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, Institute for Stem Cell Biology and Regenerative Medicine
- Member, Maternal & Child Health Research Institute (MCHRI)
- 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

- Biology and Applications of CRISPR/Cas9: Genome Editing and Epigenome Modifications: BIOS 268, GENE 268 (Spr)
- 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)

Tony Gao, Themasap Khan, Josh Tycko

Postdoctoral Faculty Sponsor

Kyle Cromer, Amanda Dudek, Aluya Oseghale, Mara Pavel-Dinu, Sriram Vaidyanathan, Volker Wiebking

Doctoral Dissertation Advisor (AC)

Renata Martin, Wai Srifa

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

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

Publications

PUBLICATIONS

- **CRISPR/Cas9 Genome Engineering in Engraftable Human Brain-Derived Neural Stem Cells.** *iScience*
  Dever, D. P., Scharenberg, S. G., Camarena, J., Kildebeck, E. J., Clark, J. T., Martin, R. M., Bak, R. O., Tang, Y., Dohse, M., Birgmeier, J. A., Jagadeesh, K. A., Bejerano, G., Tsukamoto, et al
  2019; 15: 524–35

- **Highly Efficient and Marker-free Genome Editing of Human Pluripotent Stem Cells by CRISPR-Cas9 RNP and AAV6 Donor-Mediated Homologous Recombination.** *Cell stem cell*
  Martin, R. M., Ikeda, K., Cromer, M. K., Uchida, N., Nishimura, T., Romano, R., Tong, A. J., Lemgart, V. T., Camarena, J., Pavel-Dinu, M., Sindhu, C., Wiebking, V., Vaidyanathan, et al
  2019; 24 (5): 821

- **Highly Efficient and Marker-free Genome Editing of Human Pluripotent Stem Cells by CRISPR-Cas9 RNP and AAV6 Donor-Mediated Homologous Recombination** *CELL STEM CELL*
  Martin, R. M., Ikeda, K., Cromer, M., Uchida, N., Nishimura, T., Romano, R., Tong, A. J., Lemgart, V. T., Camarena, J., Pavel-Dinu, M., Sindhu, C., Wiebking, V., Vaidyanathan, et al
  2019; 24 (5): 821-+

- **RETRO-ORBITAL INFUSION OF HUMAN MESENCHYMAL STROMAL CELLS ACCELERATES WOUND HEALING THROUGH SYSTEMIC EFFECTS**
  Kosaric, N., Srifa, W., Kiwanuka, H., Porteus, M., Gurtner, G.
  WILEY.2019: A10

- **Gene correction for SCID-X1 in long-term hematopoietic stem cells (vol 10, 1634, 2019)** *NATURE COMMUNICATIONS*
  Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C. E., Lee, C., Bao, G., Kildebeck, E. J., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
  2019; 10

- **Genome Edited Airway Stem Cells as a Durable Cell-Based Therapy to Treat Cystic Fibrosis**
  Vaidyanathan, S., Sellers, Z. M., Bravo, D. T., Le, W., Nayak, J. V., Porteus, M. H.
  CELL PRESS.2019: 83

- **Barcoded Clonal Tracking of CRISPR-Cas9 and rAAV6-Mediated Gene Targeting in Human Hematopoietic Stem and Progenitor Cells**
  Dever, D. P., Sharma, R., Lee, C. M., Aziz, A., Koehnke, T., Camarena, J., Pan, Y., Zhao, F., Bao, G., Majeti, R., Porteus, M.
  CELL PRESS.2019: 5

- **Genome Edited Human Hematopoietic Stem Cells Correct Lysosomal Storage Disorders: Proof-of-Concept and Safety Studies for Mucopolysaccharidosis Type I and Gaucher Disease**

Gomez-Ospina, N., Scharenberg, S., Mostrel, N., Raj, N., Attardi, L., Khan, S., Tomatsu, S., Lee, C., Bao, G., Porteus, M. H.
CELL PRESS.2019: 329

- **Can't Live without "U": Genetic Engineering of UMPS to Create Auxotrophy in Human Cells**
  Wiebking, V., Patterson, J. O., Martin, R., Chanda, M. K., Lee, C. M., Srifa, W., Bao, G., Porteus, M. H.
  CELL PRESS.2019: 454
- **Targeting beta-Globin Gene into alpha-Globin Locus in Human Hematopoietic Stem and Progenitor Cells**
  Cromer, M., Camarena, J., Martin, R. M., Lesch, B. J., Lemgart, V. T., Bak, R. O., Dever, D. P., Porteus, M. H.
  CELL PRESS.2019: 400
- **Advantages of DNA Barcoding versus Integration Site Analysis for In Vivo Clone Tracking after Transplantation**
  Enstrom, M. R., Adair, J. E., Haworth, K. G., Schefter, L., Tam, K., Porteus, M. H., Kiem, H.
  CELL PRESS.2019: 198–99
- **Towards The Clinical Translation of Gene Correction in Hematopoietic Stem Cells for Sickle Cell Disease Treatment**
  Lattanzi, A., Dever, D. P., Camarena, J., Lahiri, P., Segal, H., Talbott, N., Srifa, W., Cromer, K., Lee, C., Bao, G., Bathia, N., Uchida, N., Tisdale, et al
  CELL PRESS.2019: 448
- **Efficient Genome Editing of the PKLR Locus in Human Long-Term Hematopoietic Stem Cells Using Specific CRISPR/CAS9 RNP and AAV6-Delivery of Donor Templates to Treat Pyruvate Kinase Deficiency**
  Fananas-Baquero, S., Quintana-Bustamante, O., Alberquilla, O., Sanchez-Dominguez, R., Dever, D. P., Camarena, J., Bueren, J., Porteus, M., Segovia, J. C.
  CELL PRESS.2019: 451
- **Gene correction for SCID-X1 in long-term hematopoietic stem cells** *NATURE COMMUNICATIONS*
  Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C. E., Lee, C., Bao, G., Kildebeck, E. J., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
  2019; 10
- **Gene correction for SCID-X1 in long-term hematopoietic stem cells.** *Nature communications*
  Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C. E., Lee, C., Bao, G., Kildebeck, E. J., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
  2019; 10 (1): 1634
- **A New Class of Medicines through DNA Editing** *NEW ENGLAND JOURNAL OF MEDICINE*
  Porteus, M. H.
  2019; 380 (10): 947–59
- **Identification of preexisting adaptive immunity to Cas9 proteins in humans.** *Nature medicine*
  Charlesworth, C. T., Deshpande, P. S., Dever, D. P., Camarena, J., Lemgart, V. T., Cromer, M. K., Vakulskas, C. A., Collingwood, M. A., Zhang, L., Bode, N. M., Behlke, M. A., Dejene, B., Cieniewicz, et al
  2019
- **Human genome-edited hematopoietic stem cells phenotypically correct Mucopolysaccharidosis type I.** *Nature communications*
  Gomez-Ospina, N., Scharenberg, S. G., Mostrel, N., Bak, R. O., Mantri, S., Quadros, R. M., Gurumurthy, C. B., Lee, C., Bao, G., Suarez, C. J., Khan, S., Sawamoto, K., Tomatsu, et al
  2019; 10 (1): 4045
- **Author Correction: Gene correction for SCID-X1 in long-term hematopoietic stem cells.** *Nature communications*
  Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C. E., Lee, C., Bao, G., Kildebeck, E. J., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
  2019; 10 (1): 2021
- **Highly efficient editing of the #-globin gene in patient-derived hematopoietic stem and progenitor cells to treat sickle cell disease.** *Nucleic acids research*
  Park, S. H., Lee, C. M., Dever, D. P., Davis, T. H., Camarena, J., Srifa, W., Zhang, Y., Paikari, A., Chang, A. K., Porteus, M. H., Sheehan, V. A., Bao, G.
  2019
- **Efficient scarless genome editing in human pluripotent stem cells.** *Nature methods*
  Ikeda, K., Uchida, N., Nishimura, T., White, J., Martin, R. M., Nakauchi, H., Sebastiano, V., Weinberg, K. I., Porteus, M. H.
  2018; 15 (12): 1045–47

- **Efficient CRISPR/Cas9-Mediated Gene Editing of Pklr in Human Hematopoietic Progenitors and Stem Cells for the Gene Therapy of Pyruvate Kinase Deficiency**
  Quintana Bustamante, O., Fananas-Baquero, S., Dever, D. P., Omaira, A., Camarena, J., Sanchez-Dominguez, R., Morin, M., Fernandez, V., Angel Moreno-Pelayo, M., Bueren, J. A., Porteus, M., Segovia, J. C.
  AMER SOC HEMATOLOGY.2018

- **Highly Efficient Editing of the Beta-Globin Gene in Patient Derived Hematopoietic Stem and Progenitor Cells to Treat Sickle Cell Disease**
  Park, S., Lee, C. M., Dever, D. P., Davis, T. H., Camarena, J., Zhang, Y., Porteus, M., Sheehan, V. A., Bao, G.
  AMER SOC HEMATOLOGY.2018

- **An Engineered Cell-Traceable Model of Reticular Dysgenesis in Human Hematopoietic Stem Cells Linking Metabolism and Differentiation**
  Wang, W., Awani, A., Reich, L., Nakauchi, Y., Thomas, D., Dever, D. P., Porteus, M., Weinacht, K. G.
  AMER SOC HEMATOLOGY.2018

- **Pharmacological Inhibition of Nlk (Nemo-like Kinase) Rescues Erythropoietic Defects in Pre-Clinical Models of Diamond Blackfan Anemia**
  Wilkes, M. C., Chen, J., Siva, K., Veretti, G., Dever, D. P., Youn, M., Chae, H., Mercado, J. D., Saxena, M., Narla, A., Glader, B., Porteus, M., Repellin, et al
  AMER SOC HEMATOLOGY.2018

- **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

- **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

- **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

- **Multiplexed genetic engineering of human hematopoietic stem and progenitor cells using CRISPR/Cas9 and AAV6** *ELIFE*

  Bak, R. O., Dever, D. P., Reinisch, A., Hernandez, D., Majeti, R., Porteus, M. H.

2017; 6

- **CRISPR-Mediated Integration of Large Gene Cassettes Using AAV Donor Vectors** *CELL REPORTS*
  Bak, R. O., Porteus, M. H.
  2017; 20 (3): 750–56

- **Toward Responsible Human Genome Editing** *JAMA-JOURNAL OF THE AMERICAN MEDICAL ASSOCIATION*
  Hynes, R. O., Coller, B. S., Porteus, M.
  2017; 317 (18): 1829–30

- **Multiplexing CRISPR-Cas9 Genome Editing in Human Hematopoietic Stem and Effector Cells**
  Dever, D. P., Bak, R. O., Reinisch, A., Cruz, D., Majeti, R., Porteus, M.
  CELL PRESS.2017: 343

- **Maximizing Translation of Cas9 mRNA Therapeutics by Sequence Engineering and Chemical Modification**
  Vaidyanathan, S., Azizian, K. T., Henderson, J., Shin, D., Lebedev, A., Hogrefe, R. I., McCaffrey, A. P., Porteus, M. H.
  CELL PRESS.2017: 167

- **Correction of X-Linked Severe Combined Immunodeficiency in Human Hematopoietic Stem and Progenitor Cells**
  Pavel-Dinu, M., Wiebking, V. A., Gomez-Ospina, N., Bak, R. O., Dejene, B. T., Mantri, S., Nicolas, C. E., Punjya, N. R., Lee, C., Weinberg, K. I., Bao, G., DeRavin, S., Porteus, et al
  CELL PRESS.2017: 345–46

- **Priming Hematopoietic Stem and Progenitor Cells for CRISPR/Cas9-Mediated Homologous Recombination**
  Camarena, J., Charlesworth, C. T., Bak, R. O., Carte, J., Potter, J., Dever, D. D., Porteus, M. H.
  CELL PRESS.2017: 78

- **CRISPR-Based Gene Correction to Treat IPEX Syndrome**
  Goodwin, M., Shipp, S., Froessl, L., Porteus, M., Roncarolo, M., Bacchetta, R.
  CELL PRESS.2017: 168

- **CRISPR-Mediated Integration of Large Gene Cassettes Using AAV Donor Vectors**
  Bak, R. O., Porteus, M. H.
  CELL PRESS.2017: 237–38

- **Genome Editing in Cardiovascular Biology.** *Circulation research*
  Seeger, T., Porteus, M., Wu, J. C.
  2017; 120 (5): 778-780

- **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

- **Closing In on Treatment for Hemophilia B.** *The New England journal of medicine*
  Porteus, M.
  2017; 377 (23): 2274–75

- **The changing landscape of gene editing in hematopoietic stem cells: a step towards Cas9 clinical translation.** *Current opinion in hematology*
  Dever, D. P., Porteus, M. H.
  2017

- **Genome Editing for the beta-Hemoglobinopathies.** *Advances in experimental medicine and biology*
  Porteus, M. H.
  2017; 1013: 203–17

- **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

- **Anti-Fungal Prophylaxis Using Intermediate Dose Ambisome Is Associated with Delayed Methotrexate Clearance in Pediatric Patients Undergoing Allogeneic Hematopoietic Stem Cell Transplantation**
  Schultz, L. M., Kumar, K., Stone, S., Callard, E., Witkowski, J., Shinn, L., Pinner, L., Franklin, S., Kula, J., Patel, N., Kumar, P., Weinberg, K. I., Porteus, et al
  AMER SOC HEMATOLOGY.2016

- **Induction of Fetal Hemoglobin Synthesis By Crispr/Cas9-Mediated Disruption of the beta-Globin Locus Architecture**
  Antoniani, C., Meneghini, V., Lattanzi, A., Pavani, G., Felix, T., Amor, F., Romano, O., Magrin, E., Weber, L., Cradick, T., Lundberg, A. S., Porteus, M., Amendola, et al
  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.
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