

2015 Research Annual Report

Oncology

RESEARCH AND TRAINING DETAILS



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Research Highlights

John Perentesis, MD

Dr. Perentesis leads the cancer programs for Cincinnati Children's, and his current major initiatives include development of the clinical and research initiative for the Proton Therapy Center, the Cincinnati Children's Cancer and Blood Diseases Institute (CBDI) Liberty Campus cancer center and next generation patient care delivery system, the Advanced Cancer Therapies Center, and the CBDI/pathology clinical leukemia and tumor sequencing/precision medicine center. In addition, he leads the international clinical and research programs. His academic work focuses on new anticancer drug discovery and development, and oncology translational clinical research at the national level. His laboratory program focuses on complementary studies of leukemia biology and genomics, and pharmacogenetics of childhood cancer therapy.

Significant Publications:

Emoto C, Fukuda T, Mizuno T, Cox S, Schniedewind B, Christians U, Widemann BC, Fisher MJ, Weiss B, Perentesis J, Vinks AA. Age-Dependent Changes in Sirolimus Metabolite Formation in Patients with Neurofibromatosis Type 1. *Ther Drug Monit*. 2015 Jun;37(3):395-9.

Fouladi M, Perentesis JP, Wagner LM, Vinks AA, Reid JM, Ahern C, Thomas G, Mercer CA, Krueger DA, Houghton PJ, Doyle LA, Chen H, Weigel B, Blaney SM. A Phase I Study of Cixutumumab (Imc-A12) in Combination with Temsirolimus (Cci-779) in Children with Recurrent Solid Tumors: A Children's Oncology Group Phase I Consortium Report. *Clin Cancer Res.* 2015 Apr 1;21(7):1558-65.

Horton TM, Perentesis JP, Gamis AS, Alonzo TA, Gerbing RB, Ballard J, Adlard K, Howard DS, Smith FO, Jenkins G, Kelder A, Schuurhuis GJ, Moscow JA. A Phase 2 Study of Bortezomib Combined with Either Idarubicin/Cytarabine or Cytarabine/Etoposide in Children with Relapsed, Refractory or Secondary Acute Myeloid Leukemia: A Report from the Children's Oncology Group. *Pediatr Blood Cancer*. 2014 Oct;61(10):1754-60.

Michael Absalon, MD, PhD

Based on a phase-1 pilot study of CPX-351, a chemotherapy nanoparticle established in 2013 for children with relapsed/refractory hematologic malignancies, early results from this first-in-children study have been used to develop an international phase-2 study of the agent for children and young adults with relapsed AML within the Children's Oncology Group.

Denise Adams, MD

Ahmad N, Adams DM, Wang J, Prakash R, Karim NA. Hepatic Epithelioid Hemangioendothelioma in a Patient with Hemochromatosis. *J Natl Compr Canc Netw.* 2014 Sep;12(9):1203-7.

This paper depicts the efficacy of sirolimus for a patients with metastatic Epithelioid Hemangioendotheliomas who had failed other treatment options in a disease with limited therapy options.

Fernandes VM, Fargo JH, Saini S, Guerrera MF, Marcus L, Luchtman-Jones L, Adams D, Meier ER. Kaposiform Lymphangiomatosis: Unifying Features of a Heterogeneous Disorder. *Pediatr Blood Cancer*. 2015 May;62(5):901-4.

This paper explores the heterogeneity of a rare newly identified vascular anomaly (KLA) and the use of sirolimus for this entity.

Jeng MR, Fuh B, Blatt J, Gupta A, Merrow AC, Hammill A, Adams D. Malignant Transformation of Infantile Hemangioma to Angiosarcoma: Response to Chemotherapy with Bevacizumab. *Pediatr Blood Cancer.* 2014 Nov;61(11):2115-7.

This paper is critical as it describes transformation of a benign common vascular tumor (hemangioma) into an aggressive

rare vascular tumor (angiosarcoma) which has changed our standard of practice for diffuse liver hemangiomas.

National/International leadership positions:

American Society of Pediatric Hematology/Oncology

- Program Committee Co-Chair
- · Certification Committee Chair
- Vascular Anomaly special interest Group Chair

International Society for the Study of Vascular Anomalies

• Scientific Committee Member

Honors

Best Doctors Cincinnati: 2014 and 2015

World Orphan Drug Congress USA Heroes Wall Award

2014 Nominee, Making A Difference Award 2014 - HVMC Team - Cincinnati Children's Hospital Medical Center

2014 Robert Schöbinger Prize for the best clinical paper, 20th International Workshop on Vascular Anomalies, Melbourne, Australia

Lionel Chow, MD, PhD

Dave N, Chow LM, Gudelsky GA, LaSance K, Qi X, Desai PB. Preclinical Pharmacological Evaluation of Letrozole as a Novel Treatment for Gliomas. *Mol Cancer Ther.* 2015 Apr;14(4):857-64.

This publication provides pre-clinical evidence for the repurposing of letrozole in the treatment of patients with high-grade astrocytoma. A clinical trial based on this work is being planned.

Gass D, Dewire M, Chow L, Rose SR, Lawson S, Stevenson C, Pai AL, Jones B, Sutton M, Lane A, Pruitt D, Fouladi M, Hummel TR. Pediatric Tectal Plate Gliomas: A Review of Clinical Outcomes, Endocrinopathies, and Neuropsychological Sequelae. *J Neurooncol.* 2015 Mar;122(1):169-77.

This publication reviews our institution's experience and long term outcome with tectal plate glioma. This information will help guide the pediatric neuro-oncology community in the appropriate treatments and follow-up for these patients.

Salloum R, DeWire M, Lane A, Goldman S, Hummel T, Chow L, Miles L, Sutton M, Stevenson C, Fouladi M, Leach J. Patterns of Progression in Pediatric Patients with High-Grade Glioma or Diffuse Intrinsic Pontine Glioma Treated with Bevacizumab-Based Therapy at Diagnosis. *J Neurooncol*. 2015 Feb;121(3):591-8.

This publication describes previously unrecognized aggressive invasive behavior of high-grade glioma and DIPG in children treated with bevacizumab. These results have contributed to the decreased use of this drug to treat these tumors in children.

Biplab Dasgupta, PhD

Pooya S, Liu X, Kumar VB, Anderson J, Imai F, Zhang W, Ciraolo G, Ratner N, Setchell KD, Yutaka Y, Jankowski MP, Dasgupta B*. The Tumor Suppressor *LKB1* regulates Myelination through Mitochondrial Metabolism. *Nat Commun.* 2014 Sep 26;5:4993.

This research showed that the tumor suppressor gene Lkb1 unexpectedly controls myelination in the peripheral nervous

system by regulating mitochondrial metabolism.

Invited Lecture:

• FASEB Conference, AMPK in Health and Disease, Lucca, Italy

Jennifer Davis, DO

Dr. Davis served on the St. Baldrick's Foundation 2015 Grant Review Committee, and in 2014 served on the ASPHO Education Committee, Special Interest Group for Vascular Anomalies. During her Hematology/Oncology fellowship, Dr. Davis served on the AAP SOMSRFT National Committee representing the residents and fellows in the AAP from District IV where she served as Co-Chair for the National SOMSRFT Advocacy Project *ImmuneWise*.

Dr. Davis is a Procter Scholar and former St. Baldrick's Fellow. She was selected to receive the American Society of Hematology (ASH) Abstract Achievement Award at the 54th Annual Meeting, and the American Society of Pediatric Hematology Organization (ASPHO) Travel Award at the 26th Annual Meeting where she delivered the Plenary Address during her Hematology Oncology Fellowship.

Mariko DeWire, MD

DeWire M, Fouladi M, Turner DC, Wetmore C, Hawkins C, Jacobs C, Yuan Y, Liu D, Goldman S, Fisher P, Rytting M, Bouffet E, Khakoo Y, Hwang El, Foreman N, Stewart CF, Gilbert MR, Gilbertson R, Gajjar A. An Open-Label, Two-Stage, Phase li Study of Bevacizumab and Lapatinib in Children with Recurrent or Refractory Ependymoma: A Collaborative Ependymoma Research Network Study (CERN). *J Neurooncol*. 2015 May;123(1):85-91.

This publication reports the combination of bevacizumab and lapatinib was well tolerated in children diagnosed with relapsed or refractory ependymoma, yet ineffective.

Salloum R, DeWire M, Lane A, Goldman S, Hummel T, Chow L, Miles L, Sutton M, Stevenson C, Fouladi M, Leach J. Patterns of Progression in Pediatric Patients with High-Grade Glioma or Diffuse Intrinsic Pontine Glioma Treated with Bevacizumab-Based Therapy at Diagnosis. *J Neurooncol.* 2015 Feb;121(3):591-8.

This publication retrospectively reviewed patients diagnosed with diffuse intrinsic pontine glioma (DIPG) and high-grade glioma (HGG) and treated with beavizumab based therapy. The patterns of progression were observed and concluded that Bevacizumab may lead to an increased incidence of distant and diffuse disease.

National/International leadership positions:

- CNS committee, Children's Oncology Group
- Principal Investigator, Pediatric Brain Tumor Consortium
- Clinical Trial Auditor, Pediatric Brain Tumor Consortium

Rachid Drissi, PhD

Margol AS, Robison NJ, Gnanachandran J, Hung LT, Kennedy RJ, Vali M, Dhall G, Finlay JL, Erdreich-Epstein A, Krieger MD, Drissi R, Fouladi M, Gilles FH, Judkins AR, Sposto R, Asgharzadeh S. Tumor-Associated Macrophages in Shh Subgroup of Medulloblastomas. *Clin Cancer Res.* 2015 Mar 15;21(6):1457-1465.

This data show that SHH tumors have a unique tumor microenvironment among medulloblastoma subgroups. The interactions of TAMs and SHH medulloblastoma cells may contribute to tumor growth revealing TAMs as a potential therapeutic target.

National/International leadership positions:

• Biology Co-Chair for the Pediatric Brain Tumor Consortium

Maryam Fouladi, MD

Fouladi M, Perentesis JP, Wagner LM, Vinks AA, Reid JM, Ahern C, Thomas G, Mercer CA, Krueger DA, Houghton PJ, Doyle LA, Chen H, Weigel B, Blaney SM. A Phase I Study of Cixutumumab (Imc-A12) in Combination with Temsirolimus (Cci-779) in Children with Recurrent Solid Tumors: A Children's Oncology Group Phase I Consortium Report. Clin Cancer Res. 2015 Apr 1;21(7):1558-65.

Hoffman LM, Fouladi M. Towards Risk-Adapted Therapy for Rhabdoid Tumour Subgroups. *Lancet Oncol.* 2015 May;16(5):486-8.

Packer RJ, Rood BR, Turner DC, Stewart CF, Fisher M, Smith C, Young-Pouissant T, Goldman S, Lulla R, Banerjee A, Pollack I, Kun L, Onar-Thomas A, Wu S, Boyett JM, Fouladi M. Phase I and Pharmacokinetic Trial of Ptc299 in Pediatric Patients with Refractory or Recurrent Central Nervous System Tumors: A Pbtc Study. *J Neurooncol.* 2015 Jan;121(1):217-24.

James Geller, MD

Abruzzo TA, Geller JI, Kimbrough DA, Michaels S, Corrêa ZM, Cornell K, Augsburger JJ. Adjunctive techniques for optimization of ocular hemodynamics in children undergoing ophthalmic artery infusion chemotherapy. *J Neurointerv Surg.* 2014 Sep Oct;7(10):770-6.

In advancing selective intra-ophthalmic artery infusion chemotherapy (SOAIC) at Cincinnati Children's for the treatment of intraocular retinoblastoma, the retinoblastoma team has safely and effectively optimized the infusion technique now promoting 100% technical success in drug delivery, making this option available to all infants and children with intraocular retinoblastoma needing such therapy.

Cost NG, DeFoor WR, Jr., Crotty EJ, Geller JI. The Initial Experience with Renal Nephrometry in Children, Adolescents, and Young Adults with Renal Tumors. *Pediatr Blood Cancer*. 2014 Aug;61(8):1434-9.

Predictive imaging scoring achieved with Renal Nephrometry of pediatric renal tumors, presented for the first time in this manuscript, may provide the means for the clinical research community to move forward with formal study of renal sparing surgery (patient selection for possible partial nephrectomy), rather than continuing with current approaches that rely heavily on surgery via complete nephrectomy for nearly all pediatric patients with kidney malignancies.

Lombardi AJ, Sutton ME, Tiao GM, Geller JI. Vincristine-Associated Neurological Morbidity in the Treatment of Hepatoblastoma. *J Pediatr Hematol Oncol.* 2015 May;37(4):e258-63.

The use of vincristine for the treatment of hepatoblastoma remains controversial with unclear benefit; and we have now presented a clearer picture of risks, advancing risk/benefit considerations for the international intergroup efforts as we design the first 'world study' of hepatoblastoma via the Children's Oncology Group (COG), Society of Pediatric Oncology in Europe (SIOPEL), and the Japanese Liver Tumor Group (JPLT).

National/International leadership positions:

At the national level, Dr. Geller participates as a member of the Children's Oncology Group (COG) Renal Tumor (steering committee member), Rare Tumor including Liver Tumor, Retinoblastoma and Central Nervous System Committees.

For the COG Renal Tumor Committee (RTC), he functions as liaison to the COG Developmental Therapeutics Committee (DVL), Pediatric Preclinical Testing Program (PPTP), the NCI-sponsored Pediatric Match Trial, as well as chair to both the RTC Developmental Therapeutics Working Group and the Task Force to develop the next COG High Risk Renal Tumors Protocols (anaplastic Wilms tumor, rhabdoid tumor, relapsed Wilms tumor) and Chair our first cross-collaborative group

study of translocation renal cell carcinoma, slotted to be co-sponsored by COG and ECOG through our National Cancer Therapy Network (NCTN). (AREN1621) In addition, he is the Vice Chair of the pivotal Renal Biology, Banking and Classification study (AREN03B2) and participates as a voting member of the RTC Biology Subcommittee.

Dr. Geller serves as the oncology lead for COG in developing the next liver tumor trial, an international effort titled Pediatric Hepatic International Tumor Trial (PHITT; AHEP1531), and helps lead biological therapy integration initiatives.

For the Renal, Liver and DVL committees, he currently Chair 3 National Phase 1 and 2 trials studying novel therapy in pediatric and adolescent solid and CNS tumors: ADVL1111 – Phase 1 study of tivantinib, ADVL1315 – Phase 1 study of axitinib, ADVL1522 – Phase 2 study of IMGN901.

Adrienne Hammill, MD, PhD

Adams DM, Hammill A. Other Vascular Tumors. Semin Pediatr Surg. 2014 Aug;23(4):173-7.

A comprehensive review of vascular tumors.

Jeng MR, Fuh B, Blatt J, Gupta A, Merrow AC, Hammill A, Adams D. Malignant Transformation of Infantile Hemangioma to Angiosarcoma: Response to Chemotherapy with Bevacizumab. *Pediatr Blood Cancer*. 2014 Nov;61(11):2115-7.

1st report of malignant transformation, as well as chemotherapeutic tx.

National/International leadership positions:

ASPHO

• Vascular SIG - Vice-Chair of Practice Committee

Sturge-Weber Syndrome Task Force

· Chair, Clinicial Consensus Committee

Invited Speaker – Grand Rounds, El Paso Children's Hospital – May 2015

Invited Speaker at NINDS-Funded Research Meeting on SWS – "Leveraging a Gene Discovery: an agenda for future research" – April 2015

Trent Hummel, MD

Prada CE, Hufnagel RB, Hummel TR, Lovell AM, Hopkin RJ, Saal HM, Schorry EK. The Use of Magnetic Resonance Imaging Screening for Optic Pathway Gliomas in Children with Neurofibromatosis Type 1. *J Pediatr*. 2015 Jul 29. pii: S0022-3476(15)00716-7.

Impact: Early identification of OPG by screening MRI prior to the development of vision loss may lead to improved visual outcomes - children with negative brain and orbital MRI screening at age 15 months or later did not develop symptomatic OPGs.

Gass D, Dewire M, Chow L, Rose SR, Lawson S, Stevenson C, Pai AL, Jones B, Sutton M, Lane A, Pruitt D, Fouladi M, Hummel TR. Pediatric tectal plate gliomas: a review of clinical outcomes, endocrinopathies, and neuropsychological sequelae. *J Neurooncol.* 2015 Mar;122(1):169-77.

Impact: While tectal plate gliomas have historically been considered indolent tumors that are rarely progressive, 23% of patients in our study experienced disease progression and required further therapy

Salloum R, DeWire M, Lane A, Goldman S, Hummel T, Chow L, Miles L, Sutton M, Stevenson C, Fouladi M, Leach J. Patterns of progression in pediatric patients with high-grade glioma or diffuse intrinsic pontine glioma treated with

Bevacizumab-based therapy at diagnosis. J Neurooncol. 2015 Feb;121(3):591-8.

Impact: Bevacizumab may lead to a higher incidence of distant and diffuse metastatic disease in newly-diagnosed children with high grade gliomas or DIPG who received Bevacizumab-based therapy.

National/International leadership positions:

Pediatric Brain Tumor Consortium:

- Member of Scientific Committee
- · Site Co-Principal Investigator

Jennifer Mangino, MD

Dr. Mangino was a recipient of the 2015 Cancer Family Care Unsung Hero Award.

Alferiev IS, Iyer R, Croucher JL, Adamo RF, Zhang K, Mangino JL, Kolla V, Fishbein I, Brodeur GM, Levy RJ, Chorny M. Nanoparticle-Mediated Delivery of a Rapidly Activatable Prodrug of Sn-38 for Neuroblastoma Therapy. *Biomaterials*. 2015 May;51:22-9.

This publication details a new method of packaging and delivering a potent anti-cancer drug.

lyer R, Croucher JL, Chorny M, Mangino JL, Alferiev IS, Levy RJ, Kolla V, Brodeur GM. Nanoparticle Delivery of an Sn38 Conjugate Is More Effective Than Irinotecan in a Mouse Model of Neuroblastoma. Cancer Lett. 2015 May 1;360(2):205-12.

Irinotecan is a frequent chemotherapeutic agent used in relapsed Neuroblastoma, and this paper details a nanoparticle pro-drug that is even more potent than standard Irinotecan in an animal model.

Benjamin Mizukawa, MD

Dr. Mizukawa is a St. Baldrick's Foundation Scholar.

Zhang S, Konstantinidis DG, Yang JQ, Mizukawa B, Kalim K, Lang RA, Kalfa TA, Zheng Y, Guo F. Gene Targeting Rhoa Reveals Its Essential Role in Coordinating Mitochondrial Function and Thymocyte Development. *J Immunol*. 2014 Dec 15;193(12):5973-82.

Identified a critical role for RhoA GTPase signaling in thymocyte development and survival through its regulation of mitochondrial function and reactive oxygen species generation.

Rajaram Nagarajan, MD

Bielack SS, Smeland S, Whelan JS, Marina N, Jovic G, Hook JM, Krailo MD, Gebhart M, Papai S, Meyer J, Nadel H, Randall RL, Deffenbaugh C, Nagarajan R, Brennan B, Letson GD, Teot LA, Goorin A, Baumhoer D, Werner M, Lau CC, Sundby Hall K, Gelderblom H, Meyers P, Gorlick R, Windhager R, Helmke K, Eriksson M, Hoogerbrugge PM, Schomberg P, Tunn PU, Kuehne T, Jürgens H, van den Berg H, Böhling T, Picton S, Renard M, Reichardt P, Gerss J, Butterfass-Bahloul T, Morris C, Hogendoorn PC, Seddon B, Calaminus G, Michelagnoli M, Dhooge C, Sydes MR, Bernstein M. MAP plus maintenance pegylated interferon-alpha-2b (MAPifn) versus MAP alone in patients with resectable high-grade osteosarcoma and good histological response to preoperative MAP: First results of the EURAMOS-1 Good Response randomized controlled trial. *J Clin Oncol.* 2015 Jul 10;33(20):2279-87.

This publication describes results of the largest pediatric osteosarcoma trial for those patients with a good histologic response to neoadjuvant therapy.

Whitlow PG, Caparas M, Cullen P, Trask C, Schulte F, Embry L, Nagarajan R, Johnston DL, Sung L. Strategies to Improve Success of Pediatric Cancer Co-operative Group Quality of Life Studies: A Report from the Children's Oncology Group.

Qual Life Res. 2015 Jun;24(6):1297-301.

This publication describes the state of past and current QOL of studies in COG.

Whelan JS, Bielack SS, Marina N, Smeland S, Jovic G, Hook JM, Krailo M, Anninga J, Butterfass-Bahloul T, Böhling T, Calaminus G, Capra M, Deffenbaugh C, Dhooge C, Eriksson M, Flanagan AM, Gelderblom H, Goorin A, Gorlick R, Gosheger G, Grimer RJ, Hall KS, Helmke K, Hogendoorn PC, Jundt G, Kager L, Kuehne T, Lau CC, Letson GD, Meyer J, Meyers PA, Morris C, Mottl H, Nadel H, Nagarajan R, Randall RL, Schomberg P, Schwarz R, Teot LA, Sydes MR, Bernstein M,EUROMAS collaborators. EURAMOS-1, an international randomised study for osteosarcoma: results from prerandomisation treatment. *Ann Oncol.* 2015 Feb;26(2):407-14.

This publication describes the largest international pediatric osteosarcoma trial.

National/International leadership positions:

Physician Champion for the National Association of Children's Hospitals and Related Institutions/Children's Hospital Association (NACHRI) Hematology/Oncology Quality Improvement Collaborative in Catheter Related Blood Stream Infections (2009 to present)

Children's Oncology Group

- Cincinnati Children's Cancer Control Local Responsible Investigator for the Cancer Control Committee.
- Member of the Children's Oncology Group Protocol AML1031 study committee: AML 1031 "A Phase III Randomized Trial for Patients with *de novo* AML using Bortezomib (IND# 58443, NSC# 681239) and Sorafenib (BAY 43-9006, IND#69896, NSC# 724772) for Patients with FLT3 ITD".
- Member of the Children's Oncology Group Protocol AEPI05N2 study committee: AEPI05N2 "Genetic Epidemiology of Osteosarcoma".
- Member of the Children's Oncology Group Protocol AOST0331 study committee: AOST 0331 "A randomized trial of the European and American Osteosarcoma Study Group to optimize treatment strategies for resectable osteosarcoma based on histological response to pre-operative chemotherapy " (IND# 12697) A Phase III Intergroup Study.

Committee Chair for the University of Cincinnati Cancer Survivorship Research Program – Cincinnati Cancer Center (July 2014 to present).

Maureen O'Brien, MD

Bukowinski AJ, Burns KC, Parsons K, Perentesis JP, O'Brien MM. Toxicity of Cancer Therapy in Adolescents and Young Adults (AYAs). Semin Oncol Nurs. 2015 Aug;31(3):216-26.

Comprehensive review of the challenges and research opportunities in AYA pharmacology.

Smith FO, O'Brien MM. Thiopurines for the Treatment of Acute Lymphoblastic Leukemia in Children: What's Old Is New. *JAMA Oncol.* 2015 Jun 1;1(3):281-2.

Invited review of the challenge of optimizing thiopurine maintenance therapy in ALL.

Alcamo AM, Pinchasik DE, Mo JQ, Grimley MS, O'Brien MM. Successful Treatment of Disseminated Adenovirus Infection in an Infant With Acute Lymphoblastic Leukemia. *J Pediatr Hematol Oncol.* 2015 Apr; 37(3):e178-81.

Case report highlighting the importance of viral surveillance in infant ALL.

Thomas TO, Chandrakasan S, O'Brien M, Jefferies JL, Ryan TD, Wilmot I, Baker ML, Madueme PC, Morales D, Lorts A. The

use of a Berlin Heart EXCOR LVAD in a child receiving chemotherapy for Castleman's disease. *Pediatr Transplant.* 2015 Feb;19(1):E15-8.

Case report highlighting the only published case where chemotherapy was safely and successfully administered to a child with and LVAD, demonstrating the collaboration and expertise of the Heart Institute and CBDI

National/International leadership positions:

- Children's Oncology Group (COG) Myeloid Steering Committee and Novel Agents Committee
- National Study Chair COG AAML1522: Phase 2 Study of Lenalidomide in Relapsed/Refractory Pediatric Acute Myeloid Leukemia
- Study Committee COG AAML1532 (Risk Stratified Therapy for Acute Myeloid Leukemia in Down Syndrome)
- Children's Oncology Group Acute Lymphoblastic Leukemia Committee
- ALL 2018 Frontline ALL trial Task Force
- Study Committee COG AALL1131 (Risk-Stratified Randomized Phase III Testing of Blinatumomab (IND#117467, NSC-765986) in First Relapse of Childhood B-Lymphoblastic Leukemia (B-ALL))
- Therapeutic Advances in Childhood Leukemia (TACL consortium)
- Site Principal Investigator
- National Study Vice-Chair: T2015-001 (Phase 1 trial of inotuzumab ozogamicin in relapsed pediatric acute lymphoblastic leukemia)

Christine Phillips, MD

Fouladi M, Perentesis JP, Phillips CL, Leary S, Reid JM, McGovern RM, Ingle AM, Ahern CH, Ames MM, Houghton P, Doyle LA, Weigel B, Blaney SM. A Phase I Trial of Mk-2206 in Children with Refractory Malignancies: A Children's Oncology Group Study. *Pediatr Blood Cancer*. 2014 Jul;61(7):1246-51.

Publication of first pediatric trial of MK-2206, an AKT inhibitor, in children with refractory cancer.

Invited member of CRTI Study Section of the American Society of Hematology.

Joseph Pressey, MD

Barnes MJ, Pressey J, Adams J, Hensler MA, Madan-Swain A. Physician and Nurse Beliefs of Phase 1 Trials in Pediatric Oncology. *Cancer Nurs.* 2014 Sep-Oct;37(5):E48-52.

This manuscript describes potential barriers to the enrollment of cancer patients on critical Phase 1 therapy trials.

Srivastava RK, Kaylani SZ, Edrees N, Li C, Talwelkar SS, Xu J, Palle K, Pressey JG, Athar M. Gli Inhibitor Gant-61 Diminishes Embryonal and Alveolar Rhabdomyosarcoma Growth by Inhibiting Shh/Akt-Mtor Axis. *Oncotarget.* 2014 Dec 15;5(23):12151-65.

This manuscript demonstrates the therapeutic effect of a novel therapeutic agent in rhabdomyosarcoma (RMS) and the role of crosstalk between signaling pathways in RMS tumorigenesis.

Lisa Privette Vinnedge, PhD

Privette Vinnedge LM, Benight NM, Wagh PK, Pease NA, Nashu MA, Serrano-Lopez J, Adams AK, Cancelas JA, Waltz SE, Wells SI. The Dek Oncogene Promotes Cellular Proliferation through Paracrine Wnt Signaling in Ron Receptor-Positive

Breast Cancers. Oncogene. 2015 Apr 30;34(18):2325-36.

This manuscript was the first to study the oncogenic activities of DEK in solid tumors *in vivo* and linked it to other well-established signaling pathways including RON receptor tyrosine kinase and Wnt signaling.

William Seibel, MD

Evelyn C, Duan X, Biesiada J, Seibel W, Meller J, Zheng Y. Rational Design of Small Molecule Inhibitors Targeting the Ras GEF, SOS1. *Chem Biol.* 2014 Dec 18; 21(12): 1618-28.

This paper describes the discovery of the first inhibitors of SOS1, a key regulator of RAS activation, which is in turn a key driver in cancer cell growth and metastasis.

James MA, Seibel WL, Kupert E, Hu X, Potharla VY, Anderson MW. A novel, soluble compound, C25, sensitizes to TRAIL-induced apoptosis through upregulation of DR5 expression. *Anticancer Drugs*. 2015 Jun;26(5):518-30.

This paper describes the discovery of a novel inhibitor of NFkB and the characterization of its efficacy in potentiating other chemotherapeutics via upregulation of DR5. Such compounds might be combined with many existing chemotherapeutics allowing them to be used at a lower dose, thereby retaining activity, but lessening side effects.

Chen Y, Tang H, Seibel W, Papoian R, Oh K, Li X, Zhang J, Golczak M, Palczewski K, Kiser PD. Identification and characterization of novel inhibitors of Mammalian aspartyl aminopeptidase. *Mol Pharmacol.* 2014 Aug; 86(2):231-42.

This paper describes the de novo design and screening of inhibitors of DAP, which will allow evaluation of the therapeutic potential for this target in diabetic retinopathy.

Brian Turpin, MD

Trout A, Sharp S, Turpin B, Zhang B, Gelfand M. Optimizing the interval between G-CSF therapy and F18 FDG-PET imaging in children and young adult patients receiving chemotherapy for sarcoma. *Pediatr Radiol.* 2015 Jul;45(7):1001-6.

This article addresses the challenge of the use of functional imaging in sarcoma response assessment when interference due to the obligatory use of marrow stimulating factors is present.

Andersson C, Kvist PH, McElhinney K, Baylis R, Gram LK, Pelzer H, Lauritzen B, Holm TL, Hogan S, Wu D, Turpin B, Miller W, Palumbo JS. Factor XIII Transglutaminase Supports the Resolution of Mucosal Damage in Experimental Colitis. *PLoS One.* 2015 Jun 22;10(6):e0128113.

This article demonstrates that FXIII is a significant determinant of mucosal healing, and interventions supporting FXIII activity may be a means to limit colitis pathology and improve resolution of mucosal damage, thus possibly abrogating development of inflammation driven cancers.

Turpin B*, Miller W*, Rosenfeldt L, Kombrinck K, Flick MJ, Steinbrecher KA, Harmel-Laws E, Mullins ES, Shaw M, Witte DP, Revenko A, Monia N, Palumbo JS. (* signifies equal contributors). Thrombin drives tumorigenesis in colitis-associated colon cancer. *Cancer Res.* 2014 Jun 1;74(11):3020-30.

This publication broadens the understanding of the role of hematologic factors in tumor development through detailed studies of the role of thrombin on early oncogenesis in inflammation driven cancer.

Brian Weiss, MD

Weiss B, Widemann BC, Wolters P, Dombi E, Vinks A, Cantor A, Perentesis J, Schorry E, Ullrich N, Gutmann DH, Tonsgard J, Viskochil D, Korf B, Packer RJ, Fisher MJ. Sirolimus for Progressive Neurofibromatosis Type 1-Associated Plexiform Neurofibromas: A Neurofibromatosis Clinical Trials Consortium Phase II Study. *Neuro Oncol.* 2015 Apr;17(4):596-603.

This paper summarized an important study of Sirolimus for NF1 plexiform neurofibroma in which Sirolimus prolonged time to progression for patients with progressive plexiforms.

Dandoy CE, Davies SM, Flesch L, Hayward M, Koons C, Coleman K, Jacobs J, McKenna LA, Olomajeye A, Olson C, Powers J, Shoemaker K, Jodele S, Alessandrini E, Weiss B. A Team-Based Approach to Reducing Cardiac Monitor Alarms. *Pediatrics*. 2014 Dec;134(6):e1686-94.

This improvement science article addressed a common safety issue in novel ways. As senior author, Dr. Weiss supervised and worked closely with Dr. Dandoy on this project, and on this article.

Salloum R, Garrison A, von Allmen D, Sheridan R, Towbin AJ, Adams D, Weiss B. Relapsed Perinatal Neuroblastoma after Expectant Observation. *Pediatr Blood Cancer*. 2015 Jan;62(1):160-2.

A recently published report of the only patient with low-risk neuroblastoma on the COG observation trial to have relapsed with high-risk neuroblastoma.

National/International leadership positions:

- Member, COG Neuroblastoma Committee
- Study Chair: 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.
- Study Chair: NF106: A Phase 2 Trial of the MEK Inhibitor PD-0325901 in Adolescents and Adults with NF1- Associated Morbid Plexiform Neurofibromas.

Susanne Wells, PhD

Adams AK, Hallenbeck GE, Casper KA, Patil YJ, Wilson KM, Kimple RJ, Lambert PF, Witte DP, Xiao W, Gillison ML, Wikenheiser-Brokamp KA, Wise-Draper TM, Wells SI. Dek Promotes Hpv-Positive and -Negative Head and Neck Cancer Cell Proliferation. *Oncogene*. 2015 Feb 12;34(7):868-77.

DEK loss in mouse and human models of head and neck cancer attenuates tumor cell proliferation.

Chlon TM, Hoskins EE, Mayhew CN, Wikenheiser-Brokamp KA, Davies SM, Mehta P, Myers KC, Wells JM, Wells SI. High-Risk Human Papillomavirus E6 Protein Promotes Reprogramming of Fanconi Anemia Patient Cells through Repression of P53 but Does Not Allow for Sustained Growth of Induced Pluripotent Stem Cells. *J Virol*. 2014 Oct;88(19):11315-26.

Pluripotent stem cell reprogramming requires an intact FA pathway.

Lombardi AJ, Hoskins EE, Foglesong GD, Wikenheiser-Brokamp KA, Wiesmuller L, Hanenberg H, Andreassen PR, Jacobs AJ, Olson SB, Keeble WW, Hays LE, Wells SI. Acquisition of Relative Interstrand Crosslinker Resistance and Parp Inhibitor Sensitivity in Fanconi Anemia Head and Neck Cancers. *Clin Cancer Res.* 2015 Apr 15;21(8):1962-72.

Head and neck cancer in FA evolves to overcome crosslinker sensitivity, but develops PARP sensitivity.

Significant Publications

Dandoy CE, Davies SM, Flesch L, Hayward M, Koons C, Coleman K, Jacobs J, McKenna LA, Olomajeye A, Olson C, Powers J, Shoemaker K, Jodele S, Alessandrini E, **Weiss B**. A Team-Based Approach to Reducing Cardiac Monitor Alarms. *Pediatrics*. 2014 Dec;134(6):e1686-94.

This is the lead annual publication for our safety pillar of activities. It describes a novel approach using latest human factors research methodologies to reduce critical distraction from inappropriate alarms in the patient care setting. In

other high reliability settings, e.g., the airline and nuclear power industries, this reduction of distraction from false alarms results in dramatic reduction in worker errors, and major improvements in safety.

Emoto C, Fukuda T, Mizuno T, Cox S, Schniedewind B, Christians U, Widemann BC, Fisher MJ, Weiss B, Perentesis J, Vinks AA. Age-Dependent Changes in Sirolimus Metabolite Formation in Patients with Neurofibromatosis Type 1. *Ther Drug Monit*. 2015 Jun;37(3):395-9.

This publication is representative of our national leadership in translational cancer pharmacology and genomics. The paper describes leading-edge work in developmental pharmacology - the identification of the full spectrum of anticancer drug metabolites and how they change with age. Most previous studies have focused only on the differences in levels of the parent drugs between adults and children. This important new area provides a full picture of the drug and the metabolites which may have a more profound impact on outcomes including cure and side effects. This is also the foundation of the major integrated collaboration between multiple divisions: patient genomics and clinical trials are conducted in the oncology division; the pharmacology and pathology divisions assay drugs and metabolites along with pharmacokinetic modeling; and the biomedical informatics division integrates and develops high density data predictive models. This work is at the leading edge for the major new CBDI program in cancer genomics and patient-derived xenografts/avatars.

Fouladi M, Perentesis JP, Wagner LM, Vinks AA, Reid JM, Ahern C, Thomas G, Mercer CA, Krueger DA, Houghton PJ, Doyle LA, Chen H, Weigel B, Blaney SM. A Phase I Study of Cixutumumab (Imc-A12) in Combination with Temsirolimus (Cci-779) in Children with Recurrent Solid Tumors: A Children's Oncology Group Phase I Consortium Report. Clin Cancer Res. 2015 Apr 1;21(7):1558-65.

The oncology division at Cincinnati Children's is the nation's largest center for new anticancer drug trials and tumor genomic-guided therapies. This high impact manuscript is representative of multiple publications delineating new anticancer drug development led by our group. These early clinical trials not only yield insight into to cancer biology and response, but serve as the foundation for the next series of standard of care anticancer regimens. In many circumstances, the therapies being developed in oncology represent what will be available nationally in three to five years.

Pooya S, Liu X, Kumar VB, Anderson J, Imai F, Zhang W, Ciraolo G, Ratner N, Setchell KD, Yoshida Y, Jankowski MP, Dasgupta B. The Tumour Suppressor Lkb1 Regulates Myelination through Mitochondrial Metabolism. *Nat Commun.* 2014 Sep 26;5:4993.

Cancer research efforts in the CBDI are seamlessly integrated with a team science approach. This high impact publication reflects a major focus of oncology - and the CBDI - into basic investigations of the biochemistry and quantum biology of tumor metabolism. In turn, these insights are directly tied to the development of novel pediatric anticancer agents.

Privette Vinnedge LM, Benight NM, Wagh PK, Pease NA, Nashu MA, Serrano-Lopez J, Adams AK, Cancelas JA, Waltz SE, Wells SI. The Dek Oncogene Promotes Cellular Proliferation through Paracrine Wnt Signaling in Ron Receptor-Positive Breast Cancers. *Oncogene*. 2015 Apr 30;34(18):2325-36.

This high impact publication explores and provides insight into essential - and targetable - mechanisms underpinning cellular proliferation driven by paracrine signaling. This work serves not only as a model for epithelial cancers but has strong implications for similar mechanisms and pathways in other tumors as well. This work is also integrated into drug development and drug discovery efforts within the CBDI.

Division Publications

- Abruzzo TA, Geller JI, Kimbrough DA, Michaels S, Correa ZM, Cornell K, Augsburger JJ. Adjunctive techniques for optimization of ocular hemodynamics in children undergoing ophthalmic artery infusion chemotherapy. J Neurointerv Surg. 2014; .
- 2. Adams AK, Hallenbeck GE, Casper KA, Patil YJ, Wilson KM, Kimple RJ, Lambert PF, Witte DP, Xiao W, Gillison ML, Wikenheiser-Brokamp KA, Wise-Draper TM, Wells SI. **DEK promotes HPV-positive and -negative head and neck cancer cell proliferation**. *Oncogene*. 2015; 34:868-77.
- 3. Adams DM, Hammill A. Other vascular tumors. Semin Pediatr Surg. 2014; 23:173-7.
- 4. Ahmad N, Adams DM, Wang J, Prakash R, Karim NA. Hepatic epithelioid hemangioendothelioma in a patient with hemochromatosis. *J Natl Compr Canc Netw.* 2014; 12:1203-7.
- 5. Alcamo AM, Pinchasik DE, Mo JQ, Grimley MS, O'Brien MM. Successful Treatment of Disseminated Adenovirus Infection in an Infant With Acute Lymphoblastic Leukemia. *J Pediatr Hematol Oncol*. 2014; 37:e178–e181.
- Alferiev IS, Iyer R, Croucher JL, Adamo RF, Zhang K, Mangino JL, Kolla V, Fishbein I, Brodeur GM, Levy RJ, Chorny M. Nanoparticle-mediated delivery of a rapidly activatable prodrug of SN-38 for neuroblastoma therapy.
 Biomaterials. 2015; 51:22-9.
- 7. Andersson C, Kvist PH, McElhinney K, Baylis R, Gram LK, Pelzer H, Lauritzen B, Holm TL, Hogan S, Wu D, Turpin B, Miller W, Palumbo JS. Factor XIII Transglutaminase Supports the Resolution of Mucosal Damage in Experimental Colitis. *PLoS One*. 2015; 10:e0128113.
- 8. Antonini TN, Raj SP, Oberjohn KS, Cassedy A, Makoroff KL, Fouladi M, Wade SL. A pilot randomized trial of an online parenting skills program for pediatric traumatic brain injury: improvements in parenting and child behavior. *Behav Ther.* 2014; 45:455-68.
- 9. Barnes MJ, Pressey J, Adams J, Hensler MA, Madan-Swain A. Physician and nurse beliefs of phase 1 trials in pediatric oncology. *Cancer Nurs*. 2014; 37:E48-52.
- 10. Brink A, Correa ZM, Geller J, Abruzzo T, Augsburger JJ. Managing the consequences of aggressive conservative treatment for refractory retinoblastoma with vitreous seeding. *Arq Bras Oftalmol*. 2014; 77:256-8.
- 11. Chamlin SL, Mancini AJ, Lai JS, Beaumont JL, Cella D, Adams D, Drolet B, Baselga E, Frieden IJ, Garzon M, Holland K, Horii KA, Lucky AW, McCuaig C, Metry D, Morel KD, Newell BD, Nopper AJ, Powell J, Siegel D, Haggstrom AN. Development and Validation of a Quality-of-Life Instrument for Infantile Hemangiomas. *J Invest Dermatol*. 2015; 135:1533-9.
- 12. Chen Y, Tang H, Seibel W, Papoian R, Li X, Lambert NA, Palczewski K. A High-Throughput Drug Screening Strategy for Detecting Rhodopsin P23H Mutant Rescue and Degradation. *Invest Ophthalmol Vis Sci.* 2015; 56:2553-67.
- 13. Chen Y, Tang H, Seibel W, Papoian R, Oh K, Li X, Zhang J, Golczak M, Palczewski K, Kiser PD. **Identification and characterization of novel inhibitors of Mammalian aspartyl aminopeptidase**. *Mol Pharmacol*. 2014; 86:231-42.
- 14. Chlon TM, Hoskins EE, Mayhew CN, Wikenheiser-Brokamp KA, Davies SM, Mehta P, Myers KC, Wells JM, Wells SI. High-risk human papillomavirus E6 protein promotes reprogramming of Fanconi anemia patient cells through repression of p53 but does not allow for sustained growth of induced pluripotent stem cells. J Virol. 2014; 88:11315-26.

- 15. Cooper TM, Alonzo TA, Gerbing RB, Perentesis JP, Whitlock JA, Taub JW, Horton TM, Gamis AS, Meshinchi S, Loken MR, Razzouk BI. AAML0523: a report from the Children's Oncology Group on the efficacy of clofarabine in combination with cytarabine in pediatric patients with recurrent acute myeloid leukemia. *Cancer*. 2014; 120:2482-9.
- 16. Cost NG, DeFoor WR, Jr., Crotty EJ, Geller Jl. The initial experience with RENAL Nephrometry in children, adolescents, and young adults with renal tumors. *Pediatr Blood Cancer*. 2014; 61:1434-9.
- 17. Cost NG, Liss ZJ, Bean CM, Geller JI, Minevich EA, Noh PH. Prechemotherapy robotic-assisted laparoscopic radical nephrectomy for an adolescent with Wilms tumor. *J Pediatr Hematol Oncol*. 2015; 37:e125-7.
- 18. Cripe TP, Ngo MC, Geller JI, Louis CU, Currier MA, Racadio JM, Towbin AJ, Rooney CM, Pelusio A, Moon A, Hwang TH, Burke JM, Bell JC, Kirn DH, Breitbach CJ. Phase 1 study of intratumoral Pexa-Vec (JX-594), an oncolytic and immunotherapeutic vaccinia virus, in pediatric cancer patients. *Mol Ther*. 2015; 23:602-8.
- 19. Dandoy CE, Davies SM, Flesch L, Hayward M, Koons C, Coleman K, Jacobs J, McKenna LA, Olomajeye A, Olson C, Powers J, Shoemaker K, Jodele S, Alessandrini E, Weiss B. A team-based approach to reducing cardiac monitor alarms. *Pediatrics*. 2014; 134:e1686-94.
- 20. Dave N, Chow LM, Gudelsky GA, LaSance K, Qi X, Desai PB. Preclinical pharmacological evaluation of letrozole as a novel treatment for gliomas. *Mol Cancer Ther.* 2015; 14:857-64.
- 21. DeWire M, Fouladi M, Turner DC, Wetmore C, Hawkins C, Jacobs C, Yuan Y, Liu D, Goldman S, Fisher P, Rytting M, Bouffet E, Khakoo Y, Hwang El, Foreman N, Stewart CF, Gilbert MR, Gilbertson R, Gajjar A. An open-label, two-stage, phase II study of bevacizumab and lapatinib in children with recurrent or refractory ependymoma: a collaborative ependymoma research network study (CERN). *J Neurooncol*. 2015; 123:85-91.
- 22. DeWire M, Green DM, Sklar CA, Merchant TE, Wallace D, Lin T, Vern-Gross T, Kun LE, Krasin MJ, Boyett JM, Wright KD, Wetmore C, Broniscer A, Gajjar A. Pubertal development and primary ovarian insufficiency in female survivors of embryonal brain tumors following risk-adapted craniospinal irradiation and adjuvant chemotherapy. *Pediatr Blood Cancer*. 2014; 62:329–334.
- 23. Dillehay KL, Seibel WL, Zhao D, Lu S, Dong Z. **Target validation and structure-activity analysis of a series of novel PCNA inhibitors**. *Pharmacol Res Perspect*. 2015; 3:e00115.
- 24. Doros LA, Rossi CT, Yang J, Field A, Williams GM, Messinger Y, Cajaiba MM, Perlman EJ, K AS, Cathro HP, Legallo RD, LaFortune KA, Chikwava KR, Faria P, Geller JI, Dome JS, Mullen EA, Gratias EJ, Dehner LP, Hill DA. DICER1 mutations in childhood cystic nephroma and its relationship to DICER1-renal sarcoma. *Mod Pathol*. 2014; 27:1267-80.
- 25. DuBois SG, Groshen S, Park JR, Haas-Kogan DA, Yang X, Geier E, Chen E, Giacomini K, Weiss B, Cohn SL, Granger MM, Yanik GA, Hawkins R, Courtier J, Jackson H, Goodarzian F, Shimada H, Czarnecki S, Tsao-Wei D, Villablanca JG, Marachelian A, Matthay KK. Phase I Study of Vorinostat as a Radiation Sensitizer with 131I-Metaiodobenzylguanidine (131I-MIBG) for Patients with Relapsed or Refractory Neuroblastoma. Clin Cancer Res. 2015; 21:2715-21.
- 26. Emoto C, Fukuda T, Mizuno T, Cox S, Schniedewind B, Christians U, Widemann BC, Fisher MJ, Weiss B, Perentesis J, Vinks AA. Age-dependent changes in sirolimus metabolite formation in patients with neurofibromatosis type 1. *Ther Drug Monit*. 2015; 37:395-9.
- 27. Evelyn CR, Biesiada J, Duan X, Tang H, Shang X, Papoian R, Seibel WL, Nelson S, Meller J, Zheng Y. Combined

- Rational Design and a High Throughput Screening Platform for Identifying Chemical Inhibitors of a Rasactivating Enzyme. *J Biol Chem.* 2015; 290:12879-98.
- 28. Evelyn CR, Duan X, Biesiada J, Seibel WL, Meller J, Zheng Y. Rational design of small molecule inhibitors targeting the Ras GEF, SOS1. Chem Biol. 2014; 21:1618-28.
- 29. Fernandes VM, Fargo JH, Saini S, Guerrera MF, Marcus L, Luchtman-Jones L, Adams D, Meier ER. **Kaposiform lymphangiomatosis: unifying features of a heterogeneous disorder**. *Pediatr Blood Cancer*. 2015; 62:901-4.
- 30. Ferrer FA, Herbst KW, Fernandez CV, Khanna G, Dome JS, Naranjo A, Mullen EA, Geller JI, Gratias EJ, Shamberger R, Ritchey M, Ehrlich PF. Feasibility of using CT volume as a predictor of specimen weight in a subgroup of patients with low risk Wilms tumors registered on COG Study AREN03B2: implications for central venous catheter placement. *J Pediatr Urol*. 2014; 10:969-73.
- 31. Fouladi M, Perentesis JP, Phillips CL, Leary S, Reid JM, McGovern RM, Ingle AM, Ahern CH, Ames MM, Houghton P, Doyle LA, Weigel B, Blaney SM. A phase I trial of MK-2206 in children with refractory malignancies: a Children's Oncology Group study. *Pediatr Blood Cancer*. 2014; 61:1246-51.
- 32. Fouladi M, Perentesis JP, Wagner LM, Vinks AA, Reid JM, Ahern C, Thomas G, Mercer CA, Krueger DA, Houghton PJ, Doyle LA, Chen H, Weigel B, Blaney SM. A Phase I Study of Cixutumumab (IMC-A12) in Combination with Temsirolimus (CCI-779) in Children with Recurrent Solid Tumors: A Children's Oncology Group Phase I Consortium Report. Clin Cancer Res. 2015; 21:1558-65.
- 33. Gass D, Dewire M, Chow L, Rose SR, Lawson S, Stevenson C, Pai AL, Jones B, Sutton M, Lane A, Pruitt D, Fouladi M, Hummel TR. Pediatric tectal plate gliomas: a review of clinical outcomes, endocrinopathies, and neuropsychological sequelae. *J Neurooncol*. 2015; 122:169-77.
- 34. Grasso CS, Tang Y, Truffaux N, Berlow NE, Liu L, Debily MA, Quist MJ, Davis LE, Huang EC, Woo PJ, Ponnuswami A, Chen S, Johung TB, Sun W, Kogiso M, Du Y, Qi L, Huang Y, Hutt-Cabezas M, Warren KE, Le Dret L, Meltzer PS, Mao H, Quezado M, van Vuurden DG, Abraham J, Fouladi M, Svalina MN, Wang N, Hawkins C, Nazarian J, Alonso MM, Raabe EH, Hulleman E, Spellman PT, Li XN, Keller C, Pal R, Grill J, Monje M. Functionally defined therapeutic targets in diffuse intrinsic pontine glioma. *Nat Med*. 2015; 21:555-9.
- Hanmod SS, Wang G, Edwards H, Buck SA, Ge Y, Taub JW, Wang Z. Targeting histone deacetylases (HDACs) and Wee1 for treating high-risk neuroblastoma. Pediatr Blood Cancer. 2015; 62:52-9.
- 36. Hoffman LM, Fouladi M. Towards risk-adapted therapy for rhabdoid tumour subgroups. *Lancet Oncol*. 2015; 16:486-8.
- 37. Hoffman LM, Salloum R, Fouladi M. Molecular biology of pediatric brain tumors and impact on novel therapies. *Curr Neurol Neurosci Rep.* 2015; 15:10.
- 38. Horton TM, Perentesis JP, Gamis AS, Alonzo TA, Gerbing RB, Ballard J, Adlard K, Howard DS, Smith FO, Jenkins G, Kelder A, Schuurhuis GJ, Moscow JA. A Phase 2 study of bortezomib combined with either idarubicin/cytarabine or cytarabine/etoposide in children with relapsed, refractory or secondary acute myeloid leukemia: a report from the Children's Oncology Group. Pediatr Blood Cancer. 2014; 61:1754-60.
- 39. Iyer R, Croucher JL, Chorny M, Mangino JL, Alferiev IS, Levy RJ, Kolla V, Brodeur GM. Nanoparticle delivery of an SN38 conjugate is more effective than irinotecan in a mouse model of neuroblastoma. Cancer Lett. 2015; 360:205-12.
- 40. James MA, Seibel WL, Kupert E, Hu XX, Potharla VY, Anderson MW. A novel, soluble compound, C25, sensitizes to

TRAIL-induced apoptosis through upregulation of DR5 expression. Anticancer Drugs. 2015; 26:518-30.

- 41. Jeng MR, Fuh B, Blatt J, Gupta A, Merrow AC, Hammill A, Adams D. Malignant transformation of infantile hemangioma to angiosarcoma: response to chemotherapy with bevacizumab. *Pediatr Blood Cancer*. 2014; 61:2115-7.
- 42. Johnston D, Gerbing R, Alonzo T, Aplenc R, Nagarajan R, Schulte F, Cullen P, Sung L. Patient-Reported Outcome Coordinator Did Not Improve Quality of Life Assessment Response Rates: A Report from the Children's Oncology Group. *PLoS One*. 2015; 10:e0125290.
- 43. Kesselheim JC, Atlas M, Adams D, Aygun B, Barfield R, Eisenman K, Fulbright J, Garvey K, Kersun L, Nageswara Rao A, Reilly A, Sharma M, Shereck E, Wang M, Watt T, Leavey P. Humanism and professionalism education for pediatric hematology-oncology fellows: A model for pediatric subspecialty training. *Pediatr Blood Cancer*. 2014; 62:335–340.
- 44. Kilburn LB, Kocak M, Decker RL, Wetmore C, Chintagumpala M, Su J, Goldman S, Banerjee A, Gilbertson R, Fouladi M, Kun L, Boyett JM, Blaney SM. A phase 1 and pharmacokinetic study of enzastaurin in pediatric patients with refractory primary central nervous system tumors: a pediatric brain tumor consortium study. *Neuro Oncol.* 2015; 17:303-11.
- 45. Lombardi AJ, Hoskins EE, Foglesong GD, Wikenheiser-Brokamp KA, Wiesmuller L, Hanenberg H, Andreassen PR, Jacobs AJ, Olson SB, Keeble WW, Hays LE, Wells SI. Acquisition of Relative Interstrand Crosslinker Resistance and PARP Inhibitor Sensitivity in Fanconi Anemia Head and Neck Cancers. Clin Cancer Res. 2015; 21:1962-72.
- 46. Lombardi AJ, Sutton ME, Tiao GM, Geller JI. Vincristine-associated neurological morbidity in the treatment of hepatoblastoma. *J Pediatr Hematol Oncol.* 2015; 37:e258-63.
- 47. Margol AS, Robison NJ, Gnanachandran J, Hung LT, Kennedy RJ, Vali M, Dhall G, Finlay JL, Erdreich-Epstein A, Krieger MD, Drissi R, Fouladi M, Gilles FH, Judkins AR, Sposto R, Asgharzadeh S. Tumor-associated macrophages in SHH subgroup of medulloblastomas. *Clin Cancer Res.* 2015; 21:1457-65.
- 48. Maschietto M, Charlton J, Perotti D, Radice P, Geller JI, Pritchard-Jones K, Weeks M. The IGF signalling pathway in Wilms tumours--a report from the ENCCA Renal Tumours Biology-driven drug development workshop.

 Oncotarget. 2014; 5:8014-26.
- 49. Merrow AC, Gupta A, Adams DM. Additional imaging features of intramuscular capillary-type hemangioma: the importance of ultrasound. *Pediatr Radiol*. 2014; 44:1472-4.
- 50. Ni Y, Wright J, Perentesis J, Lingren T, Deleger L, Kaiser M, Kohane I, Solti I. Increasing the efficiency of trial-patient matching: automated clinical trial eligibility pre-screening for pediatric oncology patients. *BMC Med Inform Decis Mak*. 2015; 15:28.
- 51. Nikanjam M, Stewart CF, Takimoto CH, Synold TW, Beaty O, Fouladi M, Capparelli EV. Population pharmacokinetic analysis of oxaliplatin in adults and children identifies important covariates for dosing. *Cancer Chemother Pharmacol.* 2015; 75:495-503.
- 52. Packer RJ, Rood BR, Turner DC, Stewart CF, Fisher M, Smith C, Young-Pouissant T, Goldman S, Lulla R, Banerjee A, Pollack I, Kun L, Onar-Thomas A, Wu S, Boyett JM, Fouladi M. Phase I and pharmacokinetic trial of PTC299 in pediatric patients with refractory or recurrent central nervous system tumors: a PBTC study. *J Neurooncol*. 2015; 121:217-24.
- 53. Patil SA, Pfeffer SR, Seibel WL, Pfeffer LM, Miller DD. Identification of imidazoquinoline derivatives as potent

- antiglioma agents. Med Chem. 2015; 11:400-6.
- 54. Plotkin SR, Albers AC, Babovic-Vuksanovic D, Blakeley JO, Breakefield XO, Dunn CM, Evans DG, Fisher MJ, Friedman JM, Giovannini M, Gutmann DH, Kalamarides M, McClatchey Al, Messiaen L, Morrison H, Parkinson DB, Stemmer-Rachamimov AO, Van Raamsdonk CD, Riccardi VM, Rosser T, Schindeler A, Smith MJ, Stevenson DA, Ullrich NJ, van der Vaart T, Weiss B, Widemann BC, Zhu Y, Bakker AC, Lloyd AC. Update from the 2013 International Neurofibromatosis Conference. *Am J Med Genet A*. 2014; 164A:2969-78.
- 55. Pooya S, Liu X, Kumar VB, Anderson J, Imai F, Zhang W, Ciraolo G, Ratner N, Setchell KD, Yoshida Y, Jankowski MP, Dasgupta B. The tumour suppressor LKB1 regulates myelination through mitochondrial metabolism. *Nat Commun*. 2014; 5:4993.
- 56. Privette Vinnedge LM, Benight NM, Wagh PK, Pease NA, Nashu MA, Serrano-Lopez J, Adams AK, Cancelas JA, Waltz SE, Wells SI. The DEK oncogene promotes cellular proliferation through paracrine Wnt signaling in Ron receptor-positive breast cancers. *Oncogene*. 2015; 34:2325-36.
- 57. Salloum R, DeWire M, Lane A, Goldman S, Hummel T, Chow L, Miles L, Sutton M, Stevenson C, Fouladi M, Leach J. Patterns of progression in pediatric patients with high-grade glioma or diffuse intrinsic pontine glioma treated with Bevacizumab-based therapy at diagnosis. *J Neurooncol*. 2015; 121:591-8.
- 58. Salloum R, Garrison A, von Allmen D, Sheridan R, Towbin AJ, Adams D, Weiss B. Relapsed perinatal neuroblastoma after expectant observation. *Pediatr Blood Cancer*. 2015; 62:160-2.
- 59. Sauter SL, Wells SI, Zhang X, Hoskins EE, Davies SM, Myers KC, Mueller R, Panicker G, Unger ER, Sivaprasad U, Brown DR, Mehta PA, Butsch Kovacic M. **Oral human papillomavirus is common in individuals with Fanconi anemia**. *Cancer Epidemiol Biomarkers Prev.* 2015; 24:864-72.
- 60. Servaes S, Khanna G, Naranjo A, Geller JI, Ehrlich PF, Gow KW, Perlman EJ, Dome JS, Gratias E, Mullen EA.

 Comparison of diagnostic performance of CT and MRI for abdominal staging of pediatric renal tumors: a report from the Children's Oncology Group. *Pediatr Radiol.* 2015; 45:166-72.
- 61. Srivastava RK, Kaylani SZ, Edrees N, Li C, Talwelkar SS, Xu J, Palle K, Pressey JG, Athar M. **GLI inhibitor GANT-61** diminishes embryonal and alveolar rhabdomyosarcoma growth by inhibiting Shh/AKT-mTOR axis. *Oncotarget*. 2014; 5:12151-65.
- 62. Su JM, Thompson P, Adesina A, Li XN, Kilburn L, Onar-Thomas A, Kocak M, Chyla B, McKeegan E, Warren KE, Goldman S, Pollack IF, Fouladi M, Chen A, Giranda V, Boyett J, Kun L, Blaney SM. A phase I trial of veliparib (ABT-888) and temozolomide in children with recurrent CNS tumors: a pediatric brain tumor consortium report. *Neuro Oncol*. 2014; 16:1661-8.
- 63. Thomas TO, Chandrakasan S, O'Brien M, Jefferies JL, Ryan TD, Wilmot I, Baker ML, Madueme PC, Morales D, Lorts A. The use of a Berlin Heart EXCOR LVAD in a child receiving chemotherapy for Castleman's disease. *Pediatr Transplant*. 2015; 19:E15-8.
- 64. Wagner LM, Fouladi M, Ahmed A, Krailo MD, Weigel B, DuBois SG, Doyle LA, Chen H, Blaney SM. Phase II study of cixutumumab in combination with temsirolimus in pediatric patients and young adults with recurrent or refractory sarcoma: a report from the Children's Oncology Group. *Pediatr Blood Cancer*. 2015; 62:440-4.
- 65. Weiss B, Widemann BC, Wolters P, Dombi E, Vinks A, Cantor A, Perentesis J, Schorry E, Ullrich N, Gutmann DH, Tonsgard J, Viskochil D, Korf B, Packer RJ, Fisher MJ. Sirolimus for progressive neurofibromatosis type 1-associated plexiform neurofibromas: a neurofibromatosis Clinical Trials Consortium phase II study. *Neuro*

Oncol. 2015; 17:596-603.

- 66. Whelan JS, Bielack SS, Marina N, Smeland S, Jovic G, Hook JM, Krailo M, Anninga J, Butterfass-Bahloul T, Bohling T, Calaminus G, Capra M, Deffenbaugh C, Dhooge C, Eriksson M, Flanagan AM, Gelderblom H, Goorin A, Gorlick R, Gosheger G, Grimer RJ, Hall KS, Helmke K, Hogendoorn PC, Jundt G, Kager L, Kuehne T, Lau CC, Letson GD, Meyer J, Meyers PA, Morris C, Mottl H, Nadel H, Nagarajan R, Randall RL, Schomberg P, Schwarz R, Teot LA, Sydes MR, Bernstein M, collaborators E. **EURAMOS-1**, an international randomised study for osteosarcoma: results from pre-randomisation treatment. *Ann Oncol*. 2015; 26:407-14.
- 67. Whitlow PG, Caparas M, Cullen P, Trask C, Schulte F, Embry L, Nagarajan R, Johnston DL, Sung L. Strategies to improve success of pediatric cancer cooperative group quality of life studies: a report from the Children's Oncology Group. Qual Life Res. 2015; 24:1297-301.
- 68. Yanik GA, Villablanca JG, Maris JM, Weiss B, Groshen S, Marachelian A, Park JR, Tsao-Wei D, Hawkins R, Shulkin BL, Jackson H, Goodarzian F, Shimada H, Courtier J, Hutchinson R, Haas-Koga D, Hasenauer CB, Czarnecki S, Katzenstein HM, Matthay KK. 131I-metaiodobenzylguanidine with intensive chemotherapy and autologous stem cell transplantation for high-risk neuroblastoma. A new approaches to neuroblastoma therapy (NANT) phase II study. Biol Blood Marrow Transplant. 2015; 21:673-81.
- 69. Zhang J, Dong Z, Mundla SR, Hu XE, Seibel W, Papoian R, Palczewski K, Golczak M. Expansion of first-in-class drug candidates that sequester toxic all-trans-retinal and prevent light-induced retinal degeneration. *Mol Pharmacol*. 2015; 87:477-91.
- 70. Zhang S, Konstantinidis DG, Yang JQ, Mizukawa B, Kalim K, Lang RA, Kalfa TA, Zheng Y, Guo F. **Gene targeting RhoA reveals its essential role in coordinating mitochondrial function and thymocyte development**. *J Immunol*. 2014; 193:5973-82.
- 71. Zhukova N, Ramaswamy V, Remke M, Martin DC, Castelo-Branco P, Zhang CH, Fraser M, Tse K, Poon R, Shih DJ, Baskin B, Ray PN, Bouffet E, Dirks P, von Bueren AO, Pfaff E, Korshunov A, Jones DT, Northcott PA, Kool M, Pugh TJ, Pomeroy SL, Cho YJ, Pietsch T, Gessi M, Rutkowski S, Bognar L, Cho BK, Eberhart CG, Conter CF, Fouladi M, French PJ, Grajkowska WA, Gupta N, Hauser P, Jabado N, Vasiljevic A, Jung S, Kim SK, Klekner A, Kumabe T, Lach B, Leonard JR, Liau LM, Massimi L, Pollack IF, Ra YS, Rubin JB, Van Meir EG, Wang KC, Weiss WA, Zitterbart K, Bristow RG, Alman B, Hawkins CE, Malkin D, Clifford SC, Pfister SM, Taylor MD, Tabori U. WNT activation by lithium abrogates TP53 mutation associated radiation resistance in medulloblastoma. *Acta Neuropathol Commun*. 2014; 2:174.
- 72. Zukotynski K, Fahey F, Kocak M, Kun L, Boyett J, Fouladi M, Vajapeyam S, Treves T, Poussaint TY. **18F-FDG PET** and MR imaging associations across a spectrum of pediatric brain tumors: a report from the pediatric brain tumor consortium. *J Nucl Med*. 2014; 55:1473-80.

Faculty, Staff, and Trainees

Faculty Members

John Perentesis, MD, FAAP, Professor

Leadership Deb Kleisinger Endowed Chair of Novel Cancer Treatments; Executive Co-Director, Cancer and Blood Diseases Institute; Director, Division of Oncology; Director, Leukemia/Lymphoma Program; Cincinnati Children's Principal Investigator, Children's Oncology Group (COG); Cincinnati Children's Principal Investigator, National Cancer Institute Pediatric Phase I Consortium

Research Interests Novel therapeutic approaches to AML and NHL; New anticancer drug development; molecular oncogenesis and pharmacogenetics in high risk leukemia, lymphoma and pediatric cancers

Michael Absalon, MD, PhD, Assistant Professor

Leadership Director, Medical Education Program; Associate Director, Leukemia/Lymphoma Program

Research Interests New therapeutics; relapsed leukemia and lymphoma, post-transplant lymphoproliferative disease, T-cell lymphoma

Denise M. Adams, MD, Professor

Leadership Marjory J. Johnson Chair Vascular Tumor Translational Research; Medical Director, Comprehensive Hemangiomas and Vascular Malformation Clinic; Director, Hematology/Oncology Fellowship Program

Research Interests Development of clinical trials for complicated vascular anomaly phenotypes; Angiogenesis, endothelial cell proliferation, vascular anomalies, mTOR inhibition as a therapeutic approach to complex vascular anomalies

Karen Burns, MD, Assistant Professor

Leadership Director, Cancer Survivorship Center

Research Interests Childhood cancer survival; fertility preservation and outcomes; adolescent and young adult outcomes and quality of life; leukemia/lymphoma therapy

Lionel Chow, MD, PhD, Assistant Professor

Leadership St. Baldrick's Foundation Scholar

Research Interests Mechanisms of growth of high-grade astrocytoma and their treatment with novel molecularly targeted inhibitors. In particular, early events of tumorigenesis and in overcoming therapeutic resistance.

Biplab Dasgupta, PhD, Associate Professor

Research Interests Role of AMPK in brain development and function; Metabolic reprograming in glioblastoma

Jennifer Davis, DO, Instructor

Leadership St. Baldrick's Foundation Fellow

Research Interests Lymphatics/lymphangiogenesis; use of zebrafish to develop animal models to identify new genetic targets for novel therapies to treat rare vascular tumors and metastatic disease for which currently there is no cure

Mariko DeWire, MD, Assistant Professor

Research Interests Developmental therapeutics, clinical trial development and quality of life in pediatric patients diagnosed with brain tumors, especially high grade gliomas and diffuse intrinsic pontine glioma

Rachid Drissi, PhD, Assistant Professor

Research Interests Role of telomeres, telomerase and epigenetic alterations in pediatric brain tumors.

Maryam Fouladi, MD, MSc, Professor

Leadership Marjory J. Johnson Chair of Brain Tumor Translational Research; Medical Director, Neuro-Oncology Program; Cincinnati Children's Principal Investigator, Collaborative Ependymoma Research Network (CERN)

Research Interests Early Phase I trials and molecular therapeutics in oncology

James I. Geller, MD, Associate Professor

Leadership Medical Director, Kidney and Liver Tumors Program; Co-Medical Director, Retinoblastoma Program; Associate Director, Global Cancer Programs

Research Interests Development of novel therapy options for infants, children, adolescents and young adults with solid tumors, with a specific focus in kidney, liver and retinal tumors.

Adrienne Hammill, MD, PhD, Assistant Professor

Research Interests Vascular malformations, in particular, Cerebrovascular Diseases (HHT, SWS, CCM)

Santosh Hanmod, MD, Instructor

Sara Helmig, MD, Instructor

Trent Hummel, MD, Assistant Professor

Research Interests Developing novel therapeutics to treat children with central nervous system tumors including those with very poor prognosis tumors such as high-grade gliomas and diffuse intrinsic pontine gliomas

Beatrice Lampkin, MD, Professor Emerita

Research Interests Blood and bone marrow morphology and the significance thereof in relationship to patients' case histories

Jennifer Mangino, MD, Assistant Professor

Research Interests Developing trials to bring new therapies to patients with relapsed/refractory cancer (particularly adolescent and young adult patients) and maximizing our supportive care practices (particularly surrounding nutrition and exercise)

Benjamin Mizukawa, MD, Assistant Professor

Research Interests Genetic mutations and signaling protein alterations involved in leukemia initiation and progression; use of animal models of human leukemia to predict response and resistance to novel targeted therapies

Rajaram Nagarajan, MD, Assistant Professor

Leadership Associate Director, Oncology Clinical Operations; Director of Cancer Control and Outcomes Research, Cancer Survivorship Center

Research Interests quality of life, late effects of cancer therapy and outcomes research; bone sarcoma treatments and outcomes

Maureen O'Brien, MD, Associate Professor

Leadership Associate Director, Leukemia/Lymphoma Program

Research Interests Development and clinical evaluation of novel targeted therapies for children and adolescents with relapsed and refractory acute leukemias

Christine Phillips, MD, Assistant Professor

Research Interests Leukemia and lymphoma, including leukemia pharmacogenetics and translational and clinical development of new drugs for high-risk and relapsed leukemias

Joseph Pressey, MD, Associate Professor

Leadership Director, Musculoskeletal Tumor Program; Program Leader, Young Adult Oncology Center **Research Interests** Development of therapies for high-risk sarcomas

Lisa Privette-Vinnedge, PhD, Assistant Professor

Leadership Director, Office of Postdoctoral Affairs

Research Interests Elucidating the molecular mechanisms through which the DEK oncogene drives breast cancer progression; investigation of clinical use of DEK as a cancer biomarker and therapeutic target; pre-clinical testing of novel cancer therapeutics

William Seibel, PhD, Assistant Professor

Leadership Project Manager, Drug Discovery; Head of Compound Library Services

Research Interests Use of modeling, cheminformatics and screening to identify new drug leads against novel therapeutic targets

Rachana Shah, MD, Instructor

Research Interests Developmental therapeutics with special focus on immunotherapy/immune checkpoint inhibitors; High Intensity Focused Ultrasound (HIFU)

Brian Turpin, DO, Assistant Professor

Research Interests Genomic alterations in childhood and adolescent/young adult tumors, and the development of novel therapeutics with a particular focus in the use of genomic-alteration guided pharmaceuticals and immunotherapy in sarcomas

Brian D. Weiss, MD, Associate Professor

Leadership Associate Director for Safety and Compliance, Cancer and Blood Diseases Institute; Medical Director,

Neuroblastoma Program; Cincinnati Children's Principal Investigator, New Approaches to Neuroblastoma Therapy Consortium (NANT); Associate Director of Clinical Research, Division of Oncology

Research Interests Targeted therapies for neuroblastoma and NF1-related tumors, and novel methods to improve the quality and safety of cancer care in children

Susanne Wells, PhD, Professor

Leadership Director, Epithelial Carcinogenesis and Stem Cell Program

Research Interests Reprogramming the DNA damage response through oncogenes and tumor suppressors

Jordan Wright, MD, Instructor

Joint Appointment Faculty Members

Ahna Pai, PhD, Associate Professor (Adherence Psychology)

Saulius Sumanas, PhD, Assistant Professor (Developmental Biology)

Mary Sutton, MD, Associate Professor (Neurology)

Rachel Thienprayoon, MD, Assistant Professor (Anesthesia)

Jonathan Tolentino, MD, Assistant Professor (UC Internal Medicine)

Clinical Staff Members

- Faiza Ali, MD
- Carina Braeutigam, MD
- Carol Chute, CNP
- Sarah Fitzgerald, MD
- Cai Ling Wang, MD

Trainees

- Andrew Bukowinski, MD, PL-IV, Children's Hospital of Pittsburg, PA
- Rishi Raj Chhipa, PhD, PGY, Roswell Park Cancer Center
- Timothy Chlon, PhD, PGY, Northwestern University
- Michelle Glaunert, , PGY, Perdue University
- Lindsey Hoffman, DO, PL-VII, Children's Hospital Colorado
- Neelum Jeste, MD, PL-IV, Washington University/St. Louis Children's Hospital
- Anne Lombardi, MD, PL-VII, Baylor College of Medicine
- Dana Lounder, MD, PL-IV, University of Pittsburgh Medical Center
- Marie Matrka, PhD, PGY
- Samantha Michaels, MD, PL-IV, University of Kentucky
- Ranjithmenon Muraleedharan, PhD, PGY, University of Nottingham, UK
- Nicole Oatman, BS, PGY, University of Arizona

- Annmarie Ramkisson, PhD, PGY, University of Toronto
- Sonya Ruiz Torres, , PGY
- Ralph Salloum, MD, PLVIII, Detroit Medical Center/Wayne State University
- Eric Smith, , PGY
- Angela White, , PGY, North Carolina Wesleyan College

Grants, Contracts, and Industry Agreements

Grant and Contract Awards Annual Direct

Absalon, M

A Phase I Study Using Plerixafor as a Chemosensitizing Agent for Relapsed Acute Leukemia and MDS in Pediatric Patients

Children's HealthCare of Atlanta, Inc.

8/1/2011-7/31/2014

\$2,582

Adams, D

Phase II: Vincristine vs. Sirolimus for High Risk Kaposiform Hemangioendothelioma

Food and Drug Administration

R01 FD004363 9/10/2014-8/31/2018 \$264,320

Chow, L

Using a Novel Mouse Model to Develop an Effective Therapy for Angiosarcoma

Cancer Free Kids

7/1/2014-6/30/2015 \$50,000

Molecular Targeting of Pediatric High-Grade Glioma

St. Baldrick's Foundation

7/1/2014-6/30/2016 \$115,000

Molecular Targeting of High-Grade Astrocytoma

The Sontag Foundation

10/1/2011-9/30/2015 \$130,435

DasGupta, B

Regulation of Forebrain Neurogenesis by the Energy Sensor AMP Kinase

R01 NS072591	7/1/2012-6/30/2017	\$216,562
Novel Combinatorial Therapeutics in G	Blioblastoma	
University of Cincinnati		
	7/1/2014-6/30/2015	\$60,000
DeWire, M		
Health Related Quality of Life in Pediat	ric DNS Malignancies: A Feasibility Study Utilizing	PROMIS
Alex's Lemonade Stand Foundation		
	9/2/2014-3/1/2015	\$20,000
Drissi, R		
Epigenetic Alterations as Therapeutic	Targets in Pediatric Brain Tumors	
The Cure Starts Now Foundation		
	5/1/2015-4/30/2016	\$100,000
Fouladi, M		
Pediatric Brain Tumor Consortium (PE	STC)	
National Institutes of Health(St Jude's Cl	nildren's Hospital)	
UM1 CA081457	4/1/2014-3/31/2019	\$120,463
Pediatric Brain Tumor Consortium (PE	BTC) - per patient	
National Institutes of Health(St Jude's Cl	nildren's Hospital)	
UM1 CA081457	4/1/2014-3/31/2019	\$48,500
COG NCTN Network Group Operations	Center	
Children's Hospital of Philadelphia		
U10 CA180886	4/11/2014-2/28/2019	\$12,073
Establishment of an International Diffu	se Intrinsic Pontine Glioma (DIPG) Registry	
The Cure Starts Now Foundation		
	1/1/2012-12/31/2015	\$176,186
Hammill, A		

National Institutes of Health(The Regents of the University of California)

U54 NS065705	9/30/2014-7/31/2019	\$1,350
Irwin, M		
Pet Center Research Study		
CancerFree Kids		
	07/01/2014-06/30/2015	\$10,000
Mizukawa, B		
Targeted Inhibition of Cdc42 G	TPase in the Acute Myeloid Leukemia Stem Cell	
St. Baldrick's Foundation		
	7/1/2013-6/30/2016	\$110,000
Perentesis, J		
COG Phase 1 & Pilot Consortiu	ım studies	
Cookies for Kids' Cancer(Childre	en's Hospital of Philadelphia)	
	5/1/2014-4/30/2017	\$23,100
Childrens' Oncology Group Ch	air Award - Per Patient	
National Institutes of Health(Chi	ldren's Hospital of Philadelphia)	
U10 CA098543	3/1/2012-2/28/2017	\$2,499
Community Clinical Oncology	Program Research Base	
National Institutes of Health(Chi	ldren's Hospital of Philadelphia)	
U10 CA095861	7/24/2012-5/31/2016	\$96
COG Phase 1 & Pilot Consortiu	ım studies per patient	
National Institutes of Health(Chi	ldren's Hospital of Philadelphia)	
UM1 CA097452	7/24/2012-07/31/2017	\$54,894
COG NCTN Network Group Op	erations Center - Per Case Reimbursement	
National Institutes of Health(Chi	ldren's Hospital of Philadelphia)	
U10 CA180886	4/11/2014-2/28/2019	\$120,371
COG St. Baldrick Supplementa	I Reimbursement	
St. Baldrick's Foundation(Childre	en's Hospital of Philadelphia)	

3/1/2012-2/28/2017

\$40,040

Weiss, B		
Neuroblastoma Biology Study		
National Institutes of Health(Children's	Hospital Los Angeles)	
P50 CA081403	9/1/2011-8/31/2014	\$6,931
Randomized Phase II study of 131 I-M	IIBG vs 131 I-MIBG with Vincristine and Ironotecan	
National Institutes of Health(Children's	Hospital Los Angeles)	
R01 CA172067	4/4/2013-3/31/2018	\$14,946
COG NCTN Network Group Operation	s Center	
National Institutes of Health(Children's	Hospital of Philadelphia)	
U10 CA098543	4/11/2014-2/28/2019	\$11,550
Wells, S		
Metabolic Predictors of Childhood Ca	ncer Development and Outcome	
Hyundai Hope on Wheels		
	10/1/2014-9/30/2016	\$125,000
Role and Regulaton of the Human DE	K Proto-Oncogene	
National Institutes of Health		
R01 CA116316	9/5/2012-6/30/2017	\$169,088
Williams, J		
St. Baldrick's Foundation 3rd Year Fe	llowship Award	
St. Baldrick's Foundation		
	7/1/2014-6/30/2015	\$95,250
	Current Year Direct	\$2,101,230
Industry Contracts		
Adams, D		
Pierre Fabre Pharmaceuticals, Inc		\$1,694

\$16,230

\$3,850

Fouladi, M

Novartis Pharmaceuticals

Pfizer, Inc./St. Jude's Children's Hospital

Gel	ler.	J

Total	\$2,400,991
Current Year Direct Receipts	\$299,755
Wells, S Bacterial Robotics, LLC	\$1,925
Novartis Pharmaceuticals	\$16,610
Weiss, B	
Turpin, B Sarcoma Alliance for Research through Collaboration	\$1,200
Roche Laboratories Inc / CHOP	\$11,550
Pfizer, Inc. / CHOP	\$10,010
Novartis Pharmaceuticals / CHOP	\$4,620
Morphotek Inc / CHOP	\$20,532
Millennium Pharmaceuticals, Inc. / CHOP	\$4,620
GlaxoSmithKline / CHOP	\$4,004
Eisai, Inc. / CHOP	\$3,080
Perentesis, J Amgen, Inc.	\$6,041
Porentacia I	
Novartis	\$4,820
Amgen, Inc.	\$15,561
Epizyme, Inc.	\$22,391
O'Brien, M Seattle Genetics, Inc.	\$104,323
Hummel, T GlaxoSmithKline	\$16,957
Novartis Pharmaceuticals	\$29,737