Data on adults included 644 (598 allo + 46 auto) evaluable patients out of total 835 patients reported. In pediatric cohort 165 patients suffered from malignancies, 356 patients were transplanted for non-malignant diseases; 437 underwent a first SCT, 87 had a subsequent transplant. In adult group 626 were treated for hematological malignancies and 18 for non-malignant diseases (SAA or thalassemia). No data on auto-SCT in adults were published after 2004. The majority of pediatric patients received treosulfan in dose 39-45 mg/m2 (332 patients, 62%). Most of adult patients treated after 2007 received dose of 42 mg/m2.

Results: The main indications for treosulfan use in pediatric population were non-malignant diseases (68%) or second SCT, while among adults older age (>50 years) and/or comorbidities disqualifying from myeloablative conditioning. No correlation between the given treosulfan dose and the grade III/IV toxicity was observed both in children and in adults. No association between dose and GVHD, OS, DFS, relapse incidence and TRM was found both in children and in adults.

Conclusions: Treosulfan-based conditioning with its low toxicity profile and dose-dependent myelotoxicity is a good option in children treated with non-malignant diseases. Additionally, both children and adults not eligible for conventional transplant regimen can be offered this treatment with acceptable results. Toxicity and survival were similar in children and adults, while acute and chronic GVHD incidence were higher in adult population.

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Rarity of Donor-Derived Malignancy after Allogeneic BMT with High-Dose Post-Transplantation Cyclophosphamide

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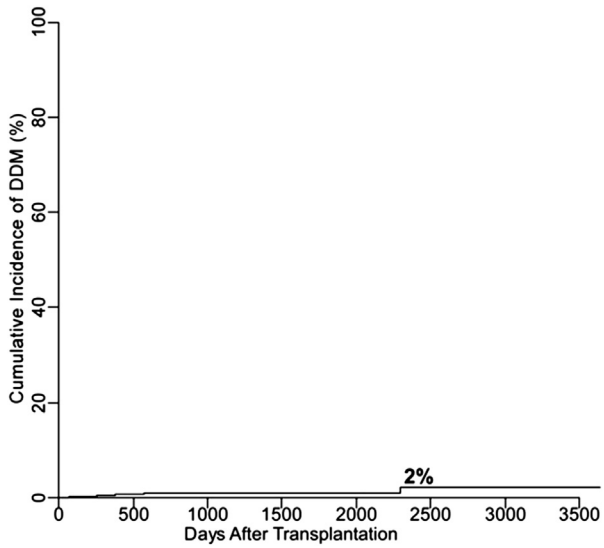
Donor-derived malignancy (DDM) is a rare but often fatal complication of alloBMT, with a reported incidence of 0.1-5%. AlloBMT utilizing high-dose post-transplantation cyclophosphamide (PT/Cy) as GVHD prophylaxis produces excellent rates of engraftment and low rates of acute and chronic GVHD. Because exposing the allograft to cytotoxic chemotherapy may theoretically increase the risk of DDM, we evaluated the incidence of DDM after alloBMT with PT/Cy. From 2000-2012, 790 patients (median age 51y, range 1-74y) received T-cell replete alloBMT with high-dose PT/Cy at Johns Hopkins, including 313 (40%) who received PT/Cy as sole GVHD prophylaxis. Of these transplants, 349 (44%) were HLA-haploidentical and 346 (44%) were myeloablative. Median donor age was 41y (range 13-79y). With a median follow-up of 3y (range, 0.8-9.4y) in patients without events, the 3 year PFS and OS probabilities were 42% and 56% respectively. Five cases (5/790=0.6%) of DDM were identified



as well as one case of clonal, donor-derived LGL leukemia that resolved without any therapy. By competing-risk analysis, the probability of DDM was 0.6% at 1 y, 0.8% at 5 y, and 2% overall (Figure). In the 5 identified cases of DDM, the median patient age was 41y (range 18-65 y) at BMT and median donor age was 41y (31-67y). These patients were initially transplanted for ALL (1), NHL (3), or Hodgkin lymphoma (1). Two patients received myeloablative conditioning and 3 received additional GVHD prophylaxis with mycophenolate mofetil and tacrolimus. The median time from BMT to the diagnosis of DDM was 1.3y (range 0.5-6.3y). DDMs consisted of MDS (1), AML (3), and CMML (1). All of the patients received treatment for their DDM; 2 are long term survivors and 3 died of their DDM. The incidence of developing a DDM after high-dose PT/Cy is rare, and is within the range reported for other transplant platforms.

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Ex −Vivo T Cell Depleted Allogeneic (TCD) Hematopoietic Stem Cell Transplantation for Advanced Chronic Myelofibrosis: MSKCC Experience

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Introduction: Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is the only potentially curative treatment option for MF. The role of ex −vivo TCD allo-HSCT hasn't been reported in patients with advanced MF.

Patients: Between 5/1990-4/2013, 12 pts underwent TCD transplant at MSKCC for MF; 9 had primary MF, 2 post ET and 1 post MDS. Median age was 56 (42.7-65.5). Disease status prior to transplant per DIPSS was: intermediate-1 (4), intermediate-2 (6), and high-risk (2). Splenectomy prior to transplant was performed in 8 patients. JAK2 V617F mutation status was known on five patients and was detected on 3. Five pts received a TCD marrow graft and were conditioned with a TBI-based regimen and 7 pts received TCD peripheral blood graft and were conditioned with a chemotherapy