Stanford



# Ami J. Shah

Clinical Professor, Pediatrics - Stem Cell Transplantation


### CLINICAL OFFICE (PRIMARY)

- **Pediatric Stem Cell Transplantation**

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## Bio

### BIO

Dr. Shah joined Stanford University in 2015 as a Clinical Professor of Pediatrics in the Division of Hematology/ Oncology, Stem Cell Transplantation and Regenerative Medicine. Dr. Shah completed medical school at the University of North Carolina- Chapel Hill. She completed her training in Pediatrics and Pediatric Hematology/ Oncology at Childrens Hospital Los Angeles. Her areas of clinical expertise have been in stem cell transplantation for malignant and non-malignant disorders. She has been actively involved with the care and treatment of children with primary immune deficiencies and is the site PI for the Primary Immune Deficiencies Consortium (PIDTC). She has experience in numerous gene therapy trials for primary immune deficiencies, hemoglobinopathies and other genetic diseases. She has a specific interest in the long term outcomes following HSCT, in specific the neurocognitive function post HSCT. She has been an active participant in American Society of Transplantation and Cellular Therapy (ASTCT), Children's Oncology Group (COG) and American Society of Hematology (ASH).

She has been actively involved with mentorship and graduate medical education, and currently serves as the Program Director for the Pediatric Hematology/ Oncology Fellowship and the Pediatric Stem Cell Fellowship. She also serves on the Pediatric Mentoring Group.

### CLINICAL FOCUS

- Pediatric Hematology-Oncology
- Pediatric Stem Cell Transplantation

### ACADEMIC APPOINTMENTS

- Clinical Professor, Pediatrics - Stem Cell Transplantation
- Member, Maternal & Child Health Research Institute (MCHRI)

### ADMINISTRATIVE APPOINTMENTS

- Program Director, Pediatric Stem Cell Transplantation Fellowship, (2022- present)
- Program Director, Pediatric Hematology/ Oncology Fellowship, Stanford School of Medicine, (2021- present)
- Associate Program Director Pediatric Hematology/ Oncology Fellowship, Stanford School of Medicine, (2020-2021)

- Quality Director of the Bass Center, Lucile Packard Childrens Hospital, (2015-2021)

### HONORS AND AWARDS

- Laura Bachrach Faculty Mentoring Award, Stanford University, Lucile Packard Children's Hospital (2019)

### BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS

- Medical Advisory Board, Make A Wish Greater Bay Area (2020 - present)
- Medical Advisory Committee, Ronald McDonald House (2018 - 2020)
- Board Member, Make A Wish Greater Los Angeles (2010 - 2013)

### PROFESSIONAL EDUCATION

- Fellowship: Childrens Hospital Los Angeles Pediatric Residency (1996) CA
- Residency: Childrens Hospital Los Angeles Pediatric Residency (1993) CA
- Internship: Childrens Hospital Los Angeles Pediatric Residency (1991) CA
- Board Certification: Pediatric Hematology-Oncology, American Board of Pediatrics (2000)
- Medical Education: University of North Carolina Chapel Hill (1990) NC

## Research & Scholarship

### CLINICAL TRIALS

- Long Term Effects On Recipients of Hematopoietic Stem Cell Transplantation, Recruiting
- A Clinical Study to Assess the Efficacy and Safety of Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy (CALD), Not Recruiting
- A Safety and Efficacy Study Evaluating CTX001 in Participants With Transfusion-Dependent β-Thalassemia, Not Recruiting
- A Safety and Efficacy Study Evaluating CTX001 in Subjects With Severe Sickle Cell Disease, Not Recruiting
- Gene Therapy for Pyruvate Kinase Deficiency (PKD), Not Recruiting
- Ibrutinib in Combination With Corticosteroids vs Placebo in Combination With Corticosteroids in Participants With New Onset Chronic Graft Versus Host Disease (cGVHD), Not Recruiting
- Study Comparing Efficacy and Safety of Defibrotide vs Best Supportive Care in the Prevention of Hepatic Veno-Occlusive Disease in Adult and Pediatric Patients, Not Recruiting
- Clinical Trial to Evaluate the Efficacy of Gene Therapy for Pyruvate Kinase Deficiency, Not Specified

## Teaching

### GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Pediatric Hem/Onc (Fellowship Program)

## Publications

### PUBLICATIONS

- **Newborn screening reduces survival disparities in SCID after stem cell transplant: A PIDTC report.** *Journal of human immunity*
  Winestone, L. E., Logan, B. R., Liu, X., Williams, O., Dunn, E. A., Thakar, M. S., Kidd, S. A., Mousallem, T., Dorsey, M. J., O'Reilly, R. J., Kapoor, N., Forbes Satter, L., Kapadia, et al
  2026; 2 (5): e20250231

- **Can we eliminate TBI/Busulfan and GVHD in Fanconi anemia transplantation? Results from a prospective haploidentical transplant approach**
  Bertaina, A., Czechowicz, A., Hiroshima, L., Kunte, N., Klinger, E., Saini, G., John, T., Alexander, J., Shyr, D., Shah, A., Klein, O., Boyel, J., Boelens, et al
  SPRINGERNATURE.2026: 122-123

- **Durable clinical benefits with exagamglogene autotemcel for greater than 6 years of follow-up in transfusion-dependent thalassemia and sickle cell disease with recurrent vaso-occlusive crises**
  Locatelli, F., Frangoul, H., Lang, P., Sharma, A., Meisel, R., Corbacioglu, S., de la Fuente, J., Bhatia, M., Mapara, M., Molinari, L., Liem, R. I., Dedeken, L., Li, et al
  SPRINGERNATURE.2026: 14-15

- **Correction of Ineffective Erythropoiesis and Normalization of Iron Homeostasis After Exagamglogene Autotemcel in Transfusion-Dependent β-Thalassemia.** *American journal of hematology*
  Sheth, S., Corbacioglu, S., de la Fuente, J., Algeri, M., Rupprecht, J., Kuo, K. H., Shah, A. J., Lang, P., Merkeley, H., Carpenter, B., Mapara, M. Y., Liem, R. I., Grupp, et al
  2026

- **Begelomab (BEGESAND®) Salvages Steroid-Resistant Acute GVHD in Pediatric Patients.** *Journal of clinical medicine*
  Shyr, D., Chirieleison, S. M., Fernandez-Pol, S., Weinacht, K., Agarwal, R., Shah, A. J., Spinelli, M., Palmieri, R., Di Naro, A. F., Bertaina, A.
  2026; 15 (11)

- **Clinical Outcomes of Lentiviral Vector Gene Therapy for Sickle Cell Disease.** *Blood advances*
  Prueksapraopong, C., Fernandes, A., Campo Fernandez, B., Roy, S., Hollis, R. P., Habtemariam, B., Pellin, D., Ceoldo, G., Lin, T., Dang, T. T., Cornetta, K., Romero, Z., Blazar, et al
  2026

- **Durable clinical benefits with exagamglogene autotemcel in transfusion-dependent thalassemia and severe sickle cell disease**
  de la Fuente, J., Locatelli, F., Lang, P., Sharma, A., Meisel, R., Grupp, S. A., Corbacioglu, S., Mapara, M., Bhatia, M., Li, A. M., Molinari, L., Liem, R. I., Dedeken, et al
  WILEY.2026: S187-S188

- **Relationship between Social Drivers of Health and Clinician Adherence to Late Effects Screening Among Allogeneic HCT Survivors in the United States**
  Taylor, A. O., Hong, S., Jones, S., Ng, K., Gooley, T. A., Bhatt, N., Hellewell, E. M., D Sung, A., Ahmed, N., Morey, C., Fitzmaurice, S., Kennedy, V. E., Lauer, et al
  ELSEVIER SCIENCE INC.2026: S24-S25

- **Model-Based ATG Dosing Optimization in Ab Haplo-HSCT: Improved Pre-HSCT Exposure Consistency and Viral Infection Analysis**
  Shyr, D. C., Hiroshima, L., John, T. D., Barbarito, G., Agarwal, R., Alexander, J. L., Shah, A. J., Li, T., Chang, I., Arreola, V., Boelens, J., Bertaina, A.
  ELSEVIER SCIENCE INC.2026

- **Durable Clinical Benefits with Exagamglogene Autotemcel for over 5 Years of Follow-up in Transfusion-Dependent Thalassemia and Sickle Cell Disease with Recurrent Vaso-Occlusive Crises**
  Bhatia, M., Frangoul, H., Locatelli, F., Lang, P., Sharma, A., Meisel, R., Corbacioglu, S., de la Fuente, J., Mapara, M. Y., Molinari, L., Liem, R., Dedeken, L., Li, et al
  ELSEVIER SCIENCE INC.2026: S2-S3

- **Neurodevelopmental outcomes following hematopoietic cell transplantation for patients with severe combined immunodeficiency (SCID): A PIDTC study.** *Journal of human immunity*
  Brumm, V. L., Kidd, S. A., Logan, B. R., Chunara, F., Heimall, J., Griffith, L. M., Kohn, D. B., Sanchez, L., Bednarski, J. J., Martinez, C., Lugt, M. V., Kapoor, N., Wright, et al
  2026; 2 (1): e20250163

- **Hematopoietic Cell Transplantation for Wiskott-Aldrich syndrome: A PIDTC Report.** *Blood advances*
  Alexander, J. L., Dávila Saldaña, B. J., Brazauskas, R., Dammalapati, S. G., Griffith, L. M., Shah, A. J., Shimano, K. A., Ochs, H. D., Bleesing, J., Ebens, C. L., Kapadia, M., Bauchat, A., Kapoor, et al
  2025

- **Outcomes Following Matched Sibling Donor Transplantation for Severe Combined Immunodeficiency: A Report from the PIDTC.** *Blood advances*
  Rayes, A., Logan, B. R., Liu, X., Dara, J., Buckley, R. H., Oved, J. H., Kapoor, N., Kapadia, M., Chandra, S., Martinez, C. A., Bunin, N., Chandrakasan, S., Chen, et al
  2025

- **DURABLE CLINICAL BENEFITS IN TRANSFUSION-DEPENDENT B-THALASSEMIA WITH EXAGAMGLOGENE AUTOTEMCEL**
  Locatelli, F., Lang, P., Meisel, R., Corbacioglu, S., Wall, D., Li, A., de la fuente, J., Shah, A., Ben Carpenter, Kwiatkowski, J., Mapara, M., Liem, R., Handgreitinger, R., et al

SPRINGERNATURE.2025: 102-103

- **DURABLE CLINICAL BENEFITS IN SEVERE SICKLE CELL DISEASE WITH EXAGAMGLOGENE AUTOTEMCEL**
  Frangoul, H., Locatelli, F., Sharma, A., Bhatia, M., Mapara, M., Molinari, L., Wall, D., Dedeken, L., Liem, R., Shah, A., Telfer, P., Corbacioglu, S., Rondelli, et al
  SPRINGERNATURE.2025: 100-101

- **Pre-transplant CAR T-cell therapy is associated with inferior survival and increased non-relapse mortality in pediatric B-ALL: A single-center retrospective analysis**
  Chok, R., Huang, M., Aftandilian, C., Schultz, L., Shyr, D., Lacayo, N., Shah, A., Saab, R., Gruber, T., Mackall, C., Bertaina, A., Davis, K., Klein, et al
  ELSEVIER.2025: 6077-6078

- **Long-term follow-up demonstrates durable clinical benefits of exagamglogene autotemcel for sickle cell disease with recurrent vaso-occlusive crises: Final results of climb SCD-121**
  Grupp, S., Locatelli, F., Sharma, A., Mapara, M., Bhatia, M., Liem, R., Dedeken, L., Molinari, L., Eckrich, M., Kuo, K., Telfer, P., Shah, A., Cavazzana, et al
  ELSEVIER.2025: 2537-2538

- **Correction of ineffective erythropoiesis and durable clinical benefit with exagamglogene autotemcel for transfusion-dependent β-thalassemia**
  Locatelli, F., Meisel, R., Corbacioglu, S., De La Fuente, J., Algeri, M., Rupprecht, J., Kuo, K., Shah, A., Lang, P., Merkeley, H., Carpenter, B., Mapara, M., Liem, et al
  ELSEVIER.2025: 6089-6090

- **Durable clinical benefits with exagamglogene autotemcel for transfusion-dependent thalassemia and severe sickle cell disease**
  de la Fuente, J., Frangoul, H., Locatelli, F., Lang, P., Wall, D., Sharma, A., Meisel, R., Corbacioglu, S., Mapara, M., Bhatia, M., Molinari, L., Liem, R. I., Dedeken, et al
  WILEY.2025: S19-S20

- **Improved outcome of HSCT in STAT1 gain-of-function disease following JAK inhibition bridging.** *Journal of human immunity*
  Buddingh, E. P., Slatter, M., Becerra, J. C., Garcia, L. A., von Asmuth, E., Baris, S., de la Calle-Martín, O., Chan, A. Y., Chan, S. B., Chandrakasan, S., Chellapandian, D., Dara, J., Farmand, et al
  2025; 1 (3)

- **Improvements in Health-Related Quality of Life in Patients with Transfusion-Dependent beta-Thalassemia After Exagamglogene Autotemcel.** *Blood advances*
  de la Fuente, J., Frangoul, H., Lang, P., Wall, D., Meisel, R., Corbacioglu, S., Li, A. M., Shah, A. J., Carpenter, B., Kwiatkowski, J. L., Mapara, M. Y., Liem, R. I., Rupprecht, et al
  2025

- **Pediatric Transplant and Cellular Therapy Consortium RESILIENT Conference on Pediatric Chronic Graft-Versus-Host Disease Survivorship After Hematopoietic Cell Transplantation: Part III. Long-term Impact of Chronic GVHD on Endocrinologic, Cardiovascular, and Metabolic Outcomes in Survivors of Pediatric HCT.** *Transplantation and cellular therapy*
  Khandelwalhl, P., Fish, J. D., Gorfinkel, L., Guilcher, G. M., Howell, J., Nishitani, M., Nuechterlein, B., Obeng, E., Shah, A. J., Cuvelier, G. D., Rotz, S., Williams, K. M., Duncan, et al
  2025

- **A Patient With NEMO Deficiency, Disseminated M. szulgai, and Post-HSCT Inflammatory Disease.** *Pediatric transplantation*
  Rosenthal, A., Goyal, A., Chen, S. F., Lewis, D. B., Shah, A.
  2025; 29 (1): e70020

- **Improved outcome of HSCT in STAT1 gain-of-function disease following JAK inhibition as bridging treatment, An IEWP/ PIDTC Study**
  *Improved outcome of HSCT in STAT1 Gain of function following JAK inhibition as briding treatment, an IEWP/ PIDTC study*
  Buddingh, E. P.
  2025; 1 (3)

- **RP-L301, a Lentiviral-Mediated Gene Therapy for Pyruvate Kinase Deficiency (PKD): A Phase 2 Clinical Trial Design**
  Shah, A. J., Lorenzo, J., Grace, R. F., Beers, E., Bartels, M., Glenthoej, A., Navarro, S., Llanos, L., Gaisse, B., Sanchez, S., Zubicaray, J., Chien, M., Bustamante, et al
  ELSEVIER.2024: 35831-35832

- **Durable Clinical Benefits with Exagamglogene Autotemcel for Severe Sickle Cell Disease**

Frangoul, H., Locatelli, F., Sharma, A., Bhatia, M., Mapara, M. Y., Liem, R. I., Wall, D., Molinari, L., Dedeken, L., Kuo, K. H. M., Telfer, P., Shah, A. J., Cavazzana, et al

ELSEVIER.2024

- **Durable Clinical Benefits with Exagamglogene Autotemcel for Transfusion-Dependent β-Thalassemia**

  Locatelli, F., Lang, P., Meisel, R., Wall, D., Corbacioglu, S., Li, A. M., de la Fuente, J., Shah, A. J., Carpenter, B., Kwiatkowski, J. L., Mapara, M. Y., Liem, R. I., Cappellini, et al

  ELSEVIER.2024: 512-514

- **OUTCOME OF PATIENTS WITH SIGNAL-TRANSDUCER AND ACTIVATOR OF OF TRANSCRIPTION 1 MUTATIONS (STAT1) GAIN-OF-FUNCTION VARIANTS AFTER HAEMATOPOIETIC STEM CELL TRANSPLANTATION - AN IEWP/PIDTC STUDY**

  Buddingh, E., Slatter, M., Aldave Becerra, J., Alonso Garcia, L., von Asmuth, E., Baris, S., de la Calle-Martin, O., Chan, A., Chan, B., Chandrakasan, S., Chellapandian, D., Dara, J., Landsverk, et al

  SPRINGERNATURE.2024: 94-95

- **Neurodevelopmental Outcomes (ND) in Patients with Severe Combined Immunodeficiency (SCID) Following Hematopoietic Cell Transplantation (HCT) in the Era of Newborn Screening. A PIDTC Study**

  Shah, A., Brumm, V., Kidd, S., Logan, B., Chunara, F., Heimall, J., Schofield, H., Sanchez, L., Bednarski, J., Chandra, S., Chellapandian, D., Ebens, C., Lust, et al

  ACADEMIC PRESS INC ELSEVIER SCIENCE.2024

- **Provider Perspectives of Long-Term Follow-Up Care of Patients with Severe Combined Immunodeficiency**

  Ige, K., Buchbinder, D., Izadi, N., Butte, M., Chen, X., Collins, C., Cowan, M., Dimitriades, V., Franck, L., Nguyen, K., Shah, A., Puck, J., Smith, et al

  ACADEMIC PRESS INC ELSEVIER SCIENCE.2024

- **Exagamglogene Autotemcel for Severe Sickle Cell Disease.** *The New England journal of medicine*

  Frangoul, H., Locatelli, F., Sharma, A., Bhatia, M., Mapara, M., Molinari, L., Wall, D., Liem, R. I., Telfer, P., Shah, A. J., Cavazzana, M., Corbacioglu, S., Rondelli, et al

  2024

- **Exagamglogene Autotemcel for Transfusion-Dependent β-Thalassemia.** *The New England journal of medicine*

  Locatelli, F., Lang, P., Wall, D., Meisel, R., Corbacioglu, S., Li, A. M., de la Fuente, J., Shah, A. J., Carpenter, B., Kwiatkowski, J. L., Mapara, M., Liem, R. I., Cappellini, et al

  2024

- **Gene Therapy for Adult and Pediatric Patients with Severe Pyruvate Kinase Deficiency: Results from a Global Study of RP-L301**

  Shah, A., Sevilla, J., Lopez Lorenzo, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Quintana Bustamante, O., Gonzalez Vicent, M., Iriondo, et al

  CELL PRESS.2024: 2-3

- **Relevance of lymphocyte proliferation to PHA in severe combined immunodeficiency (SCID) and T cell lymphopenia.** *Clinical immunology (Orlando, Fla.)*

  Abraham, R. S., Basu, A., Heimall, J. R., Dunn, E., Yip, A., Kapadia, M., Kapoor, N., Satter, L. F., Buckley, R., O'Reilly, R., Cuvelier, G. D., Chandra, S., Bednarski, et al

  2024: 109942

- **Diagnosis and management of pyruvate kinase deficiency: international expert guidelines.** *The Lancet. Haematology*

  Al-Samkari, H., Shehata, N., Lang-Robertson, K., Bianchi, P., Glenthoj, A., Sheth, S., Neufeld, E. J., Rees, D. C., Chonat, S., Kuo, K. H., Rothman, J. A., Barcellini, W., van Beers, et al

  2024

- **Exagamglogene Autotemcel for Severe Sickle Cell Disease**

  Frangoul, H., Locatelli, F., Sharma, A., Bhatia, M., Mapara, M., Molinari, L., Wall, D., Liem, R. I., Telfer, P., Shah, A. J., Cavazzana, M., Corbacioglu, S., Rondelli, et al

  AMER SOC HEMATOLOGY.2023

- **Exagamglogene Autotemcel for Transfusion-Dependent ß-Thalassemia**

  Locatelli, F., Lang, P., Corbacioglu, S., Wall, D., Meisel, R., Li, A. M., de La Fuente, J., Shah, A. J., Carpenter, B., Kwiatkowski, J. L., Mapara, M., Liem, R. I., Cappellini, et al

  AMER SOC HEMATOLOGY.2023

- **LENTIVIRAL-MEDIATED GENE THERAPY FOR SEVERE PYRUVATE KINASE DEFICIENCY: RESULTS FROM AN ONGOING GLOBAL PHASE 1 STUDY**
  Lopez Lorenzo, J., Shah, A., Sevilla, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Quintana Bustamante, O., Zeini, M., Choi, et al
  SPRINGERNATURE.2023: 275-276

- **Busulfan and subsequent malignancy: An evidence-based risk assessment.** *Pediatric blood & cancer*
  Long-Boyle, J. R., Kohn, D. B., Shah, A. J., Spencer, S. M., Sevilla, J., Booth, C., López Lorenzo, J. L., Nicoletti, E., Shah, A., Reatz, M., Matos, J., Schwartz, J. D.
  2023: e30738

- **Post-Transplant Late Complications Increase Over Time for Patients with SCID: A Primary Immune Deficiency Treatment Consortium (PIDTC) Landmark Study.** *The Journal of allergy and clinical immunology*
  Eissa, H., Thakar, M., Shah, A. J., Logan, B., Griffith, L. M., Dong, H., Parrott, R. E., O'Reilly, R., Dara, J., Kapoor, N., Satter, L. F., Chandra, S., Kapadia, et al
  2023

- **Genotype, Oxidase Status, and Preceding Infection or Autoinflammation Do Not Affect Allogeneic HCT Outcomes for CGD.** *Blood*
  Leiding, J. W., Arnold, D. E., Parikh, S. H., Logan, B. R., Marsh, R. A., Griffith, L. M., Wu, R., Kidd, S., Mallhi, K. K., Chellapandian, D., Si Lim, S., Grunebaum, E., Falcone, et al
  2023

- **Measuring the effect of newborn screening on survival after haematopoietic cell transplantation for severe combined immunodeficiency: a 36-year longitudinal study from the Primary Immune Deficiency Treatment Consortium.** *Lancet (London, England)*
  Thakar, M. S., Logan, B. R., Puck, J. M., Dunn, E. A., Buckley, R. H., Cowan, M. J., O'Reilly, R. J., Kapoor, N., Satter, L. F., Pai, S., Heimall, J., Chandra, S., Ebens, et al
  2023

- **LENTIVIRAL-MEDIATED GENE THERAPY FOR SEVERE PYRUVATE KINASE DEFICIENCY: GLOBAL PHASE 1 STUDY RESULTS**
  Shah, A., Lorenzo, J., Sevilla, J., Navarro, S., Llanos, L., Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Bustamante, O. Q., Zeini, M., Choi, et al
  WILEY.2023: S133-S134

- **Viral-specific T cells for Cytomegalovirus retinitis following hematopoietic stem cell transplantation: A success story.** *Pediatric blood & cancer*
  Montiel-Esparza, R., Michalak, S. M., Le, A. H., Or, C., Nguyen, Q. D., Khoury, R., Grimley, M. S., Bertaina, A., Klinger, E., Shah, A. J., Wood, E. H.
  2023: e30429

- **Global Phase 1 Study Results of Lentiviral Mediated Gene Therapy for Severe Pyruvate Kinase Deficiency**
  Shah, A. J., Lopez Lorenzo, J., Sevilla, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Quintana Bustamante, O., Zeini, M., Choi, et al
  CELL PRESS.2023: 118-119

- **Chronic granulomatous disease identified in the evaluation of atypical Kawasaki disease in an infant**
  Solomon, B., Reddy, A., Konvinse, K., Shah, A., Gernez, Y., Mony, V.
  ACADEMIC PRESS INC ELSEVIER SCIENCE.2023: 133

- **PMD 10428247** *European Hematology Association*
  Franco, L., Peter, L., Selim, c., Amanda, L., Josu, d., Donna, w., Roland, M., Shah, A. j., Robert, L., Marcus, M., Ben, C., Kwiatkowski, J., Maria, et al
  2023

- **The diagnosis of severe combined immunodeficiency: Implementation of the PIDTC 2022 Definitions.** *The Journal of allergy and clinical immunology*
  Dvorak, C. C., Haddad, E., Heimall, J., Dunn, E., Cowan, M. J., Pai, S., Kapoor, N., Satter, L. F., Buckley, R. H., O'Reilly, R. J., Chandra, S., Bednarski, J. J., Williams, et al
  2022

- **The diagnosis of severe combined immunodeficiency (SCID): The Primary Immune Deficiency Treatment Consortium (PIDTC) 2022 Definitions.** *The Journal of allergy and clinical immunology*
  Dvorak, C. C., Haddad, E., Heimall, J., Dunn, E., Buckley, R. H., Kohn, D. B., Cowan, M. J., Pai, S., Griffith, L. M., Cuvelier, G. D., Eissa, H., Shah, A. J., O'Reilly, et al

2022

- **Lentiviral-mediated Gene Therapy for Adults and Children with Severe Pyruvate Kinase Deficiency: Results from an Ongoing Global Phase 1 Study**
  Shah, A. J., Lopez Lorenzo, J., Sevilla, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Bustamante, O., Zeini, M., Choi, et al
  AMER SOC HEMATOLOGY.2022: 4902-4903

- **Efficacy and Safety of a Single Dose of Exagamglogene Autotemcel for Transfusion-Dependent beta-Thalassemia**
  Locatelli, F., Lang, P., Li, A., Corbacioglu, S., de la Fuente, J., Wall, D. A., Liem, R., Meisel, R., Mapara, M. Y., Shah, A. J., Cappellini, M., Kattamis, A., Sheth, et al
  AMER SOC HEMATOLOGY.2022: 4899-4901

- **Mitapivat versus Placebo for Pyruvate Kinase Deficiency.** *The New England journal of medicine*
  Shah, A. J., Schwartz, J. D., Segovia, J. C.
  2022; 386 (26): 2538-2539

- **Sequential Stem Cell-Kidney Transplantation in Schimke Immuno-osseous Dysplasia.** *The New England journal of medicine*
  Bertaina, A., Grimm, P. C., Weinberg, K., Parkman, R., Kristovich, K. M., Barbarito, G., Lippner, E., Dhamdhere, G., Ramachandran, V., Spatz, J. M., Fathallah-Shaykh, S., Atkinson, T. P., Al-Uzri, et al
  2022; 386 (24): 2295-2302

- **Outcomes Following Treatment for Adenosine Deaminase Deficient Severe Combined Immunodeficiency: A Report from the PIDTC.** *Blood*
  Cuvelier, G. D., Logan, B. R., Prockop, S., Buckley, R. H., Kuo, C. Y., Griffith, L. M., Liu, X., Yip, A., Hershfield, M., Ayoub, P., Moore, T. B., Dorsey, M., O'Reilly, et al
  2022

- **Male-Specific Late Effects in Adult Hematopoietic Cell Transplantation Recipients: A Systematic Review from the Late Effects and Quality of Life Working Committee of the Center for International Blood and Marrow Transplant Research and Transplant Complications Working Party of the European Society of Blood and Marrow Transplantation** *TRANSPLANTATION AND CELLULAR THERAPY*
  Phelan, R., Im, A., Hunter, R. L., Inamoto, Y., Lupo-Stanghellini, M., Rovo, A., Badawy, S. M., Burns, L., Eissa, H., Murthy, H. S., Prasad, P., Sharma, A., Suelzer, et al
  2022; 28 (6)

- **Male-specific late effects in adult hematopoietic cell transplantation recipients: a systematic review from the Late Effects and Quality of Life Working Committee of the Center for International Blood and Marrow Transplant Research and Transplant Complications Working Party of the European Society of Blood and Marrow Transplantation.** *Bone marrow transplantation*
  Phelan, R., Im, A., Hunter, R. L., Inamoto, Y., Lupo-Stanghellini, M. T., Rovo, A., Badawy, S. M., Burns, L., Eissa, H., Murthy, H. S., Prasad, P., Sharma, A., Suelzer, et al
  2022

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