



## MINUTES

### CIBMTR WORKING COMMITTEE FOR CHRONIC LEUKEMIA

Honolulu, HI

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

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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. **CK21-01** Jain T, Estrada-Merly N, Queralt Salas M, Kim S, DeVos J, Chen M, Fang X, Kumar R, Andrade Campos M, Elmariah H, Agrawal V, Aljurf M, Ulrike Bacher V, Badar T, Badawy S, Ballen K, Beitinjaneh A, Bhatt V, Bredeson C, DeFilipp Z, Dholaria B, Farhadfar N, Farhan S, Gandhi A, Ganguly S, Gergis U, Grunwald M, Hamad N, Hamilton B, Inamoto Y, Iqbal M, Jamy O, Juckett M, Kharfan-Dabaja MA, Krem M, Lad D, Liesveld J, Al Malki M, Malone AK, Murthy H, Ortí G, Patel S, Pawarode A, Perales M, van der Poel M, Ringden O, Rizzieri D, Roivo A, Savani B, Savoie M, Seo S, Solh M, Ustun C, Verdonck L, Wingard J, Wirk B, Bejanyan N, Jones R, Nishihori T, Oran B, Nakamura R, Scott B, Saber W, Gupta V. Donor types and outcomes of transplantation in myelofibrosis: A CIBMTR study. *Blood Advances*. 2024 Aug 27; 8(16):4281-4293. doi:10.1182/bloodadvances.2024013451. Epub 2024 Jun 25. PMC11372592.
- b. **GS19-02** Graft failure in MDS and acute leukemia patients after allogeneic stem cell transplantation receiving post transplant cyclophosphamide (M Krem/ R Maziarz). *Submitted*.

#### 4. Studies in progress (Attachment 3)

- a. **CK16-01b** Identification of germline predisposition mutations in young myelodysplastic syndrome patients (L Godley). **Analysis.**
- b. **CK22-01** Impact of somatic mutations on outcomes after allogeneic hematopoietic cell transplantation in patients with myelodysplastic syndrome with ring sideroblasts (MDS-RS) and MDS/myeloproliferative neoplasm with RS and thrombocytosis (MDS/MPN-RS-T) (S Arslan/ R Nakamura). **Protocol Development.**
- c. **CK22-02** Toxicity and survival of AML/MDS patients receiving allogeneic stem cell transplantation using reduced-intensity conditioning: A propensity score analysis. (P Kongtim/ A Portuguese/ S Ciurea/ B Scott). **Data File Preparation.**
- d. **CK23-01** Identifying the Optimal Graft-versus-Host Disease Regimen in Allogeneic Transplantation for Myelofibrosis (S Patel/ D Courier). **Protocol Received.**
- e. **CK23-02** The mutational landscape in Myelodysplastic Syndrome arising from Aplastic Anemia and its impact on Allogeneic Stem Cell Transplantation Outcomes (B Ball/ R Nakamura). **Protocol Received.**
- f. **CK24-01** Identifying the optimal stem cell dosing for peripheral blood stem cell transplantation with post-transplant cyclophosphamide. (H Elmariah/ A Gandhi/ N Bejanyan/ R Marziarz). **Protocol Received.**
- g. **CK24-02** Outcomes of allogeneic hematopoietic stem cell transplantation in patients with DDX41-mutated myelodysplastic syndrome and acute myeloid leukemia. (R Stubbins/ E Wong/ L Fox/ L Gowda/ S Seropian). **Protocol Received.**
- h. **CK24-03** Comparison of reduced intensity conditioning regimens for haploidentical donor hematopoietic cell transplant with post-transplant cyclophosphamide in patients with acute myeloid leukemia or myelodysplastic syndromes. (H Elmariah/ S Arslan/ M Al Malki/ N Bejanyan). **Protocol Development.**
- i. **CK24-04** Comparison of post-transplant cyclophosphamide-based reduced intensity conditioning regimens for older patients with acute myelogenous leukemia and MDS. (L Bachier/ S Solomon). **Protocol Development.**

## 5. Future/proposed studies

- a. **PROP 2410-22; 2410-178** Impact of Post-Transplant Cyclophosphamide on Outcomes in Patients with CMML Undergoing Allogeneic Stem Cell Transplant (Y Berry/ S Farhan/ I Varadarajan/ K Ballen) (Attachment 4)

*Dr. Farhan's Presentation:*

- ***Discussed the role of allogeneic stem cell transplant in CMML.***
- *Highlighted survival outcomes and the impact of GVHD prophylaxis with Ptcy.*
- *Research questions focused on the impact of Ptcy-based GVHD prophylaxis and advances in pre- and post-transplant care.*
- *Cohort includes 940 who got CNI-based GVHD prophylaxis and 458 who got Ptcy-based.*
- *Key issue raised: 1) (which risk stratification system will be used (e.g. CPSS or others)?); 2) impact of splenomegaly needs to be considered; 3) how to handle correlation between donor type (i.e. haploidentical donors) and use of Ptcy; 4) need to include rates of graft failure as an outcome*

- b. **PROP 2402-01** Outcomes of Second Transplant for myelofibrosis (H Ali/ S Otoukesh) (Attachment 5)

*Dr. Ali's Presentation:*

- **Evaluate outcomes of second transplant for myelofibrosis.**
- *Discussed survival, non-relapse mortality, and relapse risk.*
- *Highlighted the importance of patient selection, donor choices, and timing for second transplant.*
- *Key issue raised: 1) key factor to consider is time from 1<sup>st</sup> HCT to 2<sup>nd</sup> HCT; 2) disease phenotype at the time of 2<sup>nd</sup> HCT; 3) How is this analysis unique when compared to recent EBMT publication*

- c. **PROP 2409-16; 2410-261** Propensity score matched analysis comparing survival by pre-transplant treatment in Myeloid Neoplasms in the venetoclax era (H Elmariah/ W Saber/ N Premnath/ M Juckett) (Attachment 6)

*Dr. Premnath's Presentation:*

- **Comparison of survival by pre-transplant treatment in myeloid neoplasms in the Venetoclax era.**
- *The main hypothesis is venetoclax-based pre-transplant regimens, leads to superior outcomes in the post-allogeneic stem cell transplant compared to alternative first-line therapy.*
- *Focused on disease-free survival, overall survival, relapse, non-relapse mortality, and mixed donor chimerism.*
- *Propensity score-based matching for various factors.*
- *Key issue raised: 1) impact on practice; 2) what is included in the control arm; 3) heterogeneity is introduced by including AML and MDS and across all disease stages*

- d. **PROP 2409-22** Myelodysplastic Neoplasms with Hypoplasia (MDS-h) or Fibrosis (MDS-f): Distinct Clinical Entities Compared to Other MDS Subtypes (A Law/ S Rodriguez Rodriguez) (Attachment 7)

*Dr. Sergio Rodriguez's Presentation:*

- **Discussed myelodysplastic neoplasms with hypoplasia or fibrosis.**
- *Highlighted differences in survival and engraftment outcomes compared to other MDS subtypes.*
- *Focused on overall survival, platelet engraftment, graft failure, relapse, non-relapse mortality, and GVHD.*
- *Key issue raised: 1) accuracy of reporting of the histology; 2) availabilities of molecular data; 3) impact of PNH clone size*

- e. **PROP 2410-17** Evaluating transplant outcome in high risk chronic phase CML (Z Gong/ Y Lei) (Attachment 8)

*Dr. Zimu Gong's Presentation:*

- ***Evaluated transplant outcome in high-risk chronic phase CML.***
- *Discussed the impact of cytogenetic aberrations and the timing of transplant.*
- *Focused on overall survival, relapse-free survival, and GRFS.*
- *Key issue raised: Availability of variables needed to define “high risk” disease, is a concern; molecular data; details of TKI therapies; more details are needed on the control arm (non-transplanted patients)*

- f. **PROP 2410-46; 2410-167** Outcomes of Patients with CLL/SLL Who Receive Allogeneic Hematopoietic Stem Cell Transplant in the Modern Era of Therapies (A Kittai/ S Jaglowski/ J Huang/ M Shadman) (Attachment 9)

*Dr. Jennifer Wong's Presentation:*

- ***Discussed outcomes of patients with CLL/SLL who receive allogeneic hematopoietic stem cell transplant in the modern era of therapies.***
- *Focusing on progression-free survival, overall survival, relapse, non-relapse mortality, and GVHD.*
- *Focused on the impact of targeted therapies and chemoimmunotherapy pre-HCT on HCT outcomes.*
- *Key issue raised: Lead time bias is a concern*

- g. **PROP 2410-148** An international study comparing the efficacy and utility of anti-CD19 CAR-T versus allogeneic stem cell transplantation for Richter Transformation (A Kittai/ J Woyach) (Attachment 10)

*Dr. Adam Katai's Presentation:*

- *Compared the efficacy and utility of anti-CD19 CAR T versus allogeneic transplant for Richter's transformation.*
- *Discussed overall survival, progression-free survival, non-relapse mortality, and safety.*
- *Highlighted the importance of matching patients based on prior treatments and response status.*
- ***Questions and Comments:***
  - *Participants raised questions about the impact of pre-transplant treatments, the role of molecular genetics, and the feasibility of including certain patient populations.*
  - *Discussions on the importance of matching patients based on various factors and the potential impact of newer therapies on transplant outcomes.*

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

- i. **PROP 2409-18** The Impact of Pre-Transplant JAK Inhibition on Outcomes in Allogeneic Stem Cell Transplant for Myelofibrosis (A Ali). ***Dropped due to overlap with current study/publication.***
- j. **PROP 2409-25** Outcomes of GATA2+ MDS transplants in the PTCy era (N Hossain/ P Munshi). ***Dropped due to small sample size.***
- k. **PROP 2410-04** Factors associated with survival following allogeneic transplant for TP53-mutated myelodysplastic syndrome (M Shah/ G Murthy). ***Dropped due to small sample size.***

- l. **PROP 2410-25** Impact of BTKi pre and post CAR T-cell therapy (S Srikantan/ S Farhan). ***Dropped due to small sample size.***
- m. **PROP 2410-60** Risk Factors for Graft Failure following Allogeneic Hematopoietic Cell Transplantation in Patients With BCR-ABL negative Myeloproliferative neoplasms (R Mishra/ T Jain). ***Dropped due to overlap with current study/publication.***
- n. **PROP 2410-79** Impact of Tacrolimus-Methotrexate Versus Post-transplant Cyclophosphamide on Engraftment, Graft Failure, and GVHD Prevention in Myelofibrosis Patients Undergoing Allogeneic Hematopoietic Cell Transplantation (M Pandey/ A Ashraf). ***Dropped due to overlap with current study/publication.***
- o. **PROP 2410-89** The real-world and associated factors of outcomes of relapsed/refractory CLL treated with standard-of-care lisocabtagene maraleucel (E Bezerra/ A Kittai). ***Dropped due to small sample size.***
- p. **PROP2410-101** Risk Factors for Treatment Failure Post-Allogeneic Hematopoietic Cell Transplantation for Myelofibrosis (A Trunk/ C Brunstein). ***Dropped due to overlap with current study/publication.***
- q. **PROP 2410-123** Impact of post-transplant cyclophosphamide (PT-Cy)-based prophylaxis in matched sibling and matched unrelated donors for patients older than 60-years-old with myelodysplastic syndrome. (W Chai-Ho/ G Schiller). ***Dropped due to overlap with current study/publication.***
- r. **PROP 2410-145** Trends in Utilization of Allogeneic Stem Cell Transplant in the Treatment of Myelodysplastic Syndrome (N Punwani). ***Dropped due to overlap with current study/publication.***
- s. **PROP 2410-149** Prognostic impact of IPSS-M relative to IPSS-R in the era of modern MDS treatments for allogeneic HCT (P Munshi/ K Pratz). ***Dropped due to supplemental data needed.***
- t. **PROP 2410-152** Describing allogeneic transplant outcomes in patients with myelofibrosis who undergo pre-transplant treatment with hypomethylating agents. (A Vartanov). ***Dropped due to small sample size.***
- u. **PROP 2410-193** Impact of Ruxolitinib on GVHD and Overall Survival in Patients with Myelofibrosis Following Allogeneic Stem Cell Transplant (J L Reagan, M R Christopher). ***Dropped due to overlap with current study/publication.***
- v. **PROP 2410-237** Comprehensive CIBMTR Analysis of Post-Allogeneic Transplant Treatment with Azacitidine in Chronic Myelomonocytic Leukemia (M Kulasekaran/ G Hildebrandt). ***Dropped due to small sample size.***

## 6. Other business