



Matthew Porteus

Sutardja Chuk Professor of Definitive and Curative Medicine
Pediatrics - Stem Cell Transplantation

CONTACT INFORMATION

- **Administrative Contact**

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Bio

BIO

Dr. Porteus was raised in California and was a local graduate of Gunn High School before completing A.B. degree in "History and Science" at Harvard University where he graduated Magna Cum Laude and wrote an thesis entitled "Safe or Dangerous Chimeras: The recombinant DNA controversy as a conflict between differing socially constructed interpretations of recombinant DNA technology." He then returned to the area and completed his combined MD, PhD at Stanford Medical School with his PhD focused on understanding the molecular basis of mammalian forebrain development with his PhD thesis entitled "Isolation and Characterization of TES-1/DLX-2: A Novel Homeobox Gene Expressed During Mammalian Forebrain Development." After completion of his dual degree program, he was an intern and resident in Pediatrics at Boston Children's Hospital and then completed his Pediatric Hematology/Oncology fellowship in the combined Boston Children's Hospital/Dana Farber Cancer Institute program. For his fellowship and post-doctoral research he worked with Dr. David Baltimore at MIT and CalTech where he began his studies in developing homologous recombination as a strategy to correct disease causing mutations in stem cells as definitive and curative therapy for children with genetic diseases of the blood, particularly sickle cell disease. Following his training with Dr. Baltimore, he took an independent faculty position at UT Southwestern in the Departments of Pediatrics and Biochemistry before again returning to Stanford in 2010 as an Associate Professor. During this time his work has been the first to demonstrate that gene correction could be achieved in human cells at frequencies that were high enough to potentially cure patients and is considered one of the pioneers and founders of the field of genome editing—a field that now encompasses thousands of labs and several new companies throughout the world. His research program continues to focus on developing genome editing by homologous recombination as curative therapy for children with genetic diseases but also has interests in the clonal dynamics of heterogeneous populations and the use of genome editing to better understand diseases that affect children including infant leukemias and genetic diseases that affect the muscle. Clinically, Dr. Porteus attends at the Lucille Packard Children's Hospital where he takes care of pediatric patients undergoing hematopoietic stem cell transplantation.

ACADEMIC APPOINTMENTS

- Professor, Pediatrics - Stem Cell Transplantation
- Member, Bio-X
- Member, Cardiovascular Institute
- Member, Institute for Stem Cell Biology and Regenerative Medicine
- Member, Maternal & Child Health Research Institute (MCHRI)

- Faculty Fellow, Sarafan ChEM-H
- Member, Stanford Cancer Institute
- Member, Wu Tsai Neurosciences Institute

LINKS

- Porteus Lab: <http://porteuslab.stanford.edu/>

Publications

PUBLICATIONS

- **Integration of affinity-dependent modulation and CD5/TRAC knockout generates fratricide-resistant and highly cytotoxic anti-CD5 CAR-T cells**
Kim, S., Jeong, J., Park, J., Lee, Y., Kim, H., Kang, H., Porteus, M. H., Feldman, S., Koh, Y.
AMER ASSOC CANCER RESEARCH.2026
- **T-allo10 Cell Addback Post-Abdepleted-HSCT Drives Lasting Leukemia-Free Survival with Distinct Immune Regulation Biomarkers**
Bertaina, A., Bacchetta, R., Shyr, D. C., Saini, G., Lee, J., Agarwal, R., Klein, O. R., Wang, G., Margittai-Gotesman, D., Al Dakheel, A., Barbarito, G., Oppizzi, L., Limaye, et al
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- **Harnessing DNA barcoding to enhance and sustain polyclonality in gene-edited hematopoietic stem cells.** *Molecular therapy. Methods & clinical development*
Ojeda-Perez, I., Bustos, A., Selvaraj, S., Fañanas-Baquero, S., Alberquilla-Fernandez, O., Serrano, L. J., Bonafont, J., Garcia-Torralba, A., Torres-Ruiz, R., Rodriguez-Perales, S., Lang, V., Trigueros, C., Mayo-Garcia, et al
2025; 33 (4): 101615
- **Novel humanized loss-of-function NF1 mouse model of juvenile myelomonocytic leukemia.** *Blood advances*
Sinha, R., Patil, R. V., Romano, R., Sharma, D., Lee, E., Perriman, R., Takeda, S., Lesch, B. J., Yao, Z., Liu, Y. L., Cromer, M. K., Porteus, M. H., Bertaina, et al
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- **THERAPEUTIC DOSES OF T-ALLO10 IMMUNOTHERAPY POST-ABDEPLETED HSCT ENHANCE IMMUNE RECONSTITUTION AND ACHIEVE 100% LEUKEMIA-FREE SURVIVAL IN PEDIATRIC AND YOUNG ADULT PATIENTS WITH HEMATOLOGIC MALIGNANCIES**
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SPRINGER NATURE.2025: 74-75
- **Unveiling the cut-and-repair cycle of designer nucleases in human stem and T cells via CLEAR-time dPCR.** *Nature communications*
White, N., Chalk, J. A., Hu, Y. T., Pins, S. M., Joseph, C. R., Antoniou, P., Wimberger, S., Svensson, S., Caetano-Silva, S. P., Mudde, A. C., Rai, R., Selvaraj, S., Feist, et al
2025; 16 (1): 9571
- **Hematopoietic stem cell transplantation using briquilimab (Anti-CD117 Antibody-Conditioning), immunosuppression and TCRαβ+ T-cell/CD19+B-cell depleted haploidentical grafts in patients with fanconi anemia: An approach without irradiation, busulfan and calcineurin inhibitors.**
Agarwal, R., Bertaina, A., Soco, C., Long-Boyle, J., Saini, G., Kunte, N., Hiroshima, L., Chan, Y., Willner, H., Krampf, M., Van Hentenryck, M., Perriman, R., Istomina, et al
ELSEVIER.2025: 2432-2433
- **IL10 gene mutations do not impair Tr1 differentiation in vivo and can be functionally corrected by precise gene editing**
Strubbe, S., Boss, A., Wang, B., Mantilla, M., Freeborn, R., Guevara Becerra, M., Gharahdaghi, N., Uhlig, H. H., Porteus, M., Arkwright, P. D., Roncarolo, M., Bacchetta, R.
OXFORD UNIV PRESS.2025
- **A human arteriovenous differentiation roadmap reveals vein developmental mechanisms and vascular effects of viruses.** *bioRxiv : the preprint server for biology*
Ang, L. T., Zheng, S. L., Liu, K. J., Masaltseva, A., Winters, J., von Creyzt, I., Jha, S. K., Yin, Q., Qian, C., Xiong, X., Dailamy, A., Xi, E., Alcocer, et al
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- **Overcoming Barriers to Commercially Pre-Viable Gene and Cell Therapies for Rare and Ultra-Rare Diseases.** *Molecular therapy : the journal of the American Society of Gene Therapy*
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- **Global Observatory for Genome Editing Summit Statement: Call for a Charter on Emerging Technologies and Human Dignity.** *The CRISPR journal*
Baylis, F., Evans, J., Hunt, T., Hurlbut, J. B., Jasanoff, S., Kysar, D., Littler, K., Lwoff, L., Mills, P., Moses, J., Porteus, M., Saha, K., Snead, et al
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- **Irradiation- and busulfan-free stem cell transplantation in Fanconi anemia using an anti-CD117 antibody: a phase 1b trial.** *Nature medicine*
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- **Rewiring endogenous genes in CAR T cells for tumour-restricted payload delivery.** *Nature*
Chen, A. X., Yap, K. M., Kim, J. S., Sek, K., Huang, Y. K., Dunbar, P. A., Wiebking, V., Armitage, J. D., Munoz, I., Todd, K. L., Derrick, E. B., Nguyen, D., Tong, et al
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- **A Reset for Bioethics: A Statement from the Global Observatory for Genome Editing.** *The CRISPR journal*
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- **Allele-Specific CRISPR/AAV6 Gene Correction of Dominant *JAK2*-V617F Mutation in Polycythemia Vera**
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- **DNA repair precision after gene editing, unveiled**
Turchiano, G., White, N., Chalk, A., Hu, Y., Pins, S., Antoniou, P., Wimberger, S., Svensson, S., Silva, S., Mudde, A., Rai, R., Selvaraj, S., Feist, et al
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- **Fast Quantification of Genome-Editing Outcomes using CRISPR-based detection**
Blanluet, C., Sperber, H., Selvaraj, S., Porteus, M.
CELL PRESS.2025
- **A hybrid gene correction strategy for Cystic Fibrosis**
Amaya, A., Sun, Y., Chatterjee, S., Chen, Z., Siegart, D., Porteus, M.
CELL PRESS.2025
- **Improved Prediction and Activation of Homology-Directed Repair in Translational Ex-vivo Systems**
Turk, R., Kurgan, G., Glenn, S., Woodley, J., Selvaraj, S., Schmiderer, L., Vargas, J., Sturgeon, M., Thommandru, B., Murugan, K., Wallace, K., Rettig, G., Vakulskas, et al
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- **Multilayered HIV-1 resistance in HSPCs through CCR5 Knockout and B cell secretion of HIV-inhibiting antibodies.** *Nature communications*
Feist, W. N., Luna, S. E., Ben-Efraim, K., Filsinger Interrante, M. V., Amorin, A., Johnston, N. M., Bruun, T. U., Utz, A., Ghanim, H. Y., Lesch, B. J., McLaughlin, T. M., Dudek, A. M., Porteus, et al
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- **Human striatal progenitor cells that contain inducible safeguards and overexpress BDNF rescue Huntington's disease phenotypes.** *Molecular therapy. Methods & clinical development*
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- **Engineering synthetic signaling receptors to enable erythropoietin-free erythropoiesis.** *Nature communications*
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- **Immune composition of the mononuclear cell fraction of human umbilical cord blood.** *Frontiers in immunology*
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- **TET2 regulates early and late transitions in exhausted CD8+ T cell differentiation and limits CAR T cell function.** *Science advances*
Dimitri, A. J., Baxter, A. E., Chen, G. M., Hopkins, C. R., Rouin, G. T., Huang, H., Kong, W., Holliday, C. H., Wiebking, V., Bartoszek, R., Drury, S., Dalton, K., Koucky, et al
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- **Evaluation of Bone Marrow in Fanconi Anemia Patients Treated with Briquilimab Antibody-Based Conditioning and TCR $\alpha\beta$ B-Cell Depleted Haploidentical Grafts**
Soco, C., Krampf, M. R., Chan, Y., Hoang, H., Kunte, N., Saini, G., Weinberg, K. I., Parkman, R., Bertaina, A., Agarwal, R., Porteus, M., Czechowicz, A. D.
ELSEVIER.2024: 2015-2016
- **Targeting the *JAK2*-V617F Mutation in Polycythemia Vera Using CRISPR/AAV6 Genome Editing**
Ekman, F., Selvaraj, S., Gotlib, J., Cromer, M., Porteus, M. H.
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- **T-allo10 Immunotherapy Results in Enhanced Early Immune Reconstitution and Reduced GvHD with Excellent Clinical Outcomes Post A β -Depleted HSCT in Pediatric and Young Adult Hematologic Malignancy Patients**
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- **Developing a Genome Editing Strategy for the Treatment of Alpha-Thalassemia Major**
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- **Cell and gene therapy accessibility.** *Science (New York, N.Y.)*
Rouce, R. H., Porteus, M. H.
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- **Enhancement of erythropoietic output by Cas9-mediated insertion of a natural variant in haematopoietic stem and progenitor cells.** *Nature biomedical engineering*
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- **A Story of Perseverance: An Interview with Matthew Porteus.** *The CRISPR journal*
Porteus, M. H., Davies, K.
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- **Dual α -globin and truncated EPO receptor knockin restores hemoglobin production in α -thalassemia-derived red blood cells.** *bioRxiv : the preprint server for biology*
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- **Engineering Inducible Signaling Receptors for Erythropoietin-Free Erythropoiesis**
Shah, A. P., Majeti, K. R., Porteus, M. H., Cromer, K.
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- **Endogenous Human TRIM Protein Expression Influences Potency of a Knock-In Human-Rhesus Chimeric Protein Expression Platform for CXCR4-Tropic HIV-1 Cure**
Dudek, A. M., Sasu, E. J., Porteus, M. H.
CELL PRESS.2024: 570
- **Advancing X-Linked Agammaglobulinemia Therapy: A Universal Gene Targeting-Based Correction**
Ghanim, H. Y., Pavel-Dinu, M., Feist, W., Fesseha, R., Luna, S., Porteus, M. H.
CELL PRESS.2024: 772-773
- **Further Investigation of HBB Gene Editing with CRISPR/Cas9/rAAV6 in Human HSPCs to Treat Sickle Cell Disease**
Xu, L., Selvaraj, S., Porteus, M. H.
CELL PRESS.2024: 555
- **Improving Therapeutic Homology Directed Repair Genome Editing Efficiency in Human Primary Cells through Optimal Guide RNA Selection**
Selvaraj, S., Johnston, N., Schmiderer, L., Amorin, N. A., Porteus, M. H.
CELL PRESS.2024: 359-360
- **A simultaneous knockout knockin genome editing strategy in HSPCs potently inhibits CCR5- and CXCR4-tropic HIV-1 infection.** *Cell stem cell*
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- **Investigating adverse genomic and regulatory changes caused by replacement of the full-lengthCFTRcDNA using Cas9 and AAV.** *Molecular therapy. Nucleic acids*
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2024; 35 (1): 102134
- **Gene regulation in inborn errors of immunity: Implications for gene therapy design and efficacy.** *Immunological reviews*
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- **Transient inhibition of 53BP1 increases the frequency of targeted integration in human hematopoietic stem and progenitor cells.** *Nature communications*
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- **Epitope-engineered human hematopoietic stem cells are shielded from CD123-targeted immunotherapy.** *The Journal of experimental medicine*
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- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *Molecular biology of the cell*
Riepe, C., Wąchalska, M., Deol, K. K., Amaya, A. K., Porteus, M. H., Olzmann, J. A., Kopito, R. R.
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- **Radiation and Busulfan-Free Hematopoietic Stem Cell Transplantation Using Briquilimab (JSP191) Anti-CD117 Antibody-Conditioning, Transient Immunosuppression and TCR $\alpha \beta$ + T-Cell/CD19+B-Cell Depleted Haploidentical Grafts in Patients with Fanconi Anemia**
Agarwal, R., Bertaina, A., Soco, C., Saini, G., Kunte, N., Hiroshima, L., Chan, Y., Willner, H., Krampf, M. L., Nofal, R., Barbarito, G., Sen, S., Felber, et al
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- **Using Inducible Signaling Receptors to Increase Erythropoietic Output from Genome-Edited Hematopoietic Stem Cells**
Majeti, K. R., Shah, A. P., Luna, S. E., Soupene, E., Chu, S. N., Sharma, D., Porteus, M., Cromer, K.
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- **Using Human Genetics to Develop Strategies to Increase Erythropoietic Output from Genome-Edited Hematopoietic Stem and Progenitor Cells**
Luna, S. E., Camarena, J., Hampton, J. P., Majeti, K. R., Charlesworth, C. T., Soupene, E., Sheehan, V. A., Cromer, K., Porteus, M.
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- **Dual α -Globin and Truncated EPO Receptor Knockin Restores Hemoglobin Production in α -Thalassemia-Derived Hematopoietic Stem and Progenitor Cells**
Chu, S. N., Soupene, E., Wienert, B., Yin, H., Sharma, D., Jia, K., Homma, S., Hampton, J. P., Conklin, B., MacKenzie, T. C., Porteus, M., Cromer, K.
AMER SOC HEMATOLOGY.2023
- **One Year Follow-up on the First Patient Treated with Nula-Cel: An Autologous CRISPR/Cas9 Gene Corrected CD34+Cell Product to Treat Sickle Cell Disease**
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- **Towards Automated Engineering of Donor-Derived T Lymphocytes into CRISPR/Cas9-Mediated CAR T Cells in a Closed-System**
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- **Molecular dynamics of genome editing with CRISPR-Cas9 and rAAV6 virus in human HSPCs to treat sickle cell disease.** *Molecular therapy. Methods & clinical development*
Xu, L., Lahiri, P., Skowronski, J., Bhatia, N., Lattanzi, A., Porteus, M. H.
2023; 30: 317-331
- **High-efficiency transgene integration by homology-directed repair in human primary cells using DNA-PKcs inhibition.** *Nature biotechnology*
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- **Combined lineage tracing and scRNA-seq reveals unexpected first heart field predominance of human iPSC differentiation.** *eLife*
Galdos, F. X., Lee, C., Lee, S., Paige, S., Goodyer, W., Xu, S., Samad, T., Escobar, G. V., Darsha, A., Beck, A., Bak, R. O., Porteus, M. H., Wu, et al
2023; 12
- **A PILOT TO INVESTIGATE RESTING STATE IN PATIENTS WITH SICKLE CELL DISEASE POST STEM CELL TRANSPLANT**
Alva, H., Marzelli, M., Foland-Ross, L., Porteus, M., Reiss, A.
WILEY.2023: S168
- **Discovery of Key Transcriptional Regulators of Alloantigen-Inducible Tregs Used for Cell Therapy**
Cepika, A., Amaya, L., Waichler, C., Narula, M., Thomas, B. C., Chen, P. P., Mantilla, M. M., Pavel-Dinu, M., Freeborn, R., Porteus, M. H., Bacchetta, R., Mueller, F., Greenleaf, et al
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- **Base-Editing as a Safe and Highly Effective Alternative Treatment for X-SCID Compared to CRISPR-Cas9 Nuclease Editing with an AAV Donor**
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- **CRISPR/Cas9 Based Genome Editing to Replace the Full-Length CFTR cDNA Shows Promising Restoration of CFTR Function and Safety in Pre-Clinical Studies**
Vaidyanathan, S., Paranjapye, A., Kerschner, J. L., Thrasher, A. J., Turchiano, G., Harris, A., Porteus, M. H.
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- **Measuring Small Molecule Improvements in Genome Editing for Pyruvate Kinase Deficiency Using DNA Barcoding Templates**
Ojeda Perez, I., Selvaraj, S., Bustos, A., Javier Serrano, L., Bonafont, J., Alberquilla-Fernandez, O., Amarin, N. A., Garcia-Torralba, A., Torres-Ruiz, R., Rodriguez-Perales, S., Trigueros, C., Mayo-Garcia, R., Quintana-Bustamante, et al
CELL PRESS.2023: 540
- **Engineering a Potential Curative Treatment for Hemophilia A Using an AAV Dual Targeting Strategy**
Johnston, N., Amarin, A., Nguyen, L., Ghanim, H., Porteus, M. H.
CELL PRESS.2023: 267
- **Increasing Erythropoietic Output from Genome-Edited Hematopoietic Stem and Progenitor Cells Using a Truncated EPO Receptor**
Luna, S. E., Camarena, J., Hampton, J. P., Majeti, K. R., Charlesworth, C. T., Soupene, E., Sheehan, V., Cromer, K., Porteus, M. H.
CELL PRESS.2023: 538-539
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