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

- **Integration of affinity-dependent modulation and CD5/TRAC knockout generates fratricide-resistant and highly cytotoxic anti-CD5 CAR-T cells**
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- **T-allo10 Cell Addback Post-Abdepleted-HSCT Drives Lasting Leukemia-Free Survival with Distinct Immune Regulation Biomarkers**
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- **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
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- **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.
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- **A human arteriovenous differentiation roadmap reveals vein developmental mechanisms and vascular effects of viruses.** *bioRxiv : the preprint server for biology*
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- **Rewiring endogenous genes in CAR T cells for tumour-restricted payload delivery.** *Nature*
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  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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  Blanluet, C., Sperber, H., Selvaraj, S., Porteus, M.
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- **A hybrid gene correction strategy for Cystic Fibrosis**
  Amaya, A., Sun, Y., Chatterjee, S., Chen, Z., Siegwart, D., Porteus, M.
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- **Allele-Specific CRISPR/AAV6 Gene Correction of Dominant JAK2-V617F Mutation in Polycythemia Vera**
  Ekman, F., Selvaraj, S., Gotlib, J., Cromer, K., Porteus, M.
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- **Engineered Hematopoietic Stem Cells Give Rise to Therapeutic Antibody Secreting B Cells**
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- **Multilayered HIV-1 resistance in HSPCs through CCR5 Knockout and B cell secretion of HIV-inhibiting antibodies.** *Nature communications*
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- **T-allo10 Immunotherapy Results in Enhanced Early Immune Reconstitution and Reduced GvHD with Excellent Clinical Outcomes Post Aβ-Depleted HSCT in Pediatric and Young Adult Hematologic Malignancy Patients**
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- **Evaluation of Bone Marrow in Fanconi Anemia Patients Treated with Briquilimab Antibody-Based Conditioning and TCRαβ⁺ T-Cell/CD19⁺ B-Cell Depleted Haploidentical Grafts**
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- **Human assembloids reveal the consequences of CACNA1G gene variants in the thalamocortical pathway.** *Neuron*
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  Porteus, M. H., Davies, K.
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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.
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- **Advancing X-Linked Agammaglobulinemia Therapy: A Universal Gene Targeting-Based Correction**
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  Xu, L., Selvaraj, S., Porteus, M. H.
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- **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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- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *Molecular biology of the cell*
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  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**
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- **Using Human Genetics to Develop Strategies to Increase Erythropoietic Output from Genome-Edited Hematopoietic Stem and Progenitor Cells**
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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.
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- **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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- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *bioRxiv : the preprint server for biology*
  Riepe, C., Wachalska, M., Deol, K. K., Amaya, A. K., Porteus, M. H., Olzmann, J. A., Kopito, R. R.
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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.
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- **A PILOT TO INVESTIGATE RESTING STATE IN PATIENTS WITH SICKLE CELL DISEASE POST STEM CELL TRANSPLANT**
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  Bzhilyanskaya, V., Brault, J., Liu, S., Kozhushko, N., Lawson, A., Pavel-Dinu, M., Clark, A. B., Meis, R. J., Ma, M., Lazzarotto, C. R., Tsai, S. Q., Wu, X., Dahl, et al
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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., 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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