Stanford

Weinacht,Katja Gabriele

Assistant Professor of Pediatrics (Stem Cell Transplantation and Regenerative Medicine)

Pediatrics - Stem Cell Transplantation

CLINICAL OFFICES

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ACADEMIC CONTACT INFORMATION

- **Administrative Assistant**

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Bio

CLINICAL FOCUS

- Pediatric Hematology-Oncology

ACADEMIC APPOINTMENTS

- Assistant Professor, Pediatrics - Stem Cell Transplantation
- Member, Bio-X
- Member, Maternal & Child Health Research Institute (MCHRI)

ADMINISTRATIVE APPOINTMENTS

- Attending Physician, Pediatric SCTRM Stanford School of Medicine, (2016- present)
- Assistant Professor, Pediatrics Stanford School of Medicine, (2012-2016)

HONORS AND AWARDS

- Mentored Clinical Scientist Research Career Development Award (K08), NIAID (2016-21)
- Research Fellowship Award, Charles King Trust (2013-15)
- Amy-Potter Research Fellowship Award, Boston Children’s Hospital, Harvard Medical School (2013-14)
- Research Fellowship Award, Primary Immunodeficiency Treatment Consortium (2013-14)
- Amy-Potter Research Fellowship Award, Boston Children’s Hospital, Harvard Medical School (2012-13)
- Dan Heller Teaching Award, Harvard Medical School (2009)

BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS

- Member, American Society of Pediatric Hematology/Oncology (ASPHO) (2009 - present)
- Member, Children’s Oncology Group (COG) (2009 - present)
- Associate Member, American Society of Hematology (ASH) (2009 - present)
- Member, Primary Immunodeficiency Treatment Consortium (PIDTC) (2012 - present)
- Member, International Society of Stem Cell Research (ISSCR) (2014 - present)

PROFESSIONAL EDUCATION

- Fellowship: Boston Childrens Hospital Pediatric Hematology and Oncology Fellowship (2012) MA
- Residency: Mass General Hospital for Children Pediatric Residency (2009) MA
- Board Certification, American Board of Pediatric Hematology-Oncology , 2013
- Board Certification, American Board of Pediatrics , 2009
- Research Fellowship, Pediatric Immunology, Laboratory Dr. Luigi D. Notarangelo, Boston Children's Hospital, Harvard Medical School, Boston, MA , 2010-2012
- Clinical Fellowship, Pediatric Hematology/Oncology, Boston Children's Hospital, Dana-Farber Cancer Institute, Harvard Medical School, Boston, MA , 2009-2012
- Residency, Pediatrics, Massachusetts General Hospital, Harvard Medical School, Boston, MA , 2006-2009
- Postdoctoral Fellow, Channing Laboratory, Brigham and Women's Hospital, Harvard Medical School, Boston, MA , 2005-2006
- PhD (Medicine), Technische Universitaet Muenchen, School of Medicine, Munich, Germany , 2004
- MD, Technische Universitaet Muenchen, School of Medicine, Munich, Germany , 2002

PATENTS

- Comstock LE, Coyne MJ, Weinacht KG, Kasper DL, Tzianabos AO. "United States Patent 10/388,390 Method of Overexpression of Zwitterionic Polysaccharides", Brigham and Women's Hospital, Mar 13, 2003

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

I am a pediatric hematologist-oncologist with special interest in the niche of diseases that intersect immune dysfunction, primary immunodeficiency and bone marrow failure. My clinical practice focuses on pediatric patients requiring a hematopoietic stem cell transplantation, patients with DiGeorge Syndrome and patients with genetic immune diseases presenting with autoimmunity. As a physician-scientist, I strive to advance our insights into the mechanisms leading to immunodeficiency, autoimmunity and tolerance on a molecular level and to translate our research into novel targeted therapies patients.

My work is a natural extension of my clinical training in pediatric hematology-oncology combined with my scientific background in immunology and microbiology. After completing my clinical training at Boston Children's Hospital/Dana-Farber Cancer Institute, I joined the laboratory of Luigi D. Notarangelo in the division of immunology, Boston Children's Hospital/Harvard Stem Cell Institute, where I have acquired skills in the field of reprogramming, tissue engineering and gene correction. In my laboratory, we now use iPSC-based disease models to study how defects in mitochondrial metabolism and oxidative stress affect hematopoietic stem and progenitor cell development and cell death with the goal of identifying therapeutic targets. A separate focus of my laboratory is devoted to understanding the thymic developmental defects in DiGeorge syndrome.

CLINICAL TRIALS

- Natural History Study of SCID Disorders, Recruiting
- Patients Treated for Chronic Granulomatous Disease (CGD) Since 1995, Recruiting
- Patients Treated for SCID (1968-Present), Recruiting
- Patients Treated for Wiskott-Aldrich Syndrome (WAS) Since 1990, Not Recruiting

Teaching

STANFORD ADVISEES

Postdoctoral Faculty Sponsor

Wenqing Wang

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Immunology (Phd Program)
- Immunology/Rheumatology (Fellowship Program)
- Medicine (Masters Program)
- Microbiology and Immunology (Phd Program)
- Molecular and Cellular Physiology (Phd Program)
- Pediatric Hem/Onc (Fellowship Program)
- Stem Cell Biology and Regenerative Medicine (Phd Program)

Publications

PUBLICATIONS

- **Calm in the midst of cytokine storm: a collaborative approach to the diagnosis and treatment of hemophagocytic lymphohistiocytosis and macrophage activation syndrome.** *Pediatric rheumatology online journal*
  Halyabar, O., Chang, M. H., Schoettler, M. L., Schwartz, M. A., Baris, E. H., Benson, L. A., Biggs, C. M., Gorman, M., Lehmann, L., Lo, M. S., Nigrovic, P. A., Platt, C. D., Priebe, et al
  2019; 17 (1): 7

- **Combined liver and hematopoietic stem cell transplantation in X-linked hyper IgM syndrome.** *The Journal of allergy and clinical immunology*
  Bucciol, G., Nicholas, S. K., Calvo, P. L., Cant, A., Edgar, J. D., Espanol, T., Ferrua, F., Galicchio, M., Gennery, A. R., Hadzic, N., Hanson, I. C., Kusminsky, G., Lange, et al
  2019

- **Engineering Regenerative Thymic Tissues to Restore Long-Term T Cell Lymphopoiesis**
  Gai, H., Gras-Pena, R., Verma, Y., Fateh, V., Ikeda, K., Dejene, B., Min, D., Wang, J., Swigut, T., Weinberg, K. I., Hollander, G. A., Heilshorn, S., Roncarolo, et al
  AMER SOC HEMATOLOGY.2018

- **An Engineered Cell-Traceable Model of Reticular Dysgenesis in Human Hematopoietic Stem Cells Linking Metabolism and Differentiation**
  Wang, W., Awani, A., Reich, L., Nakauchi, Y., Thomas, D., Dever, D. P., Porteus, M., Weinacht, K. G.
  AMER SOC HEMATOLOGY.2018

- **Jakinibs for the treatment of immune dysregulation in patients with gain-of-function signal transducer and activator of transcription 1 (STAT1) or STAT3 mutations.** *The Journal of allergy and clinical immunology*
  Forbes, L. R., Vogel, T. P., Cooper, M. A., Castro-Wagner, J., Schussler, E., Weinacht, K. G., Plant, A. S., Su, H. C., Allenspach, E. J., Slatter, M., Abinun, M., Lilic, D., Cunningham-Rundles, et al
  2018

- **Recent outcome of hematopoietic cell transplantation for Wiskott-Aldrich syndrome is excellent in all donor types: A Primary Immune Deficiency Treatment Consortium (PIDTC) Study**
  Pai, S., Brazauskas, R., Bleesing, J., Dvorak, C. C., Kletzel, M., Petrovic, A., Prockop, S. E., Quinones, R., Goldman, F. D., Quigg, T. C., Hanson, I. C., Stenger, E., Thakar, et al
  SPRINGER/PLENUM PUBLISHERS.2018: 399–400

- **Resolution of CGD Related Colitis after Allogeneic Hematopoietic Stem Cell Transplantation in Patients with Chronic Granulomatous Disease-Early Results From the 6903 Study of the Primary Immune Deficiency Treatment Consortium (PIDTC)**
  Leiding, J. W., Logan, B. R., Yin, Z., Arbuckle, E., Bleesing, J. J., Sullivan, K. E., Heimall, J., Burroughs, L., Skoda-Smith, S., Chandrakasan, S., Yu, L. C., Oshrine, B. R., Cuvelier, et al
  ELSEVIER SCIENCE INC.2018: S53–S54

- **Human Ipsc-Derived Thymic Epithelial Progenitor Cells as Stem Cell-Based Therapyto Restore Thymic Function in Hematopoietic Stem Cell Transplant Recipients**
  Gai, H., Sebastiano, V., Weinacht, K. G.
  ELSEVIER SCIENCE INC.2018: S364

- **Ruxolitinib reverses Dysregulated T Helper Cell Responses and controls Autoimmunity caused by a Novel STAT1 Gain of Function Mutation** *Journal of Allergy and Clinical Immunology*

Weinacht, K. G., Charbonnier, L. M., Alroqi, F., Plant, A., Qiao, Q., Wu, H., Ma, C., Torgerson, T. R., Rosenweig, S. D., Flesier, T. A., Notarangelo, L. D., Hanson, I. C., Forbes, et al

2017

- **Reticular dysgenesis-associated AK2 protects hematopoietic stem and progenitor cell development from oxidative stress** *JOURNAL OF EXPERIMENTAL MEDICINE*

  Rissone, A., Weinacht, K. G., La Marca, G., Bishop, K., Giocaliere, E., Jagadeesh, J., Felgentreff, K., Dobbs, K., Al-Herz, W., Jones, M., Chandrasekharappa, S., Kirby, M., Wincovitch, et al

  2015; 212 (8): 1185-1202

- **Diagnosis of immunodeficiency caused by a purine nucleoside phosphorylase defect by using tandem mass spectrometry on dried blood spots** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*

  La Marca, G., Canessa, C., Giocaliere, E., Romano, F., Malvagia, S., Funghini, S., Moriondo, M., Valleriani, C., Lippi, F., Ombrone, D., Della Bona, M. L., Speckmann, C., Borte, et al

  2014; 134 (1): 155-?

- **Differential role of nonhomologous end joining factors in the generation, DNA damage response, and myeloid differentiation of human induced pluripotent stem cells** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*

  Felgentreff, K., Du, L., Weinacht, K. G., Dobbs, K., Bartish, M., Giliani, S., Schlaeger, T., DeVine, A., Schambach, A., Woodbine, L. J., Davies, G., Baxi, S. N., van der Burg, et al

  2014; 111 (24): 8889-8894

- **Primary Immune Deficiency Treatment Consortium (PIDTC) report.** *journal of allergy and clinical immunology*

  Griffith, L. M., Cowan, M. J., Notarangelo, L. D., Kohn, D. B., Puck, J. M., Pai, S., Ballard, B., Bauer, S. C., Bleesing, J. J., Boyle, M., Brower, A., Buckley, R. H., van der Burg, et al

  2014; 133 (2): 335-347 e11

- **Whole-exome sequencing identifies tetratricopeptide repeat domain 7A (TTC7A) mutations for combined immunodeficiency with intestinal atresias** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*

  Chen, R., Giliani, S., Lanzi, G., Mias, G. I., Lonardi, S., Dobbs, K., Manis, J., Im, H., Gallagher, J. E., Phanstiel, D. H., Euskirchen, G., Lacroute, P., Bettinger, et al

  2013; 132 (3): 656-?

- **First reported case of Omenn syndrome in a patient with reticular dysgenesis** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*

  Henderson, L. A., Frugoni, F., Hopkins, G., Al-Herz, W., Weinacht, K., Comeau, A. M., Bonilla, F. A., Notarangelo, L. D., Pai, S.

  2013; 131 (4): 1227-1230

- **The role of induced pluripotent stem cells in research and therapy of primary immunodeficiencies.** *Current opinion in immunology*

  Weinacht, K. G., Brauer, P. M., Felgentreff, K., Devine, A., Gennery, A. R., Giliani, S., Al-Herz, W., Schambach, A., Zúñiga-Pflücker, J. C., Notarangelo, L. D.

  2012; 24 (5): 617-624

- **Trans locus inhibitors limit concomitant polysaccharide synthesis in the human gut symbiont Bacteroides fragilis** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*

  Chatzidaki-Livanis, M., Weinacht, K. G., Comstock, L. E.

  2010; 107 (26): 11976-11980

- **Tyrosine site-specific recombinases mediate DNA inversions affecting the expression of outer surface proteins of Bacteroides fragilis** *MOLECULAR MICROBIOLOGY*

  Weinacht, K. G., Roche, H., Krinos, C. M., Coyne, M. J., Parkhill, J., Comstock, L. E.

  2004; 53 (5): 1319-1330

- **Mpi recombinase globally modulates the surface architecture of a human commensal bacterium** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*

  Coyne, M. J., Weinacht, K. G., Krinos, C. M., Comstock, L. E.

  2003; 100 (18): 10446-10451

- **Extensive surface diversity of a commensal microorganism by multiple DNA inversions** *NATURE*

  Krinos, C. M., Coyne, M. J., Weinacht, K. G., Tzianabos, A. O., Kasper, D. L., Comstock, L. E.

  2001; 414 (6863): 555-558

- **[Drug hypersensitivity].** *Terapevticheskii? arkhiv*

  TAREEV, E. M., VINOGRADOVA, O. M., Semenkova, E. N., SOLOV'EVA, A. P.

1975; 47 (4): 5-12