



2018 Annual Report

Division of Hematology, Oncology and Bone Marrow Transplant

DIVISION DESCRIPTION

The Division of Hematology, Oncology and Bone Marrow Transplant provides state-of-the-art integrated care, utilizing conventional treatments (such as chemotherapy, radiation and surgery), cellular therapies, molecular- and radioisotope-targeted therapies and stem cell transplant, aimed at curing malignancies and treating hematological disorders in children.

Research interests include ways to recognize and destroy cancer cells using immunologic, cellular and molecular biologic pathways, bone marrow transplantation as immunotherapy, CAR T-cell therapy, systemic targeted radiotherapy, cancer survivorship, neuro-oncology and palliative care.

2018 HIGHLIGHTS

- **Christian Capitini, MD**, and co-investigator Sean Fain, PhD (Medical Physics), received a five-year, \$1.75 million grant from the National Institutes of Health-National Cancer Institute (NIH-NCI) for their project, "Combining hu14.18-IL2 and NK Cell Infusions to Treat Neuroblastoma." This project will use murine allogeneic hematopoietic stem cell transplant (alloHSCT) models to develop evidence for a clinically applicable combined strategy that utilizes the immunocytokine hu14.18-IL2 to enhance the graft-versus-tumor effect of immunologically activated, ex-vivo activated natural killer (NK) cells, and to track the localization of these NK cells using a novel 19F-MRI platform.

Dr. Capitini and co-investigators Dr. Fain and **Paul Sondel, MD, PhD**, also received a four-year, \$792,000 Research Scholar Grant from the American Cancer Society (ACS), for the project, "Ex Vivo Activated NK Cells and Immunocytokine for Pediatric Cancers." This grant will provide preclinical evidence for a potential platform for incorporating recently developed immunotherapies, namely immunocytokine and natural killer (NK) cells, for treatment of neuroblastoma and osteosarcoma.

In addition, Dr. Capitini and Krishanu Saha, PhD (Biomedical Engineering), received a one-year \$50,000 grant from the UW Carbone Cancer Center (UWCCC) Leukemia Research Funding Opportunity for their project, "Optimization of CD19 CAR NK Cells for B Cell Leukemia." This project will compare the ability of natural killer (NK) cells to mediate anti-leukemia effects using a CD19 chimeric antigen receptor (CAR) versus CAR T cells.

Finally, Dr. Capitini was named the 2018 winner of the University of Wisconsin Department of Pediatrics Gerard B. Odell Research Award.

- Medical student Katharine Tippins (mentor: **Christian Capitini, MD**) received a four-month, \$5,000 Summer Student Fellowship from St. Baldrick's Foundation for her project, "Generating an NK-mediated Graft-Versus-Tumor Effect Against Osteosarcoma."

- A new phase I clinical trial led by **Kenneth DeSantes, MD**, and **Paul Sondel, MD, PhD**, that is the first to combine natural killer (NK) cell therapy with an immunocytokine to target children with relapsed/refractory neuroblastoma, including those with bulky tumors, is now open and recruiting at American Family Children's Hospital. A \$136,000 grant from Solving Kids' Cancer, The Catherine Elizabeth Blair Memorial Foundation, and Wade's Army is supporting the novel, "first in human" immunotherapy.
- At its residency graduation ceremony, the University of Wisconsin Department of Psychiatry honored **Carol Diamond, MD**, for supervising first-year psychiatry residents on their Pediatrics Hematology/Oncology rotation for nearly 20 years.
- **Mario Otto, MD, PhD**, received a one-year, \$100,000 research award from the St. Baldrick's Foundation for project, "Improving Anti-Cancer Immune Responses to Targeted Radionuclide Therapy," which will combine molecular-targeted radiotherapy with immunomodulatory agents to facilitate radiation-damage induced anti-tumor immune responses.

Sean Rinella, MPH, a research assistant in Dr. Otto's lab, received a two-year grant from the UW Institute for Clinical and Translational Research (ICTR) Predoctoral TL1 Program to support his project, "Development of pre-clinical models and clinical applications for T cell receptor alpha beta depleted haploidentical stem cell transplant."

- **Paul Sondel, MD, PhD**, received a U54 subaward from the Children's Hospital of Philadelphia and the National Institutes of Health/National Cancer Institute (NIH/NCI). The subaward, which will provide ~\$2.1 million to UW-Madison over five years, is part of a \$12.1 multi-institutional consortium, "Discovery and Development of Optimal Immunotherapeutic Strategies for Childhood Cancers," led by principal investigators at the Center for Childhood Cancer Research at Children's Hospital of Philadelphia and at Stanford University. Dr. Sondel and team members **Amy Erbe-Gurel, PhD**, **Jacquelyn Hank, PhD**, Zachary Morris, MD, PhD (Human Oncology), and **Alexander Rakhmievich, MD, PhD**, will lead Project 3, "Discovery and Development of Pediatric Cancer Antigen Targets Recognized by Adaptive Immune Response," and support two additional projects of the cooperative agreement.

In addition, Dr. Sondel and the University of Wisconsin Pediatric Dream Team received renewal funding through the Stand Up to Cancer-St. Baldrick's Pediatric Dream Team Translational Cancer Research Award, supported by the American Association for Cancer Research (AACR) and St. Baldrick's Foundation. This award provides \$250,000 per year to the University of Wisconsin Dream Team, for a four-year total of \$1,000,000. Key members of the Wisconsin team are: **Kenneth DeSantes, MD** (co-principal investigator); **Christian Capitini, MD**; **Mario Otto, MD, PhD**; **Inga Hofmann, MD**; Peiman Hematti, MD (Medicine); Jacques Galipeau, MD (Medicine); **Amy Erbe-Gurel, PhD**; **Jacquelyn Hank, PhD**; Alexander Rakhmievich, MD, PhD (Human Oncology); and **Kimberly McDowell, MD, PhD**.

Dr. Sondel is also a co-principal investigator, along with Jacques Galipeau, MD (Medicine) and collaborators Douglas McNeel, MD, PhD (Medicine), and David Beebe, PhD (Biomedical Engineering), on a new three-year, \$600,000 Collaborative Health Sciences Program grant from the Partnership Education and Research Committee (PERC) of the Wisconsin Partnership Program. This grant, "UW Innovations in Malignancy Personalized Advanced Cell Therapies (UW-IMPACT)," allows for collaboration between the three labs to generate data and examine the potential for the use of autologous B-cells for cancer immunotherapy, in combination with DNA vaccines and immunocytokines, for personalized cell therapies for otherwise incurable adult and pediatric malignancies, including prostate cancer and neuroblastoma.

Finally, Dr. Sondel received a one-year, \$100,000 Impact Grant from Hyundai Hope on Wheels to support his project “Innate and Adaptive Immunotherapy to Eradicate Immunologically Cold Neuroblastoma.” His team will develop combination immunotherapy regimens, activating both innate and adaptive immune responses, to eradicate immunologically cold neuroblastomas in mice in order to translate these regimens to potent anti-neuroblastoma clinical therapy.

- Medical student Claire Baniel (mentor: **Paul Sondel, MD, PhD**) received a one-year, \$43,000, 2018-2019 Medical Fellows Research Award from the Howard Hughes Medical Institute for her project, "Endogenous Antibody Response: Role in the Potentiation and Outcome of Combination Anti-Melanoma In Situ Immunotherapies."
- Graduate student **Peter Carlson** (mentors: **Paul Sondel, MD, PhD**, and Zachary Morris, MD, PhD [Human Oncology]) received a three-year, \$107,143 Ruth L. Kirschstein National Research Service Award Individual Predoctoral Fellowship (F30) for MD/PhD and Other Dual Degrees from the National Institutes of Health, National Cancer Institute (NIH-NCI) to support his project, "Molecular Targeted Radiotherapy to Overcome Resistance to In Situ Cancer Vaccination."
- Medical students Megan Gokey and Do Dang (mentor: **Paul Sondel, MD, PhD**) received Shapiro Summer Research Awards for the projects, “Enhancing Detection Sensitivity of Humanized Therapeutic Antibody in Patient Samples for Improved Pharmacokinetic Evaluation,” and “Effect of Cyclophosphamide on In Situ Vaccine-Induced Tumor Immunotherapy,” respectively.
- Graduate student Alex Pieper (mentor: **Paul Sondel, MD, PhD**) was appointed to the UW Institute for Clinical and Translational Research’s TL1 program. The award supports his research project, "Influence of Treatment Regimen and Tumor Type on Concomitant Immune Tolerance."

In 2018, *U.S. News and World Report* ranked UW Health's American Family Children's Hospital in the [top 50 children's hospitals for pediatric cancer care](#).

RECENT PUBLICATIONS

Albertini MR, Yang RK, Ranheim EA, Hank JA, Zuleger CL, Weber S, Neuman H, Hartig G, Weigel T, Mahvi D, Henry MB, Quale R, McFarland T, Gan J, Carmichael L, Kim K, Loibner H, Gillies SD, **Sondel PM**. Pilot trial of the hu14.18-IL2immunocytokine in patients with completely resectable recurrent stage III or stage IV melanoma. *Cancer Immunol Immunother*. 2018 Oct;67(10):1647-1658. doi: 10.1007/s00262-018-2223-z. Epub 2018 Aug 3. PubMed PMID: 30073390; PubMed Central PMCID: PMC6168354.

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*ePub only; no print citation available when report was compiled

** Publication had previously appeared in 2017 report as an ePub

GRANT SUPPORT

Principal Investigator	Sponsor	Title	Co-Investigators
Capitini, Christian Matthew	Midwest Athletes Against Childhood Cancer	Improving graft-versus- leukemia effects of ex vivo activated NK cells through JAK/STAT blockade	
Capitini, Christian Matthew	National Institutes of Health (NIH)	Inhibiting STAT1 as a novel graft-versus- host/graft-versus- leukemia therapy	
Capitini, Christian Matthew	Novartis Pharmaceuticals	Phase II study of redirected autologous t cells engineered to contain anti-CD19 attached to TCR and 4- 1BB signaling domains in patients with chemotherapy resistant or refractory acute lymphoblastic leukemia	
Capitini, Christian Matthew	Novartis Pharmaceuticals	CTL019B2202 - A phase II, single arm, multicenter trials to determine the efficacy and safety of CTL019 in pediatric patients with relapsed and refractory B-cell acute lymphoblastic leukemia	
Capitini, Christian Matthew	Novartis Pharmaceuticals	Long-term follow-up of patients exposed to lentiviral-based CD19 directed CART cell therapy	
Capitini, Christian Matthew	Hyundai Hope on Wheels	Anti-GD2 immunocytokine and NK cell infusions for neuroblastoma	
Capitini, Christian Matthew	St. Baldrick's Foundation	Developing MSC- derived exosomes to enhance HSCT for pediatric leukemia	
Capitini, Christian Matthew	National Institutes of Health (NIH)	Combining hu14.18-IL2 and NK cell infusions to treat neuroblastoma	

Capitini, Christian Matthew	American Cancer Society	Ex vivo activated NK cells and immunocytokine for pediatric cancers	HemOnc: Paul Sondel
Capitini, Christian Matthew	St. Baldrick's Foundation	Generating an NK- mediated graft-versus- tumor effect against osteosarcoma	
Capitini, Christian Matthew	UWF - University of Wisconsin Foundation	Optimizaiton of CD19 CAR NK cells for B cell leukemia	
Slukvin, Igor I (Primate Center)	National Institutes of Health (NIH)	CCR5-mutant monkey model to facilitate the development of novel stem cell-based therapies for AIDS	HemOnc: Christian Capitini
Chinnadurai, Raghavan (UW Carbone Cancer Center)	When Everyone Survives Foundation	IFN-gamma primed donor mesenchymal stromal cells to mitigate graft versus host disease	HemOnc: Christian Capitini
DeSantes, Kenneth B	Midwest Athletes Against Childhood Cancer	Support for research data management	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & Bristol- Myers Squibb Company	AALL-1122: 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	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & St. Baldrick's Foundation	St. Baldrick's supplemental per case reimbursement	
DeSantes, Kenneth B	Solving Kids Cancer	Phase I trial of ex-vivo expanded haploidentical NK cells and Hu14.18-IL2 for children with relapsed/refractory neuroblastoma	

DeSantes, Kenneth B	Children's Hospital of Philadelphia & Millenium Pharmaceuticals	ADVL0921 - A phase II study of MLN8237, a selective aurora a kinase inhibitor in children with recurrent/refractory solid tumors and leukemias	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & Hoffmann-LaRoche, Inc.	ANHL1131 - Intergroup trial for children or adolescents with B-cell NHL or B-AL: evaluation of Rituximab efficacy and safety in high risk patients	
DeSantes, Kenneth B	Children's Hospital of Philadelphia	AALL1131 - A phase III randomized trial for newly diagnosed high risk B-precursor acute lymphoblastic leukemia (ALL) testing Clofarabine in the very high risk stratum	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & National Institutes of Health (NIH)	COG CTSU phase II supplement	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & National Institutes of Health (NIH)	COG workload intensity	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & National Institutes of Health (NIH)	COG/NCTN per case reimbursement	
DeSantes, Kenneth B	Midwest Athletes Against Childhood Cancer	Treatment of relapsed or refractory neuroblastoma with ex-vivo activated and expanded haploidentical NK cells and continuous infusion Hu14.18-IL2	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & National Institutes of Health (NIH)	COG NCORP research base, per case reimbursement	

DeSantes, Kenneth B	Children's Hospital of Philadelphia & Seattle Genetics, Inc.	A randomized phase III study of brentuximab vedotin (IND#117117) for newly diagnosed classical hodgkin lymphoma (cHL) in children and adolescents	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & Bayer Corporation	AAML1031: A phase III randomized trial for patients with de novo AML using bortezomib and sorafenib for patients with high allelic ratio FLT3/ITD	
DeSantes, Kenneth B	Macrogenics	A phase 1, open-label, dose escalation study of MGA271 in pediatric patients with B7-H3-expressing relapsed or refractory solid tumors	
DeSantes, Kenneth B	Children's Hospital of Philadelphia & Children's Oncology Group	APEC14B1 - Everychild protocol: a registry, eligibility screening, biology and outcome study	
DeSantes, Kenneth B	University of California-San Francisco & National Institutes of Health (NIH)	Primary immune deficiency treatment consortium	
DeSantes, Kenneth B	Curing Kids Cancer	A phase 1, open-label, Dose escalation study of CLR 131 in Children and Adolescents with select solid tumors, lymphoma, and malignant brain tumors	HemOnc: Mario Otto
DeSantes, Kenneth B	Children's Hospital of Philadelphia & Novartis	AALL0434: Intensified methotrexate, nelarabine and augmented BFM therapy for children and young adults with newly diagnosed T-cell acute lymphoblastic leukemia or T-cell lymphoblastic lymphoma	

DeSantes, Kenneth B	Children's Hospital of Philadelphia & St. Baldrick's Foundation	ALTE15N2, LEHRN (late effects after high-risk neuroblastoma) study	
Desantes, Kenneth B	Children's Hospital of Philadelphia & Children's Oncology Group	APEC1621SC - Pediatric MATCH (molecular analysis for therapy choice) screening	
Hematti, Peiman (UW Department of Medicine)	Novartis Pharmaceuticals	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 UW14062	
Hofmann, Inga	Midwest Athletes Against Childhood Cancer	Prognostic markers and therapeutic targets in GATA2-related myelodysplastic syndromes and leukemia	
Hofmann, Inga	EvansMDS	Prognostic markers and therapeutic targets in GATA2-related myelodysplastic syndromes	
Hofmann, Inga	Children's Hospital of Boston	Radiation- and alkylator-free hematopoietic cell transplantation for bone marrow failure due to dyskeratosis congenita / telomere disease	
Hofmann, Inga	St. Baldrick's Foundation	Advanced cellular therapies for pediatric cancer/predisposition syndromes	
Hofmann, Inga	Novartis	Eltrombopag in pediatric patients with refractory, relapsed, or treatment naive severe aplastic anemia or recurrent aplastic anemia	

Otto, Mario	Midwest Athletes Against Childhood Cancer	Targeted molecular radiotherapy to improve the outcomes in children with malignant brain tumors	
Otto, Mario	Hyundai Hope on Wheels	A novel phospholipid ether analog to combine targeted molecular radiotherapy and immunotherapy in pediatric solid tumors	
Otto, Mario	Cannonball Kids Cancer Foundation	TCR-/+ and CD19+ depleted KIR/KIR ligand-mismatched haploidentical hematopoietic stem cell transplant and zoledronate for pediatric relapsed/refractory hematologic malignancies and high risk solid tumors	
Otto, Mario	UW Carbone Cancer Center & National Institutes of Health (NIH)	TCR $\alpha\beta$ + /CD19+ depleted HSCT + zoledronate for pediatric cancers	
Otto, Mario	Wisconsin Alumni Research Foundation	Travel grant	
Otto, Mario	St. Baldrick's Foundation	Improving anti-cancer immune responses to targeted radionuclide therapy	
Otto, Mario	Cellectar, LLC	A phase 1, open-label, dose escalation study of CLR 131 in children and adolescents with select solid tumors, lymphoma, and malignant brain tumors	
Otto, Mario	UWF - University of Wisconsin Foundation	2018 - The ride award	
Morris, Zachary (UW Department of Human Oncology)	National Institutes of Health (NIH)	Immunomodulation of the tumor microenvironment with molecular targeted radiotherapy to facilitate an adaptive anti-tumor immune response to combined modality immunotherapies	HemOnc: Mario Otto, Paul Sondel

Patel, Neha J	Boehringer Ingelheim, Ltd.	Phase I open label, dose escalation trial to determine the MTD, safety, PK and efficacy of afatinib monotherapy in children aged 2 years to <18 years with recurrent/refractory neuroectodermal tumours, rhabdomyosarcoma and/or other solid tumours	
Patel, Neha J	Nationwide Children's Hospital	The "head start 4" protocol newly diagnosed children (less than 10 years old) with medulloblastoma and other central nervous system embryonal tumors, phase IV	
Puccetti, Diane M	St. Jude Medical	LCH-IV: International collaborative treatment protocol for children and adolescents with langerhans cell histiocytosis	
Sondel, Paul M	Midwest Athletes Against Childhood Cancer	Determining the influence of KIR/KIR-ligand genotypes in the outcome of high-risk neuroblastoma patients following anti-GD2 based immunotherapy	
Sondel, Paul M	St. Jude Medical	A phase I trial of the humanized anti-GD2 antibody (HU14.18K322A) in children and adolescents with neuroblastoma or melanoma	
Sondel, Paul M	National Institutes of Health (NIH)	Enhancing antibody-directed innate immunity to improve cancer outcome	

Sondel, Paul M	Alex's Lemonade Stand Foundation	Identifying how pre-existing anti-therapeutic antibodies are associated with better outcome in a clinical trial of ADCC-inducing anti-GD2 mAb	
Sondel, Paul M	St. Jude Medical	Neuroblastoma protocol 2012: therapy for children with advanced stage high-risk neuroblastoma	
Sondel, Paul M	Wisconsin Alumni Research Foundation	Combining radiotherapeutic with antitumor antibody and IL2 to create a potent in situ cancer vaccine	
Sondel, Paul M	American Association for Cancer Research & Children's Hospital of Philadelphia	Immunogenomics to create new therapies for high-risk childhood cancers	HemOnc: Kenneth DeSantes, Mario Otto, Christian Capitini, Inga Hofmann
Sondel, Paul M	Children's Hospital of Philadelphia & National Institutes of Health (NIH)	Discovery and development of optimal immunotherapeutic strategies for childhood cancer	
Sondel, Paul M	Hyundai Hope on Wheels	Innate and adaptive immunotherapy to eradicate immunologically cold neuroblastoma	
Sondel, Paul M	Hughes (Howard) Medical Institute	Endogenous antibody response: Their role in the potentiation and outcome of combination anti-melanoma in situ immunotherapies.	
Sondel, Paul M	Fred Hutchinson Cancer Research Center & National Institutes of Health (NIH)	Cancer immunotherapy trials network central operations and statistical center	
Sondel, Paul M	National Institutes of Health (NIH)	Molecular targeted radiotherapy to overcome resistance to in situ cancer vaccination	

Drezner, Marc (UW-ICTR)	National Institutes of Health (NIH)	UW - Institute for Clinical & Translational Research	AIR: Anna Huttenlocher, Robert Lemanske, Christine Seroogy GPAM: Elizabeth Cox HemOnc: Paul Sondel, Margo Hoover-Regan
Ponik, Suzanne M (UW Department of Cell and Regenerative Biology)	National Institutes of Health (NIH) & Morgridge Institute for Research	Quantitative in vivo optical imaging of tumor heterogeneity	HemOnc: Paul Sondel
Asimakopoulos, Fotios (UW Department of Medicine)	American Cancer Society	The role of TPL2 in regulating macrophage-myeloma tumor cell interactions	HemOnc: Paul Sondel