Stanford

Yusuke Nakauchi, MD, PhD

Basic Life Research Scientist, Stem Cell Bio Regenerative Med Institute

Bio

BIO

My research projects aim to investigate the biology of human leukemia. I believe my research will contribute to clarify the disease pathogenesis of leukemia and help identify the critical cells to target to both prevent the development of de novo leukemia and halt relapse.

HONORS AND AWARDS

- Allele-specific anti-HLA antibodies as theranostic agents in hematopoietic cell transplantation, SPARK (2023)
- 60th ASH Abstract Achievement Award (Oral Presentation), American Society of Hematology (2018)
- Stanford University School of Medicine, the Dean's Postdoctoral Fellowship, Stanford University (2016)
- Overseas Award, Nakayama Foundation for Human Science (2014)

EDUCATION AND CERTIFICATIONS

- Doctor of Medicine, Asahikawa Medical University , Medicine (2005)
- Doctor of Philosophy, The University of Tokyo , Medicine (Pathology/ Immunology/Microbiology) (2014)

LINKS

- Majeti Lab, Stanford University School of Medicine: http://majetilab.stanford.edu
- The Institute of Medical Science, The University of Tokyo: http://stemcell-u-tokyo.org/en/sct/
- Asahikawa Medical University: http://www.jimu.asahikawa-med.ac.jp/english/index.html
- SPARK at Stanford: https://sparkmed.stanford.edu/

Publications

PUBLICATIONS

- **A single-cell framework identifies functionally and molecularly distinct multipotent progenitors in adult human hematopoiesis.** *Cell reports*
  Ediriwickrema, A., Nakauchi, Y., Fan, A. C., Köhnke, T., Hu, X., Luca, B. A., Kim, Y., Ramakrishnan, S., Nakamoto, M., Karigane, D., Linde, M. H., Azizi, A., Newman, et al
  2025; 44 (9): 116236

- **Donor-Targeted Anti-HLA-A2 Antibody Shows Nanogram-Level Efficacy in a Novel GVHD Mouse Model.** *bioRxiv : the preprint server for biology*
  Niizuma, K., Chang, A. H., Kim, J. S., Busque, S., Majeti, R., Nakauchi, H., Nakauchi, Y.
  2025

- **Unwanted Concatemeric Knock-Ins Occur Frequently with Cas9/AAV-Mediated Gene-Editing: Detection and Prevention**

Suchy, F. P., Karigane, D., Nakauchi, Y., Higuchi, M., Zhang, J., Pekrun, K., Hsu, I., Fan, A. C., Nishimura, T., Charlesworth, C. T., Bhadury, J., Nishimura, T., Wilkinson, et al

CELL PRESS.2024: 211-212

- **Genome engineering with Cas9 and AAV repair templates generates frequent concatemeric insertions of viral vectors.** *Nature biotechnology*

  Suchy, F. P., Karigane, D., Nakauchi, Y., Higuchi, M., Zhang, J., Pekrun, K., Hsu, I., Fan, A. C., Nishimura, T., Charlesworth, C. T., Bhadury, J., Nishimura, T., Wilkinson, et al

  2024

- **Genetically Corrected RAG2-SCID Human Hematopoietic Stem Cells Restore V(D)J-Recombinase and Rescue Lymphoid Deficiency.** *Blood advances*

  Pavel-Dinu, M., Gardner, C. L., Nakauchi, Y., Kawai, T., Delmonte, O. M., Palterer, B., Bosticardo, M., Pala, F., Viel, S., Malech, H. L., Ghanim, H. Y., Bode, N. M., Kurgan, et al

  2023

- **Simplified Intrafemoral Injections Using Live Mice Allow for Continuous Bone Marrow Analysis** *Journal of Visualized Experiments*

  Nakauchi, Y., Ediriwickrema, A., Martinez-Krams, D., Zhao, F., Rangavajhula, A., Karigane, D., Majeti, R.

  2023

- **RUNX1 loss renders hematopoietic and leukemic cells dependent on interleukin-3 and sensitive to JAK inhibition.** *The Journal of clinical investigation*

  Fan, A. C., Nakauchi, Y., Bai, L., Azizi, A., Nuno, K. A., Zhao, F., Köhnke, T., Karigane, D., Cruz-Hernandez, D., Reinisch, A., Khatri, P., Majeti, R.

  2023

- **The cell type specific 5hmC landscape and dynamics of healthy human hematopoiesis and TET2-mutant pre-leukemia.** *Blood cancer discovery*

  Nakauchi, Y., Azizi, A., Thomas, D., Corces, M. R., Reinisch, A., Sharma, R., Cruz Hernandez, D., Kohnke, T., Karigane, D., Fan, A., Martinez-Krams, D., Stafford, M., Kaur, et al

  2022

- **Integrated analysis of patient samples identifies biomarkers for venetoclax efficacy and combination strategies in acute myeloid leukemia.** *Nature cancer*

  Zhang, H. n., Nakauchi, Y. n., Köhnke, T. n., Stafford, M. n., Bottomly, D. n., Thomas, R. n., Wilmot, B. n., McWeeney, S. K., Majeti, R. n., Tyner, J. W.

  2020; 1 (8): 826–39

- **Hematopoietic Stem Cells** *Harrison's Principles of Internal Medicine (Japanese 5th Edition)*

  Nakauchi, Y., Nakauchi, H.

  MEDSI.2017; 19: 89e1-4

- **Effective treatment against severe graft-versus-host disease with allele-specific anti-HLA monoclonal antibody in a humanized mouse model.** *Experimental hematology*

  Nakauchi, Y., Yamazaki, S., Napier, S. C., Usui, J., Ota, Y., Takahashi, S., Watanabe, N., Nakauchi, H.

  2015; 43 (2): 79-88 e1 4

- **Concurrent administration of intravenous systemic and intravitreal methotrexate for intraocular lymphoma with central nervous system involvement** *INTERNATIONAL JOURNAL OF HEMATOLOGY*

  Nakauchi, Y., Takase, H., Sugita, S., Mochizuki, M., Shibata, S., Ishiwata, Y., Shibuya, Y., Yasuhara, M., Miura, O., Arai, A.

  2010; 92 (1): 179-185

- **DNMT3A R882H Is Not Required for Disease Maintenance in Primary Human AML, but Is Associated With Increased Leukemia Stem Cell Frequency.** *Cancer discovery*

  Köhnke, T., Karigane, D., Hilgart, E., Fan, A. C., Kayamori, K., Miyauchi, M., Collins, C. T., Suchy, F. P., Rangavajhula, A., Feng, Y., Nakauchi, Y., Martinez-Montes, E., Fowler, et al

  2025

- **Intra-leukemic interferon signaling suppresses expansion and mediates chemoresistance in human AML.** *Blood cancer discovery*

  Karigane, D., Fan, A. C., Nishimura, T., Kayamori, K., Nakauchi, Y., Köhnke, T., Rangavajhula, A., Ediriwickrema, A., Benard, B. A., Thomas, R., Zhao, F., Stafford, M., Suchy, et al

  2025

- **Development of iPSC-derived T cells targeting EGFR neoantigens in non-small cell lung cancer.** *Molecular therapy. Methods & clinical development*

Niizuma, K., Nishimura, T., Villanueva, J., Amaya, L., Fowler, J. L., Isobe, T., Nakauchi, Y., Saavedra, B., Xu, H., Nakanishi, M., Wilkinson, A. C., Loh, K. M., Shrager, et al

2025; 33 (3): 101517

- **Highly efficient in vivo hematopoietic stem cell transduction using an optimized self-complementary adeno-associated virus** *MOLECULAR THERAPY METHODS & CLINICAL DEVELOPMENT*

  Charlesworth, C. T., Homma, S., Amaya, A. K., Dib, C., Vaidyanathan, S., Tan, T., Miyauchi, M., Nakauchi, Y., Suchy, F. P., Wang, S., Igarashi, K. J., Cromer, M., Dudek, et al

  2025; 33 (1)

- **Highly efficient in vivo hematopoietic stem cell transduction using an optimized self-complementary adeno-associated virus.** *Molecular therapy. Methods & clinical development*

  Charlesworth, C. T., Homma, S., Amaya, A. K., Dib, C., Vaidyanathan, S., Tan, T. K., Miyauchi, M., Nakauchi, Y., Suchy, F. P., Wang, S., Igarashi, K. J., Cromer, M. K., Dudek, et al

  2025; 33 (1): 101438

- **DNMT3AR882H Is Not Required for Disease Maintenance in Primary Human AML, but Is Associated With Increased Leukemia Stem Cell Frequency.** *bioRxiv : the preprint server for biology*

  Köhnke, T., Karigane, D., Hilgart, E., Fan, A. C., Kayamori, K., Miyauchi, M., Collins, C. T., Suchy, F. P., Rangavajhula, A., Feng, Y., Nakauchi, Y., Martinez-Montes, E., Fowler, et al

  2024

- **Failure of metabolic checkpoint control during late-stage granulopoiesis drives neutropenia in reticular dysgenesis.** *Blood*

  Wang, W., Arreola, M., Mathews, T., DeVilbiss, A. W., Zhao, Z., Martin-Sandoval, M., Mohammed, A., Benegiamo, G., Awani, A., Goeminne, L. J., Dever, D. P., Nakauchi, Y., Porteus, et al

  2024

- **IDENTIFICATION AND CHARACTERIZATION OF NEW MULTIPOTENT PROGENITORS IN ADULT HUMAN HEMATOPOIESIS**

  Ediriwickrema, A., Nakauchi, Y., Fan, A., Kohnke, T., Hu, X., Luca, B., Kim, Y., Ramakrishnan, S., Nakamoto, M., Karigane, D., Linde, M., Azizi, A., Newman, et al

  ELSEVIER SCIENCE INC.2024

- **ENGINEERING SEQUENTIAL MUTATIONS INTO HUMAN HSPCS YIELDS AN AGGRESSIVE MYELOID MALIGNANCY ALLOWING FOR INTERROGATION OF PRELEUKEMIC TRANSFORMATION**

  Collins, C., Nakauchi, Y., Koehnke, T., Chavez, J., Choi, S., Sharma, R., Zhao, F., Majeti, R.

  ELSEVIER SCIENCE INC.2024

- **IDENTIFICATION AND CHARACTERIZATION OF NEW MULTIPOTENT PROGENITORS IN ADULT HUMAN HEMATOPOIESIS**

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  Collins, C., Nakauchi, Y., Koehnke, T., Chavez, J., Choi, S., Sharma, R., Zhao, F., Majeti, R.

  ELSEVIER SCIENCE INC.2024

- **AML/T cell interactomics uncover correlates of patient outcomes and the key role of ICAM1 in T cell killing of AML.** *Leukemia*

  Sayitoglu, E. C., Luca, B. A., Boss, A. P., Thomas, B. C., Freeborn, R. A., Uyeda, M. J., Chen, P. P., Nakauchi, Y., Waichler, C., Lacayo, N., Bacchetta, R., Majeti, R., Gentles, et al

  2024

- **IDH1-Mutant Preleukemic Hematopoietic Stem Cells Can Be Eliminated by Inhibition of Oxidative Phosphorylation.** *Blood cancer discovery*

  Landberg, N., Köhnke, T., Feng, Y., Nakauchi, Y., Fan, A. C., Linde, M. H., Karigane, D., Lim, K., Sinha, R., Malcovati, L., Thomas, D., Majeti, R.

  2024: OF1-OF18

- **IDH1-mutant preleukemic hematopoietic stem cells can be eliminated by inhibition of oxidative phosphorylation.** *Blood cancer discovery*

  Landberg, N., Köhnke, T., Feng, Y., Nakauchi, Y., Fan, A. C., Linde, M. H., Karigane, D., Lim, K., Sinha, R., Malcovati, L., Thomas, D., Majeti, R.

  2023

- **Simplified Intrafemoral Injections Using Live Mice Allow for Continuous Bone Marrow Analysis.** *Journal of visualized experiments : JoVE*

  Nakauchi, Y., Ediriwickrema, A., Martinez-Krams, D., Zhao, F., Rangavajhula, A., Karigane, D., Majeti, R.

2023

- **Engineering Sequential Mutations into Human HSPCs Yields an Aggressive Myeloid Malignancy Allowing for Interrogation of Preleukemic Transformation**
  Collins, C. T., Nakauchi, Y., Koehnke, T., Chavez, J. S., Choi, S., Sharma, R., Zhao, F., Majeti, R.
  AMER SOC HEMATOLOGY.2023

- **BCOR Loss Confers Increased Stemness and Partially Rescues RUNX1-Deficient Phenotypes in Human Hematopoietic Stem and Progenitor Cells**
  Jackson, K. K., Fan, A. C., Karigane, D., Zhao, F., Collins, C. T., Nakauchi, Y., Kayamori, K., Rangavajhula, A. S., Koehnke, T., Majeti, R.
  AMER SOC HEMATOLOGY.2023

- **Gene Correction of DNMT3A:R882H in Primary Human AML Demonstrates That This Mutation Is Not Required for Disease Maintenance, but Is Associated with Increased Leukemia Stem Cell Frequency**
  Koehnke, T., Karigane, D., Hilgart, E., Kayamori, K., Fan, A. C., Collins, C. T., Suchy, F. P., Rangavajhula, A. S., Feng, Y., Nakauchi, Y., Martinez-Montes, E., Koldobskiy, M., Feinberg, et al
  AMER SOC HEMATOLOGY.2023

- **Intra-Leukemic IFN. Signaling Mediates Cell Cycle Suppression and Chemoresistance in AML**
  Karigane, D., Fan, A. C., Kayamori, K., Nakauchi, Y., Koehnke, T., Rangavajhula, A. S., Ediriwickrema, A., Majeti, R.
  AMER SOC HEMATOLOGY.2023

- **Targeting IDH1-Mutated Pre-Leukemic Hematopoietic Stem Cells in Myeloid Disease, Including CCUS and AML**
  Landberg, N., Koehnke, T., Nakauchi, Y., Fan, A., Karigane, D., Thomas, D., Majeti, R.
  AMER SOC HEMATOLOGY.2022: 2234-2235

- **Dysregulated lipid synthesis by oncogenic IDH1 mutation is a targetable synthetic lethal vulnerability.** *Cancer discovery*
  Thomas, D., Wu, M., Nakauchi, Y., Zheng, M., Thompson-Peach, C. A., Lim, K., Landberg, N., Kohnke, T., Robinson, N., Kaur, S., Kutyna, M., Stafford, M., Hiwase, et al
  2022

- **Reengineering Ponatinib to Minimize Cardiovascular Toxicity** *CANCER RESEARCH*
  Hnatiuk, A. P., Bruyneel, A. A. N., Tailor, D., Pandrala, M., Dheeraj, A., Li, W., Serrano, R., Feyen, D. A. M., Vu, M. M., Amatya, P., Gupta, S., Nakauchi, Y., Morgado, et al
  2022; 82 (15): 2777-2791

- **IL-3 SELECTIVELY RESCUES RUNX1-DEFICIENT HUMAN HSPCS WITH DYSREGULATED JAK/ STAT SIGNALING**
  Fan, A., Azizi, A., Nuno, K., Nakauchi, Y., Zhao, F., Cruz-Hernandez, D., Reinisch, A., Majeti, R.
  ELSEVIER SCIENCE INC.2022: S84

- **IL-3 RESCUES PROLIFERATIVE DEFECTS IN INFLAMMATION-SENSITIVE RUNX1 DEFICIENT HUMAN HEMATOPOIETIC STEM AND PROGENITOR CELLS**
  Fan, A., Azizi, A., Dutta, R., Nakauchi, Y., Nuno, K., Zhao, F., Reinisch, A., Majeti, R.
  ELSEVIER SCIENCE INC.2020: S59

- **Enasidenib drives human erythroid differentiation independently of isocitrate dehydrogenase 2.** *The Journal of clinical investigation*
  Dutta, R. n., Zhang, T. Y., Köhnke, T. n., Thomas, D. n., Linde, M. n., Gars, E. n., Stafford, M. n., Kaur, S. n., Nakauchi, Y. n., Yin, R. n., Azizi, A. n., Narla, A. n., Majeti, et al
  2020

- **Use of polyvinyl alcohol for chimeric antigen receptor T-cell expansion.** *Experimental hematology*
  Nishimura, T., Hsu, I., Martinez-Krams, D. C., Nakauchi, Y., Majeti, R., Yamazaki, S., Nakauchi, H., Wilkinson, A. C.
  2019

- **Enasidenib Drives Maturation of Human Erythroid Precursors Independently of IDH2**
  Dutta, R., Zhang, T. Y., Koehnke, T., Thomas, D., Gars, E., Stafford, M., Nakauchi, Y., Kaur, S., Yin, R., Narla, A., Majeti, R.
  AMER SOC HEMATOLOGY.2019

- **Induction of Truncating ASXL1 Mutations in Human CD34+HSPCs Mimics Human ASXL1-Mutated Clonal Hematopoiesis and Progression to Myeloid Malignancies**
  Koehnke, T., Sharma, R., Nakauchi, Y., Reinisch, A., Majeti, R.

AMER SOC HEMATOLOGY.2019

- **MODELING THE PATHOGENESIS OF RUNX1 DEFICIENCY IN INHERITED AML PREDISPOSITION SYNDROMES IN PRIMARY CELLS**
  Fan, A., Nuno, K., Nakauchi, Y., Koehnke, T., Kim, S., Reinisch, A., Cruz-Hernandez, D., Majeti, R.
  ELSEVIER SCIENCE INC.2019: S65
- **Azacitidine and Ascorbate Inhibit the Competitive Outgrowth of Human TET2 Mutant HSPCs in a Xenograft Model of Pre-Leukemia**
  Nakauchi, Y., Thomas, D., Sharma, R., Corces, M., Reinisch, A., Cruz, D., Koehnke, T., Karigane, D., Fan, A., Majeti, R.
  AMER SOC HEMATOLOGY.2018
- **IDH1 Mutant AML Is Susceptible to Targeting De Novo Lipid Synthesis Independent of 2-Hydroxyglutarate and Has a Distinct Metabolic Profile from IDH2 Mutant AML**
  Thomas, D., Nakauchi, Y., Wu, M., Zheng, M., Sinha, S., Dill, D., Peltz, G., Majeti, R.
  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
- **Large-Scale Clonal Analysis Resolves Aging of the Mouse Hematopoietic Stem Cell Compartment.** *Cell stem cell*
  Yamamoto, R. n., Wilkinson, A. C., Ooehara, J. n., Lan, X. n., Lai, C. Y., Nakauchi, Y. n., Pritchard, J. K., Nakauchi, H. n.
  2018; 22 (4): 600–607.e4
- **Establishment of a Therapeutic Anti-Pan HLA-Class II Monoclonal Antibody That Directly Induces Lymphoma Cell Death via Large Pore Formation.** *PloS one*
  Matsuoka, S., Ishii, Y., Nakao, A., Abe, M., Ohtsuji, N., Momose, S., Jin, H., Arase, H., Sugimoto, K., Nakauchi, Y., Masutani, H., Maeda, M., Yagita, et al
  2016; 11 (3): e0150496
- **A Safeguard System for Induced Pluripotent Stem Cell-Derived Rejuvenated T Cell Therapy** *STEM CELL REPORTS*
  Ando, M., Nishimura, T., Yamazaki, S., Yamaguchi, T., Kawana-Tachikawa, A., Hayama, T., Nakauchi, Y., Ando, J., Ota, Y., Takahashi, S., Nishimura, K., Ohtaka, M., Nakanishi, et al
  2015; 5 (4): 597-608

PRESENTATIONS

- Novel Strategy to Treat Graft-versus-Host Disease with Allele-Specific Anti-HLA Monoclonal Antibody - 18th Annual Winter Meeting of the Korean Society of Blood and Marrow Transplantation (2/14/2014 - 2/15/2014)
- Novel Therapeutic Approach To Graft-Versus-Host Disease With Allele-Specific Anti-HLA Monoclonal Antibody - 55th American Society of Hematology Annual Meeting and Exposition (12/7/2013 - 12/10/2013)
- TET2 Disruption Alters Human Hematopoietic Stem/ Progenitor Cells Differentiation and Self-Renewal