




## Crystal Mackall

Ernest and Amelia Gallo Family Professor and Professor of Pediatrics and of Medicine  
Pediatrics - Hematology & Oncology

 NIH Biosketch available Online

 Curriculum Vitae available Online

### CLINICAL OFFICES

- **Pediatric Oncology**

725 Welch Rd

Clinic D

Palo Alto, CA 94304

**Tel** (650) 497-8953

**Fax** (650) 724-1164

### ACADEMIC CONTACT INFORMATION

- **Administrative Contact**

Carol Sinoben - Executive Asst.

**Email** csinoben@stanford.edu

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### Bio

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#### BIO

Crystal L Mackall MD is the Ernest and Amelia Gallo Family Professor of Pediatrics and Internal Medicine at Stanford University. She serves as Founding Director of the Stanford Center for Cancer Cell Therapy, Associate Director of Stanford Cancer Institute, Leader of the Cancer Immunology and Immunotherapy Program and Director of the Parker Institute for Cancer Immunotherapy at Stanford. During a 27 year tenure culminating as Chief of the Pediatric Oncology Branch, NCI, and now at Stanford, she has led an internationally recognized translational research program spanning basic studies of T cell homeostasis and tumor immunology, and clinical trials of immune based therapies for cancer. Her work is credited with identifying an essential role for the thymus in human T cell regeneration and discovering IL-7 as the master regulator of T cell homeostasis. She has led numerous first-in-human and first-in-child clinical trials spanning dendritic cell vaccines, cytokines, and adoptive immunotherapy using NK cells and genetically modified T cells. Her group was among the first to demonstrate impressive activity of CD19-CAR in pediatric leukemia, recently demonstrated impressive activity of CD22-CARs for leukemia and has identified T cell exhaustion as a major feature limiting the activity of CAR T cells. Dr. Mackall's clinical trials are notable for the incorporation of deep biologic endpoints that further our understanding of the basis for success and failure of novel immunotherapeutics. She has published over 185 manuscripts and serves in numerous leadership positions, including co-PI on the NCI Pediatric Cancer Immunotherapy Network (U54), Leader of the NCI Pediatric Cancer Immunotherapy Trials Network, and co-Leader of the St. Baldrick's-StandUp2Cancer Pediatric Dream Team. She is Board Certified in Pediatrics, Pediatric Hematology-Oncology and Internal Medicine.

#### CLINICAL FOCUS

- Pediatric Hematology-Oncology

#### ACADEMIC APPOINTMENTS

- Professor, Pediatrics - Hematology & Oncology
- Professor, Medicine - Blood & Marrow Transplantation
- Member, Bio-X
- Member, Maternal & Child Health Research Institute (MCHRI)
- Member, Stanford Cancer Institute

## **ADMINISTRATIVE APPOINTMENTS**

- Founding Director, Stanford Center for Cancer Cell Therapy, (2017- present)
- Director, Parker Institute for Cancer Immunotherapy at Stanford, (2016- present)
- Associate Director, Stanford Cancer Institute, (2016- present)
- Leader, Cancer Immunology and Immunotherapy Program, Stanford Cancer Institute, (2016- present)
- Director, Cancer Immunotherapy Program, Department of Pediatrics, (2016- present)

## **HONORS AND AWARDS**

- Lila and Murray Gruber Memorial Cancer Research Award and Lectureship, American Academy of Dermatology (March 2018)
- BJ Kennedy Keynote Lecturer, Masonic Cancer Center, Minneapolis, MN (2018)
- Top 10 Clinical Research Award for New CAR-T Cell Therapy for Relapsed Leukemia, Top 10 Clinical Research Award (2018)
- Chair, Pediatric Cancer Working Group, American Association for Cancer Research (2017-18)
- Stephen Max Memorial Lectureship, University of Maryland (2017)
- Warren Stow Distinguished Lectureship, MD Anderson Cancer Center (2017)
- Nitschke-Kaskake Visiting Professorship, Oklahoma City Children's Hospital (2016)
- G. Burroughs Mider Lectureship, National Institutes of Health (2015)
- Member, Association of American Physicians (2015)
- William Hathaway Visiting Professor Award, University of Colorado (2015)
- Co-Leader, St.Baldrick's/StandUp2Cancer Pediatric Dream Team (2013-present)
- Alexandra Scott Lectureship in Pediatric Oncology, Children's Hospital of Philadelphia (2013)
- Director's Award, National Institutes of Health (2013)
- Great Teacher Lectureship, National Institutes of Health (2012)
- Vineberg Lectureship, Montreal Children's Hospital (2011)
- Alex Koufos Memorial Lectureship, Akron Children's Hospital (2009)
- Merit Award, National Institutes of Health (2007)
- Member, Best Doctors in American (2006-present)
- Member, American Society for Clinical Investigation (2005)
- Distinguished Alumni Award, Northeastern Ohio Universities College of Medicine (2004)
- Commendation Medal, United States Public Health Service (2003)
- Director's Award, National Cancer Institute (2003)
- Distinguished Clinical Teacher Award, National Institutes of Health (2000)
- Member, Alpha Omega Alpha, Honorary Medical Society (1984)

## **BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS**

- Executive Board, Federation of Clinical Immunology Societies (FOCIS) (2001 - 2002)
- Member, Biologic Response Modifiers Advisory Committee, Food and Drug Administration (2002 - 2003)
- Member, NIH Central Tenure Committee (2004 - 2008)
- Education Committee, American Society of Clinical Oncology (2006 - 2009)
- Member, DNA Advisory Committee, US Food and Drug Administration (2008 - 2008)
- Advisory Board for Clinical Research, NIH Clinical Center (2008 - 2012)

- Vice Chair and Chair, Scientific Committee on Immunology and Host Defense, American Society of Hematology (2011 - 2012)
- Member, Immunology Steering Committee, American Association for Cancer Research (2013 - 2014)
- Chair, Program Committee on Immunology, American Association for Cancer Research (2014 - 2015)
- Chair-Elect, Pediatric Cancer Working Group, American Association for Cancer Research (2015 - 2017)
- Member, Committee on Scientific Affairs, American Society of Hematology (2016 - 2017)
- Chair, Pediatric Cancer Working Group, American Association for Cancer Research (2017 - present)

## PROFESSIONAL EDUCATION

- Board Certification: Pediatric Hematology-Oncology, American Board of Pediatrics (1992)
- Fellowship: National Cancer Institute - Center Cancer Research (1992) MD
- Board Certification: Pediatrics, American Board of Pediatrics (1989)
- Board Certification: Internal Medicine, American Board of Internal Medicine (1989)
- Residency: Akron General Hospital (1988) OH
- MD, Northeastern Ohio Universities College of Medicine , Medicine (1984)
- Medical Education: Northeastern Ohio Universities (1984) OH
- BS, University of Akron , Natural Sciences (1980)

## PATENTS

- Rimas Orentas, Ira Pastan, Crystal Mackall. "United States Patent 61/549,516 Anti-CD22 Chimeric Antigen Receptors, patent pending", National Cancer Institute
- Rimas Orentas, Ira Pastan, Crystal Mackall, Dimiter Dimitrov. "United States Patent 61/717,960 M971 Chimeric Antigen Receptors, patent pending", National Cancer Institute
- Dimiter Dimitrov, Rimas Orentas, Crystal Mackall. "United States Patent 61/805001 Anti-CD276 polypeptides, proteins and chimeric antigen receptors, patent pending", National Cancer Institute
- Rimas Orentas, Dimiter Dimitrov, Crystal Mackall. "United States Patent 61/900,906 ALK Antibodies, Conjugates and Chimeric Antigen Receptors, patent pending", National Cancer Institute
- Terry Fry, Haiying Qin, Crystal Mackall, Rimas Orentas. "United States Patent 62/135,442 Dual Specific Anti-CD22-Anti-CD19-Chimeric Antigen Receptors, patent pending", National Cancer Institute
- Crystal Mackall, Yongzhi Cui. "United States Patent 62/216,447 Anti-CD276 Chimeric Antigen Receptors, patent pending", National Cancer Institute
- Jay Berzofsky, Lee Helman, Crystal Mackall. "United States Patent 7,867,977 A Peptide Epitope and Improvement Thereof Inducting T Cell Immunity to Alveolar Rhabdomyosarcoma in HLA-B7 positive Individuals.", National Cancer Institute
- Hokyung Kay Chung, Michael Z Lin, Crystal Mackall, Robbie Majzner, Louai Labanieh. "United States Patent 62/694,830 Chimeric Antigen Receptor Polypeptides and Methods of Using Same", Leland Stanford Junior University, Jul 16, 2018
- Crystal L Mackall, Michelle Monje, Christopher Mount, Robbie Majzner. "United States Patent 041839 CAR T cell therapy to treat H3K27M midline gliomas", Leland Stanford Junior University, Jul 12, 2018
- Tom Wandless, Rachel Lynn, Sanjay Malhotra, Evan Weber, Crystal Mackall. "United States Patent 025394 Transiently regulated CAR-T cells engineered to prevent T-cell exhaustion and improve immunotherapy", Leland Stanford Junior University, Mar 30, 2018
- Tom Wandless, Rachel Lynn, Sanjay Malhotra, Evan Weber, Crystal Mackall. "United States Patent PCT/US2018/025459 Methods of Treating T Cell Exhaustion by Inhibiting Modulating T Cell Receptor Signaling", Leland Stanford Junior University, Mar 30, 2018
- Rachel Lynn, Evan Weber, Crystal Mackall, Elena Sotillo. "United States Patent 62/599.299 Compositions and Methods for Inhibiting T Cell Exhaustion", Leland Stanford Junior University, Dec 15, 2017
- Jay Berzofsky, Lee Helman, Crystal Mackall. "United States Patent 12/092,449 Immunogenic Peptides And Methods Of Use For Treating And Preventing Cancer", National Cancer Institute, May 2, 2008

## Research & Scholarship

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### CURRENT RESEARCH AND SCHOLARLY INTERESTS

We are impressed by the potency of T cell immune responses for the treatment of cancer and our work focuses on enhancing the effectiveness of T cell based immunotherapies for cancer. Our approach is to simultaneously conduct basic studies alongside clinical trials, leveraging an iterative bench-to bedside-bench rotation

to efficiently optimize clinically relevant cancer immunotherapies. Our laboratory seeks to develop novel therapies for early phase testing in clinical trials, and also conducts intensive studies on clinical samples obtained from patients treated on immunotherapy trials. We also seek to enhance fundamental understanding of human T cell biology.

We focus primarily on using genetically engineered T cells to treat cancer, with an emphasis on chimeric antigen receptors (CARs). CARs are non-natural receptors, created using synthetic biology, that endow T cells with the capacity for antigen-specific, MHC-unrestricted killing. Some clinical results using CAR based therapies have been impressive, but we believe that further progress will emerge as a result of focus on these three major areas:

1. T cell exhaustion, a state whereby continued T cell activation leads to diminished functionality, is a fundamental barrier limiting the efficacy of many cancer immunotherapies. Our laboratory is focused on using high dimensional, single cell analyses to better define human T cell exhaustion and to enhance understanding of the biological mechanisms responsible for this phenomena. We believe that enhanced understanding of T cell exhaustion will give rise to novel approaches to prevent or reverse this phenomenon in the context of cancer immunotherapy.
2. Effective immunotherapies require a therapeutic window which allows the immune cell to preferentially or exclusively attack the neoplastic cell while sparing non-neoplastic, vital tissues. Our laboratory is focused on identifying novel targets for T cell based immunotherapies and for enhancing our understanding of the basis for differential antigen recognition using CAR T cells for cancer therapy. We are also interested in using novel approaches for combinatorial recognition, both to diminish the risk for tumor escape due to loss of antigen expression, and to allow targeting of tumor antigens that pose a risk due to co-expression on healthy, vital tissues.
3. The tumor microenvironment is potently immunosuppressive and can prevent potent antigen specific immune responses from effectively mediating antitumor effects. Our laboratory focuses on enhancing understanding of the immunosuppressive tumor microenvironment and on developing novel approaches to diminish the ability of the tumor microenvironment to limit the efficacy of T cell based immunotherapies.

## **CLINICAL TRIALS**

- Anti-CD22 Chimeric Receptor T Cells in Pediatric and Young Adults With Recurrent or Refractory CD22-expressing B Cell Malignancies, Recruiting
- Axicabtagene Ciloleucef Expanded Access Study, Recruiting
- Nivolumab With or Without Ipilimumab in Treating Younger Patients With Recurrent or Refractory Solid Tumors or Sarcomas, Recruiting
- Phase I CD19/CD22 Chimeric Antigen Receptor T Cells in Peds Recurrent/Refractory B Cell Malignancies, Recruiting
- Phase I CD19/CD22 Chimeric Antigen Receptor(CAR) T Cells in Adults With Recurrent/Refractory B Cell Malignancies, Recruiting
- Enoblituzumab (MGA271) in Children With B7-H3-expressing Solid Tumors, Not Recruiting
- Enrollment on the Childhood Cancer Research Network (CCRN) of the Children s Oncology Group, Not Recruiting
- Screening Protocol for Tumor Antigen Expression Profiling and HLA Typing for Eligibility Determination, Not Recruiting
- Study of bb2121 in Multiple Myeloma, Not Recruiting

## **Teaching**

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### **STANFORD ADVISEES**

#### **Doctoral Dissertation Reader (AC)**

Benson George, Christopher Mount

#### **Postdoctoral Faculty Sponsor**

Hima Anbunathan, Zina Good, Dorota Klysz, Amaury Leruste, Raziieh Monjezi, Johanna Lena Theruvath, Diane Tseng, Evan Weber

#### **Doctoral Dissertation Advisor (AC)**

Justin Arredondo-Guerrero, Louai Labanieh, Katherine Murphy

**Doctoral Dissertation Co-Advisor (AC)**

Vandon Duong

**Postdoctoral Research Mentor**

Dorota Klysz, Amaury Leruste, Johanna Lena Theruvath, Diane Tseng, Evan Weber

**GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS**

- Cancer Biology (Phd Program)
- Immunology (Phd Program)
- Pediatric Hem/Onc (Fellowship Program)

**Publications**

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**PUBLICATIONS**

- **Pharmacologic control of CAR-T cell function using dasatinib.** *Blood advances*  
Weber, E. W., Lynn, R. C., Sotillo, E., Lattin, J., Xu, P., Mackall, C. L.  
2019; 3 (5): 711–17
- **Driving CAR T cell translation forward.** *Science translational medicine*  
Schultz, L., Mackall, C.  
2019; 11 (481)
- **CAR T cells targeting B7-H3, a Pan-Cancer Antigen, Demonstrate Potent Preclinical Activity Against Pediatric Solid Tumors and Brain Tumors.** *Clinical cancer research : an official journal of the American Association for Cancer Research*  
Majzner, R. G., Theruvath, J. L., Nellan, A., Heitzeneder, S., Cui, Y., Mount, C. W., Rietberg, S. P., Linde, M. H., Xu, P., Rota, C., Sotillo, E., Labanieh, L., Lee, et al  
2019
- **CAR T cell therapy: inroads to response and resistance.** *Nature reviews. Immunology*  
Brown, C. E., Mackall, C. L.  
2019
- **Pregnancy-Associated Plasma Protein-A (PAPP-A) in Ewing Sarcoma: Role in Tumor Growth and Immune Evasion.** *Journal of the National Cancer Institute*  
Heitzeneder, S., Sotillo, E., Shern, J. F., Sindiri, S., Xu, P., Jones, R., Pollak, M., Noer, P. R., Lorette, J., Fazli, L., Alag, A., Meltzer, P., Lau, et al  
2019
- **Chimeric antigen receptor (CAR) T therapies for the treatment of hematologic malignancies: clinical perspective and significance.** *Journal for immunotherapy of cancer*  
Boyiadzis, M. M., Dhodapkar, M. V., Brentjens, R. J., Kochenderfer, J. N., Neelapu, S. S., Maus, M. V., Porter, D. L., Maloney, D. G., Grupp, S. A., Mackall, C. L., June, C. H., Bishop, M. R.  
2018; 6 (1): 137
- **Target Antigen Downregulation and Other Mechanisms of Failure after Axicabtagene Ciloleucel (CAR19) Therapy**  
Oak, J., Spiegel, J. Y., Sahaf, B., Natkunam, Y., Long, S. R., Hossain, N., Mackall, C. L., Kong, K. A., Miklos, D. B.  
AMER SOC HEMATOLOGY.2018
- **Low CD19 Antigen Density Diminishes Efficacy of CD19 CAR T Cells and Can be Overcome By Rational Redesign of CAR Signaling Domains**  
Majzner, R. G., Rietberg, S. P., Labanieh, L., Sotillo, E., Weber, E. W., Lynn, R. C., Theruvath, J. L., Yuan, C. M., Xu, P., Nguyen, S. M., Shah, N. N., Stetler-Stevenson, M., Fry, et al  
AMER SOC HEMATOLOGY.2018
- **1 Study of CD19/CD22 Bispecific Chimeric Antigen Receptor (CAR) Therapy in Children and Young Adults with B Cell Acute Lymphoblastic Leukemia (ALL)**

- Schultz, L. M., Davis, K. L., Baggott, C., Chaudry, C., Marcy, A., Mavroukakis, S., Sahaf, B., Kong, K. A., Muffly, L. S., Kim, S., Meyer, E. H., Fry, T. J., Qin, et al  
AMER SOC HEMATOLOGY.2018
- **Phase I Experience with a Bi-Specific CAR Targeting CD19 and CD22 in Adults with B-Cell Malignancies**  
Hossain, N., Sahaf, B., Abramian, M., Spiegel, J. Y., Kong, K., Kim, S., Mavroukakis, S., Oak, J., Natkunam, Y., Meyer, E. H., Frank, M. J., Feldman, S. A., Long, et al  
AMER SOC HEMATOLOGY.2018
  - **Elevated Axicabtagene Ciloleucel (CAR-19) Expansion By Immunophenotyping Is Associated with Toxicity in Diffuse Large B-Cell Lymphoma**  
Spiegel, J. Y., Sahaf, B., Hossain, N., Frank, M. J., Claire, G., Abramian, M., Latchford, T., Villa, B., Cancilla, J., Oak, J., Natkunam, Y., Long, S. R., Arai, et al  
AMER SOC HEMATOLOGY.2018
  - **Systematic Evaluation of Neurotoxicity in Children and Young Adults Undergoing CD22 Chimeric Antigen Receptor T-Cell Therapy** *JOURNAL OF IMMUNOTHERAPY*  
Shalabi, H., Wolters, P. L., Martin, S., Toledo-Tamula, M., Roderick, M., Struempf, K., Kane, E., Yates, B., Delbrook, C., Mackall, C. L., Lee, D. W., Fry, T. J., Shah, et al  
2018; 41 (7): 350–58
  - **Screening Clinical Cell Products for Replication Competent Retrovirus: The National Gene Vector Biorepository Experience** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*  
Cornetta, K., Duffy, L., Feldman, S. A., Mackall, C. L., Davila, M. L., Curran, K. J., Junghans, R. P., Tang, J., Kochenderfer, J. N., O'Carbhaill, R., Archer, G., Kiem, H., Shah, et al  
2018; 10: 371–78
  - **Tumor Antigen Escape from CAR T-cell Therapy.** *Cancer discovery*  
Majzner, R. G., Mackall, C. L.  
2018
  - **Antitumor Activity Associated with Prolonged Persistence of Adoptively Transferred NY-ESO-1(c259) T Cells in Synovial Sarcoma** *CANCER DISCOVERY*  
D'Angelo, S. P., Melchiori, L., Merchant, M. S., Bernstein, D., Glod, J., Kaplan, R., Grupp, S., Tap, W. D., Chagin, K., Binder, G. K., Basu, S., Lowther, D. E., Wang, et al  
2018; 8 (8): 944–57
  - **Fludarabine and neurotoxicity in engineered T-cell therapy** *GENE THERAPY*  
Lowe, K. L., Mackall, C. L., Norry, E., Amado, R., Jakobsen, B. K., Binder, G.  
2018; 25 (3): 176–91
  - **Programming CAR-T cells to kill cancer** *NATURE BIOMEDICAL ENGINEERING*  
Labanieh, L., Majzner, R. G., Mackall, C. L.  
2018; 2 (6): 377–91
  - **CAR T CELLS TARGETING B7-H3, A PAN-CANCER ANTIGEN, DEMONSTRATE POTENT PRECLINICAL ACTIVITY AGAINST PEDIATRIC SOLID TUMORS AND BRAIN TUMORS**  
Majzner, R., Nellan, A., Heitzeneder, S., Theruvath, J., Mackall, C.  
WILEY.2018
  - **B7-H3 CAR T CELLS MEDIATE IN VITRO AND IN VIVO ACTIVITY AGAINST NEUROBLASTOMA XENOGRAFTS**  
Kadapakkam, M., Majzner, R., Xu, P., Mackall, C.  
WILEY.2018
  - **CHECKPOINT MOLECULE B7-H3 IS HIGHLY EXPRESSED ON ATYPICAL RHABDOID TERATOID TUMOR (ATRT) AND IS A PROMISING CANDIDATE FOR CAR T CELL THERAPY**  
Theruvath, J., Graef, C., Heitzeneder, S., Majzner, B., Mitra, S., Cheshier, S. H., Mackall, C.  
OXFORD UNIV PRESS INC.2018: 33
  - **ANTI-GD2 CHIMERIC ANTIGEN RECEPTOR T CELLS AS A POTENT IMMUNOTHERAPY REGIMEN IN XENOGRAFT MODELS OF HISTONE 3 K27M MUTANT DIFFUSE MIDLINE GLIOMA**  
Mount, C., Majzner, R., Sundaresh, S., Arnold, E., Kadapakkam, M., Haile, S., Labanieh, L., Woo, P., Rietberg, S., Vogel, H., Monje, M., Mackall, C.  
OXFORD UNIV PRESS INC.2018: 56

- **Engineering a designer immunotherapy Tailoring cytokine selectivity by engineering receptor-ligand pairs circumvents toxicity** *SCIENCE*  
Mackall, C. L.  
2018; 359 (6379): 990–91
- **Neurotoxicity Associated with a High-Affinity GD2 CAR-Letter.** *Cancer immunology research*  
Majzner, R. G., Weber, E. W., Lynn, R. C., Xu, P., Mackall, C. L.  
2018; 6 (4): 494–95
- **Potent antitumor efficacy of anti-GD2 CAR T cells in H3-K27M+ diffuse midline gliomas.** *Nature medicine*  
Mount, C. W., Majzner, R. G., Sundaresh, S., Arnold, E. P., Kadapakkam, M., Haile, S., Labanich, L., Hulleman, E., Woo, P. J., Rietberg, S. P., Vogel, H., Monje, M., Mackall, et al  
2018
- **CHECKPOINT MOLECULE B7-H3 IS HIGHLY EXPRESSED ON MEDULLOBLASTOMA AND PROVES TO BE A PROMISING CANDIDATE FOR CAR T CELL IMMUNOTHERAPY**  
Theruvath, J., Heitzeneder, S., Majzner, R., Cui, K., Nellan, A., Graef, C., Cheshier, S. H., Mackall, C., Mitra, S. S.  
OXFORD UNIV PRESS INC.2017: 122
- **GD2-DIRECTED CHIMERIC ANTIGEN RECEPTOR T CELLS AS A POTENT IMMUNOTHERAPY REGIMEN IN XENOGRAFT MODELS OF DIFFUSE INTRINSIC PONTINE GLIOMA**  
Mount, C., Majzner, R., Sundaresh, S., Arnold, E., Kadapakkam, M., Monje-Deisseroth, M., Mackall, C.  
OXFORD UNIV PRESS INC.2017: 198
- **CHECKPOINT MOLECULE B7-H3 IS HIGHLY EXPRESSED ON MEDULLOBLASTOMA AND PROVES TO BE A PROMISING CANDIDATE FOR CAR T CELL IMMUNOTHERAPY**  
Theruvath, J., Heitzeneder, S., Majzner, R., Graef, C., Cui, K., Nellan, A., Cheshier, S. H., Mackall, C., Mitra, S.  
OXFORD UNIV PRESS INC.2017: 28–29
- **Autologous lymphapheresis for the production of chimeric antigen receptor T cells.** *Transfusion*  
Allen, E. S., Stroncek, D. F., Ren, J., Eder, A. F., West, K. A., Fry, T. J., Lee, D. W., Mackall, C. L., Conry-Cantilena, C.  
2017; 57 (5): 1133-1141
- **Harnessing the Immunotherapy Revolution for the Treatment of Childhood Cancers** *CANCER CELL*  
Majzner, R. G., Heitzeneder, S., Mackall, C. L.  
2017; 31 (4): 476-485
- **Elutriated lymphocytes for manufacturing chimeric antigen receptor T cells** *JOURNAL OF TRANSLATIONAL MEDICINE*  
Stroncek, D. F., Lee, D. W., Ren, J., Sabatino, M., Highfill, S., Khuu, H., Shah, N. N., Kaplan, R. N., Fry, T. J., Mackall, C. L.  
2017; 15
- **CNS Endothelial Cell Activation Emerges as a Driver of CAR T Cell-Associated Neurotoxicity.** *Cancer discovery*  
Mackall, C. L., Miklos, D. B.  
2017; 7 (12): 1371–73
- **Assessment of programmed death-ligand 1 expression and tumor-associated immune cells in pediatric cancer tissues.** *Cancer*  
Majzner, R. G., Simon, J. S., Grosso, J. F., Martinez, D., Pawel, B. R., Santi, M., Merchant, M. S., Geoerger, B., Hezam, I., Marty, V., Vielh, P., Daugaard, M., Sorensen, et al  
2017
- **Tumor Antigen and Receptor Densities Regulate Efficacy of a Chimeric Antigen Receptor Targeting Anaplastic Lymphoma Kinase.** *Molecular therapy : the journal of the American Society of Gene Therapy*  
Walker, A. J., Majzner, R. G., Zhang, L., Wanhainen, K., Long, A. H., Nguyen, S. M., Lopomo, P., Vigny, M., Fry, T. J., Orentas, R. J., Mackall, C. L.  
2017
- **Identification of GPC2 as an Oncoprotein and Candidate Immunotherapeutic Target in High-Risk Neuroblastoma.** *Cancer cell*  
Bosse, K. R., Raman, P., Zhu, Z., Lane, M., Martinez, D., Heitzeneder, S., Rathi, K. S., Kendsersky, N. M., Randall, M., Donovan, L., Morrissy, S., Sussman, R. T., Zhelev, et al  
2017; 32 (3): 295–309.e12
- **CD22-targeted CAR T cells induce remission in B-ALL that is naive or resistant to CD19-targeted CAR immunotherapy.** *Nature medicine*

- Fry, T. J., Shah, N. N., Orentas, R. J., Stetler-Stevenson, M., Yuan, C. M., Ramakrishna, S., Wolters, P., Martin, S., Delbrook, C., Yates, B., Shalabi, H., Fountaine, T. J., Shern, et al  
2017
- **New developments in immunotherapy for pediatric solid tumors.** *Current opinion in pediatrics*  
Schultz, L. M., Majzner, R., Davis, K. L., Mackall, C.  
2017
  - **Immunotherapy for acute lymphoblastic leukemia: from famine to feast** *BLOOD ADVANCES*  
Davis, K. L., Mackall, C. L.  
2016; 1 (3): 265–69
  - **A Prospective Evaluation of Neurocognitive Function and Neurologic Symptoms in Pediatric and Young Adult Patients with Relapsed/Refractory Acute Lymphoblastic Leukemia (ALL) Undergoing Anti-CD22 Chimeric Antigen Receptor Therapy**  
Shalabi, H., Wolters, P. L., Martin, S., Delbrook, C., Yates, B., Lee, D. W., Mackall, C. L., Fry, T. J., Shah, N. N.  
AMER SOC HEMATOLOGY.2016
  - **Tocilizumab-Refractory Cytokine Release Syndrome (CRS) Triggered By Chimeric Antigen Receptor (CAR)-Transduced T Cells May Have Distinct Cytokine Profiles Compared to Typical CRS**  
Ishii, K., Shalabi, H., Yates, B., Delbrook, C., Mackall, C. L., Fry, T. J., Shah, N. N.  
AMER SOC HEMATOLOGY.2016
  - **Long-Term Outcomes Following CD19 CAR T Cell Therapy for B-ALL Are Superior in Patients Receiving a Fludarabine/Cyclophosphamide Preparative Regimen and Post-CAR Hematopoietic Stem Cell Transplantation**  
Lee, D. W., Stetler-Stevenson, M., Yuan, C. M., Shah, N. N., Delbrook, C., Yates, B., Zhang, H., Zhang, L., Kochenderfer, J. N., Rosenberg, S. A., Fry, T. J., Stroncek, D., Mackall, et al  
AMER SOC HEMATOLOGY.2016
  - **Minimal Residual Disease Negative Complete Remissions Following Anti-CD22 Chimeric Antigen Receptor (CAR) in Children and Young Adults with Relapsed/Refractory Acute Lymphoblastic Leukemia (ALL)**  
Shah, N. N., Stetler-Stevenson, M., Yuan, C. M., Shalabi, H., Yates, B., Delbrook, C., Zhang, L., Lee, D. W., Stroncek, D., Mackall, C. L., Fry, T. J.  
AMER SOC HEMATOLOGY.2016
  - **Reduction of MDSCs with All-trans Retinoic Acid Improves CAR Therapy Efficacy for Sarcomas** *CANCER IMMUNOLOGY RESEARCH*  
Long, A. H., Highfill, S. L., Cui, Y., Smith, J. P., Walker, A. J., Ramakrishna, S., El-Etriby, R., Galli, S., Tsokos, M. G., Orentas, R. J., Mackall, C. L.  
2016; 4 (10): 869-880
  - **Induction of Immune Response after Allogeneic Wilms' Tumor 1 Dendritic Cell Vaccination and Donor Lymphocyte Infusion in Patients with Hematologic Malignancies and Post-Transplantation Relapse.** *Biology of blood and marrow transplantation*  
Shah, N. N., Loeb, D. M., Khuu, H., Stroncek, D., Ariyo, T., Raffeld, M., Delbrook, C., Mackall, C. L., Wayne, A. S., Fry, T. J.  
2016
  - **Impact of Two Measures of Micrometastatic Disease on Clinical Outcomes in Patients with Newly Diagnosed Ewing Sarcoma: A Report from the Children's Oncology Group.** *Clinical cancer research*  
Vo, K. T., Edwards, J. V., Epling, C. L., Sinclair, E., Hawkins, D. S., Grier, H. E., Janeway, K. A., Barnette, P., McIlvaine, E., Krailo, M. D., Barkauskas, D. A., Matthay, K. K., Womer, et al  
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