

# Stanford

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## Sandeep Soni

Clinical Associate Professor, Pediatrics - Stem Cell Transplantation

### CLINICAL OFFICES

- **Pediatric Stem Cell Transplantation**

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### Bio

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#### BIO

Dr. Sandeep Soni specializes in stem cell transplant and gene therapy for children with non-malignant disorders e.g , life-threatening genetic disorders, aplastic anemia and hemoglobinopathies, specifically transfusion dependent Thalassemia and severe symptomatic Sickle Cell Disease (SCD). Dr. Soni did his hematopoietic stem cell transplantation (HSCT) training at Hadassah Medical Center in Jerusalem, Israel and at MD Anderson Cancer Center in Houston, TX. He has been treating patients with cellular therapies for over 15 years. The focus of his clinical research is to develop reduced intensity conditioning (RIC) regimens to alleviate the short-term and long-term toxicities associated with HSCT, and to bring innovative gene-therapy treatments to the clinic to treat hemoglobinopathies and other rare genetic disorders. The goal of gene therapy treatments is to correct or edit the defective genes in patients' own stem cells (autologous) and infuse them back to the patient after adequate conditioning, thereby obviating the need to perform HSCT. Dr. Soni has also worked at bluebird bio, Inc. and Crispr Therapeutics, Inc. to develop their gene therapy programs. He is currently the PI of multiple gene therapy studies being conducted at LPCH, He specializes in translational research, clinical trial design and stem cell manipulations.

Cellular Therapy, Gene Therapy, Stem Cell Transplant

Sickle Cell Disease, Thalassemia

Reduced Intensity Conditioning

#### CLINICAL FOCUS

- Hematology/Oncology
- Gene Therapy for rare pediatric disorders
- Stem Cell Transplant and Gene Therapy
- Thalassemia, Sickle Cell Disease, Reduced Intensity Conditioning

#### ACADEMIC APPOINTMENTS

- Clinical Associate Professor, Pediatrics - Stem Cell Transplantation

#### HONORS AND AWARDS

- ASBMT Tandem Meetings Best Abstract Award for Clinical Research, ASBMT/CIBMTR (Feb 2015)

## BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS

- Organizing Committee, ISCT (2018 - present)
- Cell Therapy Liaison to FDA, ASBMT (2017 - present)

## PROFESSIONAL EDUCATION

- Board Certification: Hematology/Oncology, American Board of Pediatrics (2004)
- Fellowship: UT MD Anderson Cancer Center (2003) TX
- Residency: Childrens Hospital at Montefiore Pediatric Residency (2002) NY
- Medical Education: Maulana Azad Medical College (1988) India
- Internship: Maulana Azad Medical College (1987) India

## Research & Scholarship

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### CURRENT RESEARCH AND SCHOLARLY INTERESTS

Translational Medicine, Cellular/Gene Therapy Clinical Trials, Phase I studies

## Publications

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### PUBLICATIONS

- **Chemistry, manufacturing and controls for gene modified hematopoietic stem cells** *CYTOTHERAPY*  
Soni, S., Kohn, D. B.  
2019; 21 (3): 358–66
- **Unrelated Donor Transplantation in Children with Thalassemia using Reduced-Intensity Conditioning: The URTH Trial** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*  
Shenoy, S., Walters, M. C., Ngwube, A., Soni, S., Jacobsohn, D., Chaudhury, S., Grimley, M., Chan, K., Haight, A., Kasow, K. A., Parikh, S., Andreansky, M., Connelly, et al  
2018; 24 (6): 1216–22
- **Gene Therapy in Patients with Transfusion-Dependent beta-Thalassemia** *NEW ENGLAND JOURNAL OF MEDICINE*  
Thompson, A. A., Walters, M. C., Kwiatkowski, J., Rasko, J. J., Ribeil, J., Hongeng, S., Magrin, E., Schiller, G. J., Payen, E., Semeraro, M., Moshous, D., Lefrere, F., Puy, et al  
2018; 378 (16): 1479–93
- **Outcomes of Unrelated Donor Stem Cell Transplantation with Post-Transplant Cyclophosphamide for Graft-versus-Host Disease Prophylaxis in Patients with Severe Sickle Cell Disease** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*  
Rangarajan, H. G., Abu-Arja, R., Pai, V., Guilcher, G. T., Soni, S.  
2018; 24 (2): 413–17
- **Veno-occlusive disease after high-dose busulfan-melphalan in neuroblastoma.** *Bone marrow transplantation*  
Schechter, T., Perez-Albuerna, E., Lin, T. F., Irwin, M. S., Essa, M., Desai, A. V., Frangoul, H., Yanik, G., Dupuis, L. L., Jacobsohn, D., Kletzel, M., Ranalli, M., Soni, et al  
2018
- **Phase I Study of Clofarabine and 2-Gy Total Body Irradiation as a Nonmyeloablative Preparative Regimen for Hematopoietic Stem Cell Transplantation in Pediatric Patients with Hematologic Malignancies: A Therapeutic Advances in Childhood Leukemia Consortium Study.** *Biology of blood and marrow transplantation*  
Soni, S., Abdel-Azim, H., McManus, M., Nemecek, E., Spoto, R., Woolfrey, A., Frangoul, H.  
2017
- **Gene Therapy in a Patient with Sickle Cell Disease.** *The New England journal of medicine*  
Ribeil, J. A., Hacein-Bey-Abina, S., Payen, E., Magnani, A., Semeraro, M., Magrin, E., Caccavelli, L., Neven, B., Bourget, P., El Nemer, W., Bartolucci, P., Weber, L., Puy, et al  
2017; 376 (9): 848–55

- **Combined Umbilical Cord Blood and Bone Marrow from HLA-Identical Sibling Donors for Hematopoietic Stem Cell Transplantation in Children With Hemoglobinopathies** *PEDIATRIC BLOOD & CANCER*  
Soni, S., Boulad, F., Cowan, M. J., Scaradavou, A., Dahake, J., Edwards, S., Walters, M. C.  
2014; 61 (9): 1690-1694
- **Outcomes of Matched Sibling Donor Hematopoietic Stem Cell Transplantation for Severe Sickle Cell Disease with Myeloablative Conditioning and Intermediate-Dose of Rabbit Anti-Thymocyte Globulin** *PEDIATRIC BLOOD & CANCER*  
Soni, S., Gross, T. G., Rangarajan, H., Baker, K. S., Sturm, M., Rhodes, M.  
2014; 61 (9): 1685-1689
- **Successful unrelated umbilical cord blood transplantation for class 3 beta-thalassemia major using a reduced-toxicity regimen** *PEDIATRIC TRANSPLANTATION*  
Soni, S., Breslin, N., Cheerva, A.  
2014; 18 (2): E41-E43
- **Busulfan and melphalan as consolidation therapy with autologous peripheral blood stem cell transplantation following Children's Oncology Group (COG) induction platform for high-risk neuroblastoma: Early results from a single institution** *PEDIATRIC TRANSPLANTATION*  
Soni, S., Pai, V., Gross, T. G., Ranalli, M.  
2014; 18 (2): 217-220
- **Outcome of children who experience disease relapse following allogeneic hematopoietic SCT for hematologic malignancies** *BONE MARROW TRANSPLANTATION*  
Bajwa, R., Schechter, T., Soni, S., Gassas, A., Doyle, J., Sisler, I., Godder, K., Tatman, D., Rumelhart, S., Domm, J., Miao, Y., Frangoul, H.  
2013; 48 (5): 661-665
- **Twice daily i.v. bolus tacrolimus infusion for GVHD prophylaxis in children undergoing stem cell transplantation** *BONE MARROW TRANSPLANTATION*  
Skeens, M., Pai, V., Garee, A., Termuhlen, A. M., Bajwa, R. P., Gross, T. G., Soni, S.  
2012; 47 (11): 1415-1418
- **Advancement of Pediatric Blood and Marrow Transplantation Research in North America: Priorities of the Pediatric Blood and Marrow Transplant Consortium** *BIOLOGY OF BLOOD AND MARROW TRANSPLANTATION*  
Pulsipher, M. A., Horwitz, E. M., Haight, A. E., Kadota, R., Chen, A. R., Frangoul, H., Cooper, L. J., Jacobsohn, D. A., Goyal, R. K., Mitchell, D., Nieder, M. L., Yanik, G., Cowan, et al  
2010; 16 (9): 1212-1221
- **Pre-stem cell transplantation enzyme replacement therapy in Hurler syndrome does not lead to significant antibody formation or delayed recovery of the endogenous enzyme post-transplant: A case report** *PEDIATRIC TRANSPLANTATION*  
Soni, S., Hente, M., Breslin, N., Hersh, J., Whitley, C., Cheerva, A., Bertolone, S.  
2007; 11 (5): 563-567