UT Southwestern
Medical Center

Department of Pediatrics

Pediatric Hematology and Oncology

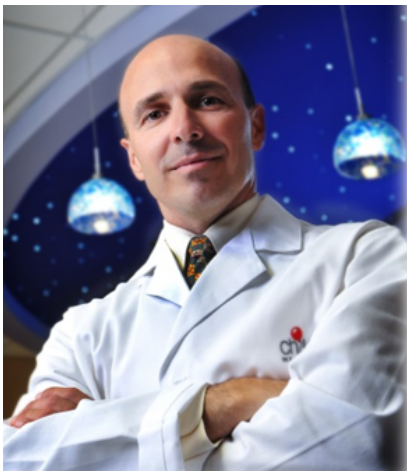
2023 Annual Report

Our mission is to provide world-class care to children with cancer and blood disorders while striving to generate scientific knowledge to improve their lives and educate future generations of the doctors and scientists who will care for them.

Pediatric Hematology and Oncology – 2023 Annual Report

Under the direction of Stephen X. Skapek, M.D., the faculty, fellows, and numerous support and administrative staff in the Division of Pediatric Hematology and Oncology are dedicated to fulfilling our overall goal of providing unrivaled care to children with cancer and blood disorders and developing programs that are exceptional and widely emulated. Specifically, we discharge the following 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 the 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.
Professor, Division Chief

As the largest cancer and blood disease program in North Texas and one of the largest in the United States, the Division annually provides care for more than 400 children with a newly diagnosed cancer and close to 1,600 children with a newly diagnosed blood disease. Our physicians primarily provide care in the Pauline Allen Gill Center for Cancer and Blood Disorders at Children's Health in Dallas and Plano. We continue to work toward increasing our geographic footprint by widening the scope and scale of clinical care we can provide in Plano and other suburban sites. This includes a new outpatient hematology clinic at UT Southwestern Frisco and plans for a similar clinic at the RedBird facility. We also continue to look for opportunities to increase regional outreach by providing educational and consultative resources for primary and referring physicians in the region.

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

Our education mission includes medical students, pediatric residents, and hematology/oncology fellows.

Numbers at a Glance

Faculty: 35* New Faculty: 6* Promotions: 2* *U.S. News & World Report*: #15	Clinical Fellows: 12* Clinical Sites: 2	Inpatient Encounters: 17,445* Outpatient Encounters: 17,383*
Research Funding Total* Grants: $9,731,392 Contracts: N/A Total # Proposals Awarded: 19 Public Federal (# K Awards): N/A Public State of Texas: N/A Other: 19 Revenue from Active Clinical Trials as of 12/31/2023: $8,999,192	Active Clinical Trials: 197*	Conferences/Lectures/ Presentations: 64 Publications: 91* Citations: 7,116*

*Sources: Orbit report of RHi032 Organizational Research Dashboard, UTSW Office of Research and Grants Support, Faculty Affairs, Education Alumni Affairs, Clinical Business Operations-invoice creation period for 2023, CVs

Honors/Awards

Best Pediatric Specialists in Dallas, *D Magazine*

- Laura Klesse
- Patrick Leavey
- Kathleen Ludwig
- Tamra Slone
- Tanya Watt
- Jonathan Wickiser
- Naomi Winick
- Ayesha Zia

Texas Super Doctors, *Texas Monthly Magazine*

- Kenneth Chen
- Kathryn Dickerson (Rising Star)
- Samuel John (Rising Star)
- Patrick Leavey
- Kathleen Ludwig (Rising Star)
- Naomi Winick

Ashley Bui

- Elizabeth Schorry First-time Attendee Conference Award – Children's Tumor Foundation

Matthew Campbell

- KL2 Clinical Scholars Program – UT Southwestern

Katy Dickerson

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

Jessica Garcia

- Travel Award 2023 – HTRS Research Colloquium

Andrew Koh

- Standing member – Interspecies Microbial and Interactions and Infections study section (NIH/NIAID)

Patrick Leavey

- Immediate Past President – Active Medical/Dental Staff at Children's Medical Center Dallas

Xin Li

- Pathway to Independence Award – NIH

Tamra Slone

- President-Elect – Active Medical/Dental Staff, Children's Medical Center Plano

Sisi Zheng

- Blood Cancer Discovery Award for Outstanding Journal Article – American Association for Cancer Research

Yanbin Zheng

- Independent Investigator Grant Award – Rally Foundation and Infinite Love for Kids Fighting Cancer

Top Conference Locations

1. **International Society of Thrombosis and Hemostasis, Montreal, June 2023**
2. **American Society of Pediatric Hematology/Oncology, Fort Worth, May 2023**
3. **Annual Meeting of the Society of Pediatric Radiology, Austin, May 2023**
4. **Multiple International Speaker Invitations including Hans Knoll Institute, Germany, and University of Exeter, England (January-December 2023)**

Education and Training

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

Third-Year Medical Students

During their third year, medical students at 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 tumors, bone tumors, and other childhood cancers.

Fourth-Year Medical Students

Fourth-year medical students have the option to participate in a four-week elective in the Outpatient Hematology/Oncology Clinics in the Pauline Allen Gill Center for Cancer and Blood Disorders at Children's Health. 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 pediatric patients are often more acutely ill. With prior approval, this elective is also available for a limited number of fourth-year students from other medical schools.

Residents

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

Division faculty are consistently praised by the residents for their devotion to education. Over the course of the four-week rotation, several afternoons each week are devoted to enhanced learning opportunities, which may include lectures, pathology reviews, and bedside teaching. The curriculum covers most, if not all, of the American Board of Pediatrics Content Outlines 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 several 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 a tumor board.

Fellows

The Division provides an excellent opportunity for clinical fellowship training. Children's Medical Center Dallas, our primary pediatric teaching hospital, is the principal site for the clinical training of our fellows. Adjacent to UT Southwestern Medical Center, this hospital is consistently ranked by *U.S. News & 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 group of world-renowned investigators, including distinguished faculty who are Nobel Laureates and many more who are members of the National Academy of Sciences, the Institute of Medicine, and the Howard Hughes Medical Institute. Nearly all of our fellows secure funding to support or, in some cases, extend their research training.

Research Activities

The Division of Pediatric Hematology and Oncology is nationally distinguished for its design and conduct of NIH-funded multicenter clinical trials involving childhood cancer and blood diseases.

Laboratory Research

Faculty are conducting molecular and cellular biology experiments in cancer and blood diseases. 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 and Oncology and across the entire UT Southwestern Medical Center campus, including the NCI-designated Harold C. Simmons Comprehensive Cancer Center and the Children's Medical Center Research Institute at UT Southwestern.

Active areas of basic research include:

- Using complementary preclinical models to dissect the key "vulnerabilities" in rhabdomyosarcoma, Ewing sarcoma, and other soft-tissue sarcomas.
- Understanding the molecular machinery by which normal cells can undergo "senescence" as a tumor suppressor mechanism in the presence of a cancer-causing oncogene
- Identifying novel proteins that can be "targeted" as novel therapies in childhood cancer
- Uncovering how hematopoietic and embryonic stem cells are controlled and how these control mechanisms can go awry in cancer and blood diseases
- Using novel model systems to elucidate the host and bacterial factors that cause invasive bacterial and fungal infections
- Understanding how gut microbiota can augment anti-tumor immune responses in the context of cancer immunotherapy

- Understanding how the immune system impacts both outcomes and the development of specific late effects in pediatric leukemia patients
- Developing novel chimeric-antigen receptor T-cell therapy for acute myelogenous leukemia and pediatric solid tumors

Clinical Research

Physicians in the Division are engaged in a wide range of clinical research efforts spanning the cancer and blood disease programs. These efforts are supported by robust infrastructure provided by the Clinical Research Office (CRO) within the Gill Center, the Pediatric Hematology/Oncology Multicenter Research Study Core, and the Simmons Cancer Center, the only NCI-designated cancer center in North Texas. Currently, the research portfolio has over 150 oncology trials and 20 to 30 hematology trials open for enrollment for Gill Center patients.

Active areas of clinical research include:

- Prospective clinical trials for children with cancer, conducted under the umbrella of the NCI-sponsored Children's Oncology Group
- Prospective, early phase clinical trials for children with hematological malignancies, conducted as part of the Therapeutic Advances in Childhood Leukemia and Lymphoma (TACL) consortium and other academic and industry partners
- Prospective therapeutic trials for children and young adults with neurofibromatosis as part of the Neurofibromatosis Clinical Trials Consortium
- Clinical trials focused on children with neuroblastoma as part of the New Approaches to Neuroblastoma Therapy (NANT) consortium
- Prospective observational studies and clinical trials for children with sickle cell disease, rare anemias, venous thromboembolism and hemophilia, and von Willebrand disease
- Retrospective research studies investigating molecular and clinical factors influencing late effects in childhood cancer survivors
- Early phase clinical trials of immunotherapeutics for childhood cancer, including the use of CAR T-cells for childhood leukemia

Research Funding

Clinical and laboratory research efforts are funded by a wide variety of national, regional and local organizations, such as the National Cancer Institute; National Heart, Lung, and Blood Institute; National Eye Institute; Cancer Prevention and Research Institute of Texas; American Cancer Society; St. Baldrick's Foundation; Children's Cancer Fund of Dallas; Children's Medical Center Foundation; Wipe Out Kids' Cancer; 1 Million 4 Anna Foundation; and Hyundai Hope on Wheels Foundation.

Patient Care

The Pauline Allen Gill Center for Cancer and Blood Disorders (CCBD) at Children's Health in Dallas and Plano is the clinical site for pediatric hematology and oncology care at UT Southwestern. The largest 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.

Core Clinical Programs in Hematology and Oncology

- Brain Tumor
- Bone and Soft Tissue Sarcoma
- Bone Marrow Failure
- Genitourinary Neoplasms
- Hemophilia 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 Programs

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

A multidisciplinary approach is used in the Gill Center to plan and deliver clinical care that is targeted to meet the needs of each child. Among the services offered are child life, social work, child 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 public facility which serves Dallas County and is the site of the newborn nursery. Approximately 16,000 newborns are delivered at Parkland each year. New sites for hematology consultations include the newborn nursery at William P. Clements Jr. University Hospital and Texas Health Presbyterian Hospital Dallas.

Clinical Activities

The Pediatric Hematology/Oncology services at the CCBD have experienced continued growth over the last three years as a result of that expansion at Children's Medical Center Plano and the continued success of the Cellular and Immunotherapy Program. The average daily census was 44 total patients (eight patients at Plano and 36 patients at Dallas) during 2023, and a record 57 hematopoietic stem cell transplants were performed. Additional clinical activity highlights include implementing a fertility preservation program and reaching the landmark of delivering the services' 100th cell-based immunotherapy treatment.

Patient Statistics

Hematology and Oncology Patient Stats by Type of Visit by Academic and Calendar Year by Location.

Dallas

	2019	2020	2021	2022	2023
Inpatient consultations (heme only)	--	1877	2,148	2,359	2,549
Inpatient follow-up visits	13,885	12,622	13,459	12,831	12,140
New outpatient visits	1,515	1,594	1,722	1,686	1,720
Follow-up outpatient visits	12,519	10,708	11,730	11,756	11,872

Plano CMC

	2019	2020	2021	2022	2023
Inpatient consultations	--	--	--	--	--
Inpatient follow-up visits	124	131	1,628	2,383	2,714
New outpatient visits	365	312	564	617	753
Follow-up outpatient visits	1,981	2,027	2,987	3,612	3,981

Top Peer-Reviewed Publications and Book Chapters

1. Choi Y, Lichterman JN, Coughlin LA, Poulides N, Li W, Del Valle P, Palmer SN, Gan S, Kim J, Zhan X, Gao Y, Evers BM, Hooper LV, Pasare C, **Koh AY**. Immune checkpoint blockade induces gut microbiota translocation that augments extraintestinal antitumor immunity. Sci Immunol. 2023 Mar 10;8(81):eabo2003. PMID: 36867675

2. Gupta S, Dai Y, Chen Z, Winestone LE, Teachey DT, Bona K, Aplenc R, Rabin KR, Zweidler-McKay P, Carroll AJ, Heerema NA, Gastier-Foster J, Borowitz MJ, Wood BL, Maloney KW, Mattano LA Jr, Larsen EC, Angiolillo AL, Burke MJ, Salzer WL, Winter SS, Brown PA, Guest EM, Dunsmore KP, Kairalla JA, **Winick NJ**, Carroll WL, Raetz EA, Hunger SP, Loh ML, Devidas M. Racial and ethnic disparities in childhood and young adult acute lymphocytic leukaemia: secondary analyses of eight Children's Oncology Group cohort trials. Lancet Haematol. 2023 Feb;10(2):e129-e141. PMID: 36725118

3. Lamble AJ, Myers RM, Taraseviciute A, **John S**, Yates B, Steinberg SM, **Sheppard J**, Kovach AE, Wood B, Borowitz MJ, Stetler-Stevenson M, Yuan CM, Pillai V, Foley T, Chung P, Chen L, Lee DW, Annesley C, DiNofia A, Grupp SA, Verneris MR, Gore L, Laetsch TW, Bhojwani D, Brown PA, Pulsipher MA, Rheingold SR, Gardner RA, Shah NN. Preinfusion factors impacting relapse immunophenotype following CD19 CAR T cells. Blood Adv. 2023 Feb 28;7(4):575-585. PMID: 35482927

4. Warren KE, Vezina G, Krailo M, Springer L, Buxton A, Peer CJ, Figg WD, William-Hughes C, Kessel S, Fouladi M, Gajjar A, **Bowers D**. Phase II randomized trial of lenalidomide in children with pilocytic astrocytomas and optic pathway gliomas: A report from the children's oncology group. J Clin Oncol. 2023 Jun 20;41(18):3374-3383. PMID: 37126770

5. Whitworth H, Amankwah EK, Betensky M, Castellucci LA, Cuker A, Goldenberg NA, Male C, Rinzler E, **Zia A**, Raffini L. Updated guidance for efficacy and safety outcomes for clinical trials in venous thromboembolism in children: communication from the ISTH SSC Subcommittee on Pediatric and Neonatal Thrombosis and Hemostasis. J Thromb Haemost. 2023 Jun;21(6):1666-1673. PMID: 36921919