median age of 16 (2-80) months at transplantation with a median follow-up of 8.9 (3-22.6) years after last HCT. Post-transplant leukocyte IDUA enzyme levels below the lower reference were seen in 25% of patients due to mixed-chimerism or the use of a carrier donor. Following successful HCT, the clinical course of HS patients is strikingly improved, evident in all organ systems. Residual disease burden is present in the majority of the patients with high variability between patients. A better cognitive status at HCT was a major predictor for superior cognitive development after HCT (figure 1). Significant predictors for superior long-term outcome in all organ systems were the presence of "normal IDUA enzyme levels obtained after HCT" and a "younger age at transplantation". See the association between the leukocyte IDUA enzyme level obtained after HCT and surgical intervention for cord compression and growth, in figure 2 and 3 respectively.

**Conclusion:** Although HCT significantly improved the clinical course in HS patients, residual disease burden was observed in the majority of transplanted HS patients. Using exclusively non-carrier donors and accepting only full donor-chimerism will improve the prognosis of HS patients. Reducing the age at HCT through newborn screening could further improve the outcomes of HS patients after HCT.

### Allogeneic Stem Cell Transplantation for Children with Sickle Cell Disease Achieves Quality of Life Similar to Normal Children and Is Cost Effective

Staci Arnold 1, Zhezhen Jin 1, Alan Weinberg 3, 4, Jacquelyn Bishop 5, Stephen Sands 1, Maureen Licursi 1, Monica Bhatia 6, Andrew Kung 6, Prakash Satwani 6, 1Columbia University, New York, NY; 5Pediatrics, New York Presbyterian Hospital, New York, NY; 2Biostatistics, University Medical Center, New York, NY; 3Columbia University Medical Center, New York, NY; 4Health Evidence and Policy, Mount Sinai Hospital, New York, NY; 5Health Policy and Management, Columbia University Medical Center, New York, NY; 6Pediatrics, New York Presbyterian Hospital, New York, NY; 6Pediatrics, Columbia University, New York, NY

Allogeneic stem cell transplantation (alloSCT) remains the only curative option for sickle cell disease (SCD). However, no systematic analysis exists comparing cost and quality of life (QOL) among this population. We investigated the QOL outcomes and health care utilization associated with alloSCT in children with SCD.

Internal financial data from 2002-2011 was analyzed retrospectively across two groups — post-alloSCT patients (>day+365) and patients with SCD referred for alloSCT and/or HLA typed. Surviving alloSCT recipients (A) and SCD controls (B) were surveyed with age appropriate Pediatric Quality of Life Inventory (PedsQL) and EuroQOL (EQ-5Da) questionnaires. Group A siblings without SCD (C) were also surveyed as unaffected controls. Mean QOL scores were calculated for each group with a max score of 100. Utility scores were determined based on EQ-5D responses. These scores and costs for groups A and B were used to calculate cost per quality adjusted life month (QALM) for the cohort of patients surveyed. Wilcoxon test was used to determine statistical significance.

Group A, B, and C had 16 (mean age - 14yrs), 19 (mean age - 12yrs), and 14 children (mean age - 14yrs), respectively. SCD therapy included hydroxyurea (group A n=8, group B n=10) and chronic transfusions (group A n=7, group B n=2). Mean PedsQL scaled scores were 83, 81, and 88, respectively. Mean EQ-5D visual analogue scale scores were 92, 87, 96, respectively. Mean utility scores were 0.87, 0.91, and 0.89, respectively. All QOL scores were not statistically significant (p = 0.2638). Healthcare utilization among groups A and B was previously reported (see details in table below). The median inpatient cost per QALM for group A was $0 and $514 for group B (p = 0.0023). Outpatient cost per QALM for group A was $353 and $236 for group B (p = 0.3506).

SCD patients’ post-alloSCT QOL scores are similar to unaffected siblings, indicating that QOL has normalized. Controls with SCD also had scores similar to unaffected controls. However, a statistically significant difference exists in the inpatient cost per QALM post-alloSCT compared to controls with SCD. Outpatient was not significant which may reflect the limitations of the study period as post-alloSCT QOL and cost can change over time (Felder-Puig 2006; Majhail 2010). Ultimately, this study provides the first combined analysis of QOL as an outcome and the economic impact of alloSCT for pediatric SCD patients. Further analysis is ongoing to affirm that alloSCT is a beneficial and cost effective management option for patients.

### Low Day 100 Transplant-Related Mortality and Relapse Rate Following Clofarabine in Combination with Cytarabine, Total Body Irradiation and Allogeneic Stem Cell Transplantation in Children, Adolescents and Young Adults with Poor-Risk Acute Leukemia

Nan Chen 1, Kavita Radhakrishnan 2, Jennifer Krajewski 1, Angela Ricci 1, Mark Geyer 5, Lauren Harrison 1, M Fevzi Ozkaynak 1, Alexandra Cheerva 6, Julie-An Talano 7, Theodore B. Moore 8, Alfred P. Gillio 9, Mark Walters 10, Lee Ann Baxter-Lowe 11, Mitchell S. Cairo 1, 2, 3, 4, 5, 6, 7, 8, 9, 10, 11. 1Pediatrics, New York Medical College, Valhalla, NY; 2Internal Medicine, University of San Francisco, San Francisco, CA; 3Pediatrics, Hackensack University Medical Center, Hackensack, NJ; 4Pediatrics, Boston Children’s Hospital, Boston, MA; 5Medicine, Massachusetts General Hospital, Harvard Medical School, Boston, MA; 6Pediatric Hem/Onc, University of Louisville, Louisville, KY; 7Pediatric Hematology/Oncology and BMT, Children’s Hospital of Wisconsin, Milwaukee, WI; 8Pediatrics, David Geffen School of Medicine at UCLA, Los Angeles, CA; 9Ped. Heme Onc, HUMC, Hackensack, NJ; 10Hematology/Oncology, Children’s Hospital & Research Center, Oakland, Oakland, CA; 11Immunogenetics and Transplantation Lab, UCSF, San Francisco, CA; 12Microbiology and Immunology, New York Medical College, Valhalla, NY; 13Pathology, New York Medical College, Valhalla, NY; 14Cell Biology and Anatomy, New York Medical College, Valhalla, NY; 15Medicine, New York Medical College, Valhalla, NY

**Background:** Children, adolescents, and young adults (CAYA) with Acute Leukemia in third complete remission...