



## Matthew Porteus

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

### CONTACT INFORMATION

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### Bio

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#### 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

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### PUBLICATIONS

- **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  
2023; 220 (12)
- **Small molecule correctors divert CFTR-F508del from ERAD by stabilizing sequential folding states.** *Molecular biology of the cell*  
Riepe, C., Wachalska, M., Deol, K. K., Amaya, A. K., Porteus, M. H., Olzmann, J. A., Kopito, R. R.  
2023: mbcE23080336
- **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.  
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.  
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., Amarin, A., Nguygen, 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., Amarin, 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.  
2023
- **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  
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., Taylor, 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., Raftry, 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., Taylor, D., Pandrala, M., Dheeraj, A., Li, W., Serrano, R., Feyen, D. A., Vu, M. M., Amatya, P., Gupta, S., Nakauchi, Y., Morgado, 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.  
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.  
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*  
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**  
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-Hagan, et al  
2021
- **Gene replacement of alpha-globin with beta-globin restores hemoglobin balance in beta-thalassemia-derived hematopoietic stem and progenitor cells.** *Nature medicine*  
Cromer, M. K., Camarena, J., Martin, R. M., Lesch, B. J., Vakulskas, C. A., Bode, N. M., Kurgan, G., Collingwood, M. A., Rettig, G. R., Behlke, M. A., Lemgart, V. T., Zhang, Y., Goyal, et al  
2021
- **Correction of X-CGD patient HSPCs by targeted CYBB cDNA insertion using CRISPR/Cas9 with 53BP1 inhibition for enhanced homology-directed repair.** *Gene therapy*  
Sweeney, C. L., Pavel-Dinu, M., Choi, U., Brault, J., Liu, T., Koontz, S., Li, L., Theobald, N., Lee, J., Bello, E. A., Wu, X., Meis, R. J., Dahl, et al  
2021
- **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-Hagan, et al  
2021
- **Enhanced Homology-directed Repair for Highly Efficient Gene Editing in Hematopoietic Stem/Progenitor Cells.** *Blood*  
De Ravin, S. S., Brault, J., Meis, R. J., Liu, S., Li, L., Pavel-Dinu, M., Lazzarotto, C. R., Liu, T. Q., Koontz, S., Choi, U., Sweeney, C. L., Theobald, N., Lee, et al  
2021
- **Cas9-AAV6 gene correction of beta-globin in autologous HSCs improves sickle cell disease erythropoiesis in mice.** *Nature communications*  
Wilkinson, A. C., Dever, D. P., Baik, R., Camarena, J., Hsu, I., Charlesworth, C. T., Morita, C., Nakauchi, H., Porteus, M. H.  
2021; 12 (1): 686
- **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  
2021
- **The TRACE-Seq method tracks recombination alleles and identifies clonal reconstitution dynamics of gene targeted human hematopoietic stem cells.** *Nature communications*  
Sharma, R. n., Dever, D. P., Lee, C. M., Azizi, A. n., Pan, Y. n., Camarena, J. n., Köhnke, T. n., Bao, G. n., Porteus, M. H., Majeti, R. n.  
2021; 12 (1): 472

- **Answered and Unanswered Questions in Early-Stage Viral Vector Transduction Biology and Innate Primary Cell Toxicity for Ex-Vivo Gene Editing.** *Frontiers in immunology*  
Dudek, A. M., Porteus, M. H.  
2021; 12: 660302
- **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*  
Vaidyanathan, S. n., Baik, R. n., Chen, L. n., Bravo, D. T., Suarez, C. J., Abazari, S. M., Salahudeen, A. A., Dudek, A. M., Teran, C. A., Davis, T. H., Lee, C. M., Bao, G. n., Randell, et al  
2021
- **Correction of Recessive Dystrophic Epidermolysis Bullosa by homology-directed repair-mediated genome editing.** *Molecular therapy : the journal of the American Society of Gene Therapy*  
Bonafont, J. n., Mencía, A. n., Chacón-Solano, E. n., Srifa, W. n., Vaidyanathan, S. n., Romano, R. n., Garcia, M. n., Hervás-Salcedo, R. n., Ugalde, L. n., Duarte, B. n., Porteus, M. H., Del Rio, M. n., Larcher, et al  
2021
- **Improved Genome Editing through Inhibition of FANCM and Members of the BTR Dissolvase Complex.** *Molecular therapy : the journal of the American Society of Gene Therapy*  
de Alencastro, G. n., Puzzo, F. n., Pavel-Dinu, M. n., Zhang, F. n., Pillay, S. n., Majzoub, K. n., Tiffany, M. n., Jang, H. n., Sheikali, A. n., Cromer, M. K., Meetei, R. n., Carette, J. E., Porteus, et al  
2021; 29 (3): 1016–27
- **CD34 expression does not correlate with immunophenotypic stem cell or progenitor content in human cord blood products.** *Blood advances*  
Mantri, S., Reinisch, A., Dejene, B. T., Lyell, D. J., DiGiusto, D. L., Agarwal-Hashmi, R., Majeti, R., Weinberg, K. I., Porteus, M. H.  
2020; 4 (21): 5357–61
- **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.  
2020; 103: 50–52
- **Genome-edited Human Hematopoietic Stem Cells Correct Lysosomal Storage Disorders**  
Poletto, E., Scharenberg, S. G., Channabasavaiah, G. B., Porteus, M., Gomez-Ospina, N.  
WILEY.2020: S213–S214
- **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.  
ELSEVIER SCIENCE INC.2020: S52
- **Metabolic engineering generates a transgene-free safety switch for cell therapy.** *Nature biotechnology*  
Wiebking, V., Patterson, J. O., Martin, R., Chanda, M. K., Lee, C. M., Srifa, W., Bao, G., Porteus, M. H.  
2020
- **DNA Barcoding in Nonhuman Primates Reveals Important Limitations in Retrovirus Integration Site Analysis.** *Molecular therapy. Methods & clinical development*  
Adair, J. E., Enstrom, M. R., Haworth, K. G., Schefter, L. E., Shahbazi, R., Humphrys, D. R., Porter, S., Tam, K., Porteus, M. H., Kiem, H.  
2020; 17: 796–809
- **Improving the safety of human pluripotent stem cell therapies using genome-edited orthogonal safeguards.** *Nature communications*  
Martin, R. M., Fowler, J. L., Cromer, M. K., Lesch, B. J., Ponce, E., Uchida, N., Nishimura, T., Porteus, M. H., Loh, K. M.  
2020; 11 (1): 2713
- **Cas9-AAV6-engineered human mesenchymal stromal cells improved cutaneous wound healing in diabetic mice.** *Nature communications*  
Srifa, W., Kosaric, N., Amarin, A., Jadi, O., Park, Y., Mantri, S., Camarena, J., Gurtner, G. C., Porteus, M.  
2020; 11 (1): 2470
- **Lentivector versus CRISPR/Cas9/AAV6 Gene Editing in X-Linked Severe Combined Immunodeficiency CD34(+) Hematopoietic Cells**  
Liu, T., Brault, J., Pavel-Dinu, M., Liu, S., Meis, R. J., Koontz, S., Corsino, C., Bosticardo, M., Kleinstiver, B., Notarangelo, L. D., Wu, X., Dahl, G. A., Porteus, et al  
CELL PRESS.2020: 355–56

- **Precise COL7A1 Gene Correction in Primary Patient Cells as a Therapeutic Option for Epidermolysis Bullosa**  
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.  
CELL PRESS.2020: 325–26
- **Clinical Production of Ex-Vivo Gene Corrected Hematopoietic Stem and Progenitor Cells Using a cGMP-Compliant Semi-Closed Manufacturing Process**  
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  
CELL PRESS.2020: 68–69
- **A Genomic Editing-Based Therapeutic Approach for RAG2 Deficiency**  
Pavel-Dinu, M., Gardner, C., La Guardia, T. A., Vakulskas, C. A., Lee, C., Bao, G., Sheikali, A., Menezes, T., Notarangelo, L. D., Porteus, M. H.  
CELL PRESS.2020: 55–56
- **CX3CR1 Haploinsufficiency Improves the Ability of Hematopoietic Stem and Progenitor Cells to Generate a Microglia-Like Progeny Upon Transplantation**  
Montepeloso, A., Mattioli, D., Molina-Estevez, J., Pellin, D., Belloni, M., Gomez-Ospina, N., Porteus, M., Genovese, P., Peviani, M., Biffi, A.  
CELL PRESS.2020: 486
- **Insertion of the CFTR cDNA in the Endogenous Locus in Airway Stem Cells Using CRISPR/Cas9 Restores CFTR Function to Wild-Type Levels in Differentiated Epithelia**  
Vaidyanathan, S., Sellers, Z. M., Bravo, D. T., Le, W., Randell, S. H., Desai, T. J., Kuo, C. J., Nayak, J. V., Porteus, M. H.  
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