Stanford

Matthew Porteus

Professor of Pediatrics (Stem Cell Transplantation)

Pediatrics - Stem Cell Transplantation

NIH Biosketch available Online

CLINICAL OFFICES

- **Pediatric Stem Cell Transplantation**

  725 Welch Rd

  Palo Alto, CA 94304

  Tel (650) 497-8953 **Fax** (650) 724-1164

ACADEMIC CONTACT INFORMATION

- **Administrative Contact**

  Ginger Exley - Administrative Assistant

  Email gexley@stanford.edu

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.

CLINICAL FOCUS

- Hematopoietic Stem Cell Transplantation
- Pediatric Hematology-Oncology

ACADEMIC APPOINTMENTS

- Professor, Pediatrics - Stem Cell Transplantation
- Member, Bio-X

- Member, Cardiovascular Institute
- Member, Child Health Research Institute
- Member, Institute for Stem Cell Biology and Regenerative Medicine
- Member, Stanford Cancer Institute
- Faculty Fellow, Stanford ChEM-H
- Member, Wu Tsai Neurosciences Institute

PROFESSIONAL EDUCATION

- Board Certification: Pediatric Hematology-Oncology, American Board of Pediatrics (2000)
- Fellowship: Children's Hospital Boston (1999) MA
- Residency: Children's Hospital Boston (1996) MA
- Medical Education: Stanford University School of Medicine (1994) CA

LINKS

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

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

Genome Editing and Population Dynamics for Gene Therapy and Cancer Research

CLINICAL TRIALS

- Natural History Study of SCID Disorders, Recruiting
- Patients Treated for Chronic Granulomatous Disease (CGD) Since 1995, Recruiting
- Patients Treated for SCID (1968-Present), Recruiting
- A Multicenter, Open-label Study of CMX001 Treatment of Serious Diseases or Conditions Caused by dsDNA Viruses, Not Recruiting
- Patients Treated for Wiskott-Aldrich Syndrome (WAS) Since 1990, Not Recruiting

Teaching

COURSES

2018-19

- Physician Scientist Hour: INDE 217 (Aut, Win, Spr)

2017-18

- Biology and Applications of CRISPR/Cas9: Genome Editing and Epigenome Modifications: BIOS 268 (Spr)
- Physician Scientist Hour: INDE 217 (Aut, Win, Spr)

2016-17

- Physician Scientist Hour: INDE 217 (Aut, Win, Spr)

2015-16

- Physician Scientist Hour: INDE 217 (Aut)

STANFORD ADVISEES

Doctoral Dissertation Reader (AC)

Josh Tycko

Postdoctoral Faculty Sponsor

Michael Cromer, Camille Sindhu-Bertrand, Sriram Vaidyanathan, Volker Wiebking

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Cancer Biology (Phd Program)
- Pediatric Hem/Onc (Fellowship Program)
- Stem Cell Biology and Regenerative Medicine (Phd Program)

Publications

PUBLICATIONS

- **SCID genotype and 6-month posttransplant CD4 count predict survival and immune recovery** *BLOOD*
  Haddad, E., Logan, B. R., Griffith, L. M., Buckley, R. H., Parrott, R. E., Prockop, S. E., Small, T. N., Chaisson, J., Dvorak, C. C., Mumane, M., Kapoor, N., Abdel-Azim, H., Hanson, et al
  2018; 132 (17): 1737–49

- **Optimization of CRISPR/Cas9 Delivery to Human Hematopoietic Stem and Progenitor Cells for Therapeutic Genomic Rearrangements.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Lattanzi, A., Meneghini, V., Pavani, G., Amor, F., Ramadier, S., Felix, T., Antoniani, C., Masson, C., Alibeu, O., Lee, C., Porteus, M. H., Bao, G., Amendola, et al
  2018

- **Uridine Depletion and Chemical Modification Increase Cas9 mRNA Activity and Reduce Immunogenicity without HPLC Purification.** *Molecular therapy. Nucleic acids*
  Vaidyanathan, S., Azizian, K. T., Haque, A. K., Henderson, J. M., Hendel, A., Shore, S., Antony, J. S., Hogrefe, R. I., Kormann, M. S., Porteus, M. H., McCaffrey, A. P.
  2018; 12: 530–42

- **Gene Editing on Center Stage** *TRENDS IN GENETICS*
  Bak, R. O., Gomez-Ospina, N., Porteus, M. H.
  2018; 34 (8): 600–611

- **Engineering the Hematopoietic System for Lysosomal Storage Disorders: Correction of Mucopolysaccharidosis Type I Using Genome-Edited, Human Hematopoietic Stem and Progenitor Cells**
  Gomez-Ospina, N., Scharenberg, S., Mostrel, N., Mantri, S., Nicolas, C., Porteus, M. M.
  CELL PRESS.2018: 310–11

- **CRISPR-Mediated Targeted Insertion of Cybb cDNAs into the Cybb Locus for Correction of X-CGD Patient CD34(+) Cells**
  Sweeney, C. L., Choi, U., Pavel-Dinu, M., Koontz, S., Li, L., Theobald, N., Lee, J., Wu, X., Porteus, M. H., Malech, H. L., De Ravin, S.
  CELL PRESS.2018: 233

- **Induction of Fetal Hemoglobin Synthesis by CRISPR/Cas9-mediated Editing of the Human beta-globin Locus**
  Meneghini, V., Lattanzi, A., Antoniani, C., Felix, T., Romano, O., Magrin, E., Weber, L., Pavani, G., El Hoss, S., Porteus, M., Amendola, M., El Nemer, W., Cavazzana, et al
  CELL PRESS.2018: 378

- **Genome Editing for IL-10 Deficiency in Purified Hematopoietic Stem Cells**
  Romano, R., Pavel-Dinu, M., Bacchetta, R., Porteus, M. H., Roncarolo, M.
  CELL PRESS.2018: 237–38

- **Genome Editing Using CRISPR/Cas9 and rAAV6 to Functionally Correct Wiskott-Aldrich Syndrome in Human HSPCs**
  Wiebking, V., Mantri, S., Weinberg, K. I., Porteus, M. H.
  CELL PRESS.2018: 376–77

- **CRISPR-Mediated Genetic Engineering of Human Mesenchymal Stromal Cells for Therapeutic Protein Delivery in Chronic Wounds**
  Srifa, W., Kosaric, N., Gurtner, G., Porteus, M.

CELL PRESS.2018: 33–34

- **Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated editing of the human beta-globin locus** *BLOOD*
  Antoniani, C., Meneghini, V., Lattanzi, A., Felix, T., Romano, O., Magrin, E., Weber, L., Pavani, G., El Hoss, S., Kurita, R., Nakamura, Y., Cradick, T. J., Lundberg, et al
  2018; 131 (17): 1960–73

- **MLL leukemia induction by t(9;11) chromosomal translocation in human hematopoietic stem cells using genome editing** *BLOOD ADVANCES*
  Schneidawind, C., Jeong, J., Schneidawind, D., Kim, I., Duque-Afonso, J., Wong, S., Iwasaki, M., Breese, E. H., Zehnder, J. L., Porteus, M., Cleary, M. L.
  2018; 2 (8): 832–45

- **Genome Editing of Long-Term Human Hematopoietic Stem Cells for X-Linked Severe Combined Immunodeficiency**
  Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C., Lee, C. M., Bao, G., Kildebeck, E., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
  SPRINGER/PLENUM PUBLISHERS.2018: 365–66

- **FOXP3 Gene Transfer in T cells and FOXP3 Gene Editing in HSC as Novel Treatment Options for IPEX Syndrome**
  Goodwin, M., Sato, Y., Passerini, L., Barzaghi, F., Lee, E., Suzette, S. K., Roncarolo, M., Porteus, M., Bacchetta, R.
  SPRINGER/PLENUM PUBLISHERS.2018: 427

- **Engineering blood stem cells for autologous transplants for lysosomal diseases: Correction of mucopolysaccharidosis type I using genome-edited hematopoietic stem and progenitor cells**
  Gomez-Ospina, N., Scharenberg, S. G., Mantri, S., Nicolas, C., Bak, R. O., Porteus, M. H.
  ACADEMIC PRESS INC ELSEVIER SCIENCE.2018: S54–S55

- **Global Transcriptional Response to CRISPR/Cas9-AAV6-Based Genome Editing in CD34+ Hematopoietic Stem and Progenitor Cells.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Cromer, M. K., Vaidyanathan, S., Ryan, D. E., Curry, B., Lucas, A. B., Camarena, J., Kaushik, M., Hay, S. R., Martin, R. M., Steinfeld, I., Bak, R. O., Dever, D. P., Hendel, et al
  2018

- **Protect NIH's DNA advisory committee.** *Science (New York, N.Y.)*
  Adelman, Z. N., Albritton, L. M., Boris-Lawrie, K., Buchmeier, M. J., Cannon, P., Cho, M., DiGiusto, D., Donahue, J. K., Federoff, H. J., Hammarskjold, M., Hardison, A. D., Hearing, P., Lee, et al
  2018; 362 (6413): 409–10

- **A high-fidelity Cas9 mutant delivered as a ribonucleoprotein complex enables efficient gene editing in human hematopoietic stem and progenitor cells.** *Nature medicine*
  Vakulskas, C. A., Dever, D. P., Rettig, G. R., Turk, R., Jacobi, A. M., Collingwood, M. A., Bode, N. M., McNeill, M. S., Yan, S., Camarena, J., Lee, C. M., Park, S. H., Wiebking, et al
  2018; 24 (8): 1216–24

- **Priming Human Repopulating Hematopoietic Stem and Progenitor Cells for Cas9/sgRNA Gene Targeting** *Molecular Therapy Nucleic Acids*
  Charlesworth, C. T., Camarena, J., Cromer, M. K., Vaidyanathan, S., Bak, R. O., Carte, J. M., Potter, J., Dever, D. P., Porteus, M. H.
  2018; 12: 89-104

- **CRISPR/Cas9 genome editing in human hematopoietic stem cells.** *Nature protocols*
  Bak, R. O., Dever, D. P., Porteus, M. H.
  2018; 13 (2): 358–76

- **Closing In on Treatment for Hemophilia B** *NEW ENGLAND JOURNAL OF MEDICINE*
  Porteus, M.
  2017; 377 (23): 2274–75

- **A Comprehensive TALEN-Based Knockout Library for Generating Human Induced Pluripotent Stem Cell-Based Models for Cardiovascular Diseases.** *Circulation research*
  Karakikes, I., Termglinchan, V., Cepeda, D. A., Lee, J., Diecke, S., Hendel, A., Itzhaki, I., Ameen, M., Shrestha, R., Wu, H., Ma, N., Shao, N., Seeger, et al
  2017

- **Multiplexed genetic engineering of human hematopoietic stem and progenitor cells using CRISPR/Cas9 and AAV6.** *eLife*
  Bak, R. O., Dever, D. P., Reinisch, A., Cruz Hernandez, D., Majeti, R., Porteus, M. H.

2017; 6

- **Dual-Method Clone Tracking in Nonhuman Primates Confirms Long-Term Hematopoietic Reconstitution Initiated By Early Engrafting Clones** *58th Annual Meeting and Exposition of the American-Society-of-Hematology*
  Adair, J. E., Norgaard, Z. K., Haworth, K. G., Schefter, L. E., Tam, K., Porteus, M., Kiem, H.
  AMER SOC HEMATOLOGY.2016

- **CRISPR/Cas9 ß-globin gene targeting in human haematopoietic stem cells.** *Nature*
  Dever, D. P., Bak, R. O., Reinisch, A., Camarena, J., Washington, G., Nicolas, C. E., Pavel-Dinu, M., Saxena, N., Wilkens, A. B., Mantri, S., Uchida, N., Hendel, A., Narla, et al
  2016

- **Invasive Fungal Disease in Pediatric Patients Undergoing Allogeneic Hematopoietic Stem Cell Transplant** *JOURNAL OF PEDIATRIC HEMATOLOGY ONCOLOGY*
  Aftandilian, C., Weinberg, K., Willert, J., Kharbanda, S., Porteus, M., Maldonado, Y., Agarwal, R.
  2016; 38 (7): 574-580

- **Phosphorylation of residues inside the SNARE complex suppresses secretory vesicle fusion.** *EMBO journal*
  Malmersjö, S., Di Palma, S., Diao, J., Lai, Y., Pfuetzner, R. A., Wang, A. L., McMahon, M. A., Hayer, A., Porteus, M., Bodenmiller, B., Brunger, A. T., Meyer, T.
  2016; 35 (16): 1810-1821

- **Ethical and regulatory aspects of genome editing** *BLOOD*
  Kohn, D. B., Porteus, M. H., Scharenberg, A. M.
  2016; 127 (21): 2553-2560

- **Activation of proto-oncogenes by disruption of chromosome neighborhoods** *SCIENCE*
  Hnisz, D., Weintraub, A. S., Day, D. S., Valton, A., Bak, R. O., Li, C. H., Goldmann, J., Lajoie, B. R., Fan, Z. P., Sigova, A. A., Reddy, J., Borges-Rivera, D., Lee, et al
  2016; 351 (6280): 1454-1458

- **Mutations in the nuclear bile acid receptor FXR cause progressive familial intrahepatic cholestasis.** *Nature communications*
  Gomez-Ospina, N., Potter, C. J., Xiao, R., Manickam, K., Kim, M., Kim, K. H., Shneider, B. L., Picarsic, J. L., Jacobson, T. A., Zhang, J., He, W., Liu, P., Knisely, et al
  2016; 7: 10713-?

- **Genome Editing: A New Approach to Human Therapeutics** *ANNUAL REVIEW OF PHARMACOLOGY AND TOXICOLOGY, VOL 56*
  Porteus, M.
  2016; 56: 163-190

- **TALENs Facilitate Single-step Seamless SDF Correction of F508del CFTR in Airway Epithelial Submucosal Gland Cell-derived CF-iPSCs.** *Molecular therapy. Nucleic acids*
  Suzuki, S., Sargent, R. G., Illek, B., Fischer, H., Esmaeili-Shandiz, A., Yezzi, M. J., Lee, A., Yang, Y., Kim, S., Renz, P., Qi, Z., Yu, J., Muench, et al
  2016; 5

- **Stem Cell-Specific Mechanisms Ensure Genomic Fidelity within HSCs and upon Aging of HSCs** *CELL REPORTS*
  Moehrle, B. M., Nattamai, K., Brown, A., Florian, M. C., Ryan, M., Vogel, M., Bliederhaeuser, C., Soller, K., Prows, D. R., Abdollahi, A., Schleimer, D., Walter, D., Milsom, et al
  2015; 13 (11): 2412-2424

- **Towards a new era in medicine: therapeutic genome editing** *GENOME BIOLOGY*
  Porteus, M. H.
  2015; 16

- **MLL leukemia induction by genome editing of human CD34+ hematopoietic cells.** *Blood*
  Buechele, C., Breese, E. H., Schneidawind, D., Lin, C., Jeong, J., Duque-Afonso, J., Wong, S. H., Smith, K. S., Negrin, R. S., Porteus, M., Cleary, M. L.
  2015; 126 (14): 1683-1694

- **Chemically modified guide RNAs enhance CRISPR-Cas genome editing in human primary cells.** *Nature biotechnology*
  Hendel, A., Bak, R. O., Clark, J. T., Kennedy, A. B., Ryan, D. E., Roy, S., Steinfeld, I., Lunstad, B. D., Kaiser, R. J., Wilkens, A. B., Bacchetta, R., Tsalenko, A., Dellinger, et al
  2015; 33 (9): 985-989

- **Chemically modified guide RNAs enhance CRISPR-Cas genome editing in human primary cells.** *Nature biotechnology*
  Hendel, A., Bak, R. O., Clark, J. T., Kennedy, A. B., Ryan, D. E., Roy, S., Steinfeld, I., Lunstad, B. D., Kaiser, R. J., Wilkens, A. B., Bacchetta, R., Tsalenko, A., Dellinger, et al
  2015; 33 (9): 985-989

- **A Pediatric Case of T-Cell Prolymphocytic Leukemia** *PEDIATRIC BLOOD & CANCER*
  Mitton, B., Coutre, S., Willert, J., Schlis, K., Porteus, M., Kharbanda, S., Agarwal-Hashmi, R.
  2015; 62 (6): 1061-1062

- **Genome editing of the germline: broadening the discussion.** *Molecular therapy*
  Porteus, M. H., Dann, C. T.
  2015; 23 (6): 980-982

- **Genome editing technologies: defining a path to clinic.** *Molecular therapy*
  Corrigan-Curay, J., O'Reilly, M., Kohn, D. B., Cannon, P. M., Bao, G., Bushman, F. D., Carroll, D., Cathomen, T., Joung, J. K., Roth, D., Sadelain, M., Scharenberg, A. M., von Kalle, et al
  2015; 23 (5): 796-806

- **Genome Editing Technologies: Defining a Path to Clinic: Genomic Editing: Establishing Preclinical Toxicology Standards, Bethesda, Maryland 10 June 2014.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Corrigan-Curay, J., O'Reilly, M., Kohn, D. B., Cannon, P. M., Bao, G., Bushman, F. D., Carroll, D., Cathomen, T., Joung, J. K., Roth, D., Sadelain, M., Scharenberg, A. M., von Kalle, et al
  2015; 23 (5): 796-806

- **Editing the genome to introduce a beneficial naturally occurring mutation associated with increased fetal globin** *NATURE COMMUNICATIONS*
  Wienert, B., Funnell, A. P., Norton, L. J., Pearson, R. C., Wilkinson-White, L. E., Lester, K., Vadolas, J., Porteus, M. H., Matthews, J. M., Quinlan, K. G., Crossley, M.
  2015; 6

- **Improved outcomes after autologous bone marrow transplantation for children with relapsed or refractory hodgkin lymphoma: twenty years experience at a single institution.** *Biology of blood and marrow transplantation*
  Garfin, P. M., Link, M. P., Donaldson, S. S., Advani, R. H., Luna-Fineman, S., Kharbanda, S., Porteus, M., Weinberg, K. I., Agarwal-Hashmi, R.
  2015; 21 (2): 326-334

- **Quantifying on- and off-target genome editing.** *Trends in biotechnology*
  Hendel, A., Fine, E. J., Bao, G., Porteus, M. H.
  2015; 33 (2): 132-140

- **Quantifying on- and off-target genome editing** *TRENDS IN BIOTECHNOLOGY*
  Hendel, A., Fine, E. J., Bao, G., Porteus, M. H.
  2015; 33 (2): 132-140

- **Promoterless gene targeting without nucleases ameliorates haemophilia B in mice.** *Nature*
  BARZEL, A., Paulk, N. K., Shi, Y., Huang, Y., Chu, K., Zhang, F., Valdmanis, P. N., Spector, L. P., Porteus, M. H., Gaensler, K. M., Kay, M. A.
  2015; 517 (7534): 360-364

- **Promoterless gene targeting without nucleases ameliorates haemophilia B in mice.** *Nature*
  BARZEL, A., Paulk, N. K., Shi, Y., Huang, Y., Chu, K., Zhang, F., Valdmanis, P. N., Spector, L. P., Porteus, M. H., Gaensler, K. M., Kay, M. A.
  2015; 517 (7534): 360-364

- **Strategies to increase genome editing frequencies and to facilitate the identification of edited cells.** *Methods in molecular biology (Clifton, N.J.)*
  Porteus, M.
  2015; 1239: 281-289

- **Use of Genome Engineering to Create Patient Specific MLL Translocations in Primary Human Hematopoietic Stem and Progenitor Cells.** *PloS one*
  Breese, E. H., Buechele, C., Dawson, C., Cleary, M. L., Porteus, M. H.
  2015; 10 (9)

- **Use of Genome Engineering to Create Patient Specific MLL Translocations in Primary Human Hematopoietic Stem and Progenitor Cells.** *PloS one*
  Breese, E. H., Buechele, C., Dawson, C., Cleary, M. L., Porteus, M. H.

2015; 10 (9)

- **Editing the genome to introduce a beneficial naturally occurring mutation associated with increased fetal globin.** *Nature communications*
  Wienert, B., Funnell, A. P., Norton, L. J., Pearson, R. C., Wilkinson-White, L. E., Lester, K., Vadolas, J., Porteus, M. H., Matthews, J. M., Quinlan, K. G., Crossley, M.
  2015; 6: 7085-?

- **Novel Integrated Autologous Hematopoietic Stem Cell Tracking in Nonhuman Primates Reveals Successive Pattern of Multi-Lineage Reconstitution after Total Body Irradiation** *56th Annual Meeting and Exposition of the American-Society-of-Hematology*
  Adair, J. E., Porter, S., Haworth, K., Tam, K., Kiem, H., Porteus, M. H.
  AMER SOC HEMATOLOGY.2014

- **Genome Editing in Mouse Spermatogonial Stem/Progenitor Cells Using Engineered Nucleases** *PLOS ONE*
  Fanslow, D. A., Wirt, S. E., Barker, J. C., Connelly, J. P., Porteus, M. H., Dann, C. T.
  2014; 9 (11)

- **Genome Editing of Mouse Fibroblasts by Homologous Recombination for Sustained Secretion of PDGF-B and Augmentation of Wound Healing.** *Plastic and reconstructive surgery*
  Barker, J. C., Barker, A. D., Bills, J., Huang, J., Wight-Carter, M., Delgado, I., Noble, D. L., Huang, L. J., Porteus, M. H., Davis, K. E.
  2014; 134 (3): 389e-401e

- **Newborn screening for severe combined immunodeficiency in 11 screening programs in the United States.** *JAMA-the journal of the American Medical Association*
  Kwan, A., Abraham, R. S., Currier, R., Brower, A., Andruszewski, K., Abbott, J. K., Baker, M., Ballow, M., Bartoshesky, L. E., Bonilla, F. A., Brokopp, C., Brooks, E., Caggana, et al
  2014; 312 (7): 729-738

- **Transplantation Outcomes for Severe Combined Immunodeficiency, 2000-2009** *NEW ENGLAND JOURNAL OF MEDICINE*
  Pai, S., Logan, B. R., Griffith, L. M., Buckley, R. H., Parrott, R. E., Dvorak, C. C., Kapoor, N., Hanson, I. C., Filipovich, A. H., Jyonouchi, S., Sullivan, K. E., Small, T. N., Burroughs, et al
  2014; 371 (5): 434-446

- **Quantifying Genome-Editing Outcomes at Endogenous Loci with SMRT Sequencing.** *Cell reports*
  Hendel, A., Kildebeck, E. J., Fine, E. J., Clark, J. T., Punjya, N., Sebastiano, V., Bao, G., Porteus, M. H.
  2014; 7 (1): 293-305

- **SAPTA: a new design tool for improving TALE nuclease activity.** *Nucleic acids research*
  Lin, Y., Fine, E. J., Zheng, Z., Antico, C. J., Voit, R. A., Porteus, M. H., Cradick, T. J., Bao, G.
  2014; 42 (6)

- **Nuclease-mediated gene editing by homologous recombination of the human globin locus.** *Nucleic acids research*
  Voit, R. A., Hendel, A., Pruett-Miller, S. M., Porteus, M. H.
  2014; 42 (2): 1365-1378

- **Gene/cell therapy approaches for Immune Dysregulation Polyendocrinopathy Enteropathy X-linked syndrome.** *Current gene therapy*
  Passerini, L., Santoni de Sio, F. R., Porteus, M. H., Bacchetta, R.
  2014; 14 (6): 422-428

- **Gene/Cell Therapy Approaches for Immune Dysregulation Polyendocrinopathy Enteropathy X-Linked Syndrome** *CURRENT GENE THERAPY*
  Passerini, L., de Sio, F. R., Porteus, M. H., Bacchetta, R.
  2014; 14 (6): 422-428

- **Phosphorylation of EXO1 by CDKs 1 and 2 regulates DNA end resection and repair pathway choice.** *Nature communications*
  Tomimatsu, N., Mukherjee, B., Catherine Hardebeck, M., Ilcheva, M., Vanessa Camacho, C., Louise Harris, J., Porteus, M., Llorente, B., Khanna, K. K., Burma, S.
  2014; 5: 3561-?

- **Lentiviral and targeted cellular barcoding reveals ongoing clonal dynamics of cell lines in vitro and in vivo** *GENOME BIOLOGY*
  Porter, S. N., Baker, L. C., Mittelman, D., Porteus, M. H.
  2014; 15 (5)

- **Phosphorylation of EXO1 by CDKs 1 and 2 regulates DNA end resection and repair pathway choice.** *Nature communications*

Tomimatsu, N., Mukherjee, B., Catherine Hardebeck, M., Ilcheva, M., Vanessa Camacho, C., Louise Harris, J., Porteus, M., Llorente, B., Khanna, K. K., Burma, S.
2014; 5: 3561-?

- **An Erythroid Enhancer of BCL11A Subject to Genetic Variation Determines Fetal Hemoglobin Level** *SCIENCE*
  Bauer, D. E., Kamran, S. C., Lessard, S., Xu, J., Fujiwara, Y., Lin, C., Shao, Z., Canver, M. C., Smith, E. C., Pinello, L., Sabo, P. J., Vierstra, J., Voit, et al
  2013; 342 (6155): 253-257

- **Receptor-mediated delivery of engineered nucleases for genome modification** *NUCLEIC ACIDS RESEARCH*
  Chen, Z., Jaafar, L., Agyekum, D. G., Xiao, H., Wade, M. F., Kumaran, R. I., Spector, D. L., Bao, G., Porteus, M. H., Dynan, W. S., Meiler, S. E.
  2013; 41 (19)

- **Newborn screening for severe combined immunodeficiency and T-cell lymphopenia in California: Results of the first 2 years** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
  Kwan, A., Church, J. A., Cowan, M. J., Agarwal, R., Kapoor, N., Kohn, D. B., Lewis, D. B., McGhee, S. A., Moore, T. B., Stiehm, E. R., Porteus, M., Aznar, C. P., Currier, et al
  2013; 132 (1): 140-U245

- **Generation of an HIV Resistant T-cell Line by Targeted "Stacking" of Restriction Factors** *MOLECULAR THERAPY*
  Voit, R. A., McMahon, M. A., Sawyer, S. L., Porteus, M. H.
  2013; 21 (4): 786-795

- **Expanding the Repertoire of Target Sites for Zinc Finger Nuclease-mediated Genome Modification** *MOLECULAR THERAPY-NUCLEIC ACIDS*
  Wilson, K. A., McEwen, A. E., Pruett-Miller, S. M., Zhang, J., Kildebeck, E. J., Porteus, M. H.
  2013; 2

- **Design and Development of Artificial Zinc Finger Transcription Factors and Zinc Finger Nucleases to the hTERT Locus** *MOLECULAR THERAPY-NUCLEIC ACIDS*
  Wilson, K. A., Chateau, M. L., Porteus, M. H.
  2013; 2

- **A Crisper Look at Genome Editing: RNA-guided Genome Modification** *MOLECULAR THERAPY*
  Damian, M., Porteus, M. H.
  2013; 21 (4): 719-721

- **A crisper look at genome editing: RNA-guided genome modification.** *Molecular therapy : the journal of the American Society of Gene Therapy*
  Damian, M., Porteus, M. H.
  2013; 21 (4): 720-722

- **A survey of ex vivo/in vitro transduction efficiency of mammalian primary cells and cell lines with Nine natural adeno-associated virus (AAV1-9) and one engineered adeno-associated virus serotype** *VIROLOGY JOURNAL*
  Ellis, B. L., Hirsch, M. L., Barker, J. C., Connelly, J. P., Steininger, R. J., Porteus, M. H.
  2013; 10

- **Zinc-finger nuclease-mediated gene correction using single AAV vector transduction and enhancement by Food and Drug Administration-approved drugs** *GENE THERAPY*
  Ellis, B. L., HIRSCH, M. L., Porter, S. N., Samulski, R. J., Porteus, M. H.
  2013; 20 (1): 35-42

- **Gene therapy for primary immunodeficiencies** *CURRENT OPINION IN PEDIATRICS*
  Kildebeck, E., Checketts, J., Porteus, M.
  2012; 24 (6): 731-738

- **Engineering the immune system to cure genetic diseases, HIV, and cancer Editorial overview** *CURRENT OPINION IN IMMUNOLOGY*
  Porteus, M. H., Fischer, A.
  2012; 24 (5): 576-579

- **Development of nuclease-mediated site-specific genome modification.** *Current opinion in immunology*
  Wirt, S. E., Porteus, M. H.
  2012; 24 (5): 609-616

- **Gene editing: not just for translation anymore.** *Nature methods*

McMahon, M. A., Rahdar, M., Porteus, M.
2012; 9 (1): 28-31

- **Gene editing: not just for translation anymore** *NATURE METHODS*
  McMahon, M. A., Rahdar, M., Porteus, M.
  2012; 9 (1): 28-31
- **Viral Single-Strand DNA Induces p53-Dependent Apoptosis in Human Embryonic Stem Cells** *PLOS ONE*
  Hirsch, M. L., Fagan, B. M., Dumitru, R., Bower, J. J., Yadav, S., Porteus, M. H., Pevny, L. H., Samulski, R. J.
  2011; 6 (11)
- **Zinc fingers hit off target.** *Nature medicine*
  Cheng, L., Blazar, B., High, K., Porteus, M.
  2011; 17 (10): 1192-1193
- **Seeing the light: integrating genome engineering with double-strand break repair** *NATURE METHODS*
  Porteus, M.
  2011; 8 (8): 628-630
- **Translating the Lessons From Gene Therapy to the Development of Regenerative Medicine** *MOLECULAR THERAPY*
  Porteus, M.
  2011; 19 (3): 439-441
- **Homologous recombination-based gene therapy for the primary immunodeficiencies** *YEAR IN HUMAN AND MEDICAL GENETICS: INBORN ERRORS OF IMMUNITY II*
  Porteus, M.
  2011; 1246: 131-140
- **Creating Higher Titer Lentivirus with Caffeine** *HUMAN GENE THERAPY*
  Ellis, B. L., Potts, P. R., Porteus, M. H.
  2011; 22 (1): 93-100
- **Self-complementary AAV mediates gene targeting and enhances endonuclease delivery for double-strand break repair** *GENE THERAPY*
  HIRSCH, M. L., Green, L., Porteus, M. H., Samulski, R. J.
  2010; 17 (9): 1175-1180
- **Gene Correction by Homologous Recombination With Zinc Finger Nucleases in Primary Cells From a Mouse Model of a Generic Recessive Genetic Disease** *MOLECULAR THERAPY*
  Connelly, J. P., Barker, J. C., Pruett-Miller, S., Porteus, M. H.
  2010; 18 (6): 1103-1110
- **Gene Targeting of a Disease-Related Gene in Human Induced Pluripotent Stem and Embryonic Stem Cells** *CELL STEM CELL*
  Zou, J., Maeder, M. L., Mali, P., Pruett-Miller, S. M., Thibodeau-Beganny, S., Chou, B., Chen, G., Ye, Z., Park, I., Daley, G. Q., Porteus, M. H., Joung, J. K., Cheng, et al
  2009; 5 (1): 97-110
- **Attenuation of Zinc Finger Nuclease Toxicity by Small-Molecule Regulation of Protein Levels** *PLOS GENETICS*
  Pruett-Miller, S. M., Reading, D. W., Porter, S. N., Porteus, M. H.
  2009; 5 (2)
- **Rapid "Open-Source" engineering of customized zinc-finger nucleases for highly efficient gene modification** *MOLECULAR CELL*
  Maeder, M. L., Thibodeau-Beganny, S., Osiak, A., Wright, D. A., Anthony, R. M., Eichtinger, M., Jiang, T., Foley, J. E., Winfrey, R. J., Townsend, J. A., Unger-Wallace, E., Sander, J. D., Mueller-Lerch, et al
  2008; 31 (2): 294-301
- **Comparison of zinc finger nucleases for use in gene targeting in mammalian cells** *MOLECULAR THERAPY*
  Pruett-Miller, S. M., Connelly, J. P., Maeder, M. L., Joung, J. K., Porteus, M. H.
  2008; 16 (4): 707-717
- **Spermatogonial Stem Cell Self-Renewal Requires OCT4, a Factor Downregulated During Retinoic Acid-Induced Differentiation** *STEM CELLS*
  Dann, C. T., Alvarado, A. L., Molyneux, L. A., Denard, B. S., Garbers, D. L., Porteus, M. H.

2008; 26 (11): 2928-2937

- **A look to future directions in gene therapy research for monogenic diseases** *PLOS GENETICS*
  Porteus, M. H., Connelly, J. P., Pruett, S. M.
  2006; 2 (9): 1285-1292
- **Human SMC5/6 complex promotes sister chromatid homologous recombination by recruiting the SMC1/3 cohesin complex to double-strand breaks** *EMBO JOURNAL*
  Potts, P. R., Porteus, M. H., Yu, H.
  2006; 25 (14): 3377-3388
- **Standardized reagents and protocols for engineering zinc finger nucleases by modular assembly** *NATURE PROTOCOLS*
  Wright, D. A., Thibodeau-Beganny, S., Sander, J. D., Winfrey, R. J., Hirsh, A. S., Eichtinger, M., Fu, F., Porteus, M. H., Dobbs, D., Voytas, D. F., Joung, J. K.
  2006; 1 (3): 1637-1652
- **Gene targeting using zinc finger nucleases** *NATURE BIOTECHNOLOGY*
  Porteus, M. H., Carroll, D.
  2005; 23 (8): 967-973
- **Zinc finger nucleases: custom-designed molecular scissors for genome engineering of plant and mammalian cells** *NUCLEIC ACIDS RESEARCH*
  Durai, S., Mani, M., Kandavelou, K., Wu, J., Porteus, M. H., Chandrasegaran, S.
  2005; 33 (18): 5978-5990
- **Chimeric nucleases stimulate gene targeting in human cells** *SCIENCE*
  Porteus, M. H., Baltimore, D.
  2003; 300 (5620): 763-763
- **Efficient gene targeting mediated by adeno-associated virus and DNA double-strand breaks** *MOLECULAR AND CELLULAR BIOLOGY*
  Porteus, M. H., Cathomen, T., Weitzman, M. D., Baltimore, D.
  2003; 23 (10): 3558-3565
- **Sequence, organization, and transcription of the Dlx-1 and Dlx-2 locus** *GENOMICS*
  McGuinness, T., Porteus, M. H., Smiga, S., Bulfone, A., Kingsley, C., Qiu, M. S., Liu, J. K., Long, J. E., Xu, D. W., Rubenstein, J. L.
  1996; 35 (3): 473-485
- **COCCIDIOIDAL ANTIGEN-REACTIVE CD4(+) T-LYMPHOCYTES IN THE CEREBROSPINAL-FLUID IN COCCIDIOIDES-IMMITIS MENINGITIS** *JOURNAL OF MEDICAL AND VETERINARY MYCOLOGY*
  Vollmer, T. L., Gaiser, C., DELLOCA, R. L., Porteus, M., Steinman, L., Stevens, D. A.
  1995; 33 (1): 43-48
- **The spatial localization of Dlx-2 during tooth development** *CONNECTIVE TISSUE RESEARCH*
  Thomas, B. L., Porteus, M. H., Rubenstein, J. L., Sharpe, P. T.
  1995; 32 (1-4): 27-34
- **DLX-P, MASH-1, AND MAP-5 EXPRESSION AND BROMODEOXYURIDINE INCORPORATION DEFINE MOLECULARLY DISTINCT CELL-POPULATIONS IN THE EMBRYONIC MOUSE FOREBRAIN** *JOURNAL OF NEUROSCIENCE*
  Porteus, M. H., Bulfone, A., Liu, J. K., Puelles, L., Lo, L. C., Rubenstein, J. L.
  1994; 14 (11): 6370-6383
- **SPATIALLY RESTRICTED EXPRESSION OF DLX-1, DLX-2(TES-1), GBX-2, AND WNT-3 IN THE EMBRYONIC DAY 12.5 MOUSE FOREBRAIN DEFINES POTENTIAL TRANSVERSE AND LONGITUDINAL SEGMENTAL BOUNDARIES** *JOURNAL OF NEUROSCIENCE*
  Bulfone, A., Puelles, L., Porteus, M. H., Frohman, M. A., Martin, G. R., Rubenstein, J. L.
  1993; 13 (7): 3155-3172
- **THE MOUSE DLX-2 (TES-1) GENE IS EXPRESSED IN SPATIALLY RESTRICTED DOMAINS OF THE FOREBRAIN, FACE AND LIMBS IN MIDGESTATION MOUSE EMBRYOS** *MECHANISMS OF DEVELOPMENT*
  Bulfone, A., Kim, H. J., Puelles, L., Porteus, M. H., Grippo, J. F., Rubenstein, J. L.
  1993; 40 (3): 129-140
- **DLX2 (TES1), A HOMEOBOX GENE OF THE DISTAL-LESS FAMILY, ASSIGNED TO CONSERVED REGIONS ON HUMAN AND MOUSE CHROMOSOMES-2** *GENOMICS*

Ozcelik, T., Porteus, M. H., Rubenstein, J. L., FRANCKE, U.
1992; 13 (4): 1157-1161

- **ISOLATION AND CHARACTERIZATION OF A LIBRARY OF CDNA CLONES THAT ARE PREFERENTIALLY EXPRESSED IN THE EMBRYONIC TELENCEPHALON** *MOLECULAR BRAIN RESEARCH*
  Porteus, M. H., BRICE, A. E., Bulfone, A., Usdin, T. B., CIARANELLO, R. D., Rubenstein, J. L.
  1992; 12 (1-3): 7-22

- **ISOLATION AND CHARACTERIZATION OF A NOVEL CDNA CLONE ENCODING A HOMEODOMAIN THAT IS DEVELOPMENTALLY REGULATED IN THE VENTRAL FOREBRAIN** *NEURON*
  Porteus, M. H., Bulfone, A., CIARANELLO, R. D., Rubenstein, J. L.
  1991; 7 (2): 221-229

- **SUBTRACTIVE HYBRIDIZATION SYSTEM USING SINGLE-STRANDED PHAGEMIDS WITH DIRECTIONAL INSERTS** *NUCLEIC ACIDS RESEARCH*
  Rubenstein, J. L., BRICE, A. E., CIARANELLO, R. D., Denney, D., Porteus, M. H., Usdin, T. B.
  1990; 18 (16): 4833-4842

- **VALIDATION OF A MODEL OF NON-RHEGMATOGENOUS RETINAL-DETACHMENT** *CURRENT EYE RESEARCH*
  Marmor, M. F., Porteus, M., Negi, A., Immel, J.
  1984; 3 (3): 515-518