



## MINUTES

### CIBMTR WORKING COMMITTEE FOR NON-MALIGNANT DISEASES WORKING COMMITTEE

Honolulu, HI

Friday, February 14, 2025, 1:00 – 3:00 PM HST

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## 1. Introduction

- a. Minutes from February 2024 meeting (Attachment 1)

## 2. Accrual summary (Attachment 2)

## 3. Presentations, Published or Submitted papers

**NM20-01** Hematopoietic stem cell transplantation for Fanconi anemia (S Rotz/H Eissa). **Presented at ASH 2024.**

## 4. Studies in progress (Attachment 3)

- a. **NM15-01** Outcome of allogeneic Hematopoietic Cell Transplant (HCT) in Erythropoietic Porphyria (A Saad/H Abdel-Azim/J Bloomer). **Manuscript Preparation.**
- b. **NM17-01** Late effects after hematopoietic stem cell transplantation in patients with HLH (A Horne/KS Baker/K Beutel). **Protocol Development.**
- c. **NM18-01** Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant disease (A Prakash/D Wall/K Paulson). **Protocol Development.**
- d. **AC18-02** Prospective Cohort study of Recipients of Autologous Hematopoietic cell Transplant for Systemic Sclerosis (G Georges/K Sullivan). **Manuscript Preparation.**

- e. **NM20-01** Hematopoietic stem cell transplantation for Fanconi anemia (S Rotz/H Eissa). **Manuscript Preparation**
- f. **NM22-01** Outcomes after second or greater allogeneic stem cell transplants in patients with severe aplastic anemia: A contemporary analysis (H Rangarajan/P Satwani). **Protocol Development.**
- g. **NM23-01** Impact of conditioning intensity and donor type on outcomes in patients with severe aplastic anemia undergoing upfront or salvage hematopoietic stem cell transplant (A Rayes/ S Otoukesh/ R Nakamura/ M Pulsipher). **Protocol Development.**
- h. **NM24-01** The outcomes of PTCY based GVHD prophylaxis for allogeneic stem cell transplantation in patients with severe aplastic anemia patients who lack a HLA-matched sibling donor (N Khaire/ L Gowda/ A Mirza/ R Kumar/ B Ball). **Protocol Development.**
- i. **NM24-02** Impact of somatic mutations in aplastic anemia after allogeneic stem cell transplantation (B Ball/ R Nakamura). **Protocol Development.**

## 5. Proposed studies

- a. **PROP 2408-01; 2409-26; 2410-217** Outcomes after Allogeneic Hematopoietic Stem Cell Transplant in Diamond-Blackfan Anemia: A contemporary CIBMTR analysis (J Koo/ N Gloude/ N Hossain/ P Munshi/ H Rangarajan/ N C Shah) (Attachment 4)
  - *Presented by Dr. Nicholas Gloude*
  - **Hypothesis:** *Reduced-intensity conditioning (RIC) regimens provide comparable overall survival to myeloablative conditioning (MAC) regimens for patients with Diamond Blackfan Anemia*
  - **Objectives:** *Estimate two-year overall survival, evaluate the impact of donor type, and determine the incidence of HSCT-related complications.*
- b. **PROP 2409-17; 2410-218; 2410-251** Outcomes of Autologous Hematopoietic Stem Cell Transplantation in Multiple Sclerosis (E Ayala/ M Iqbal/ S Mirza/ T Nishihori/ S Hosahalli Vasanna/ J Dalal) (Attachment 5)
  - *Presented by Dr. Smitha Vasanna.*
  - **Hypothesis:** *The intensity of conditioning regimen (myeloablative vs. non-myeloablative) has no impact on post auto transplant outcomes for MS*
  - **Objectives:** *Compare progression-free survival, assess health-related quality of life, and evaluate secondary outcomes such as overall survival and second malignancies.*
- c. **PROP 2410-11** Impact of mixed chimerism post stem cell transplantation on the long term outcome of patients with Fanconi anemia (M Ayas) (Attachment 6)
  - *Presented by Dr. Mouhab Ayas.*
  - **Hypothesis:** *Mixed chimerism post-transplant for Fanconi Anemia has no harmful effect on outcomes compared to full donor chimerism.*
  - **Objectives:** *Determine the impact of mixed chimerism on GVHD, graft failure, incidence of MDS/AML, and solid malignancies.*

- d. **PROP 2410-246** The Impact of Donor-derived Clonal Hematopoietic Mutations in children and patients less than 18 years of age following allogeneic Hematopoietic Stem Cell Transplantation for non-hematological and non-malignant hematological Conditions (M Kulasekaran/ G Hildebrandt) (Attachment 7)
- *Presented by Dr. Monika Kulasekaran.*
  - **Hypothesis:** *Comprehensive molecular profiling and risk assessment of donors are necessary.*
  - **Objectives:** *Evaluate the prevalence of donor clonal hematopoiesis mutations and correlate their impact on long-term outcomes, include secondary malignancies.*

***Proposed studies; not accepted for consideration at this time***

- e. **PROP 2410-27** Effect of age, donor source and preparative regimen on outcome of hematopoietic cell transplantation in patients with Glanzmann Thrombasthenia (D Citla-Sridhar/ J Dalal). ***Dropped due to small sample size.***
- f. **PROP 2410-56** Effect of mixed host-donor chimerism on graft failure/rejection after hematopoietic cell transplantation for non-malignant hematological disorders (A Lipsitt/ A Sharma). ***Dropped due to heterogeneous population and overlap with current study.***
- g. **PROP 2410-160** Post-transplant cyclophosphamide vs. TCR  $\alpha\beta$ /CD19 deplete Haploidentical Transplant in Inborn Errors of Immunity: A CIBMTR Analysis (H Rangarajan/ M Albert). ***Dropped due to supplemental data needed.***
- h. **PROP 2410-169** Evaluating hematopoietic cell transplant outcomes in patients with HbSC sickle cell disease: A CIBMTR Study (S Hosahalli Vasanna/ J Dalal). ***Dropped due to small sample size.***
- i. **PROP 2410-192** Evaluating Outcomes of Autologous Hematopoietic Cell Transplantation in patients with Severe Systemic Sclerosis (Scleroderma) in the contemporary era (M Iqbal/ E Ayala). ***Dropped due to overlap with current study.***
- j. **PROP 2410-205** Real-world gene therapy experience for sickle cell disease and comparing it with allogeneic stem cell transplant for toxicities (K Chetlapalli/ L Gowda). ***Dropped due to small sample size.***
- k. **PROP 2410-212** Allogeneic transplant outcomes in Vexas Syndrome (S R Cingam/ J M Lewis-Gonzalez). ***Dropped due to small sample size.***
- l. **PROP 2410-233** Evaluating outcomes of Hematopoietic Cell Transplantation in VEXAS syndrome (H Murthy/ Y Moreno Vanegas). ***Dropped due to small sample size.***
- m. **PROP 2410-264** Transplant-Related Outcomes in Patients Undergoing Allogeneic Stem Cell Transplant vs. Gene Therapy for Sickle Cell Disease and Thalassemia (M Gallogly/ L Metheny). ***Dropped due to small sample size.***

**6. Other business**