Stanford

Matthew Porteus

Sutardja Chuk Professor of Definitive and Curative Medicine

Pediatrics - Stem Cell Transplantation

CONTACT INFORMATION

- **Administrative Contact**

  Ginger Exley - Administrative Assistant

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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 Chidlren’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

- **Gene regulation in inborn errors of immunity: Implications for gene therapy design and efficacy.** *Immunological reviews*
  Ghanim, H. Y., Porteus, M. H.
  2024
- **Transient inhibition of 53BP1 increases the frequency of targeted integration in human hematopoietic stem and progenitor cells.** *Nature communications*
  Baik, R., Cromer, M. K., Glenn, S. E., Vakulskas, C. A., Chmielewski, K. O., Dudek, A. M., Feist, W. N., Klermund, J., Shipp, S., Cathomen, T., Dever, D. P., Porteus, M. H.
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- **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
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- **iPSC-derived hypoimmunogenic tissue resident memory Tcells mediate robust anti-tumor activity against cervical cancer.** *Cell reports. Medicine*
  Furukawa, Y., Ishii, M., Ando, J., Ikeda, K., Igarashi, K. J., Kinoshita, S., Azusawa, Y., Toyota, T., Honda, T., Nakanishi, M., Ohshima, K., Masuda, A., Yoshida, et al
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- **Epitope-engineered human hematopoietic stem cells are shielded from CD123-targeted immunotherapy.** *The Journal of experimental medicine*
  Marone, R., Landmann, E., Devaux, A., Lepore, R., Seyres, D., Zuin, J., Burgold, T., Engdahl, C., Capoferri, G., Dell'Aglio, A., Larrue, C., Simonetta, F., Rositzka, et al
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- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *Molecular biology of the cell*
  Riepe, C., W#chalska, M., Deol, K. K., Amaya, A. K., Porteus, M. H., Olzmann, J. A., Kopito, R. R.
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- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *bioRxiv : the preprint server for biology*
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  2023
- **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*
  Selvaraj, S., Feist, W. N., Viel, S., Vaidyanathan, S., Dudek, A. M., Gastou, M., Rockwood, S. J., Ekman, F. K., Oseghale, A. R., Xu, L., Pavel-Dinu, M., Luna, S. E., Cromer, et al
  2023
- **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.
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- **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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- **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.
  CELL PRESS.2023: 710-711

- **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., Amorin, N. A., Garcia-Torralba, A., Torres-Ruiz, R., Rodriguez-Perales, S., Trigueros, C., Mayo-Garcia, R., Quintana-Bustamante, et al
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- **Engineering a Potential Curative Treatment for Hemophilia A Using an AAV Dual Targeting Strategy**
  Johnston, N., Amorin, A., Nguygen, L., Ghanim, H., Porteus, M. H.
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- **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

- **Establishing Multilayered Genetic Resistance to HIV-1 by Engineering Hematopoietic Stem and Progenitor Cells for B Cell Specific Secretion of Therapeutic Antibodies**
  Feist, W., Luna, S., Ben-Efraim, K., Interrante, M., Amorin, N. A., Johnston, N., Dudek, A., Porteus, M. H.
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- **Molecular Dynamics of Genome Editing with CRISPR/Cas9 and rAAV6 Virus in Human HSPCs to Treat Sickle Cell Disease**
  Xu, L., Lahiri, P., Skowronski, J., Bhatia, N., Lattanzi, A., Porteus, M. H.
  CELL PRESS.2023: 262-263

- **Engineering Inducible Signaling Receptors to Increase Erythropoietic Output from Genome-Edited Hematopoietic Stem Cells**
  Shah, A. P., Majeti, K., Luna, S., Porteus, M. H., Cromer, K.
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- **Delivery of BDNF through a Pluripotent Stem Cell-Based Platform Ameliorates Behavioral Deficits in a Mouse Model of Huntington's Disease**
  Selvaraj, S., Simmons, D. A., Chen, T., Cao, G. Y., Camelo, T. S., McHugh, T. M., Gonzalez, S., Martin, R. M., Simanauskaite, J. M., Uchida, N., Longo, F. M., Porteus, M. H.
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- **Comparative analysis of CRISPR off-target discovery tools following ex vivo editing of CD34+ hematopoietic stem and progenitor cells.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Cromer, M. K., Majeti, K. R., Rettig, G. R., Murugan, K., Kurgan, G. L., Bode, N. M., Hampton, J. P., Vakulskas, C. A., Behlke, M. A., Porteus, M. H.
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- **Engineered Single Amino Acid Substitutions Protect Hematopoietic Stem and Progenitor Cells from CD123 Targeted Immunotherapy**
  Landmann, E., Devaux, A., Lepore, R., Marone, R., Engdahl, C., Hasiuk, M., Capoferri, G., Wiederkehr, A., Wellinger, L. C., Sinopoli, A., Do Sacramento, V., Haydn, A., Prat, et al
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- **Novel Humanized Loss-of-Function NF1 Mouse Model of Juvenile Myelomonocytic Leukemia**
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  AMER SOC HEMATOLOGY.2022: 9765-9766

- **Ultra-deep sequencing validates safety of CRISPR/Cas9 genome editing in human hematopoietic stem and progenitor cells.** *Nature communications*

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- **Reengineering Ponatinib to Minimize Cardiovascular Toxicity** *CANCER RESEARCH*
  Hnatiuk, A. P., Bruyneel, A. N., Tailor, D., Pandrala, M., Dheeraj, A., Li, W., Serrano, R., Feyen, D. M., Vu, M. M., Amatya, P., Gupta, S., Nakauchi, Y., Morgado, et al
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- **Generating human artery and vein cells from pluripotent stem cells highlights the arterial tropism of Nipah and Hendra viruses.** *Cell*
  Ang, L. T., Nguyen, A. T., Liu, K. J., Chen, A., Xiong, X., Curtis, M., Martin, R. M., Raftry, B. C., Ng, C. Y., Vogel, U., Lander, A., Lesch, B. J., Fowler, et al
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- **Author Correction: Investigation of Cas9 antibodies in the human eye.** *Nature communications*
  Toral, M. A., Charlesworth, C. T., Ng, B., Chemudupati, T., Homma, S., Nakauchi, H., Bassuk, A. G., Porteus, M. H., Mahajan, V. B.
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- **Two is Better Than One: CRISPR/Cas9 Based Gene Editing with FOXP3 Isoforms for IPEX Therapy**
  Lee, E., Borna, S., Sato, Y., Bacchetta, R., Roncarolo, M., Porteus, M.
  CELL PRESS.2022: 34

- **A Universal Correction Strategy for alpha-Thalassemia Using CRISPR/AAV-Mediated Genome Editing**
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- **A Simultaneous Knock-Out Knock-In Gene Editing Strategy in HSPCs Potently Inhibits R5-and X4-Tropic HIV Replication**
  Dudek, A. M., Feist, W. N., Porteus, M. H.
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- **Transplantation of Gene Edited Upper Airway Basal Stem Cells in Immunocompromised Mice Using Fibrinogen Based Scaffolds**
  Vaidyanathan, S., Bravo, D. T., Desai, T. J., Porteus, M. H., Nayak, J. V.
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- **CEDAR Trial in Progress: A First in Human, Phase 1/2 Study of the Correction of a Single Nucleotide Mutation in Autologous HSCs (GPH101) to Convert HbS to HbA for Treating Severe Sickle Cell Disease**
  Di Persio, J. F., Kanter, J., Leavey, P., Shyr, D. C., Thompson, A. A., Porteus, M. H., Intondi, A., Lahiri, P., Dever, D., Petrusich, A., Lehrer-Graiwer, J.
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- **Design of experiments as a decision tool for cell therapy manufacturing.** *Cytotherapy*
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  Brault, J., Liu, T., Liu, S., Lawson, A., Choi, U., Kozhushko, N., Bzhilyanskaya, V., Pavel-Dinu, M., Meis, R. J., Eckhaus, M. A., Burkett, S. S., Bosticardo, M., Kleinstiver, et al
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- **GENOME EDITING OF HEMATOPOIETIC STEM CELLS TO ENGINEER BLOOD**
  Porteus, M.
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- **Hematopoietic stem cell gene editing and expansion: state-of-the-art technologies and recent applications.** *Experimental hematology*
  Haltalli, M. L., Wilkinson, A. C., Rodriguez-Fraticelli, A., Porteus, M.
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- **Cedar Trial in Progress: A First in Human, Phase 1/2 Study of the Correction of a Single Nucleotide Mutation in Autologous HSCs (GPH101) to Convert HbS to HbA for Treating Severe SCD**
  Kanter, J., DiPersio, J. F., Leavey, P., Shyr, D. C., Thompson, A. A., Porteus, M. H., Intondi, A., Lahiri, P., Dever, D. P., Petrusich, A., Lehrer-Graiwer, J.
  AMER SOC HEMATOLOGY.2021: 1864-+

- **Clinically relevant gene editing in hematopoietic stem cells for the treatment of pyruvate kinase deficiency.** *Molecular therapy. Methods & clinical development*
  Fananas-Baquero, S., Quintana-Bustamante, O., Dever, D. P., Alberquilla, O., Sanchez-Dominguez, R., Camarena, J., Ojeda-Perez, I., Dessy-Rodriguez, M., Turk, R., Schubert, M. S., Lattanzi, A., Xu, L., Lopez-Lorenzo, et al
  2021; 22: 237-248

- **An Unusual "OR" Gate for Allosteric Regulation of Mammalian Transglutaminase 2 in the Extracellular Matrix** *JOURNAL OF THE AMERICAN CHEMICAL SOCIETY*
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- **Development of beta-globin gene correction in human hematopoietic stem cells as a potential durable treatment for sickle cell disease.** *Science translational medicine*
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- **Evaluating the Use of Fibrinogen Based Scaffolds to Transplant Airway Basal Stem Cells for the Treatment of Cystic Fibrosis**
  Vaidyanathan, S., Bravo, D. T., Nayak, J. V., Porteus, M. H.
  CELL PRESS.2021: 260-261

- **GMEB2 is a Conserved Cellular AAV Restriction Factor That Inhibits Transduction of Human Stem Cells**
  Dudek, A. M., Johnston, N. M., Vaidyanathan, S., Selvaraj, S., Porteus, M. H.
  CELL PRESS.2021: 48-49

- **Correction to: Gene Editing Rescues in Vitro T Cell Development of RAG2-Deficient Induced Pluripotent Stem Cells in an Artificial Thymic Organoid System.** *Journal of clinical immunology*
  Gardner, C. L., Pavel-Dinu, M., Dobbs, K., Bosticardo, M., Reardon, P. K., Lack, J., DeRavin, S. S., Le, K., Bello, E., Pala, F., Delmonte, O. M., Malech, H., Montel-Hagen, et al
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- **Gene replacement of alpha-globin with beta-globin restores hemoglobin balance in beta-thalassemia-derived hematopoietic stem and progenitor cells.** *Nature medicine*
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  Wilkinson, A. C., Dever, D. P., Baik, R., Camarena, J., Hsu, I., Charlesworth, C. T., Morita, C., Nakauchi, H., Porteus, M. H.
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- **Safe and Effective In Vivo Targeting and Gene Editing in Hematopoietic Stem Cells: Strategies for Accelerating Development National Institutes of Health/Bill & Melinda Gates Foundation Expert Scientific Roundtable Webinar Meeting.** *Human gene therapy*
  Cannon, P., Asokan, A., Czechowicz, A., Hammond, P., Kohn, D. B., Lieber, A., Malik, P., Marks, P., Porteus, M., Verhoeyen, E., Weissman, D., Weissman, I., Kiem, et al
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  Dudek, A. M., Porteus, M. H.
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- **Targeted replacement of full-length CFTR in human airway stem cells by CRISPR/Cas9 for pan-mutation correction in the endogenous locus.** *Molecular therapy : the journal of the American Society of Gene Therapy*
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- **Correction of Recessive Dystrophic Epidermolysis Bullosa by homology-directed repair-mediated genome editing.** *Molecular therapy : the journal of the American Society of Gene Therapy*
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- **The Binns Program for Cord Blood Research: A novel model of cord blood banking for academic biomedical research.** *Placenta*
  Mantri, S., Sheikali, A., Binns, C., Lyell, D. J., DiGiusto, D. L., Porteus, M. H., Agarwal-Hashmi, R.
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- **Genome-edited Human Hematopoietic Stem Cells Correct Lysosomal Storage Disorders**
  Poletto, E., Scharenberg, S. G., Channabasavaiah, G. B., Porteus, M., Gomez-Ospina, N.
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- **CAS9-AAV6 GENE CORRECTION OF AUTOLOGOUS HSCS IMPROVES SICKLE CELL DISEASE ERYTHROPOIESIS IN MICE**
  Wilkinson, A., Dever, D., Baik, R., Hsu, I., Camarena, J., Charlesworth, C., Morita, C., Nakauchi, H., Porteus, M.
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  Wiebking, V., Patterson, J. O., Martin, R., Chanda, M. K., Lee, C. M., Srifa, W., Bao, G., Porteus, M. H.
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- **Lentivector versus CRISPR/Cas9/AAV6 Gene Editing in X-Linked Severe Combined Immunodeficiency CD34(+) Hematopoietic Cells**
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  Bonafont, J., Mencia, A., Srifa, W., Vaidyanathan, S., Romano, R., Garcia, M., Jose Escamez, M., Duarte, B., Porteus, M., Larcher, F., Del Rio, M., Murillas, R.
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  Lahiri, P., Dever, D. P., Lattanzi, A., Talbott, N., Skowronski, J., Llontop, P., Vu, N., Cole, T., Fazeli, F., Segal, H., Soni, S., Porteus, M. H., Bhatia, et al
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