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. The numerous institutional resources enabling their ability to secure funding include a Physician Scientist Translational Cancer Research T32 grant through the UTSW Simmons Cancer Center, an NCI designated Comprehensive Cancer Center.



**2018 Incoming Fellows**  
**Maria Hanna, Peter Schoettler and Ashley Bui**

## 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 fruit fly and zebrafish models to understand the genetic defects causing rhabdomyosarcoma, Ewing sarcoma and malignant germ cell tumor
- Using complementary pre-clinical models to dissect the key “vulnerabilities” in rhabdomyosarcoma, Ewing sarcoma, and other soft tissue sarcomas
- Understanding the molecular machinery by which normal cells can undergo “senescence” as a tumor suppressor mechanism in the presence of a cancer-causing oncogene
- 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.
- Understanding how certain cancer-causing mutations influence the metabolism in childhood brain tumors and certain types of sarcoma

- Uncovering how hematopoietic and embryonic stem cells are controlled and how the control mechanisms can go awry in cancer and blood 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 in this past year focus on cancer. Our center was among only approximately 12 sites in the US that helped to demonstrate the usefulness of Kymriah, a CAR T-cell therapy targeting CD19-positive relapsed B-cell leukemia in children. Kymriah is now approved for this indication by the US Food and Drug Administration (FDA). In addition, Dr. Ted Laetsch, Assistant Professor of Pediatrics, was a lead investigator helping to develop LOXO-101 (larotrectinib) for children with cancer driven by *TRK* gene fusions. This drug was recently approved by the US FDA for TRK-fused cancers. Our **Experimental Therapeutics Program** continues 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, Assistant Professor of Pediatrics, to provide the newest therapies in the form of clinical trials to children with neurofibromatosis, a neurodevelopmental and cancer predisposition syndrome.

Finally, this year marked the initiation of a nascent **Precision Medicine Program**, co-led by Drs. Klesse and Kathleen Ludwig, Assistant Professor of Pediatrics, as a multifaceted program to identify causes of cancer in individual children and use that information to 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 **Experimental Therapeutics** and **Precision Medicine Programs** for children with cancer

The following list contains clinical studies approved by the Institutional Review Board (IRB) at UT Southwestern as of December 31, 2018, and excludes more than 70 Children's Oncology Group (COG) trials.

## James Amatruda

- Archival Studies on Germ Cell Tumor Specimens

## Victor Aquino

- Center for International Blood and Marrow Transplant Research (CIBMTR) - Consent for Participation and Donation of Blood Samples
- 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  $\alpha\beta$ +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-Risk WNT-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



## Ted Laetsch

- 20140106 (former CFZ008), Phase 1b Study of Carfilzomib in Combination with Induction Chemotherapy in Children with Relapsed or Refractory Acute Lymphoblastic Leukemia
- Assessing the precision of MR thermometry in Pediatric Solid Tumor Patients
- ADVL1322 (VEG116731), A Phase II Study of Pazopanib GW786034, NSC# 737754 in Children, Adolescents and Young Adults with Refractory Solid Tumors
- AOST1321, Phase 2 Study of Denosumab (IND#127430, NSC# 744010), a RANK Ligand Antibody, for Recurrent or Refractory Osteosarcoma
- NMTRC V0706, A Phase II Trial of Nifurtimox for Refractory or Relapsed Neuroblastoma or Medulloblastoma
- 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
- "NMTRC012: A Study Using Molecular Guided Therapy with Induction Chemotherapy followed by maintenance with DFMO for Subjects with Newly Diagnosed High-Risk Neuroblastoma
- (PEDS-PLAN – Pediatric Precision Laboratory Advanced Neuroblastoma Therapy)"
- 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)
- ONC-403-001, A Two-Part Study of TB-403 in Pediatric Subjects with Relapsed or Refractory Medulloblastoma, Neuroblastoma, Ewing Sarcoma, or Alveolar Rhabdomyosarcoma
- I5B-MC-JGDN, A Phase 1, Open-Label, Dose-Escalation Study of Olaratumab as a Single Agent and in combination with Doxorubicin, Vincristine/Irinotecan, or High-Dose Ifosfamide in Pediatric Patients with Relapsed or Refractory Solid Tumors
- EZH-102, A Phase 1 Study of the EZH2 Inhibitor Tazemetostat in Pediatric Subjects with Relapsed or Refractory INI1-Negative Tumors or Synovial Sarcoma
- NMTRC 014, NMTRC- Neuroblastoma Maintenance Therapy Trial Using Difluoromethylornithine (DFMO)
- 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
- APEC1621: NCI-COG Pediatric MATCH (Molecular Analysis for Therapy Choice)
- ADVL1722, A Phase 2, Multicenter, Open-label Study to Assess Safety and Preliminary Activity of Eribulin Mesylate in Pediatric Subjects with Relapsed/Refractory Rhabdomyosarcoma (RMS), Non-rhabdomyosarcoma Soft Tissue Sarcoma (NRSTS) and Ewing Sarcoma (EWS)
- 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)
- ADVL1711, A Phase 1/2 Study of Lenvatinib in Combination with Everolimus in Recurrent and Refractory Pediatric Solid Tumors, Including CNS Tumors
- UCART19-PALL, A Phase 1, Open Label, Non-comparative, Study to Evaluate the Safety and the Ability of UCART19 to Induce Molecular Remission in Paediatric Patients with Relapsed /Refractory B-cell Acute Lymphoblastic Leukaemia
- Long-term Follow-up Study of Patients Who Have Previously Been Exposed to UCART19 (Allogeneic Engineered T-cells Expressing a Lentiviral-based Anti-CD19 Chimeric Antigen Receptor)
- "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)
- M16-106, A Phase 1 Dose Escalation, Open-Label Study of Venetoclax in Combination with Navitoclax and Chemotherapy in Subjects with Relapsed/Refractory Acute Lymphoblastic Leukemia or Relapsed/Refractory Lymphoblastic Lymphoma (LL)
- T2014-001, A Phase I Trial of Temozolomide (CC-779, Pfizer, Inc.) in Combination with Etoposide and Cyclophosphamide in Children with Relapsed Acute Lymphoblastic Leukemia and Non-Hodgkins Lymphoma
- 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)

- T2012-002, A Pilot Study of Vincristine Sulfate Liposome Injection (Marqibo®) in Combination with UK ALL R3 Induction Chemotherapy for Children, Adolescents, and Young Adults with Relapse of Acute Lymphoblastic Leukemia
- The iCat2, GAIN Consortium Study, Multicenter Cohort Study to Evaluate Outcomes after Receipt of Targeted Therapy Matched to an Individualized Cancer Therapy (iCat) Recommendations in Children and Young Adults with Recurrent, Refractory, or High Risk Solid Tumors
- IMDZ-04-1702, A Phase 3, Randomized, Double-blind, Placebo-controlled Study to Determine the Efficacy and Safety of CMB305 in Unresectable Locally-advanced or Metastatic NY-ESO-1+ Synovial Sarcoma Subjects Following First-line Systemic Anti-cancer Therapy
- 54767414ALL2005, An Open-label, Multicenter, Phase 2 Study Evaluating the Efficacy and Safety of Daratumumab in Pediatric and Young Adult Subjects  $\geq 1$  and  $\leq 30$  Years of Age With Relapsed/Refractory Precursor B-cell or T-cell Acute Lymphoblastic Leukemia or Lymphoblastic Lymphoma



- AADM0211: Permission for Screening Tests for Participation in Children’s Oncology Group Developmental Therapeutics/Phase I Consortium Clinical Research Protocols
- T2009-012, A Phase I Dose Finding Study Of Panobinostat In Children With Refractory Hematologic Malignancies
- T2009-003, A Pilot Study of Decitabine and Vorinostat with Chemotherapy for Relapsed ALL
- "A Phase II Study of Sirolimus and Erlotinib in Recurrent/Refractory Germ Cell Tumors
- CCTL019A2205B: Long Term Follow-Up of Patients Exposed to Lentiviral-Based CD19 directed CART Cell Therapy
- CCTL019B2202: A Phase II, Single Arm, Multicenter Trial to Determine the Efficacy and Safety of CTL019 in Pediatric Patients with Relapsed and refractory B-cell Acute Lymphoblastic Leukemia
- CCTL019B2206: A Multicenter Study of Apheresis Collection of Peripheral Blood Mononuclear Cells (PBMC) in Patients with CD19 Expressing Malignancies Who Could Be Eligible for a CTL019 Clinical Research Trial
- AAML1321 (CAMN1072203), A Multi-Center, Open Label, Non-Controlled Phase II Study to Evaluate Efficacy and Safety of Oral Nilotinib in Pediatric Patients with Newly Diagnosed Ph+ Chronic Myelogenous Leukemia (CML) in Chronic Phase (CP) or with Ph+ CML in CP or Accelerated Phase (AP) Resistant or Intolerant to either Imatinib or Dasatinib
- LOXO-TRK-15003, A Phase 1/2 Study of the Oral TRK Inhibitor LOXO-101 in Pediatric Patients with Advanced Solid or Primary Central Nervous System Tumors
- Pilot Study of MR-guided High Intensity Focused Ultrasound (HIFU) Hyperthermia with Liposomal Doxorubicin (DOXIL) for Relapsed or Refractory Pediatric Solid Tumors
- CCTL019B2205J: A Phase II, Single Arm, Multicenter Trial to Determine the Efficacy and Safety of CTL019 in Pediatric Patients with Relapsed and Refractory B-cell Acute Lymphoblastic Leukemia
- LOXO-EXT-17005: A Phase 1/2 Study of the TRK Inhibitor LOXO-195 in Adult and Pediatric Subjects with Previously Treated NTRK Fusion Cancers
- CCTL019B2003I: Managed Access Program (MAP) to Provide Access to CTL019, for Acute Lymphoblastic Leukemia (ALL) or Diffuse Large B-cell Lymphoma (DLBCL) Patients without of Specification Leukapheresis Product and/or Manufactured Tisagenlecleucel Out of Specification for Commercial Release
- Panel Based Next Generation Sequencing for High Risk Pediatric Oncology 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 Sucrofosate 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"

## Andrew Martin

- ARST1431, A Randomized Phase 3 Study of Vincristine, Dactinomycin, Cyclophosphamide (VAC) Alternating with Vincristine and Irinotecan (VI) Versus VAC/VI Plus Temsirolimus (TORI, Torisel, NSC# 683864, IND# 122782) in Patients with Intermediate Risk (IR) Rhabdomyosarcoma (RMS)

## 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

## Zora Rogers

- Silent Infarct Transfusion Trial (SITT)
- Pediatric Hydroxyurea Phase III Clinical Trial: BABY HUG
- Pediatric Hydroxyurea Phase III Clinical Trial (BABY HUG) Follow-up Observational Study.
- "BABY HUG FU II: Pediatric Hydroxyurea Phase III Clinical Trial Follow-up Observational Study II
- Retrospective Study of Pediatric Aplastic Anemia
- HUGKISS: A Pilot Study of Hydroxyurea Management in Kids: Intensive Versus Stable Dosage Strategies
- DISPLACE (Part 1): Dissemination and Implementation of Stroke Prevention Looking at the Care Environment (Chart Review)
- Severe Chronic Neutropenia International Registry

## 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 Allogeneic 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 (rFVIII Fc; BII031) 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, and National Eye Institute; the Cancer Research and Protection Institute of Texas; St. Baldrick's Foundation, Children's Cancer Fund of Dallas, Children's Medical Center Foundation, Wipe-Out Kids' Cancer, 1 Million for Anna Foundation, the Haggerty Family Foundation, Hyundai Hope on Wheels Foundation, and the Barrett Family Center for Pediatric Cancer.

## Clinical Activities

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

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

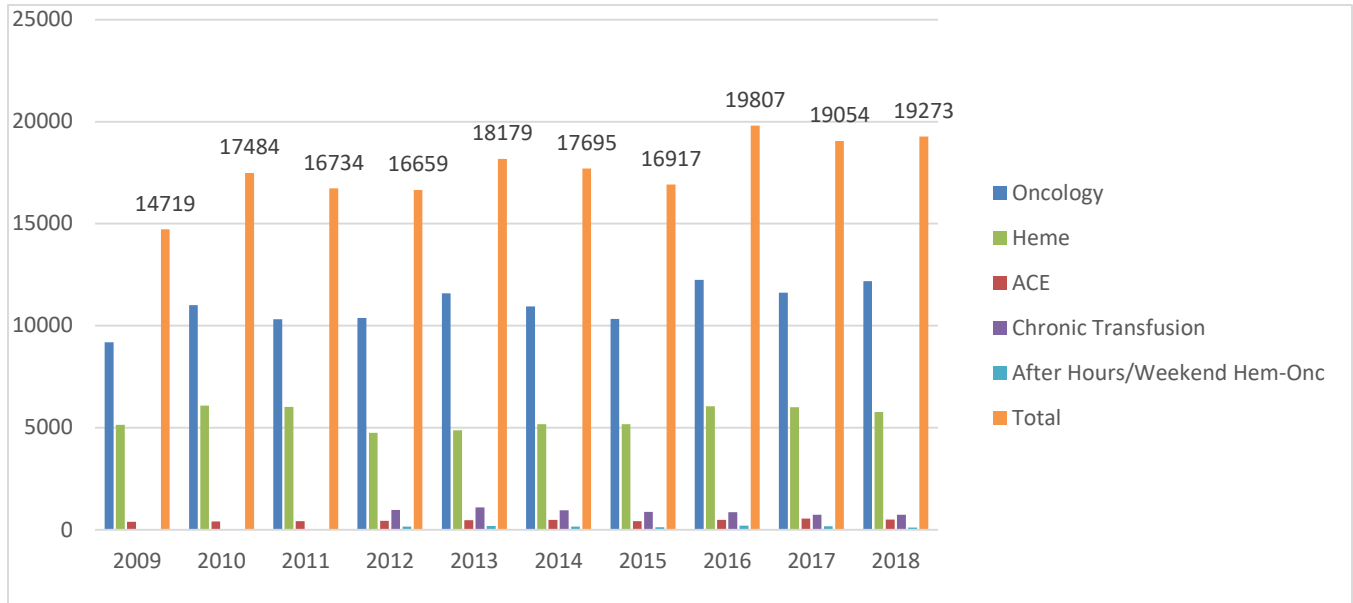
- Brain Tumor
- Bone and Soft Tissue Sarcoma
- Bone Marrow Failure
- Genitourinary Neoplasms
- Hemophilia, Hemostasis, and Thrombosis
- Hepatoblastoma
- Histiocytoses
- Iron Deficiency and other General Hematology
- Leukemia/Lymphoma
- Neuroblastoma
- Rare Tumors
- Sickle Cell Disease/Hemoglobinopathies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

## Additional Clinical/Research Programs

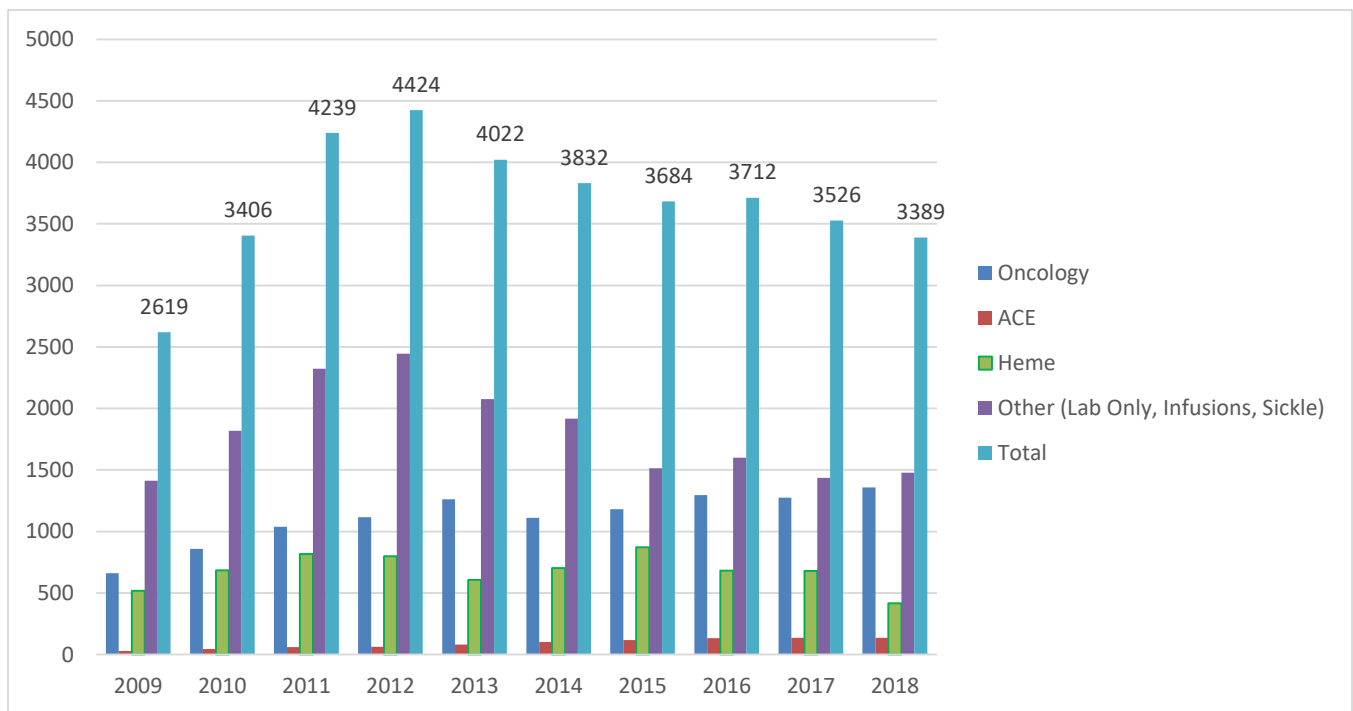
- After the Cancer Experience (ACE) Childhood Cancer Survivor Program
- Cancer Genetic Susceptibility Program
- Neurofibromatosis
- Adolescent and Young Adult Oncology
- Experimental Therapeutics Program
- Precision Medicine Program
- Palliative Care Program

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.

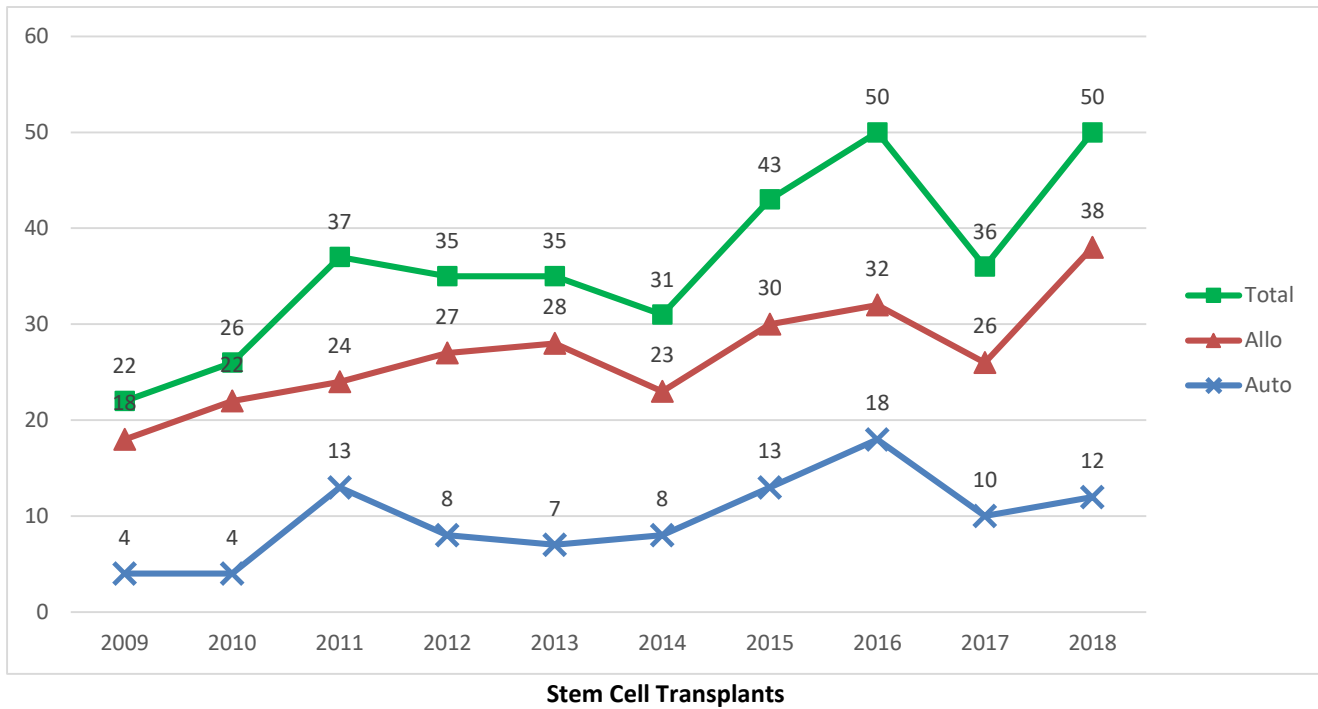
Faculty members also provide a consulting service for newborn patients with hematological conditions at Parkland Memorial Hospital, the 997-bed Dallas County hospital with approximately 16,000 newborn deliveries each year that is the site of the newborn nursery. New sites for hematology consultations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



Dallas Clinic Visits



Plano Clinic Visits



### Current Grant Support

#### James Amatruda

**Grantor:** CPRIT RP120685-C1

**Title of Project:** C1: Central Sarcoma Processing Core

**Role:** Principal Investigator

**Dates:** 09/2012 – 02/2019

**Grantor:** CPRIT RP120685-P3

**Title of Project:** P3: Functional Validation of Actionable Mutations in Sarcoma Genetic Model Systems

**Role:** Principal Investigator

**Dates:** 09/2012 – 02/2019

**Grantor:** Dana-Farber Institute/St. Baldrick's Subcontract #358099

**Title of Project:** Malignant Germ Cell Tumors International Consortium

**Role:** Principal Investigator

**Dates:** 07/2015 – 06/2020

**Grantor:** RP160249 CPRIT

**Title of Project:** DIS3L2 in Childhood Wilms Tumor: Mechanism to Medicines

**Role:** Collaborator (Mendell)

**Dates:** 03/2016 – 02/2020

**Grantor:** NIH 1 P50 CA196516-01-A1 (Project 4)

**Title of Project:** Prognostic Significance and Therapeutic Potential of DROSHA Mutations in Wilms Tumor

**Role:** Leader

**Dates:** 08/2016 – 07/2021



**Grantor:** Alex's Lemonade Stand Foundation  
**Title of Project:** DICER1-driven Cancers: Models, Mechanisms and Therapies  
**Role:** Principal Investigator  
**Dates:** 09/2016 – 08/2019

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

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

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

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

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

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

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

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

## Victor Aquino

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

## Kenneth Chen

**Grantor:** CCRAC, Micaela's Army Foundation  
**Title of Project:** Replacement therapy for miRNA-impaired Wilms tumors  
**Role:** Principal Investigator  
**Dates:** 11/2015 – Current

**Grantor:** NIH/NCI 1 K08 CA207849-01  
**Title of Project:** The Role of 5p miRNA Loss in Wilms Tumor Formation  
**Role:** Principal Investigator  
**Dates:** 07/2016 – 06/2021

## Laura Klesse

**Grantor:** Southwestern Medical Foundation / Dedman Family Scholarship Fund  
**Title of Project:** Dedman Scholar Support  
**Role:** Principal Investigator  
**Dates:** 3/2009 – Current

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

**Grantor:** The Children's Tumor Foundation  
**Title of Project:** Children's Tumor Foundation Support  
**Role:** Principal Investigator  
**Dates:** 1/2011 – Current

**Grantor:** Children's Tumor Foundation  
**Title of Project:** Developing Evidence-Based Criteria for Initiating Treatment for NF1-OPG  
**Role:** Principal Investigator  
**Dates:** 12/2017 – 11/2020

**Grantor:** Texas Neurofibromatosis Foundation  
**Title of Project:** Cardiovascular abnormalities in pediatric patients with neurofibromatosis type 1  
**Role:** Principal Investigator  
**Dates:** 06/2016 – 05/2018

## Ted Laetsch

**Grantor:** Micaela's Army Foundation  
**Title of Project:** A Clinical Trial Combining Targeted Therapy for Pediatric and Young Adult Patients with Refractory Germ Cell Tumors  
**Role:** Principal Investigator  
**Dates:** 7/2014 – Current

**Grantor:** NIH 1 R01 CA199937-01  
**Title of Project:** Image-guided doxorubicin delivery for pediatric sarcomas (MPI)  
**Role:** Co-Investigator (Chopra)  
**Dates:** 07/2015 – 06/2020

**Grantor:** University of Colorado

**Title of Project:** A Phase I/Ib Study of Eribulin in Combination with Oral Irinotecan for Adolescent and Young Adult Patients with Relapsed or Refractory Solid Tumors

**Role:** Principal Investigator

**Dates:** 09/2017 – 08/2018

**Grantor:** Hyundai Hope on Wheels

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

**Role:** Principal Investigator

**Dates:** 01/2015 – 12/2018

**Grantor:** US Department of Defense

**Title of Project:** Eliminating ex-Vivo Manipulation and Viral Transfection of T-Cells in CAR-T Cell Immunotherapy of B-Cell Malignancies

**Role:** Co-Investigator (Lux)

**Dates:** 07/2017 – 06/2019

**Grantor:** NIH / Children's Hospital of Philadelphia

**Title of Project:** Match Committee-NIH National Clinical Trials Network (NCTN) Grant

**Role:** Principal Investigator

**Dates:** 03/2018 – 02/2019

## Patrick Leavey

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

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

**Role:** Co-Investigator and Principal Investigator Biospecimen Banking Study

**Dates:** 9/2012 – 8/2018

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

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

**Role:** Principal Investigator

**Dates:** 3/2015 – 2/2019

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

**Title of Project:** Leavey - CPRIT IIRACCA

**Role:** Principal Investigator

**Dates:** 4/2014 – 2/2019

## Zora Rogers

**Grantor:** NIH/St. Jude Children's Research Hospital

**Title of Project:** Hydroxyurea Management in Kids: Intensive versus Stable Dosage Strategies" (HUG KISS)

**Role:** Principal Investigator

**Dates:** 04/2016 – 03/2019

**Grantor:** Medical University of South Carolina

**Title of Project:** DISPLACE: Dissemination and Implementation of Stroke Prevention: Looking at the Care Environment

**Role:** Principal Investigator

**Dates:** 07/01/2017- 06/30/2018

**Grantor:** NIH-National Heart, Lung and Blood Institute  
**Title of Project:** Baby Hug Follow-up Study II-Clinical Site  
**Role:** Principal Investigator  
**Dates:** 08/01/2017- 04/30/2018

## Stephen Skapek

**Grantor:** NIH/NEI  
**Title of Project:** Tgf $\beta$ 2 Controls p19Arf During Eye Development  
**Role:** Principal Investigator  
**Dates:** 4/2014 –2/2019

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

**Grantor:** Cancer Prevention Research Institute of Texas (CPRIT)  
**Title of Project:** Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas  
**Role:** Principal Investigator  
**Dates:** 9/2012 –2/2019

**Grantor:** St. Baldrick's Foundation  
**Title of Project:** Targeting LILRB4 by CAR-T cells for the treatment of pediatric AML  
**Role:** Mentor  
**Dates:** 07/2016 – 06/2019

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

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

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

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

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

**Grantor:** NIH/NHLBI

**Title of Project:** Predicting and Preventing Poor Outcomes of Venous Thromboembolism in Children

**Role:** Co- Principal Investigator

**Dates:** 05/2016 – 04/2021

**Grantor:** CHOP/NIH (NCI) UM1CA097452

**Title of Project:** COG Phase 1 Pilot Consortium

**Role:** Principal Investigator

**Dates:** 05/2017 – 03/2018

**Grantor:** CHOP/NIH U10CA180886

**Title of Project:** NIH NCTN Scientific Council

**Role:** Principal Investigator

**Dates:** 03/2017 – 02/2019

**Grantor:** CPRIT RP120685-AC

**Title of Project:** AC: Molecularly Targeted Therapy for Soft Tissue Sarcoma

**Role:** Principal Investigator

**Dates:** 09/2012 – 02/2019

**Grantor:** CPRIT RP120685-P2

**Title of Project:** P2: High Throughput Screening for Sarcoma Cell Proliferation and Survival Factors

**Role:** Principal Investigator

**Dates:** 09/2012 – 02/2019

## Tanya Watt

**Grantor:** South Plains Oncology Consortium

**Title of Project:** SPOC 2014-001 Expanded Access Study of Fenretinide

**Role:** Principal Investigator

**Dates:** 03/01/2015- 02/28/2018

## Ayesha Zia

**Grantor:** NIH/NHLBI

**Title of Project:** Predicting and Preventing Poor Outcomes of Venous Thromboembolism in Children

**Role:** Principal Investigator

**Dates:** 05/2016 – 04/2021

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

**Title of Project:** Comparative Effectiveness in the Diagnosis of VWD

**Role:** Principal Investigator

**Dates:** 12/2013 – 11/2018

**Grantor:** UT Health Science Center at Houston

**Title of Project:** HTC- Great Plains Regional Hemophilia Network

**Role:** Principal Investigator

**Dates:** 06/2017 – 05/2018

**Grantor:** UT Health Science Center at Houston

**Title of Project:** HTC- Great Plains Regional Hemophilia Network

**Role:** Principal Investigator

**Dates:** 06/2017 – 05/2018



**Grantor:** All Children's Hospital

**Title of Project:** Kids-DOTT

**Role:** Principal Investigator

**Dates:** 04/2017 – 04/2018

**Grantor:** American Thrombosis & Hemostasis Network

**Title of Project:** A Longitudinal Observational Study of Previously Treated Hemophilia Patients

**Role:** Principal Investigator

**Dates:** 07/2017 – 06/2018

## Yanbin Zheng

**Grantor:** NIH / NEI

**Title of Project:** Tgf Beta 2 controls p19Arf During Eye Development

**Role:** Co-Investigator

**Dates:** 4/2014 – 3/2018

**Grantor:** Andrew McDonough B+ Foundation

**Title of Project:** Developing a novel therapeutic strategy for rhabdomyosarcoma

**Role:** Principal Investigator

**Dates:** 1/2019 – 12/2020

## Peer-Reviewed Publications

1. Armstrong AE, Walterhouse DO, **Leavey PJ**, Reichek J, Walz AL. [Prolonged response to sorafenib in a patient with refractory metastatic osteosarcoma and a somatic PDGFRA D846V mutation.](#) *Pediatr Blood Cancer*. 2018 Oct 14:e27493. PMID: 30318721
2. Barredo JC, Hastings C, Lu X, Devidas M, Chen Y, Armstrong D, **Winick N**, et al. [Isolated late testicular relapse of B-cell acute lymphoblastic leukemia treated with intensive systemic chemotherapy and response-based testicular radiation: A Children's Oncology Group study.](#) *Pediatr Blood Cancer*. 2018 May;65(5):e26928. PMID:29286562
3. Bjornard KL, Gilchrist LS, Inaba H, Diouf B, Hockenberry MJ, Kadan-Lottick NS, **Bowers DC**, et al. [Peripheral neuropathy in children and adolescents treated for cancer.](#) *Lancet Child Adolesc Health*. 2018 Oct;2(10):744-754. PMID: 30236383
4. Blumenthal GM, Bunn PA Jr, Chaft JE, McCoach CE, Perez EA, Scagliotti GV, Carbone DP, Aerts HJWL, Aisner DL, Bergh J, Berry DA, Jarkowski A, Botwood N, Cross DAE, Diehn M, Drezner NL, Doebele RC, Blakely CM, Eberhardt WEE, Felip E, Gianni L, Keller SP, **Leavey PJ**, et al. [Current Status and Future Perspectives on Neoadjuvant Therapy in Lung Cancer.](#) *J Thorac Oncol*. 2018 Sep 27. pii: S1556-0864(18)33129-0. PMID: 30268698
5. Burke MJ, Devidas M, Maloney K, Angiolillo A, Schore R, Dunsmore K, Larsen E, Mattano LA Jr, Salzer W, Winter SS, Carroll W, **Winick NJ**, et al. [Severe pegaspargase hypersensitivity reaction rates \(grade ≥3\) with intravenous infusion vs. intramuscular injection: analysis of 54,280 doses administered to 16,534 patients on children's oncology group \(COG\) clinical trials.](#) *Leuk Lymphoma*. 2018 Jul;59(7):1624-1633. PMID: 29115886
6. **Chen KS**, Fustino NJ, Shukla AA, Stroup EK, Budhipramono A, Ateek C, Stuart SH, Yamaguchi K, Kapur P, Frazier AL, Lum L, Looijenga LHJ, **Laetsch TW**, Rakheja D, **Amatruda JF**. [EGF Receptor and mTORC1 Are Novel Therapeutic Targets in Nonseminomatous Germ Cell Tumors.](#) *Mol Cancer Ther*. 2018 May;17(5):1079-1089. PMID: 29483210

7. **Chen KS**, Stroup EK, Budhipramono A, Rakheja D, Nichols-Vinueza D, Xu L, Stuart SH, Shukla AA, Fraire C, Mendell JT, **Amatruda JF**. [Mutations in microRNA processing genes in Wilms tumors derepress the IGF2 regulator PLAG1](#). *Genes Dev*. 2018 Aug 1;32(15-16):996-1007. PMID: 30026293
8. Churchman ML, Qian M, Te Kronnie G, Zhang R, Yang W, Zhang H, Lana T, Tedrick P, Baskin R, Verbist K, Peters JL, Devidas M, Larsen E, Moore IM, Gu Z, Qu C, Yoshihara H, Porter SN, Pruett-Miller SM, Wu G, Raetz E, Martin PL, Bowman WP, **Winick N**, et al. [Germline Genetic IKZF1 Variation and Predisposition to Childhood Acute Lymphoblastic Leukemia](#). *Cancer Cell*. 2018 May 14;33(5):937-948.e8. PMID: 29681510
9. Dicken BJ, Billmire DF, Krailo M, Xia C, Shaikh F, Cullen JW, Olson TA, Pashankar F, Malogolowkin MH, **Amatruda JF**, et al. [Gonadal dysgenesis is associated with worse outcomes in patients with ovarian nondysgerminomatous tumors: A report of the Children's Oncology Group AGCT 0132 study](#). *Pediatr Blood Cancer*. 2018 Apr;65(4). PMID: 29286555
10. **Dickerson KE**, Menon NM, **Zia A**. [Abnormal Uterine Bleeding in Young Women with Blood Disorders](#). *Pediatr Clin North Am*. 2018 Jun;65(3):543-560. PMID: 29803282
11. DiNofia AM, Seif AE, Devidas M, Li Y, Hall M, Huang YV, Cahen V, Hunger SP, **Winick NJ**, et al. [Cost comparison by treatment arm and center-level variations in cost and inpatient days on the phase III high-risk B acute lymphoblastic leukemia trial AALL0232](#). *Cancer Med*. 2018 Jan;7(1):3-12. PMID: 29274118
12. Drilon A, **Laetsch TW**, Kummar S, et al. [Efficacy of Larotrectinib in TRK Fusion-Positive Cancers in Adults and Children](#). *N Engl J Med*. 2018 Feb 22;378(8):731-739. PMID: 29466156
13. Dvorak CC, Haddad E, Buckley RH, Cowan MJ, Logan B, Griffith LM, Kohn DB, Pai SY, Notarangelo L, Shearer W, Prockop S, Kapoor N, Heimall J, Chaudhury S, Shyr D, Chandra S, Cuvelier G, Moore T, Shenoy S, Goldman F, Smith AR, Sunkersett G, Vander Lugt M, Caywood E, Quigg T, Torgerson T, Chandrakasan S, Craddock J, Dávila Saldaña BJ, Gillio A, Shereck E, **Aquino V**, et al. [The genetic landscape of severe combined immunodeficiency in the United States and Canada in the current era \(2010-2018\)](#). *J Allergy Clin Immunol*. 2018 Sep 5. pii: S0091-6749(18)31271-5. PMID: 30193840
14. Frazier AL, Stoneham S, Rodriguez-Galindo C, Dang H, Xia C, Olson TA, Murray MJ, **Amatruda JF**, et al. [Comparison of carboplatin versus cisplatin in the treatment of paediatric extracranial malignant germ cell tumours: A report of the Malignant Germ Cell International Consortium](#). *Eur J Cancer*. 2018 Jul;98:30-37. PMID: 29859339
15. Germann JN, Leonard D, Heath CL, Stewart SM, **Leavey PJ**. [Hope as a Predictor of Anxiety and Depressive Symptoms Following Pediatric Cancer Diagnosis](#). *J Pediatr Psychol*. 2018 Mar 1;43(2):152-161. PMID: 29049751
16. Gupta N, Goumnerova LC, Manley P, Chi SN, Neuberg D, Puligandla M, Fangusaro J, Goldman S, Tomita T, Alden T, DiPatri A, Rubin JB, Gauvain K, Limbrick D, Leonard J, Geyer JR, Leary S, Browd S, Wang Z, Sood S, Bendel A, Nagib M, Gardner S, Karajannis MA, Harter D, Ayyanar K, Gump W, **Bowers DC**, et al. [Prospective feasibility and safety assessment of surgical biopsy for patients with newly diagnosed diffuse intrinsic pontine glioma](#). *Neuro Oncol*. 2018 Oct 9;20(11):1547-1555. PMID: 29741745
17. Gupta S, Devidas M, Loh ML, Raetz EA, Chen S, Wang C, Brown P, Carroll AJ, Heerema NA, Gastier-Foster JM, Dunsmore KP, Larsen EC, Maloney KW, Mattano LA Jr, Winter SS, **Winick NJ**, et al. [Flow-cytometric vs. -morphologic assessment of remission in childhood acute lymphoblastic leukemia: a report from the Children's Oncology Group \(COG\)](#). *Leukemia*. 2018 Jun;32(6):1370-1379. PMID: 29472723
18. Haddad E, Logan BR, Griffith LM, Buckley RH, Parrott RE, Prockop SE, Small TN, Chaisson J, Dvorak CC, Murnane M, Kapoor N, Abdel-Aziz H, Hanson IC, Martinez C, Bleesing JH, Chandra S, Smith AR, Cavanaugh ME, Jyonouchi S, Sullivan KE, Burroughs L, Skoda-Smith S, Haight AE, Tumlin AG, Quigg TC, Taylor C, Dávila Saldaña BJ, Keller MD, Seroogy CM, Desantes KB, Petrovic A, Leiding JW, Shyr DC, Decaluwe H, Teira P, Gillio AP, Knutsen AP, Moore TB, Hanna JA, Garcia MR, Lardennois A, **Leavey PJ**, et al. [PAX3-FOXO1 drives miR-486-5p and represses miR-221 contributing to pathogenesis of alveolar rhabdomyosarcoma](#). *Oncogene*. 2018 Apr;37(15):1991-2007. PMID: 29367756

19. Hawkins DS, Chi YY, Anderson JR, Tian J, Arndt CAS, Bomgaars L, Donaldson SS, Hayes-Jordan A, Mascarenhas L, McCarville MB, McCune JS, McCowage G, Million L, Morris CD, Parham DM, Rodeberg DA, Rudzinski ER, Shnorhavorian M, Spunt SL, **Skapek SX**, et al. [Addition of Vincristine and Irinotecan to Vincristine, Dactinomycin, and Cyclophosphamide Does Not Improve Outcome for Intermediate-Risk Rhabdomyosarcoma: A Report From the Children's Oncology Group](#). *J Clin Oncol*. 2018 Sep 20;36(27):2770-2777. PMID: 30091945
20. Hord J, Shah M, Badawy SM, Matthews D, Hilden J, Wayne AS, Salsberg E, **Leavey PS**; American Society of Pediatric Hematology/Oncology Workforce Advisory Taskforce. [The American Society of Pediatric Hematology/Oncology workforce assessment: Part 1-Current state of the workforce](#). *Pediatr Blood Cancer*. 2018 Feb;65(2). PMID: 29068564
21. Kletzel M, Craddock JA, **Aquino V**, et al. [SCID genotype and 6-month posttransplant CD4 count predict survival and immune recovery](#). *Blood*. 2018 Oct 25;132(17):1737-1749. PMID: 30154114
22. Kendall GC, Watson S, Xu L, LaVigne CA, Murchison W, Rakheja D, **Skapek SX**, Tirode F, Delattre O, **Amatruda JF**. [PAX3-FOXO1 transgenic zebrafish models identify HES3 as a mediator of rhabdomyosarcoma tumorigenesis](#). *Elife*. 2018 Jun 5;7. pii: e33800. PMID: 29869612
23. **Laetsch TW**, DuBois SG, Mascarenhas L, et al. [Larotrectinib for paediatric solid tumours harbouring NTRK gene fusions: phase 1 results from a multicentre, open-label, phase 1/2 study](#). *Lancet Oncol*. 2018 May;19(5):705-714. PMID: 29606586
24. **Laetsch TW**, Hawkins DS. [Larotrectinib for the treatment of TRK fusion solid tumors](#). *Expert Rev Anticancer Ther*. 2018 Oct 23:1-10. PMID: 30350734
25. **Laetsch TW**, Maude SL, Milone MC, et al. [False-positive results with select HIV-1 NAT methods following lentivirus-based tisagenlecleucel therapy](#). *Blood*. 2018 Jun 7;131(23):2596-2598. PMID: 29669777
26. **Laetsch TW**, Nagasubramanian R, Casanova M. [Targeting NTRK fusions for the treatment of congenital mesoblastic nephroma](#). *Pediatr Blood Cancer*. 2018 Jan;65(1). PMID: 28440051
27. **Laetsch TW**, Roy A, Xu L, Black JO, Coffin CM, Chi YY, Tian J, Spunt SL, Hawkins DS, Bridge JA, Parsons DW, **Skapek SX**. [Undifferentiated Sarcomas in Children Harbor Clinically Relevant Oncogenic Fusions and Gene Copy-Number Alterations: A Report from the Children's Oncology Group](#). *Clin Cancer Res*. 2018 Aug 15;24(16):3888-3897. PMID: 29691299
28. **Ludwig KF**, Du W, Sorrelle NB, et al. [Small-Molecule Inhibition of Axl Targets Tumor Immune Suppression and Enhances Chemotherapy in Pancreatic Cancer](#). *Cancer Res*. 2018 Jan 1;78(1):246-255. PMID: 29180468
29. Maude SL, **Laetsch TW**, Buechner J, et al. [Tisagenlecleucel in Children and Young Adults with B-Cell Lymphoblastic Leukemia](#). *N Engl J Med*. 2018 Feb 1;378(5):439-448. PMID: 29385370
30. Menon N, Sarode R, **Zia A**. [Rivaroxaban dose adjustment using thrombin generation in severe congenital protein C deficiency and warfarin-induced skin necrosis](#). *Blood Adv*. 2018 Jan 23;2(2):142-145. PMID: 29365322
31. Mink R, Schwartz A, Carraccio C, et al; Steering Committee of the Subspecialty Pediatrics Investigator Network (Brion L, Green M, **Journeycake J**, Yen K, Quigley R). [Creating the Subspecialty Pediatrics Investigator Network](#). *J Pediatr*. 2018 Jan;192:3-4.e2. PMID: 29246355
32. Mink RB, Schwartz A, Herman BE, et al, the Steering Committee of the Subspecialty Pediatrics Investigator Network (SPIN) (Brion L, Green M, **Journeycake J**, Yen K, Quigley R). [Validity of Level of Supervision Scales for Assessing Pediatric Fellows on the Common Pediatric Subspecialty Entrustable Professional Activities](#). *Acad Med*. 2018 Feb;93(2):283-291. PMID: 287004862

33. Mishra R, Daescu O, **Leavey P**, Rakheja D, Sengupta A. [Convolutional Neural Network for Histopathological Analysis of Osteosarcoma](#). *J Comput Biol*. 2018 Mar;25(3):313-325. PMID: 29083930
34. Pierce JL, Frazier AL, **Amatruda JF**. [Pediatric Germ Cell Tumors: A Developmental Perspective](#). *Adv Urol*. 2018 Feb 4;2018:9059382. PMID: 29515628
35. Poynter JN, Richardson M, Roesler M, Krailo M, **Amatruda JF**, Frazier AL. [Family history of cancer in children and adolescents with germ cell tumours: a report from the Children's Oncology Group](#). *Br J Cancer*. 2018 Jan;118(1):121-126. PMID: 29065103
36. Qian M, Cao X, Devidas M, Yang W, Cheng C, Dai Y, Carroll A, Heerema NA, Zhang H, Moriyama T, Gastier-Foster JM, Xu H, Raetz E, Larsen E, **Winick N**, et al. [TP53 Germline Variations Influence the Predisposition and Prognosis of B-Cell Acute Lymphoblastic Leukemia in Children](#). *J Clin Oncol*. 2018 Feb 20;36(6):591-599. PMID: 29300620
37. Robinson GW, Rudneva VA, Buchhalter I, Billups CA, Waszak SM, Smith KS, **Bowers DC**, et al. [Risk-adapted therapy for young children with medulloblastoma \(SJYC07\): therapeutic and molecular outcomes from a multicentre, phase 2 trial](#). *Lancet Oncol*. 2018 Jun;19(6):768-784. PMID: 29778738
38. Rudzinski ER, Lockwood CM, Stohr BA, Vargas SO, Sheridan R, Black JO, Rajaram V, **Laetsch TW**, Davis JL. [Pan-Trk Immunohistochemistry Identifies NTRK Rearrangements in Pediatric Mesenchymal Tumors](#). *Am J Surg Pathol*. 2018 Jul;42(7):927-935. PMID: 29683818
39. Salzer WL, Burke MJ, Devidas M, Chen S, Gore L, Larsen EC, Borowitz M, Wood B, Heerema NA, Carroll AJ, Hilden JM, Loh ML, Raetz EA, **Winick NJ**, Carroll WL, Hunger SP. [Toxicity associated with intensive postinduction therapy incorporating clofarabine in the very high-risk stratum of patients with newly diagnosed high-risk B-lymphoblastic leukemia: A report from the Children's Oncology Group study AALL1131](#). *Cancer*. 2018 Mar 15;124(6):1150-1159. PMID: 29266189
40. Shah R, Xia C, Krailo M, **Amatruda JF**, et al. [Is carboplatin-based chemotherapy as effective as cisplatin-based chemotherapy in the treatment of advanced-stage dysgerminoma in children, adolescents and young adults?](#) *Gynecol Oncol*. 2018 Aug;150(2):253-260. PMID: 29884437
41. Shields BB, Lampson EE, Sengupta AL, **Watt TC**, Mitchell RB. [Infant with an unusual pharyngeal mass](#). *Ear Nose Throat J*. 2018 Jun;97(6):154-155. PMID: 30036410
42. Shulman DS, Klega K, Imamovic-Tuco A, Clapp A, Nag A, Thorner AR, Van Allen E, Ha G, Lessnick SL, Gorlick R, Janeway KA, **Leavey PJ**, et al. [Detection of circulating tumour DNA is associated with inferior outcomes in Ewing sarcoma and osteosarcoma: a report from the Children's Oncology Group](#). *Br J Cancer*. 2018 Aug;119(5):615-621. PMID: 30131550
43. **Simms-Waldrup TR**, **Koh AY**. [Food for Gut: Microbiota Fuels Immune Reconstitution after BMT](#). *Cell Host Microbe*. 2018 Apr 11;23(4):423-424. PMID: 29649435
44. Slayton WB, Schultz KR, Kairalla JA, Devidas M, Mi X, Pulsipher MA, Chang BH, Mullighan C, Iacobucci I, Silverman LB, Borowitz MJ, Carroll AJ, Heerema NA, Gastier-Foster JM, Wood BL, Mizrahy SL, Merchant T, Brown VI, Sieger L, Siegel MJ, Raetz EA, **Winick NJ**, et al. [Dasatinib Plus Intensive Chemotherapy in Children, Adolescents, and Young Adults With Philadelphia Chromosome-Positive Acute Lymphoblastic Leukemia: Results of Children's Oncology Group Trial AALL0622](#). *J Clin Oncol*. 2018 Aug 1;36(22):2306-2314. PMID: 29812996
45. Stoneham SJ, Hale JP, Rodriguez-Galindo C, Dang H, Olson T, Murray M, **Amatruda JF**, et al. [Adolescents and Young Adults With a "Rare" Cancer: Getting Past Semantics to Optimal Care for Patients With Germ Cell Tumors](#). *Oncologist*. 2018 Feb;23(2):272. PMID: 29449513

46. Teot LA, Schneider M, Thorner AR, Tian J, Chi YY, Ducar M, Lin L, Wlodarski M, Grier HE, Fletcher CDM, van Hummelen P, **Skapek SX**, et al. [Clinical and mutational spectrum of highly differentiated, paired box 3:forkhead box protein o1 fusion-negative rhabdomyosarcoma: A report from the Children's Oncology Group](#). *Cancer*. 2018 May 1;124(9):1973-1981. PMID: 29461635
47. Waszak SM, Northcott PA, Buchhalter I, Robinson GW, Sutter C, Groebner S, Grund KB, Brugières L, Jones DTW, Pajtler KW, Morrissy AS, Kool M, Sturm D, Chavez L, Ernst A, Brabetz S, Hain M, Zichner T, Segura-Wang M, Weischenfeldt J, Rausch T, Mardin BR, Zhou X, Baciu C, Lawerenz C, Chan JA, Varlet P, Guerrini-Rousseau L, Fufts DW, Grajkowska W, Hauser P, Jabado N, Ra YS, Zitterbart K, Shringarpure SS, De La Vega FM, Bustamante CD, Ng HK, Perry A, MacDonald TJ, Hernáiz Driever P, Bendel AE, **Bowers DC**, et al. [Spectrum and prevalence of genetic predisposition in medulloblastoma: a retrospective genetic study and prospective validation in a clinical trial cohort](#). *Lancet Oncol*. 2018 Jun;19(6):785-798. PMID: 29753700
48. Williams LA, Mills L, Hooten AJ, Langer E, Roesler M, Frazier AL, Krailo M, Nelson HH, Bestrashniy J, **Amatruda JF**, Poynter JN. [Differences in DNA methylation profiles by histologic subtype of paediatric germ cell tumours: a report from the Children's Oncology Group](#). *Br J Cancer*. 2018 Oct;119(7):864-872. PMID: 30287918
49. Winter SS, Dunsmore KP, Devidas M, Wood BL, Esiashvili N, Chen Z, Eisenberg N, Briegel N, Hayashi RJ, Gastier-Foster JM, Carroll AJ, Heerema NA, Asselin BL, Gaynon PS, Borowitz MJ, Loh ML, Rabin KR, Raetz EA, Zweidler-Mckay PA, **Winick NJ**, Carroll WL, Hunger SP. [Improved Survival for Children and Young Adults With T-Lineage Acute Lymphoblastic Leukemia: Results From the Children's Oncology Group AALL0434 Methotrexate Randomization](#). *J Clin Oncol*. 2018 Oct 10;36(29):2926-2934. PMID: 30138085
50. Woldu SL, Aydin AM, Rao AV, Hutchinson RC, Singla N, Clinton TN, Krabbe LM, Passoni NM, Raj GV, Miller DS, **Amatruda JF**, et al. [Differences at Presentation and Treatment of Testicular Cancer in Hispanic Men: Institutional and National Hospital-based Analyses](#). *Urology*. 2018 Feb;112:103-111. PMID: 29079210
51. Wood B, Wu D, Crossley B, Dai Y, Williamson D, Gawad C, Borowitz MJ, Devidas M, Maloney KW, Larsen E, **Winick N**, et al. [Measurable residual disease detection by high-throughput sequencing improves risk stratification for pediatric B-ALL](#). *Blood*. 2018 Mar 22;131(12):1350-1359. PMID: 29284596
52. Xu L, **Zheng Y**, Liu J, Rakheja D, Singleterry S, **Laetsch TW**, Shern JF, Khan J, Triche TJ, Hawkins DS, **Amatruda JF**, **Skapek SX**. [Integrative Bayesian Analysis Identifies Rhabdomyosarcoma Disease Genes](#). *Cell Rep*. 2018 Jul 3;24(1):238-251. PMID: 29972784
53. Zheng DJ, Lu X, Schore RJ, Balsamo L, Devidas M, **Winick NJ**, et al. [Longitudinal analysis of quality-of-life outcomes in children during treatment for acute lymphoblastic leukemia: A report from the Children's Oncology Group AALL0932 trial](#). *Cancer*. 2018 Feb 1;124(3):571-579. PMID: 29112230