Supplementary Materials

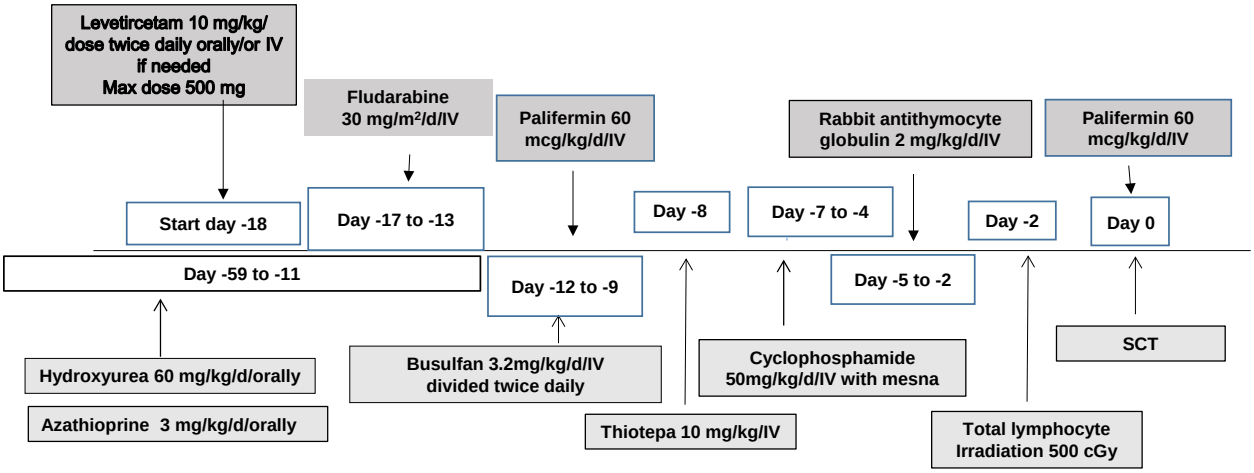
Familial Haploidentical Stem Cell Transplantation in Children and Adolescents with High Risk Sickle Cell Disease

Supplementary Fig. 1. Myeloimmunoablative conditioning regimen.

Supplementary Fig. 2. Participant flow and testing diagram

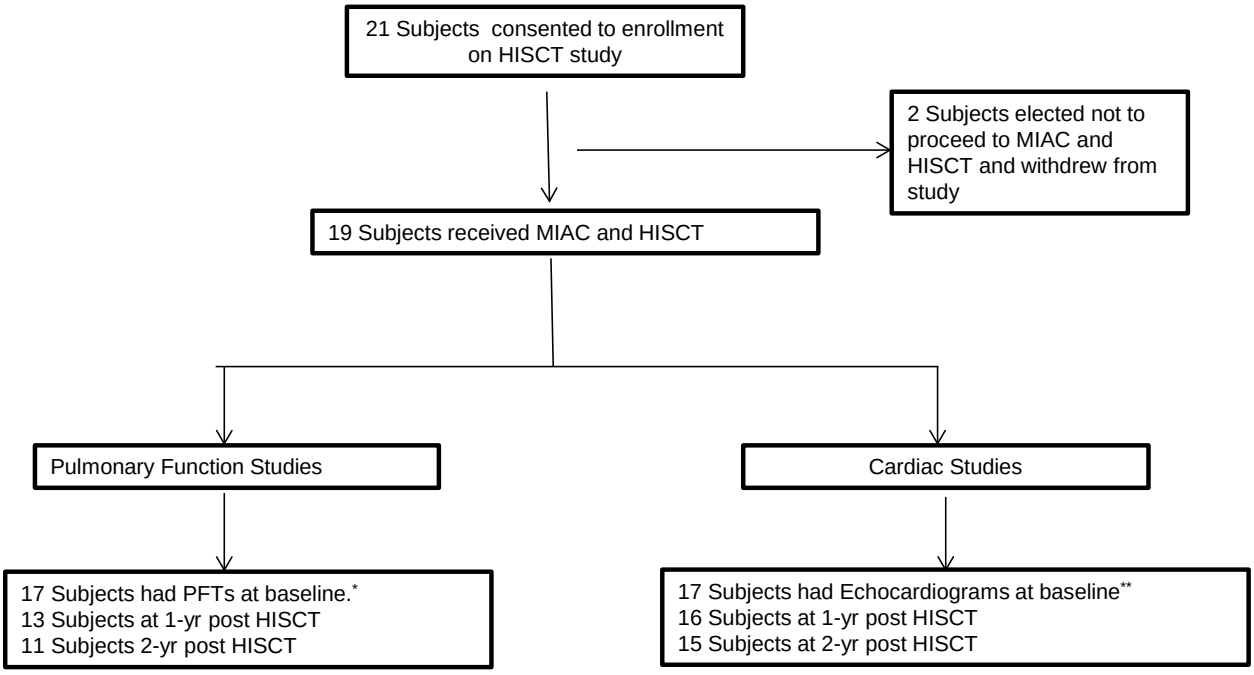
Supplementary Fig. 3. Familial Haploidentical AlloSCT Sickle Cell Disease Consortium (FHASVD) Organizational Chart

Supplementary Fig. 1. Myeloimmunoablative conditioning regimen.



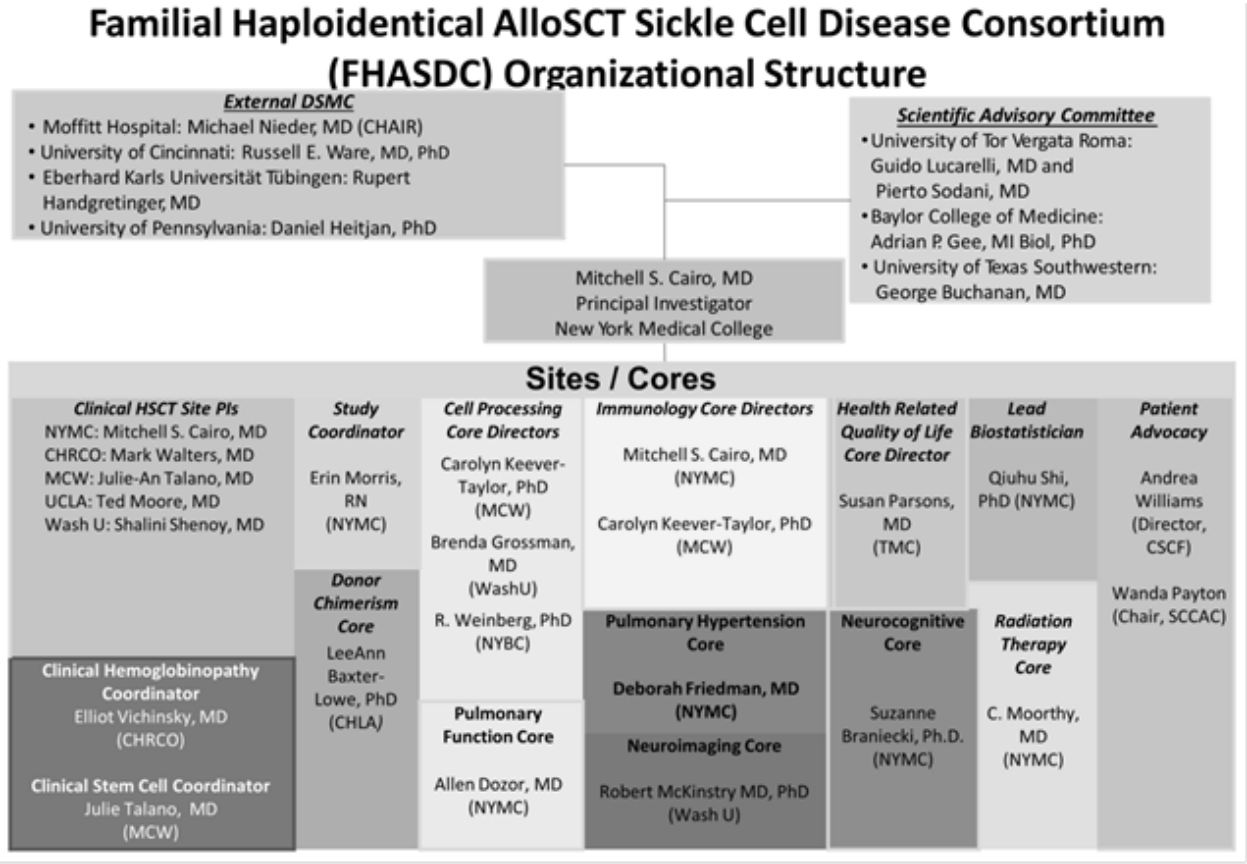
Patients who underwent stem cell transplant received hydroxyurea (60mg/kg/day/oral [maximum dose 2000mg]) and azathioprine 3mg/kg/d/oral/IV starting day -59 to day -11; levetricetam 10 mg/kg/oral/IVdose twice daily starting on day -18; fludarabine 30 mg/m^2/d/IV on days -17, -16, -15, -14, and -13 (< 10 kg 1 mg/kg/day/IV); busulfan 3.2 mg/kg/d/IV twice daily on days -12, -11, -10, and -9 (≤ 4yr 4 mg/kg/day/IV); palifermin 60/mcg/kg/d/IV days -12, -11, -10, -9 and day 0; thiotepa 10/mg/kg/IVon day -8; cyclophosphamide 50mg/kg/d/IV on days -7, -6, -5, and -4; total lymphocyte irradiation on day -2; and rabbit anti-thymocyte globulin 2 mg/kg/d/IV on day -5,-4,-3, and -2 by qualified personnel at each institution.
Abbreviations: mg, milligram; kg, kilogram; d, day; SCT, stem cell transplantation and cGy, centigray.

Supplementary Fig. 2. Participant flow and testing diagram



*Two patients were too young to do pulmonary study
**Two echocardiograms were not available for central review

Supplementary Fig. 3. Familial Haploidentical AlloSCT Sickle Cell Disease Consortium (FHASVD) Organizational Chart



The Familial Haploidentical Allogeneic Stem Cell Transplantation Sickle Cell Disease Consortium (FHASCD) was directed by Mitchell S. Cairo, MD, serving as overall Principal Investigator (PI), at New York Medical College (NYMC). There were five clinical sites, NYM (M.S. Cairo, MD, PI), Children's Hospital and Research Center at Oakland (CHRCO, M. Walters, MD, site PI), Medical College of Wisconsin (MCW, J. Talano, MD, site PI), University of California Los Angeles (UCLA, T. Moore, MD, site PI) and Washington University (WashU, S. Shenoy, MD, site PI). There were two Central Immunology Cores (NYMC and MCW, directed by M.S. Cairo, MD and C. Keever-Taylor, PhD, respectively), one central Donor Chimerism Core (Children's Hospital Los Angeles [CHLA], directed by L.A. Baxter-Lowe, PhD), one central Health-Related Quality of Life Core (HRQL) (Tufts Medical Center [TMC], directed by S. Parsons, MD), one Neuroimaging Core (WashU), directed by R. McKinstry, MD, PhD, one Neurocognitive Core (NYMC), directed by S. Braniecki, PhD, one Pulmonary Function Core (NYMC), directed by A. Dozor, MD, one Pulmonary Vascular Core (NYMC), directed by D. Friedman, D, one Biostatistical Core (NYMC), directed by Q. Shi, PhD, and one Radiation Therapy Core (NYMC) directed by C. Moorthy, MD. Three Central Processing Cores for allogeneic PBSC CD34 selection (T cell depletion) using the CliniMACS device were established at MCW (C. Keever-Taylor, PhD), WashU (B. Grossman, MD) and New York Blood Center (NYBC, R. Weinberg, PhD).

An external Data and Safety Monitoring Committee consisted of Michael Nieder, MD, Vice Chair PBMTC and Medical Director of BMT Program at All Children's Hospital, St. Petersburg, FL (Chair), Russell Ware, MD, PhD, Professor of Pediatrics, University of Cincinnati, Director, Division of Hematology, Co-Director, Cancer and Blood Diseases Institute, Cincinnati Children's Hospital, Cincinnati, OH, Rupert Handgretinger, MD, Professor and Chair of Pediatrics, University Children's Hospital, Eberhard Karls Universität Tübingen, Tübingen, Germany, and Daniel Heitjan, PhD, Professor of Biostatistics and Statistics, Director, Biostatistics, Southern Methodist University, Dallas, TX. An External Scientific Advisory Committee consisted of Guido Lucarelli, MD, Scientific Director, International Center for Transplantation in Thalassemia and Sickle Cell Anemia, Mediterranean Institute of Hematology, University of Tor Vergata Roma, Rome, Italy, Adrian P. Gee, MI Biol, PhD, Director Clinical Applications Lab Center for Cell and Gene Therapy, Baylor College of Medicine, Houston, TX and George R. Buchanan, MD, Children's Cancer Fund Distinguished Chair in Pediatric Oncology and Hematology, Professor of Pediatrics, University of Texas, Southwestern, Dallas, TX. Additionally, the FHASCD consisted of Clinical Hemoglobinopathy Coordinator, Elliot Vichinsky, MD (CHRCO) and J. Talano, MD (MCW) and two patient advocates, Andrea Williams, Director, Children's Sickle Cell Foundation (CSCF) and Wanda Payton, Chair, Sickle Cell Community Health Network of Northern California Community Advisory Council.