



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
- BMT 348 CTX001 (Vertex-SCD), Not Recruiting
- BMT 359 B-Thal (CRISPR), Not Recruiting
- BMT347 ALD, 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
- Phase III Defibrotide vs BSC in the Prevention of Hepatic Venoo-Occlusive Disease in HSCT, 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

- **Clinical Outcomes of Lentiviral Vector Gene Therapy for Sickle Cell Disease.** *Blood advances*
Prueksaprapong, 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

- **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
- **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
- **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*
Raves, 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., Handgretinger, R., et al
SPRINGER NATURE.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
SPRINGER NATURE.2025: 100-101
- **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
- **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
- **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*
Khandelwal, 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 bridging treatment, an IEWP/ PIDTC study
Buddingh, E. P.
2025; 1 (3)
 - **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
 - **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
 - **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
 - **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
SPRINGER.2024: 94-95
 - **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
 - **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

- **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., Glenthøj, 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 β -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
- **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
- **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
- **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
- **Stemming the Tide of Gastrointestinal Chronic Granulomatous Disease.** *Digestive diseases and sciences*
Cagil, Y., Tong, E., Guimaraes, C., Andrews, J., Abu El Haija, M., Triadafilopoulos, G., Yeh, A. M., Shah, A.
2022
- **Transplanted CGD Patients have Higher Burden of Disease than Conventionally Treated Patients that Resolve with Transplantation - A PIDTC Report**
Leiding, J., Danielle, A., Parikh, S., Logan, B., Marsh, R., Griffith, L., Wu, R., Mallhi, K., Chellapandian, D., Lim, S., Grunebaum, E., Broglie, L., Prasad, et al
SPRINGER/PLENUM PUBLISHERS.2022: S76-S78
- **Sequential hematopoietic stem cell transplantation (HSCT) followed by kidney transplant-3 children who received ABT-cell/CD19 B-cell depleted HSCT followed by kidney transplant from same parental donor are rejection free and immunosuppression free with donor specific tolerance > 1year post kidney transplant**
Grimm, P., Lewis, D. B., Al-Uzri, A., Fathallah-Shaykh, S., Shah, A., Agarwal, R., Weinberg, K. I., Gallo, A., Concepcion, W., Bertaina, A.
WILEY.2022
- **Who Should be Eligible for Gene Therapy Clinical Trials in Red Blood Cell Pyruvate Kinase Deficiency (PKD)?: Towards an Expanded Definition of Severe PKD.** *American journal of hematology*
Schwartz, J. D., Barcellini, W., Grace, R. F., Bianchi, P., Zanella, A., Lopez Lorenzo, J. L., Sevilla, J., Shah, A. J., Glader, B., Nicoletti, E., Ordonez, S. N., Segovia, J. C.
1800
- **Functional Immune Tolerance Induced By Sequential Hematopoietic Stem Cell-Solid Organ Transplantation**
Bertaina, A., Barbarito, G., Ramachandran, V. V., Kristovich, K., Lippner, E., Fathallah-Shaykh, S., Al-Uzri, A., Shah, A. J., Aubert, G., Slepicka, P., Oppizzi, L., Agarwal, R., Roncarolo, et al
AMER SOC HEMATOLOGY.2021: 1818-+
- **Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: Interim Results of a Global Phase 1 Study for Adult and Pediatric Patients**
Shah, A. J., Lopez Lorenzo, J., Navarro, S., Sevilla, J., Llanos, L., Perez de Camino Gaisse, B., Sanchez, S., Glader, B., Chien, M., Quintana Bustamante, O., Beard, B. C., Law, K. M., Zeini, et al
AMER SOC HEMATOLOGY.2021
- **Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency.** *The New England journal of medicine*
Kohn, D. B., Booth, C., Shaw, K. L., Xu-Bayford, J., Garabedian, E., Trevisan, V., Carbonaro-Sarracino, D. A., Soni, K., Terrazas, D., Snell, K., Ikeda, A., Leon-Rico, D., Moore, et al
2021
- **Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: Updated Results of a Global Phase 1 Study for Adult and Pediatric Patients**
Lopez Lorenzo, J., Shah, A. J., Navarro, S., Sevilla, J., Llanos, L., Perez Camino de Gaisse, B., Sanchez, S., Glader, B., Chien, M., Quintana-Bustamante, O., Beard, B. C., Law, K. M., Zeini, et al
CELL PRESS.2021: 42-43
- **Return to Work Among Young Adult Survivors of Allogeneic Hematopoietic Cell Transplantation in the United States.** *Transplantation and cellular therapy*
Bhatt, N. S., Brazauskas, R., Salit, R. B., Syrjala, K., Bo-Subait, S., Tecca, H., Badawy, S. M., Baker, K. S., Beitinjaneh, A., Bejanyan, N., Byrne, M., Dias, A., Farhadfar, et al

2021

- **Immune function in childhood cancer survivors: a Children's Oncology Group review.** *The Lancet. Child & adolescent health*
Guilcher, G. M., Rivard, L., Huang, J. T., Wright, N. A., Anderson, L., Eissa, H., Pelletier, W., Ramachandran, S., Schechter, T., Shah, A. J., Wong, K., Chow, E. J.
2021
- **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. T., Rovo, A., Badawy, S. M., Burns, L., Eissa, H., Murthy, H. S., Prasad, P., Sharma, A., Suelzer, et al
2021
- **Correction to: Infections in Infants with SCID: Isolation, Infection Screening and Prophylaxis in PIDTC Centers.** *Journal of clinical immunology*
Dorsey, M., Wright, N. A., Chaimowitz, N. S., Davila Saldana, B. J., Miller, H., Keller, M. D., Thakar, M. S., Shah, A. J., Abu-Arja, R., Andolina, J., Aquino, V., Barnum, J. L., Bednarski, et al
2020
- **Infections in Infants with SCID: Isolation, Infection Screening, and Prophylaxis in PIDTC Centers.** *Journal of clinical immunology*
Dorsey, M. J., Wright, N. A., Chaimowitz, N. S., Davila Saldana, B. J., Miller, H., Keller, M. D., Thakar, M. S., Shah, A. J., Abu-Arja, R., Andolina, J., Aquino, V., Barnum, J. L., Bednarski, et al
2020
- **Correction: Chronic Granulomatous Disease-Associated IBD Resolves and Does Not Adversely Impact Survival Following Allogeneic HCT.** *Journal of clinical immunology*
Marsh, R. A., Leiding, J. W., Logan, B. R., Griffith, L. M., Arnold, D. E., Haddad, E., Falcone, E. L., Yin, Z., Patel, K., Arbuckle, E., Bleesing, J. J., Sullivan, K. E., Heimall, et al
2020
- **CRISPR-based gene editing enables FOXP3 gene repair in IPEX patient cells.** *Science advances*
Goodwin, M., Lee, E., Lakshmanan, U., Shipp, S., Froessler, L., Barzaghi, F., Passerini, L., Narula, M., Sheikali, A., Lee, C. M., Bao, G., Bauer, C. S., Miller, et al
2020; 6 (19): eaaz0571
- **CRISPR-based gene editing enables FOXP3 gene repair in IPEX patient cells** *SCIENCE ADVANCES*
Goodwin, M., Lee, E., Lakshmanan, U., Shipp, S., Froessler, L., Barzaghi, F., Passerini, L., Narula, M., Sheikali, A., Lee, C. M., Bao, G., Bauer, C. S., Miller, et al
2020; 6 (19)
- **Excellent Outcomes Following Hematopoietic Cell Transplantation for Wiskott-Aldrich Syndrome: A PIDTC Report.** *Blood*
Burroughs, L., Petrovic, A., Brazauskas, R., Liu, X., Griffith, L. M., Ochs, H. D., Bleesing, J., Edwards, S., Dvorak, C. C., Chaudhury, S., Prockop, S., Quinones, R., Goldman, et al
2020
- **Novel nonsense IKBKG Mutation in an Infant Presenting with Pneumocystis Jiroveci Pneumonia and Disseminated Mycobacterium Szulgai Infection**
Lo, R., Rosenthal, A., Shah, A., Chen, S., Eguiguren, L., Siddiqi, A., Hernandez, J., Lewis, D.
SPRINGER/PLENUM PUBLISHERS.2020: S119–S120
- **A beta T-Cell/CD19 B-Cell Depleted Haploidentical Stem Cell Transplantation: A New Platform for Curing Rare and Monogenic Disorders**
Bertaina, A., Bacchetta, R., Lewis, D. B., Grimm, P. C., Shah, A. J., Agarwal, R., Concepcion, W., Czechowicz, A., Bhatia, N., Lahiri, P., Weinberg, K. I., Parkman, R., Porteus, et al
ELSEVIER SCIENCE INC.2020: S288
- **Transplantation Outcomes for Children with Severe Combined Immune Deficiency (SCID) Have Improved over Time: A 36-Year Summary Report By the Primary Immune Deficiency Treatment Consortium (PIDTC)**
Thakar, M. S., Logan, B., Buckley, R. H., Haddad, E., Dvorak, C. C., O'Reilly, R. J., Kapoor, N., Satter, L., Martinez, C., Pai, S., Heimall, J., Jyonouchi, S., Sullivan, et al
ELSEVIER SCIENCE INC.2020: S18–S19

- **Experience with Ruxolitinib (Jakafi (R)) As a Salvage Therapy for Graft-Versus-Host Disease in Children and Young Adults**
Mavers, M., Klinger, E., Shyr, D. C., Shah, A. J., Bertaina, A., Soni, S.
ELSEVIER SCIENCE INC.2020: S179
- **Quality of Life of Patients with Wiskott Aldrich Syndrome and X-Linked Thrombocytopenia: a Study of the Primary Immune Deficiency Consortium (PIDTC), Immune Deficiency Foundation, and the Wiskott-Aldrich Foundation.** *Journal of clinical immunology*
Shah, A. J., Sokolic, R., Logan, B., Yin, Z., Iyengar, S., Scalchunes, C., Mangurian, C., Albert, M., Cowan, M. J.
2019
- **Chronic Granulomatous Disease-Associated IBD Resolves and Does Not Adversely Impact Survival Following Allogeneic HCT.** *Journal of clinical immunology*
Marsh, R. A., Leiding, J. W., Logan, B. R., Griffith, L. M., Arnold, D. E., Haddad, E., Falcone, E. L., Yin, Z., Patel, K., Arbuckle, E., Blessing, J. J., Sullivan, K. E., Heimall, et al
2019
- **GRFS and CRFS in alternative donor hematopoietic cell transplantation for pediatric patients with acute leukemia.** *Blood advances*
Mehta, R. S., Holtan, S. G., Wang, T., Hemmer, M. T., Spellman, S. R., Arora, M., Couriel, D. R., Alousi, A. M., Pidala, J., Abdel-Azim, H., Ahmed, I., Aljurf, M., Askar, et al
2019; 3 (9): 1441–49
- **GRFS and CRFS in alternative donor hematopoietic cell transplantation for pediatric patients with acute leukemia** *BLOOD ADVANCES*
Mehta, R. S., Holtan, S. G., Wang, T., Hemmer, M. T., Spellman, S. R., Arora, M., Couriel, D. R., Alousi, A. M., Pidala, J., Abdel-Azim, H., Ahmed, I., Aljurf, M., Askar, et al
2019; 3 (9): 1441–49
- **Non-Graft-versus-Host Disease Ocular Complications after Hematopoietic Cell Transplantation: Expert Review from the Late Effects and Quality of Life Working Committee of the Center for International Blood and Marrow Transplant Research and the Transplant Complications Working Party of the European Society for Blood and Marrow Transplantation** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Inamoto, Y., Petricek, I., Burns, L., Chhabra, S., DeFilipp, Z., Hematti, P., Rovo, A., Schears, R., Shah, A., Agrawal, V., Ahmed, A., Ahmed, I., Ali, et al
2019; 25 (5): E145–E154
- **Correction: Non-GVHD ocular complications after hematopoietic cell transplantation: expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT.** *Bone marrow transplantation*
Inamoto, Y., Petricek, I., Burns, L., Chhabra, S., DeFilipp, Z., Hematti, P., Rovo, A., Schears, R., Shah, A., Agrawal, V., Al-Khinji, A., Ahmed, I., Ali, et al
2019
- **Characteristics of Late Fatal Infections after Allogeneic Hematopoietic Cell Transplantation** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Norkin, M., Shaw, B. E., Brazauskas, R., Tecca, H. R., Leather, H. L., Gea-Banacloche, J., Kamble, R. T., DeFilipp, Z., Jacobsohn, D. A., Ringden, O., Inamoto, Y., Kasow, K. A., Buchbinder, et al
2019; 25 (2): 362–68
- **Non-GVHD ocular complications after hematopoietic cell transplantation: expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT.** *Bone marrow transplantation*
Inamoto, Y., Petricek, I., Burns, L., Chhabra, S., DeFilipp, Z., Hematti, P., Rovo, A., Schears, R., Shah, A., Agrawal, V., Ahmed, A., Ahmed, I., Ali, et al
2018
- **Ocular graft-versus-host disease after hematopoietic cell transplantation: Expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT.** *Bone marrow transplantation*
Inamoto, Y., Valdes-Sanz, N., Ogawa, Y., Alves, M., Berchicci, L., Galvin, J., Greinix, H., Hale, G. A., Horn, B., Kelly, D., Liu, H., Rowley, S., Schoemans, et al
2018
- **Non-Graft-versus-Host Disease Ocular Complications after Hematopoietic Cell Transplantation: Expert Review from the Late Effects and Quality of Life Working Committee of the Center for International Blood and Marrow Transplant Research and the Transplant Complications Working Party of the European Society for Blood and Marrow Transplantation.** *Biology of blood and marrow transplantation : journal of the American Society for Blood and Marrow Transplantation*

- Inamoto, Y., Petricek, I., Burns, L., Chhabra, S., DeFilipp, Z., Hematti, P., Rovo, A., Schears, R., Shah, A., Agrawal, V., Ahmed, A., Ahmed, I., Ali, et al
2018
- **Ocular graft-versus-host disease after hematopoietic cell transplantation: expert review from the Late Effects and Quality of Life Working Committee of the CIBMTR and Transplant Complications Working Party of the EBMT.** *Biology of blood and marrow transplantation : journal of the American Society for Blood and Marrow Transplantation*
Inamoto, Y., Valdes-Sanz, N., Ogawa, Y., Alves, M., Berchicci, L., Galvin, J., Greinix, H., Hale, G. A., Horn, B., Kelly, D., Liu, H., Rowley, S., Schoemans, et al
2018
 - **Characteristics of Late Fatal Infections after Allogeneic Hematopoietic Cell Transplant.** *Biology of blood and marrow transplantation : journal of the American Society for Blood and Marrow Transplantation*
Norkin, M., Shaw, B. E., Brazauskas, R., Tecca, H. R., Leather, H. L., Gea-Banacloche, J., Kamble, R., DeFilipp, Z., Jacobsohn, D. A., Ringden, O., Inamoto, Y., Kasow, K., Buchbinder, et al
2018
 - **Infusion of Alloenergized Donor Lymphocytes after CD34-selected Haploidentical Myeloablative Hematopoietic Stem Cell Transplantation** *CLINICAL CANCER RESEARCH*
Davies, J. K., Brennan, L. L., Wingard, J. R., Cogle, C. R., Kapoor, N., Shah, A. J., Dey, B. R., Spitzer, T. R., de Lima, M., Cooper, L. J., Thall, P. F., Champlin, R. E., Nadler, et al
2018; 24 (17): 4098–4109
 - **Neurocognitive dysfunction in hematopoietic cell transplant recipients: expert review from the late effects and Quality of Life Working Committee of the CIBMTR and complications and Quality of Life Working Party of the EBMT** *BONE MARROW TRANSPLANTATION*
Buchbinder, D., Kelly, D., Duarte, R. F., Auletta, J. J., Bhatt, N., Byrne, M., DeFilipp, Z., Gabriel, M., Mahindra, A., Norkin, M., Schoemans, H., Shah, A. J., Ahmed, et al
2018; 53 (5): 535–55
 - **Lenti-D Hematopoietic Stem Cell Gene Therapy to Arrest Progression of Cerebral Adrenoleukodystrophy: Interim Results of an International Phase 2/3 Trial**
Duncan, C., Eichler, F., Musolino, P. L., Orchard, P. J., De Oliveira, S., Thrasher, A. J., Armant, M. A., Dansereau, C., Lund, T. C., Miller, W. P., Raymond, G. V., Sankar, R., Shah, et al
ELSEVIER SCIENCE INC.2018: S65–S66
 - **Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy.** *The New England journal of medicine*
Eichler, F., Duncan, C., Musolino, P. L., Orchard, P. J., De Oliveira, S., Thrasher, A. J., Armant, M., Dansereau, C., Lund, T. C., Miller, W. P., Raymond, G. V., Sankar, R., Shah, et al
2017; 377 (17): 1630-1638
 - **Anti-Fungal Prophylaxis Using Intermediate Dose Ambisome is Associated with Delayed Methotrexate Clearance in Pediatric Patients Undergoing Allogeneic Hematopoietic Stem Cell Transplantation**
Schultz, L., Kumar, K., Stone, S., Callard, E., Witkowski, J., Shinn, L., Pinner, L., Weinberg, K. I., Porteus, M. H., Agarwal, R., Shah, A. J., Kharbanda, S.
ELSEVIER SCIENCE INC.2017: S297–S298
 - **Neurocognitive Dysfunction in Hematopoietic Cell Transplant Recipients: Expert Review from the Late Effects and Quality of Life Working Committee of the Center for International Blood and Marrow Transplant Research and Complications and Quality of Life Working Party of the European Group for Blood and Marrow Transplantation.** *Biology of blood and marrow transplantation : journal of the American Society for Blood and Marrow Transplantation*
Kelly, D. L., Buchbinder, D. n., Duarte, R. F., Auletta, J. J., Bhatt, N. n., Byrne, M. n., Gabriel, M. n., Mahindra, A. n., Norkin, M. n., Schoemans, H. n., Shah, A. J., Ahmed, I. n., Atsuta, et al
2017
 - **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
 - **Late Effects Surveillance Recommendations among Survivors of Childhood Hematopoietic Cell Transplantation: A Children's Oncology Group Report.** *Biology of blood and marrow transplantation*

Chow, E. J., Anderson, L., Baker, K. S., Bhatia, S., Guilcher, G. M., Huang, J. T., Pelletier, W., Perkins, J. L., Rivard, L. S., Schechter, T., Shah, A. J., Wilson, K. D., Wong, et al
2016; 22 (5): 782-795

- **Interim Results from a Phase 2/3 Study of the Efficacy and Safety of Ex Vivo Gene Therapy With Lentiviral Vector (Lenti-D) for Childhood Cerebral Adrenoleukodystrophy**
Eichler, F., Duncan, C., Thrasher, A., Shah, A., Baruchel, A., Paker, A., Kohn, D., Bratkovic, D., Shamir, E., Raymond, G., Gaspar, H., Amartino, H., Dalle, et al
LIPPINCOTT WILLIAMS & WILKINS.2016
- **Safety, Pharmacokinetics, and Efficacy of Palifermin in Children and Adolescents with Acute Leukemias Undergoing Myeloablative Therapy and Allogeneic Hematopoietic Stem Cell Transplantation: A Pediatric Blood and Marrow Transplant Consortium Trial.** *Biology of blood and marrow transplantation : journal of the American Society for Blood and Marrow Transplantation*
Morris, J., Rudebeck, M., Neudorf, S., Moore, T., Duerst, R., Shah, A. J., Graham, M., Aquino, V., Morris, C., Olsson, B.
2016
- **Second Allogeneic Hematopoietic Cell Transplantation for Patients with Fanconi Anemia and Bone Marrow Failure** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Ayas, M., Eapen, M., Le-Rademacher, J., Carreras, J., Abdel-Azim, H., Alter, B. P., Anderlini, P., Battiwalla, M., Bierings, M., Buchbinder, D. K., Bonfim, C., Camitta, B. M., Fasth, et al
2015; 21 (10): 1790-1795
- **Tacrolimus versus Cyclosporine after Hematopoietic Cell Transplantation for Acquired Aplastic Anemia** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Inamoto, Y., Flowers, M. E. D., Wang, T., Urbano-Ispizua, A., Hemmer, M. T., Cutler, C. S., Couriel, D. R., Alousi, A. M., Antin, J. H., Gale, R., Gupta, V., Hamilton, B. K., Kharfan-Dabaja, et al
2015; 21 (10): 1776-1782
- **Unrelated donor Hematopoietic Stem Cell Transplantation for the treatment of non-malignant genetic diseases: An alemtuzumab based regimen is associated with cure of clinical disease; earlier clearance of alemtuzumab may be associated with graft rejection.** *American journal of hematology*
Abdel-Azim, H., Mahadeo, K. M., Zhao, Q., Khazal, S., Kohn, D. B., Crooks, G. M., Shah, A. J., Kapoor, N.
2015
- **A Reduced-Toxicity Regimen Is Associated with Durable Engraftment and Clinical Cure of Nonmalignant Genetic Diseases among Children Undergoing Blood and Marrow Transplantation with an HLA-Matched Related Donor.** *Biology of blood and marrow transplantation*
Mahadeo, K. M., Weinberg, K. I., Abdel-Azim, H., Miklos, D. B., Killen, R., Kohn, D., Crooks, G. M., Shah, A. J., Kharbanda, S., Agarwal, R., Kapoor, N.
2015; 21 (3): 440-444
- **Oral and dental late effects in survivors of childhood cancer: a Children's Oncology Group report.** *Supportive care in cancer*
Effinger, K. E., Migliorati, C. A., Hudson, M. M., McMullen, K. P., Kaste, S. C., Ruble, K., Guilcher, G. M., Shah, A. J., Castellino, S. M.
2014; 22 (7): 2009-2019
- **Oral and dental late effects in survivors of childhood cancer: a Children's Oncology Group report** *SUPPORTIVE CARE IN CANCER*
Effinger, K. E., Migliorati, C. A., Hudson, M. M., McMullen, K. P., Kaste, S. C., Ruble, K., Guilcher, G. M. T., Shah, A. J., Castellino, S. M.
2014; 22 (7): 2009-2019
- **Avascular Necrosis of Bone after Allogeneic Hematopoietic Cell Transplantation in Children and Adolescents** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Li, X., Brazauskas, R., Wang, Z., Al-Seraihy, A., Baker, K. S., Cahn, J., Frangoul, H. A., Gajewski, J. L., Hale, G. A., Hsu, J. W., Kamble, R. T., Lazarus, H. M., Marks, et al
2014; 20 (4): 587-592
- **Avascular Necrosis of Bone after Allogeneic Hematopoietic Cell Transplantation in Children and Adolescents** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Li, X., Brazauskas, R., Wang, Z., Al-Seraihy, A., Baker, K., Cahn, J., Frangoul, H. A., Gajewski, J. L., Hale, G. A., Hsu, J. W., Kamble, R. T., Lazarus, H. M., Marks, et al
2014; 20 (4): 587-592
- **Cytokine and chemokine patterns across 100 days after hematopoietic stem cell transplantation in children.** *Biology of blood and marrow transplantation*

DiCarlo, J., Agarwal-Hashmi, R., Shah, A., Kim, P., Craveiro, L., Killen, R., Rosenberg-Hasson, Y., Maecker, H.
2014; 20 (3): 361-369

- **Durable Engraftment, Correction of Genetic Defects and Prevention of Veno-Occlusive Disease, Following Blood and Marrow Transplantation with an HLA-Matched Sibling DONOR, Using a Reduced Toxicity Conditioning Regimen with Busulfan, Reduced Dose Cyclophos**
Mahadeo, K., Agarwal, R., Weinberg, K. I., Abdel-Azim, H., Miklos, D. B., Shah, A. J., Tappa, L., Kapoor, N.
ELSEVIER SCIENCE INC.2014: S83
- **Gene therapy for adenosine deaminase-deficient severe combined immune deficiency: clinical comparison of retroviral vectors and treatment plans** *BLOOD*
Candotti, F., Shaw, K. L., Muul, L., Carbonaro, D., Sokolic, R., Choi, C., Schurman, S. H., Garabedian, E., Kesserwan, C., Jagadeesh, G. J., Fu, P., Gschweng, E., Cooper, et al
2012; 120 (18): 3635-3646
- **THE CYTOKINE RESPONSE TO HEMATOPOIETIC STEM CELL TRANSPLANTATION** *40th Critical Care Congress*
Kim, P., Agarwal, R., Shah, A., Craver, L., Killen, R., Rosenberg-Hasson, Y., DiCarlo, J.
LIPPINCOTT WILLIAMS & WILKINS.2010: U80–U80
- **Neurocognitive Function of Patients with Severe Combined Immunodeficiency** *IMMUNOLOGY AND ALLERGY CLINICS OF NORTH AMERICA*
Shah, A. J., Kohn, D. B.
2010; 30 (1): 143-?
- **Long-Term Neurocognitive Function of Pediatric Patients with Severe Combined Immune Deficiency (SCID): Pre- and Post-Hematopoietic Stem Cell Transplant (HSCT)** *JOURNAL OF CLINICAL IMMUNOLOGY*
Lin, M., Epport, K., Azen, C., Parkman, R., Kohn, D. B., Shah, A. J.
2009; 29 (2): 231-237
- **Progressive declines in neurocognitive function among survivors of hematopoietic stem cell transplantation for pediatric hematologic malignancies** *JOURNAL OF PEDIATRIC HEMATOLOGY ONCOLOGY*
Shah, A. J., Epport, K., Azen, C., Killen, R., Wilson, K., De Clerck, D., Crooks, G., Kapoor, N., Kohn, D. B., Parkman, R., Weinberg, K. I.
2008; 30 (6): 411-418
- **Progressive declines in neurocognitive function among survivors of hematopoietic stem cell transplantation for pediatric hematologic malignancies** *JOURNAL OF PEDIATRIC HEMATOLOGY ONCOLOGY*
Shah, A. J., Epport, K., Azen, C., Killen, R., Wilson, K., De Clerck, D., Crooks, G., Kapoor, N., Kohn, D. B., Parkman, R., Weinberg, K. I.
2008; 30 (6): 411-418
- **The effects of Campath 1H upon graft-versus-host disease, infection, relapse, and immune reconstitution in recipients of pediatric unrelated transplants** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*
Shah, A. J., Kapoor, N., Crooks, G. M., Weinberg, K. I., Azim, H. A., Killen, R., Kuo, L., Rushing, T., Kohn, D. B., Parkman, R.
2007; 13 (5): 584-593
- **Campath 1H versus ATG for the prophylaxis of graft versus host disease does not increase the risk of relapse or infections.**
Shah, A. J., Kapoor, N., Crooks, G. M., Kohn, D. B., Weinberg, K. I., Killen, R., Kuo, L., Parkman, R.
AMER SOC HEMATOLOGY.2006: 818A