



## MINUTES AND OVERVIEW PLAN

### CIBMTR WORKING COMMITTEE FOR HEALTH SERVICES AND INTERNATIONAL STUDIES

Houston, TX

Thursday, February 21, 2019, 2:45 – 4:45 PM

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#### 1. Introduction

- a. Minutes and Overview Plan from February 2018 meeting (Attachment 1)
- b. Instructions for sign-in and voting

The meeting was called to order at 2:45pm by Dr. Nandita Khera. She described the goals, expectations, and limitations of the committee, and she gave an introduction of the data that are collected in CRF and TED database. She also explained the voting process, role of working committee members, rules of authorship and statistical hour allocation, and importance of the conference evaluations.

Dr. Wael Saber then gave an introduction on center specific analysis research task force and encouraged audiences to submit proposals at TCT 2020.

#### 2. Accrual summary (Attachment 2)

Due to the full agenda, the accrual summary of registration and research cases between 2008 and 2018 were not presented to the committee but were available as part of the Working Committee attachments.

#### 3. Presentations, published or submitted papers

Due to the full agenda, the 2018 presentations and published papers were mentioned, but not presented. One paper was submitted, 2 oral presentations and 2 poster presentations.

- a. **HS12-02** K Paulson, R Brazauskas, N Khera, N He, N Majhail, G Akpek, M Aljurf, D Buchbinder, L Burns, S Beattie, C Freytes, A Garcia, J Gajewski, T Hahn, J Knight, C LeMaistre, H Lazarus, D Szwajcer, M Seftel, B Wirk, W Wood, W Saber. Inferior Access to Allogeneic Transplant in Disadvantaged Populations: A CIBMTR Analysis. **Submitted**
- b. **HS15-01** D K. Buchbinder, R Brazauskas, K Bo-Subait, K K. Ballen, T E. Hahn, T D John, S K Parsons, S K. Hashmi, N Khera, W A. Wood, W Saber. Lost to Follow-up Rates Are Higher in Pediatric Than Adult Survivors, but Not By Transplant Type: A Report from the Center for International Blood and Marrow

**Not for publication or presentation**

Transplant Research. **Poster presentation at ASH in San Diego, CA, December 2018**

- c. **HS15-02** K O Bona, R Brazauskas, N He, L E. Lehmann, J Wolfe, J Dalal, S K. Hashmi, T E. Hahn, N Khera, W A. Wood, C Duncan, W Saber. Area-Based Socioeconomic Status and Pediatric Allogeneic Hematopoietic Stem Cell Transplantation Outcomes: A CIBMTR Analysis. **Oral presentation at ASH in San Diego, CA, December 2018**
- d. **HS16-02** J Tay, R Brazauskas, N He, S Beattie, C Bredeson, J Dalal, S K. Hashmi, T E. Hahn, N Khera, W A. Wood, W Saber. The Impact of Marital Status on Hematopoietic Stem Cell Transplant (HCT) Recipient Outcomes: A Surrogate for Consistent Caregiver. a CIBMTR Registry Study. **Poster presentation at ASH in San Diego, CA, December 2018**
- e. **HS17-01** S Hong, R Brazauskas, K Herbert, T E. Hahn, N S. Majhail, S J. Lee, D Rizzo, S K. Hashmi, N Khera, W A. Wood, W Saber. Community Health Status and Its Association with Patient Outcome Post Allogeneic Hematopoietic Cell Transplantation. **Oral presentation at TCT in Houston, TX, February 2019**

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

The progress of the ongoing studies during the past year was not presented in order to provide reasonable time to the new proposals for presentation and discussion. A summary of the progress was provided as an attachment to the committee members.

- a. **HS14-01** Investigating clinical outcomes and inpatient health care resource utilization of hematopoietic cell transplantation for children with acute leukemia (S Arnold/ R Aplenc/M Pulsipher/P Satwani) **Manuscript preparation**
- b. **HS15-01** Who is lost to follow-up in the Center for International Blood and Marrow Transplant Research (CIBMTR) registry? (D Buchbinder/T Hahn/K Ballen/ W Saber/ S Parsons) **Manuscript preparation**
- c. **HS15-02** Impact of socioeconomic status on pediatric stem cell transplant outcomes (K Bona/ J Wolfe/ C Duncan/ L Lehmann) **Manuscript preparation**
- d. **HS16-01** Trends in Utilization and Outcomes of Autologous and Allogeneic Hematopoietic cell Transplantation in Racial and Ethnic Minorities (N Khera/ T Hahn/ S Ailawadhi / W Saber) **Protocol Development**
- e. **HS16-02:** The Impact of Marital Status on Hematopoietic Stem Cell Transplant Recipient Outcomes: A surrogate for consistent caregiver (S M Beattie/ J Tay/ C Bredeson) **Manuscript preparation**
- f. **HS16-03** Relationship of Race/Ethnicity and Survival after Single and Double Umbilical Cord Blood Transplantation (K Ballen) **Protocol Development**
- g. **HS17-01** Association of community health status and center survival for allogeneic hematopoietic cell transplantation (S Hong/ N Singh Majhail) **Datafile preparation**

**5. Future/proposed studies**

Dr. Shahrukh K. Hashmi led this section.

- a. **PROP 1811-02** Outcomes of autologous stem cell transplantation for patients with multiple myeloma from rural America (Siddhartha Ganguly) (Attachment 7)  
Dr. Ganguly presented this study. The specific aims of this study are two-fold: 1. Determine if there are differences in receiving AHCT in Rural patients versus Urban patients with multiple myeloma. 2. Determine if there are differences in outcomes based on residence. Comment received on the definition of “rural America” which should be more specific.

- b. **PROP 1811-10** Relative mortality risk in AYA vs younger and older survivors of allogeneic HCT for acute leukemia (Seth Rotz/ Rabi Hanna/ Navneet Majhail) (Attachment 8)  
Dr. Rotz presented this study. The specific aims of this study are three-fold: 1. Determine if AYA (age 14-29 at transplant) leukemia patients who are alive and in remission 1yr post-alloHCT will have increased relative risk of mortality compared to the general US population. 2. Determine if the relative risk of mortality compared to the general US population for AYA leukemia patients who are alive and in remission 1yr post-alloHCT is greater than the RR for children age <14, or adults age 30-49. 3. Determine if the risk of late death in AYA survivors is associated with gender and measures of socioeconomic status at the time of HCT. Comment received on if relative risk of mortality AYA population compared to general population is already been studied by other studies. Dr. Rotz replied that there was a CIBMTR study focusing on the whole population survival rate but no age group.
- c. **PROP 1811-53** Factors associated with clinical trial participation among HSCT patients: a CIBMTR Analysis (Tamryn Gray, Areej El-Jawahri) (Attachment 9)  
Dr. Gray presented this study. The specific aims of this study are three-fold: 1. To describe rates of clinical trial participation based on HCT type. 2. To identify factors that are associated with clinical trial participation in patients with undergoing HCT. 3. To examine the association between clinical trial participation and overall survival (OS) and non-relapse mortality (NRM) in autologous and allogeneic HCT recipients. Comments received on the definition of clinical trial participation. Dr. Gray said the study will include patients participate in clinical trials before transplant based on the forms designed and will not include patients who are go to clinical trials for maintenance. Regarding the center effect Dr. Gray said she can't get the clinical sites' information but she could do sensitivity analysis and patients' zip code will be very informative as well. In response to a question from participant about examine the difference between younger population and adult population, Dr Gray clarified this study will only focus on adult patients because of the different decision-making process between these two groups on participating a clinical trial. In responding to one audience questioning on the disease characteristic of clinical trail participants Dr. Gray said she will look at disease status for each disease. This proposal was accepted by the working committee and leadership, will be HS19-01.
- d. **PROP 1811-114** Incidence and Predictors of Post-Transplant Emotional Distress in Patients Undergoing Hematopoietic Cell Transplant (Neel Bhatt/ Heather Tecca/ Aksha Sharma/ Bronwen E. Shaw) (Attachment 10)  
Dr. Bhatt presented this study. The specific aims of this study are two-fold: 1. To study the cumulative incidence and predictors of post-transplant emotional distress. 2. To study the association of post-HCT emotional distress at day 100 with total number of inpatient days (in first 100 days), cGVHD (at 1-year), infections (up to 1-year) and overall survival (1-year). Comments received on current CIBMTR forms are lack of anxiety and PTSD information for pre-transplant. Dr. Bhatt said he will not exclude pre-transplant depression patients. One meeting participant expressed concern regarding the data quality of emotional distress questions and how to define these variables. Dr. Saber raised concerns about how to deal with patients who died early before the endpoint. Dr. Bhatt said would treat them as competing risk.
- e. **PROP 1811-130** Socioeconomic factors and their impact on non-relapse mortality, GVHD and GVHD survival among patients who received an allogeneic transplant for AML (James Martin/ Henry Fung) (Attachment 11)  
Dr. Martin presented this study. The purpose of this study is to determine which socioeconomic variables, if any, impact NRM and acute/chronic GVHD among adult patients with AML who undergo allogeneic hematopoietic stem cell transplant (alloHSCT) while in first or second complete remission (CR1 or CR2). Dr. Khera suggested using data after 2008 since patients' zip code are more complete since then.

***Dropped proposed studies***

- a. **PROP 1811-15** Comparison of specific ethnic population (Pakistan) with western population for GVHD outcomes. *Dropped due to feasibility.*
- b. **PROP 1811-44** Evaluating the effect of delay in allogeneic stem cell transplantation due to donor unavailability on recipient stem cell transplantation outcomes. *Dropped due to feasibility and small sample size.*
- c. **PROP 1811-84** Evaluation of Factors that Contribute to Cancellation or Delay of planned Hematopoietic Stem Cell Transplantation (HSCT). *Dropped due to feasibility and small sample size.*
- d. **PROP 1811-87** Variations in the use of myeloablative versus reduced intensity conditioning in different countries among patients more than 50 years of age using the CIBMTR database. *Dropped due to low scientific impact.*
- e. **PROP 1811-149** Cost Effective Analysis of Allogeneic bone marrow transplantation with cyclophosphamide–total body irradiation versus Bulsulphan–cyclophosphamide conditioning regimens. *Dropped due to feasibility.*
- f. **PROP 1811-177** Predictors of Cost of Initial Hospitalization for Pediatric Allogeneic Hematopoietic Cell Transplantation. *Dropped due to overlapped with previous studies.*

**6. CIBMTR strategic initiative: Fostering international collaboration**

Dr. Wood led this section. Dr. Pasquini gave a brief introduction of CIBMTR international program and three international studies which were presented later.

- a. **PROP 1811-31** Haploidentical stem cell transplantation for malignant and non-malignant hematological diseases in patients without sibling donor: a multicenter prospective longitudinal study of the Brazilian bone marrow transplantation study group (Nelson Hamerschlak/ Mariana Kerbauy/ Anrezea Riberio) (Attachment 4)  
Dr. Kerbauy presented this study. The specific aims of this study are two-fold: 1. Determine if the 1year Overall Survival after Hematopoietic Stem Cell Transplantation (HCT) plus post-Cy from Haploidentical related donor (Haplo – HCT) for acute myeloid leukemia, Hodgkin Disease (Study Arm 1) and Severe Aplastic Anemia (Arm 2) is not inferior compared to matched related or unrelated allogeneic HCT donor with 10/10 and 9/10 compatibility. 2. Compare the 1year incidences of Acute GVHD (grades II-IV, and III-IV), Chronic GVHD (NIH moderate to severe), Non-relapse-related Mortality, Disease Relapse (only Arm 1), time to Neutrophil engraftment, time to Platelet Engraftment and Event-free survival between recipients of Haplo plus Post-CY and of matched related and unrelated donors. Comments received about the process of Brazilian centers report to CIBMTR and data quality control. Dr. Pasquini also explained the challenges that Brazil transplant centers faced: 1) Data manager is not a part of transplant program recognized by the government. Hospitals can't directly hire data manager. 2) People in Brazil don't speak English but FormsNet is only in English. Dr. Saber suggested comparing Brazil Haplo patients to other countries in the future when data is complete. Other discussions are about explaining Brazilian health care system and how this study can improve Brazilian transplant program.
- b. **PROP 1811-116** Outcomes after allogeneic stem cell transplants performed in Brazil from HLA-matched siblings, unrelated and mismatched related donors. Retrospective study on behalf of the Brazilian Bone Marrow Transplantation Society (SBTMO), GEDECo (Brazil-Seattle Transplant-related complications Consortium), Hospital Israelita Albert Einstein (AmigoH), Associação da Medula Óssea do Estado de São Paulo (Ameo), Program Nacional de Apoio à Atenção Oncológica (Pronon), and CIBMTR (Adriana Seber/ Nelson Hamerschlak/ Mary Flowers/ Marcelo Pasquini) (Attachment 5)  
Dr. Seber presented this study. The specific aims of this study are two-fold: 1. Compare 1-year overall survival after allogeneic HCT performed in Brazil from URD, Haplo and MSD. 2. Compare the 100-day transplant-related mortality (TRM) and the 1-year event-free survival (EFS) after allogeneic HCT

***Not for publication or presentation***

performed in Brazil from URD, Haplo and MSD. Comment received on how the study itself funded. One participant suggested to include as many transplant centers as possible.

- c. **PROP 1811-32** Comparing outcomes of myeloablative T-replete haploidentical transplantation with PT-CY protocol and ATG+G-CSF Protocol in patients with cytogenetic intermediate/high risk acute myeloid leukemia in first complete remission (Xiao-Jun Huang) (Attachment 6)  
Dr. Huang presented this study. The purpose of this study is to compare post-transplant outcomes in adult patients with int/high risk AML in CR1 undergoing T-replete haplo-HCT with PT-CY versus ATG+G-CSF. Regarding the graft type in the population of Peking University, Dr. Huang said in recent 2 years for Haplo was PB only but mixed grafts before that. Regarding adjust age group difference, Dr. Huang said he will do a subgroup analysis.

**7. Other Business**

*Not for publication or presentation*

**Working Committee Overview Plan for 2019-20**

Study number and title	Current status	Goal with date	Total hours to complete	Total hours to goal	Hours allocated to 6/30/2019	Hours allocated 7/1/2019-6/30/2020	Total Hours allocated
<b>HS14-01</b> Investigating clinical outcomes and inpatient health care resource utilization of hematopoietic cell transplantation for children with acute leukemia	Manuscript Preparation	Submitted – July 2019	10	10	10	10	20
<b>HS15-01</b> Who is lost to follow-up in the Center for International Blood and Marrow Transplant Research (CIBMTR) registry?	Manuscript Preparation	Submitted – July 2019	50	50	50	10	60
<b>HS15-02</b> Impact of Socioeconomic Status on Pediatric Stem Cell Transplant Outcomes	Manuscript Preparation	Submitted – July 2019	70	70	70	10	80
<b>HS16-01</b> Trends in Utilization and Outcomes of Autologous and Allogeneic Hematopoietic Cell Transplantation in Racial and Ethnic Minorities	Protocol Development	Data File Preparation – July 2019	280	30	30	180	210
<b>HS16-02</b> The Impact of Marital Status on Hematopoietic Stem Cell Transplant Recipient Outcomes: A surrogate for consistent caregiver	Manuscript Preparation	Submitted – July 2019	70	70	70	10	80
<b>HS16-03</b> Relationship of Race/Ethnicity and Survival after Single and Double Umbilical Cord Blood Transplantation	Protocol Development	Analysis– July 2019	280	130	130	150	280
<b>HS17-01</b> Association of community health status and center survival for allogeneic hematopoietic cell transplantation	Analysis	Submitted – July 2019	100	100	100	10	110
<b>HS18-01</b> International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens	Draft Protocol Received	Manuscript Preparation – July 2020	310	240	0	240	240

***Not for publication or presentation***

<b>HS18-02</b> Racial differences in long term survivor outcomes after Allogeneic hematopoietic cell transplantation	Draft Protocol Received	Analysis– July 2020	310	160	0	160	160
<b>HS18-03</b> Racial/ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia	Draft Protocol Received	Data File Preparation – July 2020	310	100	0	100	100

<b>Oversight Assignments for Working Committee Leadership (March 2019)</b>	
William Wood	<b>HS14-01:</b> Investigating clinical outcomes and inpatient health care resource utilization of hematopoietic cell transplantation for children with acute leukemia
	<b>HS15-02</b> Impact of Socioeconomic Status on Pediatric Stem Cell Transplant Outcomes
	<b>HS16-03</b> Relationship of race/ethnicity and survival after single and double umbilical cord blood transplantation
	<b>HS17-01</b> Association of community health status and center survival for allogeneic hematopoietic cell transplantation
	<b>HS19-01</b> Factors associated with clinical trial participation among HSCT patients: a CIBMTR Analysis
Nandita Khera	<b>HS15-01</b> Who is lost to follow-up in the Center for International Blood and Marrow Transplant Research (CIBMTR) registry?
	<b>HS16-01</b> Trends in utilization and outcomes of autologous and allogeneic hematopoietic cell transplantation in racial and ethnic minorities
	<b>HS16-02</b> The impact of marital status on hematopoietic stem cell transplant recipient outcomes: a surrogate for consistent caregiver
Shahrukh Hashmi	<b>HS18-01</b> International collaborative study to compare the prognosis for acute leukemia patients transplanted with intensified myeloablative regimens
	<b>HS18-02</b> Racial differences in long term survivor outcomes after Allogeneic hematopoietic cell transplantation
	<b>HS18-03</b> Racial/ethnic disparities in receipt of hematopoietic cell transplantation and subsequent resource utilization in children with acute leukemia