Table 1. HSCT outcomes in children with hypodiploid ALL at 5 years

| | DFS % (range) | OS % (range) | Relapse% (range) | TRM% (range) |
|---|---|---|---|---|
| | <——————————5 year estimates——————————> | | | |
| All patients | 51 (40-62) | 56 (44-67) | 27 (18-38) | 22 (14-32) |
| Patients in CR1 | 55 (40-70) | 58 (43-72) | 26 (14-40) | 20 (9-33) |
| CR1 patients with ≤43 chromosomes | 47 (29-66) | 50 (32-69) | 30 (15-47) | 23 (9-41) |

participating centers. Kaplan-Meier estimates were used to calculate overall survival (OS) and cumulative incidence methods for secondary outcomes, including neutrophil and platelet engraftment, incidence of graft-versus-host disease (GvHD) and disease-specific characteristics at most recent follow-up. The two-sided log-rank test was used for univariate comparisons.

Seventy patients (31 ALD, 5 Krabbe, 34 MLD) were available for analysis, with a median age at transplant of 6.5 years. Median follow-up for survivors was 46 months. OS at 4 years was 60% (±6%). Cumulative incidences of neutrophil and platelet engraftment were 88% (±4%) at day 60, and 73% (±6%) at day 180, resp. Acute-GvHD (grade II-IV) occurred in 16 patients (22% ±5%) at day 100 and chronic-GvHD in 9 patients (13% ±5%). Out of the 24 patients who died, 18 (67%) died of transplant-related causes and 6 (33%) died of disease progression. Higher OS was seen in patients who had no or 1 HLA mismatch (OS 81%), compared to patients who received a CBT with 2 HLA mismatches (OS 47%, p 0.02).

On 37 patients (53%), information on disease-specific characteristics was available. OS among these patients was 68% (±8), which was not significantly different from patients without disease-specific information (p=0,307). Nineteen patients (51%) were asymptomatic at transplant, 14 (38%) had mild disease and 4 (11%) were severely affected. Overall survival was worse in patients with severe disease at transplant; none of these 4 patients survived, versus 79% of mildly affected and 76% of asymptomatic patients. At most recent follow-up, disease status was stable in 15 (57.7%), had improved in 2 (7.7%) and worsened in 9 (34.6%) surviving patients. The majority of patients showed abnormalities on MRI-scanning pre-HSCT (n=25, 68%). In patients with normal MRI-scans pre-transplant OS was 69%; in patients with abnormal MRI-scans 75%.

In conclusion, we found an encouraging OS of 60% at 4 years, with relatively low rates of GvHD. Use of a mismatched donor (>1 HLA) negatively impacts survival. Data on clinical characteristics suggest that OS is strongly influenced by disease status at transplant.

## 97

### Transplant Outcomes for Children with Hypodiploid Acute Lymphoblastic Leukemia: The Cibmtr Experience

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Children with hypodiploid ALL have inferior outcomes with chemotherapy despite intensive risk adapted treatment. A case series of pediatric hypodiploid ALL (n=139) with <45 chromosomes treated by 10 different national ALL study groups (Nachman et al; 2007), reported an 8-year event free survival (EFS) of 38.5% and overall survival (OS) of 49.8%. Patients with fewer than 44 chromosomes fared significantly worse than those with 44 chromosomes (EFS of 30% vs. 52%, p=0.01). A similar report from the Medical Research Council (MRC) included 226 children and adults treated with chemotherapy (Harrison et al, 2004). Patients with 42-45 chromosomes (n=153) had a 3 year survival of 66% compared to 29% in those with 25-39 chromosomes.

We conducted a retrospective study of 78 children with hypodiploid ALL who underwent HSCT between 1990 and 2010 and reported to CIBMTR, to determine whether outcomes might be improved with transplant.

Median age at HSCT was 10 years (range 3-18). Thirty nine (50%) patients had ≤ 43 chromosomes, 12 (15%) had 44 chromosomes and 27 (35%) had 45 chromosomes. Forty three (55%) patients were transplanted in CR1 while 35 (45%) were transplanted in ≥CR2. Twenty nine patients (37%) received a graft from a related donor, 34 (44%) an unrelated donor and 15 (19%) cord blood. Demographics and transplant characteristics were similar in those with chromosomes ≤43 or ≥44 and those transplanted with early or late disease (CR1 or CR2 and beyond). All patients received a myeloablative conditioning regimen.

Multivariate analysis confirmed both disease status and number of chromosomes were independently associated with mortality; mortality risks were higher for transplants in CR2 (HR 2.16, p=0.05) and when chromosomes were <=43 (HR 2.15, p=0.05).The 5-year estimates of DFS, OS, relapse, and treatment related mortality (TRM) for the entire group, those in CR1 and for those in CR1 with ≤43 chromosomes are shown in Table 1. In the group with ≥44 chromosomes (n=13), 3 patients died and 10 were alive at last contact (9 disease free and 1 with relapsed disease). Despite the obvious limitation of small numbers of patients and the retrospective nature of our study, our results suggest that compared to historical results from chemotherapy only treatment, pediatric patients with hypodiploid ALL, may have improved outcomes when transplanted in CR1, and benefit may be most notable in those with ≤43 chromosomes.

## 98

### Interplay of Recipient-Donor Matching for HLA, Race/Ethnicity and Gender on Long Term Outcomes in 365 Pediatric Recipients of Single 4/6 Matched Unrelated Cord Blood Transplantation (UCBT) after Myeloablative Therapy

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