2021 STATUS REPORT
NON-MALIGNANT DISEASES WORKING COMMITTEE

Working Committee Leadership	
Co-Chair:	Christopher Dvorak; University of California San Francisco Medical Center; christopher.dvorak@ucsf.edu
Co-Chair:	Andrew Gennery; Newcastle General Hospital/The Royal Victoria Infirmary; a.r.gennery@ncl.ac.uk
Co-Chair:	George Georges; Fred Hutchinson Cancer Research Center; ggeorges@fredhutch.org
Scientific Director:	Mary Eapen; Medical College of Wisconsin; meapen@mcw.edu
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Statistician:	Kyle Hebert; CIBMTR Statistical Center; khebert@mcw.edu

INTRODUCTION

a. Minutes and overview plan from 2020 TCT meeting (Attachment 1)

PROPOSALS MOVING FORWARD FOR SCORING

Not applicable

PROPOSALS DROPPED BECAUSE THEY OVERLAP WITH EXISTING STUDIES OR ARE NOT FEASIBLE DUE TO LIMITATIONS OF AVAILABLE PATIENTS OR DATA

a. PROP 2008-01 Prognostic factors in unrelated donor hematopoietic stem cell transplantation for children, adolescents with acquired severe aplastic anemia (John T. Horan/ Leslie Lehman/ Malika Kapadia).

b. PROP 2010-117 CIBMTR outcomes after transplant for children with MPS-II (Troy C. Lund/ Ashish Gupta).

c. PROP 2010-287 Thrombotic events in patients with sickle cell disease after allogeneic hematopoietic stem cell transplantation (Ameet Patel/ Adetola Kassim).

d. PROP 2010-327 Outcomes of HCT for acquired pure red blood cell aplasia (PRCA) (Ryotaro Nakamura/ Raju Pillai/ Hoda Pouhassan).

e. PROP 2010-76 Hematopoietic cell transplantation for adults with primary immunodeficiency disorders (David Buchbinder/ Jolan Walter). *Overlap with EBMT study under review.*

f. PROP 2010-79 Second or greater HCT for severe aplastic anemia: A contemporary analysis (Hemalatha Rangarajan/ Rajat Kumar/ Prakash Satwani).

PROPOSALS NOT ACCEPTED FOR CONSIDERATION AT THIS TIME DUE TO RELATIVE SCIENTIFIC IMPACT COMPARED TO ONGOING STUDIES AND/OR OTHER PROPOSALS

a. PROP 2009-01 Comparison of allogeneic HCT outcomes using fully myeloablative, reduced intensity and intermediate reduced toxicity myeloablative conditioning regimens in inborn errors of immunity (Rebecca Marsh/ Mary Eapen).

b. PROP 2010-275 Impact of donor/recipient CMV serological status on survival post allogeneic hematopoietic cell transplant in children with non-malignant disorders (Hemaltha Rangarajan/ Prakash Satwani).

STUDIES IN PROGRESS

a. **NM15-01** Outcome of allogeneic hematopoietic cell transplant in erythropoietic porphyria.
Status: Manuscript Preparation. Preparation of the manuscript is currently in progress. The goal is to submit the manuscript by July 2021.

b. **NM16-03** Results of transplants from genetically-identical twin donors in persons with aplastic anemia.
Status: Analysis. The descriptive data analysis is currently being finalized. The goal is to submit the final manuscript by July 2021.

c. **NM17-01** Late effects after hematopoietic stem cell transplantation in patients with hemophagocytic lymphocytic histiocytosis.
Status: Data File Preparation. The study data file is currently being prepared. The goal is to finish data file preparation and transfer study file to EBMT July 2021.

d. **NM18-01** Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant diseases.
Status: Data File Preparation. Preparation of the North American data is complete. Possibility of adding European data to strengthen the study is being explored. As we don't have a set date for transfer of data we are not able to assign a goal for 2021.

e. **NM19-01** Conditional and cause-specific mortality of patients with severe aplastic anemia surviving at least one year after allogeneic hematopoietic cell transplantation or immunosuppressive therapy.
Status: Analysis and Manuscript Preparation. The goal is to submit the manuscript by July 2021.

f. **NM19-03** Hematopoietic stem cell transplantation for congenital amegakaryocytic thrombocytopenia.
Status: Manuscript Preparation. Submit by February 2021.

g. **AC18-02** Prospective cohort study of recipients of autologous hematopoietic cell transplant for systemic sclerosis.
Status: Data Collection. Supplemental data collection delayed – expected to complete early 2021 and prepare study file for analysis by July 2021.

h. **NM20-01** Hematopoietic stem cell transplantation for fanconi anemia.
Status: Protocol Development. Study protocol has been drafted. Development of the protocol is in progress. The goal is to finalize the protocol and complete preparation of the data file by July 2021.

PUBLICATIONS, SUBMITTED PAPERS, PRESENTATIONS

a. **NM17-03b** Brazauskas R, Scigliuolo GM, Wang HL, Cappelli B, Ruggeri A, Fitzhugh CD, Hankins JS, Kanter J, Meerpohl JJ, Panepinto JA, Rondelli D, Shenoy S, Walters MC, Wagner JE, Tisdale JF, Gluckman E, Eapen M. Risk score to predict event-free survival after hematopoietic cell transplant for sickle cell disease. Blood. 2020 Jul 30; 136(5):623-626. PMC7393258.

b. **NM14-02** Myers K, Hebert K, Antin J, Boulad F, Burroughs L, Hofmann I, Kamble R, MacMillan ML, Eapen M. Hematopoietic stem cell transplantation for Shwachman-Diamond syndrome. Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Aug 1; 26(8):1446-1451. PMC7371524.

c. **CV20-02a** Eapen M, Zhang M-J, Tang X-Y, Lee SJ, Fei M-W, Wang H-L, Hebert KM, Arora M, Chhabra S, Devine SM, Hamadani M, D'Souza A, Pasquini MC, Phelan R, Rizzo JD, Saber W, Shaw BE, Weisdorf DJ, Horowitz MM. Hematopoietic cell transplantation with cryopreserved grafts for severe aplastic anemia. Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation. 2020 Jul 1; 26(7):e161-e166. PMC7206419.

d. **NM19-02** Impact of Conditioning Regimen on Allogeneic HCT Outcomes for Hyper-inflammatory Immune Deficiency Disorders. *Submitted. Poster presentation at 2020 ASH annual meeting.*

MINUTES AND OVERVIEW PLAN
CIBMTR WORKING COMMITTEE FOR NON-MALIGNANT DISEASES
Orlando, Florida
Wednesday, February 19, 2020, 12:15pm – 2:15pm

Co-Chair:	**Christopher Dvorak, MD, University of California San Francisco Medical Center, San Francisco, CA;** **E-mail: christopher.dvorak@ucsf.edu**
Co-Chair:	**George Georges, MD, Fred Hutchinson Cancer Research Center, Seattle, WA;** **E-mail: ggeorges@fredhutch.org**
Co-Chair:	**Andrew Gennery, MD, Newcastle General Hospital / The Royal Victoria Infirmary, Newcastle, UK;** **E-mail: a.r.gennery@ncl.ac.uk**
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Statistician:	**Kyle Hebert, MS, CIBMTR Statistical Center, Milwaukee, WI;** **E-mail: khebert@mcw.edu**

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1. Introduction

The CIBMTR Working Committee for Non-Malignant Diseases met on Wednesday, February 19th, 2020 at 12:15pm. Dr. Christopher Dvorak welcomed the audience and introduced the working committee leadership. Dr. Vikram Mathews was thanked for his contributions to the committee as chair as his term as committee chair concluded with this meeting. Dr. Dvorak proceeded to take the attendees through the committee's goals, expectations, and limitations. Dr. Dvorak then explained the CIBMTR Advisory Committee metrics for committee performance. Dr. Dvorak then walked the audience through the rules for authorship on committee studies, before explaining to the attendees the differences between TED and CRF level data. There was a motion to approve the 2019 working committee meeting minutes, and a second. The motion passed.

2. Accrual summary

The accrual tables were referenced for review but not formally presented in the interest of time.

3. Presentations, published or submitted papers

Dr. Dvorak directed the audience to the working committee materials for information regarding the three committee publications from 2019.

The three committee publications from 2019, and 1 submitted paper are listed below:

a. **NM17-03** Eapen M, Brazauskas R, Walters MC, Bernaudin F, Bo-Subait K, Fitzhugh CD, Hankins JS, Kanter J, Meerpohl JJ, Bolaños-Meade J, Panepinto JA, Rondelli D, Shenoy S, Williamson J, Woolford TL, Gluckman E, Wagner JE, Tisdale JF. Effect of donor type and conditioning regimen intensity on

allogeneic transplantation outcomes in patients with sickle cell disease: a retrospective multicentre, cohort study. ***The Lancet Haematology. 2019 Nov;6(11):e585-e596.***

b. **NM17-02** Li C, Mathews V, Kim S, George B, Hebert K, Jiang H, Li C, Zhu Y, Keesler DA, Boelens JJ, Dvorak CC, Agarwal R, Auletta JJ, Goyal RK, Hanna R, Kasow K, Shenoy S, Smith AR, Walters MC, Eapen M. Related and unrelated donor transplantation for β-thalassemia major: results of an international survey. ***Blood Advances. 2019 Sep 10;3(17):2562-2570.***

c. **NM16-04** Bejanyan N, Kim S, Hebert KM, Kekre N, Abdel-Azim H, Ahmed I, Aljurf M, Badawy SM, Beitinjaneh A, Boelens JJ, Diaz MA, Dvorak CC, Gadalla S, Gajewski J, Gale RP, Ganguly S, Gennery AR, George B, Gergis U, Gómez-Almaguer D, Vicent MG, Hashem H, Kamble RT, Kasow KA, Lazarus HM, Mathews V, Orchard PJ, Pulsipher M, Ringden O, Schultz K, Teira P, Woolfrey AE, Saldaña BD, Savani B, Winiarski J, Yared J, Weisdorf DJ, Antin JH, Eapen M. Choice of conditioning regimens for bone marrow transplantation in severe aplastic anemia. ***Blood Advances. 2019 Oct 22;3(20):3123-3131.***

d. **NM14-02** Outcomes of allogeneic hematopoietic cell transplant in patients with Shwachman diamond syndrome (K Myers) **Submitted**

4. Studies in progress

Dr. Dvorak directed the attention of the attendees to the meeting materials for updates on the full list of active committee studies.

The following is the full list of the current status of the active committee studies:

a. **AA13-02** Malignancies in patients with fanconi anemia (J Wagner) **Manuscript Preparation**

b. **NM15-01** Outcome of Allogeneic Hematopoietic Cell Transplant in Erythropoietic Porphyria (A Saad/D Moshous) **Manuscript Preparation**

c. **NM16-03** Results of transplants from genetically-identical twin donors in persons with aplastic anemia (RP Gale) **Data File Preparation**

d. **NM17-01** Late effects after hematopoietic stem cell transplantation in patients with HLH (A Horne/KS Baker/K Beutel) **Protocol Development**

e. **NM18-01** Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant disease (A Prakash/ D Wall/ K Paulson) **Data File Preparation**

f. **NM19-01** Conditional and cause-specific mortality of patients with severe aplastic anemia surviving at least one year after alloHCT or immunosuppressive therapy (R Nakamura/FL Wong/S Armenian/N Young) **Data File Preparation**

g. **NM19-02** Impact of Conditioning on Allogeneic HCT Outcomes for HLH (R Marsh) **Analysis**

h. **NM19-03** Hematopoietic Stem Cell Transplantation for Congenital Amegakaryocytic Thrombocytopenia (F Boulad/M Cancio/JJ Boelens) **Analysis**

i. **AC18-02** Prospective Cohort study of Recipients of Autologous Hematopoietic cell Transplant for Systemic Sclerosis (G Georges) **Data Collection**

5. Future/proposed studies

Dr. Gennery outlined the voting process for the attendees, explaining that voting should be based both on scientific impact and on feasibility using the CIBMTR data.

a. **PROP 1906-02** CIBMTR Retrospective Study of Allogeneic Stem Cell Transplant Outcomes in Severe Aplastic Anemia (SAA) using Fludarabine, Cyclophosphamide and Alemtuzumab ('FCC') Conditioning (Shafqat Inam; Judith Marsh)

Dr. Eapen presented the proposal on behalf of the proponents. The objective of the study would be to examine and quantify the outcomes, including overall survival, graft-failure-free survival, hematopoietic recovery, graft failure, and GVHD, of patients undergoing allogeneic

HCT for severe aplastic anemia with a fludarabine, cyclophosphamide, and alemtuzumab (FCC) conditioning regimen.

The CIBMTR identified 38 CRF-level and 68 TED-level patients who underwent allogeneic HCT with the FCC regimen in the United States between 2010 and 2018. If the study were to be accepted, these patients would be added to a cohort of European patients from the center of the group of proponents. An attendee asked whether these would have homogenous graft sources, to which Dr. Eapen replied that essentially all of the European patients would have received peripheral blood grafts, while the North American patients are a mix of bone marrow and peripheral blood, with most getting marrow. A few attendees expressed concern over the small number of patients and a potential inability to draw strong conclusions. Another attendee asked whether this would be a formal comparison of the European patients against the North American ones, to which Dr. Eapen replied that a direct comparison between the two would not be the focus of the study, but the study would test for a center effect. An attendee asked whether the FCC regimen would be compared to other regimens. Dr. Eapen replied that the working committee just published a study that was an analysis of regimen choice for aplastic anemia, and the results found no effect.

b. **PROP 1910-07/Prop 1911-132** Haploidentical Donor Transplantation for Severe Aplastic Anemia: A Combined CIBMTR-EBMT Study (Akshay Sharma; Abhishek A. Mangaonkar and colleagues)

Dr. Sharma presented the proposal. The objective of the study is to assess outcomes following hematopoietic stem cell transplant for patients undergoing haploidentical transplant for severe aplastic anemia, and compare these outcomes to known baselines from historical controls for matched siblings and matched unrelated donors.

The CIBMTR identified 146 patients (TED-level) who underwent haploidentical HCT for severe aplastic anemia in the United States between 2014 and 2018. One question was whether the presenter was concerned about some of the stark differences in treatment strategies that exist between European and North American centers for these cases. Dr. Sharma replied that he believes that this would in fact be an advantage of the study, allowing the study team to look across different strategies and compare outcomes to learn. However, Dr. Sharma could not provide the details of the different strategies that were being used in Europe. The European group also published a descriptive report of use of post-transplant Cy. The next question was what would the control group be, and would a control group exist as a formal component of this study. Dr. Sharma responded that there would be no need to actually prepare data to directly compare the haploidentical transplants to the siblings or matched unrelated donors for the purposes of this study, but that the comparison could simply be made to well-established historical benchmarks for these types of transplants.

c. **PROP 1910-13** Impact of immunosuppressive therapy (IST) duration on hematopoietic cell transplantation (HCT) outcomes in patients with severe aplastic anemia (SAA) (Jessica E. Knight-Perry; Michael R. Verneris)

Dr. Verneris presented the proposal. The objective of this study would be to determine whether a difference exists in event-free survival between patients receiving less than 6 months of immunosuppression post-transplant versus greater than 6 months of post-transplant immunosuppression following allogeneic HCT for severe aplastic anemia. Secondary aims would include doing a similar comparison for individual outcomes, such as GVHD, graft failure, and infections.

The CIBMTR identified 99 patients receiving less than 6 months of post-transplant IST, and 255 patients receiving greater than 6 months of post-transplant IST, following allogeneic HCT for severe aplastic anemia in the United States on the CRF track between 2008 and 2018. A major

concern with this proposal, voiced by several attendees, was that the length of post-tranpslant IST would be heavily correlated with the presence or absence of GVHD. Over half of the cases receiving longer than 6 months of IST after transplant had some form of GVHD, compared to only about 20 percent of those receiving less than 6 months. This would make it very difficult to control for identifying intended post-transplant IST and its impact on outcomes, which could bias results. Some attendees suggested that either a time-dependent model after transplant, or a survey to centers to gather information about standard practices for treatment of GVHD would be needed to address this issue. Another concern was that only the date of final cessation of post-transplant IST is collected on CIBMTR forms, with no information regarding the date of any early taper.

d. **PROP 1911-142/Prop 1909-03/Prop 1911-118** Hematopoietic Stem Cell Transplantation for Fanconi anemia (Farid Boulad; Seth J. Rotz; Hesham Eissa and colleagues)
Dr. Boulad presented the proposal. The objective of the study would be to examine the outcomes following allogeneic HSCT in patients in the current era transplanted for Fanconi anemia. The primary focus would be use of alternative donors (i.e. not matched siblings).
The CIBMTR identified 627 patients at the CRF-level of data collection, and 1,091 patients at the TED-level, who underwent allogeneic HSCT for Fanconi anemia in the United States, Canada, South America, or Saudi Arabia between 2000 and 2018. A very small minority of these cases were transplanted for AML or MDS with Fanconi anemia as a pre-disposing condition. A concern was raised that if the study intends to look for prognostic factors, the number of patients would be somewhat low to do so with sufficient power. Dr. Boulad asserted that he believes that in fact the numbers are not small, and this would be largest cohort of its kind seen for this disease as compared to past studies. Certain groups might be smaller for certain variables, but this should be able to be addressed in the multivariate analysis.

e. **PROP 1911-150** Clinical Outcome and Health Care Utilization of Children with Hemophagocytic Lymphohistiocytosis who received Hematopoietic Stem Cell Transplantation (Ram Kalpatthi; Meghan McCormick; Archana Ramgopal; Matt Hall; Jignesh Dalal)
Dr. Dalal presented the proposal. The objective of the study would be to describe the management of pediatric patients with hemophagocytic lymphohistiocytosis proceeding to hematopoietic stem cell transplant, and to attempt to identify factors related to overall survival following transplant.
The CIBMTR identified 295 pediatric patients at the CRF-level of data collection that underwent allogeneic HCT for hemophagocytic lymphohistiocytosis in the United States between 2000 and 2018. Dr. Dalal clarified for the group that the PHIS database could be used to provide information regarding the patients' economic burden, prophylactic treatment before transplant, and relevant social factors to be used for the portion of this study aimed at describing the management of these cases. Several members of the committee questioned the validity and completeness of the PHIS data to which Dr. Dalal was unable to respond. The committee leadership asked whether conditioning regimen would be a primary focus of this study, as the committee already has an active study accepted at the 2019 meeting that is exploring the impact of regimen choice for HLH. Dr. Dalal explained that conditioning regimen would be just one factor that this study would look at in addition to several other potential prognostic factors. Of note, there are two approved / on-going HLH studies that address transplant outcomes including late effects and it was unclear how the addition of PHIS data would generate knowledge that would impact clinical practice.

6. Dropped proposed studies

The committee received the following additional study proposals, but these proposals were not selected for presentation at the TCT meeting, for the reasons outlined below:

a. **PROP 1910-09** Effect of mixed host-donor chimerism on graft failure/rejection after hematopoietic cell transplantation for non-malignant hematological disorders
Dropped due to feasibility

b. **PROP 1911-24** Influence of GVHD prophylaxis in Aplastic Anemia and transplant outcomes in the era of newer conditioning regimens
Dropped due to overlap with recent committee study NM16-04

c. **PROP 1911-35** Composite Graft versus Host Disease and Graft Failure Free Survival (GFFS) in non-malignant hematologic disorders patients undergoing allogeneic stem cell transplantation with post-transplant cyclophosphamide: The novel composite endpoint
Dropped due to heterogeneity of diseases and overlap with other disease-specific studies

d. **PROP 1911-75** Evaluation of Outcomes following Allogeneic Hematopoietic Cell Transplantation in Patients with Diamond-Blackfan Anemia and other red cell aplasias: A CIBMTR Analysis.
Dropped due to overlap with previous publications

e. **PROP 1911-104** Impact of Time to Transplant in Severe Aplastic Anemia Patients Receiving Prior Immunosuppressive Therapy
Dropped due to feasibility

f. **PROP 1911-125** Determining the Effect of Recipient-Donor Race Matching on Outcomes forSickle Cell Patients Transplanted with Matched Unrelated Donors
Dropped due to low sample size

g. **PROP 1911-138** Allogeneic hematopoietic stem cell transplantation (HCT) outcomes with total body irradiation (TBI)-conditioning regimens versus non-TBI regimens among patients with severe aplastic anemia (SAA)
Dropped due to overlap with recent study NM16-04

h. **PROP 1911-153** Impact of non-infectious encephalopathy on outcomes among children undergoing allogeneic hematopoietic stem cell transplant for non-malignant disorders.
Dropped due to feasibility

i. **PROP 1911-156** Trends of Early Mortality Within First Two Years Following Allogeneic Hematopoietic Cell Transplantation in Children and Adolescents with Non-Malignant Disorders
Dropped due to heterogeneity of diseases and overlap with other disease-specific studies

j. **PROP 1911-165** Patterns of use and outcomes of donor lymphocyte infusions for non-malignant diseases
Dropped due to low sample size

k. **PROP 1911-202** Graft versus host disease (GVHD) free and Rejection Free Survival (GRFS) and Chronic GVHD and Rejection Free Survival (CRFS) in patients undergoing allogeneic hematopoietic stem cell transplant for non-malignant disorders.
Dropped due to heterogeneity of diseases and overlap with other disease-specific studies

l. **PROP 1911-211** Comparing incidence of acute and chronic graft versus host disease (GVHD) with different conditioning and GVHD prophylaxis regimens used in alternative donor transplants for non-malignant conditions.
Dropped due to heterogeneity of diseases and overlap with other disease-specific studies

m. **PROP 1911-218** To study the outcomes after autologous hematopoietic stem cell transplantation for multiple sclerosis
Dropped due to feasibility and overlap with other recent publications

7. Concluding Notes

a. Meeting adjourned at 1:25pm.

b. Voting on proposals.
After the new proposals were presented, each participant in the meeting had an opportunity to rate each proposal using paper ballots. Based on the voting results, current scientific merit, available number of relevant cases, and the impact of the study on the field, the following proposal was accepted to move forward to be added to the committee's active studies:
PROP 1911-142/Prop 1909-03/Prop 1911-118 Hematopoietic Stem Cell Transplantation for Fanconi anemia (Farid Boulad; Seth J. Rotz; Hesham Eissa and colleagues)

c. The following proposals were not accepted as studies:

- **PROP 1906-02** CIBMTR Retrospective Study of Allogeneic Stem Cell Transplant Outcomes in Severe Aplastic Anemia (SAA) using Fludarabine, Cyclophosphamide and Alemtuzumab
- **PROP 1910-07/Prop 1911-132** Haploidentical Donor Transplantation for Severe Aplastic Anemia: A Combined CIBMTR-EBMT Study
- **PROP 1910-13** Impact of immunosuppressive therapy (IST) duration on hematopoietic cell transplantation (HCT) outcomes in patients with severe aplastic anemia (SAA)
- **PROP 1911-150** Clinical Outcome and Health Care Utilization of Children with Hemophagocytic Lymphohistiocytosis who received Hematopoietic Stem Cell Transplantation

Working Committee Overview Plan for 2020 - 2021							
Study number and title	**Current status**	**Goal with date**	**Total hours to complete**	**Total hours to 2021 goal**	**Hours allocated to 6/30/20**	**Hours allocated 7/1/20-6/30/21**	**Total Hours allocated**
AA13-02: Malignancies in patients with Fanconi Anemia	Manuscript Preparation	Submitted – July 2020 Published –July 2021	10	**10**	10	0	**10**
NM14-02: Allo HCT for Shwachman Diamond Syndrome	Submitted	Published – July 2020	10	**10**	10	0	**10**
NM15-01: Outcome of allogeneic Hematopoietic Cell Transplant (HCT) in Erythropoietic Porphyria	Manuscript Preparation	Submitted – July 2020 Published – July 2021	30	**40**	30	10	**40**
NM16-03: Results of transplants from genetically-identical twin donors in persons with aplastic anaemia	Analysis	Manuscript Preparation – July 2020 Submitted – July 2021	80	**80**	10	70	**80**
NM17-01: Late effects after hematopoietic stem cell transplantation in patients with HLH	Protocol Development	Analysis – July 2020 Manuscript Preparation – July 2021	310	**240**	160	80	**240**
NM18-01: Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant disease	Data file preparation	Analysis – July 2020 Submitted – July 2021	160	**160**	10	150	**160**
NM19-01: Conditional and cause-specific mortality of patients with severe aplastic anemia surviving at least one year after alloHCT or immunosuppressive therapy	Analysis	Analysis – July 2020 Submitted – July 2021	150	**150**	0	150	**150**
NM19-02: Impact of Reduced Intensity Conditioning on Allogeneic HCT Outcomes for HLH	Analysis	Manuscript Preparation – July 2020 Submitted – July 2021	150	**150**	80	70	**150**
NM19-03: Hematopoietic Stem Cell Transplantation for Congenital Amegakaryocytic Thrombocytopenia	Manuscript Preparation	Submitted – July 2020 Published – July 2021	80	**80**	70	10	**80**

AC18-02: Prospective Cohort study of Recipients of Autologous Hematopoietic cell Transplant for Systemic Sclerosis	Data Collection	Data File Preparation – July 2020 Submitted – July 2021	280	**280**	30	250	**280**
NM20-01: Hematopoietic Stem Cell Transplantation for Fanconi anemia	Protocol Pending	Protocol Development – July 2020 Analysis – July 2021	330	**200**	0	200	**200**

Oversight Assignments for Working Committee Leadership (March 2020)		
George Georges	**AA13-02**	Malignancies in patients with fanconi anemia
	NM16-03	Results of transplants from genetically-identical twin donors in persons with aplastic anaemia
	NM19-01	Conditional and cause-specific mortality of patients with severe aplastic anemia surviving at least one year after alloHCT or immunosuppressive therapy
	AC18-02	Prospective Cohort study of Recipients of Autologous Hematopoietic cell Transplant for Systemic Sclerosis
	NM20-01	Hematopoietic Stem Cell Transplantation for Fanconi anemia
Christopher Dvorak	**NM14-02**	Outcomes of allogeneic hematopoietic cell transplant in patients with Shwachman diamond syndrome
	NM19-02	Impact of reduced intensity conditioning on allogeneic HCT outcomes for HLH
	NM19-03	Hematopoietic stem cell transplantation for Congenital Amegakaryocytic Thrombocytopenia
Andrew Gennery	**NM15-01**	Outcome of Allogeneic Hematopoietic Cell Transplant in Erythropoietic Porphyria
	NM17-01	Late effects after hematopoietic stem cell transplantation in patients with HLH
	NM18-01	Impact of choice of serotherapy in pediatric stem cell transplantation for non-malignant disease