



Sriram Vaidyanathan

Instructor, Pediatrics - Stem Cell Transplantation

 NIH Biosketch available Online

Bio

BIO

I am a postdoctoral scholar working with Dr. Matthew Porteus. Gene therapy has been my primary research interest during my doctoral and postdoctoral training. As a doctoral student, I studied the intracellular transport of non-viral gene delivery vectors to optimize delivery. I joined the Porteus lab to further my interest in gene therapy by applying CRISPR/Cas9 based genome editing for monogenic diseases. As a postdoctoral scholar, I have been working on using CRISPR/Cas9 technology to develop an autologous gene corrected airway stem cell therapy to treat cystic fibrosis.

ACADEMIC APPOINTMENTS

- Instructor, Pediatrics - Stem Cell Transplantation

HONORS AND AWARDS

- Postdoctoral Fellowship Award, Cystic Fibrosis Foundation (May 2019-March 2021)
- K99/R00 Pathway to Independence Award, National Institutes of Health (March 2021 - Present)
- School of Medicine Dean's Fellowship, Stanford University (January - December 2017)

Publications

PUBLICATIONS

- **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 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
- **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

- **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.
CELL PRESS.2020: 569–70
- **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
- **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-+
- **High-Efficiency, Selection-free Gene Repair in Airway Stem Cells from Cystic Fibrosis Patients Rescues CFTR Function in Differentiated Epithelia.** *Cell stem cell*
Vaidyanathan, S. n., Salahudeen, A. A., Sellers, Z. M., Bravo, D. T., Choi, S. S., Batish, A. n., Le, W. n., Baik, R. n., de la O, S. n., Kaushik, M. P., Galper, N. n., Lee, C. M., Teran, et al
2019
- **Compositional Heterogeneity in Lumbar Vertebral Trabecular Bone as a Function of Disease and Treatment**
Colon-Bernal, I., Yang, P., Ahn, T., Duong, L., Pennypacker, B., Cauble, M., Vaidyanathan, S., Kozloff, K., Orr, B., Holl, M.
WILEY.2018: 294
- **Tailoring dendrimer conjugates for biomedical applications: the impact of altering hydrophobicity** *JOURNAL OF NANOPARTICLE RESEARCH*
Holl, M., Dougherty, C. A., Vaidyanathan, S.
2018; 20 (10)
- **Global Transcriptional Response to CRISPR/Cas9-AAV6-Based Genome Editing in CD34(+) Hematopoietic Stem and Progenitor Cells** *MOLECULAR THERAPY*
Cromer, M., Vaidyanathan, S., Ryan, D. E., Curry, B., Lucas, A., Camarena, J., Kaushik, M., Hay, S. R., Martin, R. M., Steinfeld, I., Bak, R. O., Dever, D. P., Hendel, et al
2018; 26 (10): 2431-2442
- **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
- **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., Henderson, J. M., Hendel, A., Shore, S., Antony, J. S., Hogrefe, R., Kormann, M. D., Porteus, M. H., McCaffrey, A. P.
2018; 12: 530-542
- **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. n., Ryan, D. E., Curry, B. n., Lucas, A. B., Camarena, J. n., Kaushik, M. n., Hay, S. R., Martin, R. M., Steinfeld, I. n., Bak, R. O., Dever, D. P., Hendel, et al
2018
- **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
- **Priming Human Repopulating Hematopoietic Stem and Progenitor Cells for Cas9/sgRNA Gene Targeting.** *Molecular therapy. Nucleic acids*
Charlesworth, C. T., Camarena, J. n., Cromer, M. K., Vaidyanathan, S. n., Bak, R. O., Carte, J. M., Potter, J. n., Dever, D. P., Porteus, M. H.
2018; 12: 89–104
- **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