



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

- **Human striatal progenitor cells that contain inducible safeguards and overexpress BDNF rescue Huntington's disease phenotypes.** *Molecular therapy. Methods & clinical development*
Simmons, D. A., Selvaraj, S., Chen, T., Cao, G., Camelo, T. S., McHugh, T. L., Gonzalez, S., Martin, R. M., Simanaukaite, J., Uchida, N., Porteus, M. H., Longo, F. M.
2025; 33 (1): 101415
- **Human striatal progenitor cells that contain inducible safeguards and overexpress BDNF rescue Huntington's disease phenotypes** *MOLECULAR THERAPY METHODS & CLINICAL DEVELOPMENT*
Simmons, D. A., Selvaraj, S., Chen, T., Cao, G., Camelo, T., Mchugh, T. M., Gonzalez, S., Martin, R. M., Simanaukaite, J., Uchida, N., Porteus, M. H., Longo, F. M.
2025; 33 (1)
- **Engineering synthetic signaling receptors to enable erythropoietin-free erythropoiesis.** *Nature communications*
Shah, A. P., Majeti, K. R., Ekman, F. K., Selvaraj, S., Sharma, D., Sinha, R., Soupene, E., Chati, P., Luna, S. E., Charlesworth, C. T., McCreary, T., Lesch, B. J., Tran, et al
2025; 16 (1): 1140
- **Durable reconstitution of sinonasal epithelium by transplant of CFTR gene corrected airway stem cells.** *bioRxiv : the preprint server for biology*
Bravo, D. T., Vaidyanathan, S., Baker, J., Sinha, V., Tsai, E., Roozdar, P., Kong, W. W., Atkinson, P. J., Patel, Z. M., Hwang, P. H., Rao, V. K., Negrin, R. S., Wine, et al
2025
- **Dual α -globin-truncated erythropoietin receptor knockin restores hemoglobin production in α -thalassemia-derived erythroid cells.** *Cell reports*
Chu, S. N., Soupene, E., Sharma, D., Sinha, R., McCreary, T., Hernandez, B., Shen, H., Wienert, B., Bowman, C., Yin, H., Lesch, B. J., Jia, K., Romero, et al
2025; 44 (1): 115141
- **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
2024; 10 (46): eadp9371
- **Developing a Genome Editing Strategy for the Treatment of Alpha-Thalassemia Major**
Chu, S., Soupene, E., Wienert, B., Sharma, D., Jia, K., Conklin, B., Mackenzie, T., Porteus, M., Cromer, K.
LIPPINCOTT WILLIAMS & WILKINS.2024: S347
- **Human assembloids reveal the consequences of CACNA1G gene variants in the thalamocortical pathway.** *Neuron*
Kim, J. I., Miura, Y., Li, M. Y., Revah, O., Selvaraj, S., Birey, F., Meng, X., Thete, M. V., Pavlov, S. D., Andersen, J., Paşca, A. M., Porteus, M. H., Huguenard, et al
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- **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
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- **Cell and gene therapy accessibility.** *Science (New York, N.Y.)*
Rouce, R. H., Porteus, M. H.
2024; 385 (6708): 475
- **Enhancement of erythropoietic output by Cas9-mediated insertion of a natural variant in haematopoietic stem and progenitor cells.** *Nature biomedical engineering*
Luna, S. E., Camarena, J., Hampton, J. P., Majeti, K. R., Charlesworth, C. T., Soupene, E., Selvaraj, S., Jia, K., Sheehan, V. A., Cromer, M. K., Porteus, M. H.
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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.
CELL PRESS.2024: 154
- **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*
Dudek, A. M., Feist, W. N., Sasu, E. J., Luna, S. E., Ben-Efraim, K., Bak, R. O., Cepika, A. M., Porteus, M. H.
2024; 31 (4): 499-518.e6
- **Lineage-tracing hematopoietic stem cell origins in vivo to efficiently make human HLF+ HOXA+ hematopoietic progenitors from pluripotent stem cells.** *Developmental cell*
Fowler, J. L., Zheng, S. L., Nguyen, A., Chen, A., Xiong, X., Chai, T., Chen, J. Y., Karigane, D., Banuelos, A. M., Niizuma, K., Kayamori, K., Nishimura, T., Cromer, et al
2024
- **Investigating adverse genomic and regulatory changes caused by replacement of the full-lengthCFTRcDNA using Cas9 and AAV.** *Molecular therapy. Nucleic acids*
Vaidyanathan, S., Kerschner, J. L., Paranjapye, A., Sinha, V., Lin, B., Bedrosian, T. A., Thrasher, A. J., Turchiano, G., Harris, A., Porteus, M. H.
2024; 35 (1): 102134
- **Gene regulation in inborn errors of immunity: Implications for gene therapy design and efficacy.** *Immunological reviews*
Ghanim, H. Y., Porteus, M. H.
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- **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.
2024; 15 (1): 111
- **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*
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2023: 101327
- **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., 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**
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AMER SOC HEMATOLOGY.2023
- **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.
AMER SOC HEMATOLOGY.2023
- **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**
Shyr, D. C., Lowsky, R., Miller, W., Schroeder, M. A., Buchholz, T., Dougall, K., Intondi, A., Charles, A., Lehrer, J., Bouge, A., Wolf, S., MacDonald, B., Din, et al
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- **Towards Automated Engineering of Donor-Derived T Lymphocytes into CRISPR/Cas9-Mediated CAR T Cells in a Closed-System**
Oseghale, A., Bertaina, A., Porteus, M.
AMER SOC HEMATOLOGY.2023
- **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*
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 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*
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 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
 CELL PRESS.2023: 370-371
- **Base-Editing as a Safe and Highly Effective Alternative Treatment for X-SCID Compared to CRISPR-Cas9 Nuclease Editing with an AAV Donor**
 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
 CELL PRESS.2023: 572
- **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
 CELL PRESS.2023: 540
- **Engineering a Potential Curative Treatment for Hemophilia A Using an AAV Dual Targeting Strategy**
 Johnston, N., Amorin, 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
- **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.
 CELL PRESS.2023: 115-116
- **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.
 CELL PRESS.2023: 419-420

- **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., Simanaukaite, J. M., Uchida, N., Longo, F. M., Porteus, M. H.
CELL PRESS.2023: 18
- **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**
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AMER SOC HEMATOLOGY.2022: 5724-5725
- **Novel Humanized Loss-of-Function NF1 Mouse Model of Juvenile Myelomonocytic Leukemia**
Sinha, R., Patil, R., Romano, R., Lee, E., Yao, Z., Liu, Y., Porteus, M. H., Bertaina, A.
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*
Cromer, M. K., Barsan, V. V., Jaeger, E., Wang, M., Hampton, J. P., Chen, F., Kennedy, D., Xiao, J., Khrebtukova, I., Granat, A., Truong, T., Porteus, M. H.
2022; 13 (1): 4724
- **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
2022; 82 (15): 2777-2791
- **A Curative DNA Code for Hematopoietic Defects: Novel Cell Therapies for Monogenic Diseases of the Blood and Immune System.** *Hematology/oncology clinics of North America*
Porteus, M. H., Pavel-Dinu, M., Pai, S.
2022
- **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., Raftery, B. C., Ng, C. Y., Vogel, U., Lander, A., Lesch, B. J., Fowler, et al
2022
- **Reengineering Ponatinib to Minimize Cardiovascular Toxicity.** *Cancer research*
Hnatiuk, A. P., Bruyneel, A. A., Tailor, D., Pandrala, M., Dheeraj, A., Li, W., Serrano, R., Feyen, D. A., Vu, M. M., Amatya, P., Gupta, S., Nakauchi, Y., Morgado, et al
2022
- **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.
2022; 13 (1): 2109
- **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**
Cromer, M., Wienert, B., Hampton, J. P., Majeti, K. R., Conklin, B. R., Porteus, M. H., MacKenzie, T. C.
CELL PRESS.2022: 328
- **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.
CELL PRESS.2022: 230

- **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.
CELL PRESS.2022: 407
- **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.
CELL PRESS.2022: 379
- **Design of experiments as a decision tool for cell therapy manufacturing.** *Cytotherapy*
Lee, E., Shah, D., Porteus, M., Wright, J. F., Bacchetta, R.
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- **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.
2022; 13 (1): 1053
- **CRISPR-Cas9-AAV versus lentivector transduction for genome modification of X-linked severe combined immunodeficiency hematopoietic stem cells.** *Frontiers in immunology*
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
2022; 13: 1067417
- **GENOME EDITING OF HEMATOPOIETIC STEM CELLS TO ENGINEER BLOOD**
Porteus, M.
ELSEVIER SCIENCE INC.2022: S29
- **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.
1800
- **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.
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- **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*
Melkonian, A., Loppinet, E., Martin, R., Porteus, M., Khosla, C.
2021; 143 (28): 10537-10540
- **Development of beta-globin gene correction in human hematopoietic stem cells as a potential durable treatment for sickle cell disease.** *Science translational medicine*
Lattanzi, A., Camarena, J., Lahiri, P., Segal, H., Srifa, W., Vakulskas, C. A., Frock, R. L., Kenrick, J., Lee, C., Talbott, N., Skowronski, J., Cromer, M. K., Charlesworth, et al
2021; 13 (598)
- **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**
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