



MINUTES

CIBMTR WORKING COMMITTEE FOR PEDIATRIC CANCER WORKING COMMITTEE

Honolulu, HI

Saturday, February 15, 2025, 1:00 – 3:00 PM

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1. Introduction

- a. Minutes from February 2024 (Attachment 1)

2. Accrual summary (Attachment 2)

3. Presentations, Publications or Submitted papers

- a. **PC19-03** Impact of Extramedullary Disease on the Outcomes after Allogeneic Hematopoietic Transplantation in Children and Young Adults with Acute Myeloid Leukemia – a CIBMTR Analysis. (K Rao/ H Rangarajan/ P Satwani/ D Chellapandian/ B Savani/ J Silva). **Poster Presentation, ASH 2024.**
- b. **AC17-01** CD-19 chimeric antigen receptor T-cells with or without hematopoietic cell transplantation for treatment of refractory acute lymphocytic leukemia (M Perales/ J Park/ S Nikiforow). **Submitted.**

- c. **PC22-01** Impact of graft versus host disease following allogeneic hematopoietic cell transplantation on leukemia free survival in hematologic malignancies within the pediatric disease risk index risk stratification (A Bauchat/ M Qayed). **Oral Presentation, Tandem 2025**

4. Studies in progress (Attachment 3)

- a. **PC19-02** Does mixed peripheral blood T Cell Chimerism predict relapse? (S Prockop/ J Boelens/ K Peggs). **Protocol Development**
- b. **PC19-03** The impact of pre-transplant extramedullary disease on the outcome of Allogeneic Hematopoietic Cell Transplantation for Acute Myeloid Leukemia in children (H Rangarajan/ P Satwani /D Chellapandian). **Manuscript Preparation.**
- c. **CT20-02** Resource utilization with chimeric antigen receptor T cells (M Battiwalla/ H Rangarajan/ C Scheckel). **Protocol Development.**
- d. **PC20-02** Germline genetics of pediatric Myelodysplastic Syndromes (J Poynter/ L Spector). **Data File Preparation.**
- e. **PC22-01** Impact of graft versus host disease following allogeneic hematopoietic cell transplantation on leukemia free survival in hematologic malignancies within the pediatric disease risk index risk stratification (A Bauchat/ M Qayed). **Manuscript Preparation**
- f. **PC22-02** Evaluating predictors of access and outcomes with hematopoietic cell transplantation in pediatric and adolescent patients with relapsed/refractory classical Hodgkin lymphoma after treatment on an initial cooperative group clinical trial (S Castellino/ J Kahn). **Protocol Development.**
- g. **PC23-01** Post-transplant cyclophosphamide vs. TCR $\alpha\beta$ /CD19+ deplete approaches for haploidentical transplant in pediatric patients with acute leukemias and myelodysplastic syndrome: A CIBMTR/EBMT collaborative study (A Li/ H Rangarajan/ P Satwani). **Data File Preparation.**
- h. **PC23-02** Comparison of bone marrow and peripheral blood stem cells as graft source in children undergoing allogeneic hematopoietic stem cell transplantation for hematological malignancies with unmanipulated haploidentical grafts utilizing post-transplant cyclophosphamide as GvHD prophylaxis (A Srinivasan/ J Krueger). **Protocol Development**
- i. **PC24-01** Transplantation and cellular therapy for children and young adults with down's syndrome and acute leukemia (L Appell/ S Rotz). **Protocol development.**

5. Future/proposed studies

- a. **PROP 2410-40** Comparison of different TBI doses in relation to MRD status in pediatric acute lymphoblastic leukemia (T Takahashi/ A Keating) (Attachment 4)
- *Presented by Dr. Takato Takahashi.*
 - **Hypothesis:** *TBI at 12Gy has comparable disease-free survival to higher TBI doses.*
 - **Objectives:** *Compare disease-free survival, overall survival, TRM, relapse, GVHD, and late effects between high dose TBI (>12Gy) to standard myeloablative TBI (12Gy).*
- b. **PROP 2410-85** Is There an Optimal CD34+ Cell Dose In Pediatric Allogeneic Hematopoietic Cell Transplantation Performed for Malignant Diseases? (E Fraint/ T Knight) (Attachment 5)
- *Presented by Dr. Ellen Fraint.*
 - **Hypothesis:** *Higher CD34+ and TNC doses improve survival, reduce relapse rate, and accelerate engraftment.*
 - **Objectives:** *Determine optimal CD34+ and TNC doses for pediatric patients undergoing allotransplant.*

- c. **PROP 2410-94** Effect of disease burden and pre-transplant therapy in pediatric patients with myelodysplastic syndrome in the current era (J Rossoff/ S Chaudhury) (Attachment 6)
- *Presented by Dr. Sonali Chaudhury.*
 - **Hypothesis:** *Lower blast percentage pre-transplant improves disease-free survival for patients receiving HCT for MDS*
 - **Objectives:** *Determine the effect of pre-transplant bone marrow blast percentage on outcomes.*
- d. **PROP 2410-176** Comparison of Risk Factors Associated with Early and Late Disease Relapse Among Patients in Complete Remission at One Month after Tisagenlecleucel (Kymriah) therapy in Pediatric, Adolescent and Young Adult (AYA) Patients Treated for Relapsed or Refractory (r/r) B Cell Acute Lymphoblastic Leukemia (B Cell ALL) (L Davis/ P Satwani) (Attachment 7)
- *Presented by Dr. Laurie Davis.*
 - **Hypothesis:** *Patients with high disease burden at D28 will experience higher rates of early and late relapse after Kymriah infusion.*
 - **Objectives:** *Identify risk factors influencing relapse in pediatric and AYA patients treated for relapsed/refractory B-cell ALL who achieved CR by D28 post-CART.*
- e. **PROP 2410-182** Impact of Planned Post-Transplant Granulocyte Colony Stimulating Factor (G-CSF) on Transplant-Related Outcomes in Pediatric Patients with Malignant Disease Undergoing Haploidentical Hematopoietic Cell Transplant (HCT) with Post-Transplant Cyclophosphamide (ptCy) for Graft vs. Host Disease (GVHD) Prophylaxis (L Davis/ P Satwani) (Attachment 8)
- *Presented by Dr. Laurie Davis.*
 - **Hypothesis:** *Planned GCSF administration will result in lower overall survival and disease-free survival, with greater relapse and non-relapse mortality in patients receiving haploidentical HCT with PTCy.*
 - **Objectives:** *Analyze hematopoietic recovery, overall survival, GVHD, and major transplant complications, comparing those who received GCSF to those who did not.*
- f. **PROP 2410-200** Hematopoietic Stem Cell Transplant Outcomes for Infant B-cell Acute Lymphoblastic Leukemia (N Lalefar/ H Rangarajan) (Attachment 9)
- *Presented by Dr. Nahal Rose Lalefar.*
 - **Hypothesis:** *Improved outcomes for infant ALL following HCT in the contemporary era.*
 - **Objectives:** *Compare leukemia-free survival (LFS) and overall survival (OS) at one year and three years post-transplant, following trends across time periods 2003-2022 and , analyze treatment-related mortality, and explore late effects.*
- g. **PROP 2410-204** Transplantation Outcomes for Children with Hypodiploid Acute Lymphoblastic Leukemia in the Modern Era (A Bidgoli/ U Kapoor) (Attachment 10)
- *Presented by Dr. Urvi Kapoor.*
 - **Hypothesis:** *Transplantation for pediatric hypodiploid ALL, when performed in the setting of disease control, offers outcomes comparable to other ALL transplants.*
 - **Objectives:** *Evaluate leukemia-free survival, relapse, non-relapse mortality, and overall survival.*

Proposed studies; not accepted for consideration at this time

- h. **PROP 2408-14** Comparing the Progression-free Survival and Overall Survival of Autologous Stem Cell Transplantation and Allogeneic Stem Cell Transplantation in Refractory Langerhans Cell Histiocytosis (M Pamukcuoglu). ***Dropped due to small sample size.***
- i. **PROP 2410-73** The impact of prior allogeneic HSCT on outcomes following subsequent CD19.CAR-T cell infusion for pediatric patients with relapsed/refractory B-cell ALL (S Naik/ M Pulsipher). ***Dropped due to overlap with current study/publication.***
- j. **PROP 2410-136** Comparison of alternative donor options in pediatric AML with varying residual disease status (T Takahashi/ A Keating). ***Dropped due to overlap with published study***
- k. **PROP 2410-207** The Impact of Hematopoietic Cell Transplantation in Complete Remission with Incomplete Count Recovery in Pediatric AML (E Krieger/ K Magee). ***Dropped due to supplemental data needed.***
- l. **PROP 2410-211** Impact of KYMRIAHA potency on incidence of relapse and cytokine release syndrome (U Kapoor/ P Satwani). ***Dropped due to supplemental data needed.***

6. Other business