Not for publication or presentation

MINUTES AND OVERVIEW PLAN
CIBMTR WORKING COMMITTEE FOR PRIMARY IMMUNE DEFICIENCIES, INBORN ERRORS OF METABOLISM AND OTHER NON-MALIGNANT MARROW DISORDERS
Orlando, Florida
Wednesday, February 22, 2017, 2:45pm – 4:45pm

Co-Chair:	**Paolo Anderlini, MD, MD Anderson Cancer Center, Houston, TX; Telephone: 713-745-4367; E-mail: panderli@mdanderson.org**
Co-Chair:	**Neena Kapoor, MD, Children's Hospital of Los Angeles, Los Angeles, CA; Telephone: 323-361-2546; E-mail: nkapoor@chla.usc.edu**
Co-Chair:	**Jaap Jan Boelens, MD, PhD, University Medical Center Utrecht, Utrecht, Netherlands; Telephone: +31 8875 54003; E-mail: j.j.boelens@umcutrecht.nl**
Co-Chair:	**Vikram Mathews, MD, DM, MBBS, Christian Medical College Hospital, Vellore, India; Telephone: +011 91 416 228 2891; E-mail: vikram@cmcvellore.ac.in**
Scientific Director:	**Mary Eapen, MBBS, MS, CIBMTR Statistical Center, Milwaukee, WI; Telephone: 414-805-0700; E-mail: meapen@mcw.edu**
Statistical Director:	**Soyoung Kim, PhD, CIBMTR Statistical Center, Milwaukee, WI; Telephone: 414-955-8271; E-mail: skim@mcw.edu**
Statistician:	**Kyle Hebert, MS, CIBMTR Statistical Center, Milwaukee, WI; Telephone: 414-805-0673; E-mail: khebert@mcw.edu**

1. Introduction

The CIBMTR Working Committee for Primary Immune Deficiencies, Inborn Errors of Metabolism and other Non-Malignant Marrow Disorders met on Wednesday, February 22, 2017 at 2:45pm. Dr. Mathews welcomed the audience and introduced the working committee leadership, reviewed the committee's goals, expectations, and limitations. The minutes from the 2016 Tandem meeting were approved. Dr. Mathews then reviewed the CIBMTR guidelines for committee membership and rules for authorship of studies. The links to additional working committee related information were also provided. Dr. Mathews then presented a list of all of the committee's publications from the past 6 years, which consisted of 19 studies covering a broad spectrum of diseases.

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. Boelens directed the audience to the working committee materials for information regarding the three committee publications from 2016. Dr. Boelens presented a brief summary and results of the paper *Sickle cell disease: an international survey of results of HLA-identical sibling hematopoietic stem cell transplantation.* Dr. Kekre presented a brief summary and results of the paper *Effect of Antithymocyte globulin source on outcomes of bone marrow transplantation for severe aplastic anemia*.

The three committee publications from 2016 are listed below:

a. **NM14-04** Gluckman E, Cappelli B, Bernaudin F, Labopin M, Volt F, Carreras J, Simoes BP, Ferster A, Dupont S, de la Fuente J, Dalle JH, Zecca M, Walters MC, Krishnamurti L, Bhatia M, Leung K, Yanik G, Kurtzberg J, Dhedin N, Kuentz M, Michel G, Apperley J, Lutz P, Neven B, Bertrand Y, Vannier JP, Ayas M, Cavazzana M, Matthes-Martin S, Rocha V, Elayoubi H, Kenzey C, Bader P, Locatelli F, Ruggeri A, Eapen M. Sickle cell disease: an international survey of results of HLA-identical sibling hematopoietic stem cell transplantation. ***Blood 2016 (In press).***

b. **ID10-02** Slack J, Albert MH, Balashov D, Belohradsky B, Bertaina A, Bleesing J, Booth C, Büchner J, Buckley RH, Chardin M, Deripapa E, Drabko K, Eapen M, Feuchtinger T, Finocchi A, Gaspar HB, Ghosh S, Gillio A, Gonzalez-Granado LI, Grunebaum E, Guengoer T, Heilmann C, Helminin M, Higuchi K, Imai K, Kalwak K, Kanazawa N, Karasu G, Kucuk ZY, Laberko A, Lange A, Mahlaoui N, Meisel R, Moshous D, Muramatsu H, Parikh S, Pasic S, Schmid I, Schuetz C, Schultz KR, Shaw PJ, Slatter MA, Sykora K-W, Tamura S, Taskinen M, Wawer A, Wolska-Kuśnierz B, Cowan MJ, Fischer A, Gennery AR. Outcome of haematopoietic stem cell transplantation for DNA-double strand breakage repair disorders: An EBMT/ESID IEWP, SCETIDE and CIBMTR/PIDTC survey. ***Journal of Allergy and Clinical Immunology 2017 (In press).***

c. **SC14-01** Natasha Kekre, Zhang Y, Zhang M, Carreras J, Anderlini P, Ahmed P, Atta EH, Ayas M, Boelens JJ, Bonfim C, Deeg HJ, Kapoor N, Lee JW, Nakamura R, Pulsipher M, Eapen M, Antin JH. Effect of Antithymocyte globulin source on outcomes of bone marrow transplantation for severe aplastic anemia. ***Haematologica. (Submitted)***

4. Studies in progress

Dr. Boelens directed the audience to the working committee materials for the complete list of studies in progress and details regarding each study. Dr. Marsh presented a brief update on the preliminary work done on the study entitled *Allogeneic hematopoietic cell transplantation for primary immune deficiencies: current patterns of practice and change over the last 10 years*.

All of the working committee active studies are listed below:

a. **AA13-01** Correlation of levels of donor cell chimerism with hemoglobinopathy symptoms following allogeneic hematopoietic cell transplantation (A Abraham) **Manuscript Preparation**

b. **AA13-02** Malignancies in patients with fanconi anemia (J Wagner) **Data File Preparation**

c. **ID11-01** Cerebral adrenoleukodystrophy: A multicenter evaluation of outcomes based on pre-transplant functional assessments and MRI (P Orchard) **Manuscript Preparation**

d. **ID12-01** Allogeneic hematopoietic cell transplantation for combined immunodeficiency and common variable immunodeficiency (G Cuvelier/G Guilcher/N Wright) **Data File Preparation**

e. **ID13-01** Second and subsequent hematopoietic cell transplants for congenital neutropenia/kostmann agranulocytosis (S Keogh/P Shaw/J Levine/J Connelly) **Analysis**

f. **NM14-01** An investigation of the long term neurological outcomes of Hematopoietic Stem Cell Transplant (HCT) in boys with X-linked Adrenoleukodystrophy (X-ALD) (R Wynn/J Boelens/P Orchard) **Data Collection**

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

h. **NM15-01** Outcome of Allogeneic Hematopoietic Cell Transplant in Erythropoietic Porphyria (A Saad/H Abdel-Azim/J Bloomer) **Manuscript Preparation**

i. **NM16-01** Combined EBMT/CIBMTR retrospective study of allogeneic stem cell transplant outcomes in older patients (age > 50 years) with severe aplastic anemia (Rice C/Marsh J/Potter V) **Analysis**

j. **NM16-02** Allogeneic hematopoietic cell transplantation for primary immune deficiencies: current patterns of practice and change over the last 10 years (Marsh R) **Protocol Development**

k. **NM16-03** Results of transplants from genetically-identical twin donors in persons with aplastic anemia (RP Gale) **Protocol Development**

l. **NM16-04** The effect of conditioning regimen on clinical outcomes of allogeneic hematopoietic cell transplantation in severe aplastic anemia (N Bejanyan/N Kekre/D Weisdorf/J Antin) **Protocol Development**

5. Future/proposed studies

a. **PROP 1611-68** Outcomes following second allogeneic hematopoietic cell transplantation in patients with hemoglobinopathies (H Rangarajan/M Thakar)

Dr. Rangarajan presented the proposal. The study will investigate characteristics and outcomes of patients with hemoglobinopathies undergoing a second allogeneic HCT. Similar investigations have been published for Thalassemia patients undergoing a second transplant following graft failure, but available literature on Sickle Cell Disease focuses on first transplants. The aim of the study is to analyze the prognostic factors that are related with better or worse transplant outcomes, including engraftment, transplant-related mortality, incidence of acute and chronic GVHD, and overall survival. The study will likely need to be descriptive due to a relatively small number of patients.

The CIBMTR identified 20 patients with Sickle Cell Disease and 21 patients with Thalassemia major that underwent a second allogeneic transplant between 2000-2014. The primary feasibility concern voiced by several during the discussion of the proposal was the available sample size. With only 41 patients (sickle cell disease and thalassemia) with heterogeneous conditioning regimens, donor and graft sources, there was concern that the study would not be able to reach strong, informational conclusions. It was also noted that in the recent Sickle Cell Disease publication, a population of roughly 1,000 patients yielded only 23 graft rejections, indicating that it is unlikely that the available sample size for this study would increase drastically over the next few years.

The proposal was not approved.

b. **PROP 1612-06** Accelerated aging phenotypes post-transplant in telomeropathies – a Center for International Blood and Marrow Transplant Research study (S Hashmi/S Gadalla)

Dr. Gadalla presented the proposal. The goal of the study is to evaluate accelerated aging phenotypes in patients that are transplanted for telomeropathies in comparison to those transplanted for other diseases, such as inborn errors of metabolism or hemoglobinopathies. Secondary objectives include evaluating the effect of conditioning regimen and GVHD prophylaxis on the risk of premature aging phenotypes in patients with telomeropathies, as well as comparing serum pre-HCT aging biomarkers. Eligibility criteria for the study will include a diagnosis of dyskeratosis congenita for all ages, as well as identification of a germline mutation in the telomere gene.

The CIBMTR identified 10 patients transplanted with a diagnosis of dyskeratosis congenita with samples available in the sample repository between 2004-2014. There were some concerns related to the scientific impact and feasibility of the study. Several attendees felt that Fanconi anemia and hemoglobinopathies were not suitable control groups for comparison due to differences in the biology of the diseases. Furthermore, inborn errors of metabolism diseases are numerous and varied in nature, and identifying an acceptable control group for comparison would be very difficult. Another major concern among attendees is that there were only 10 patients transplanted for dyskeratosis congenita with samples available in the repository. As such, even if an acceptable control group was agreed upon, any conclusions that would be reached based on the 10 patients identified would be weak. Other questions centered upon what outcomes that are needed for the study are available. The conclusion reached was that the study would require extensive supplemental data collection. However, Dr. Gadalla was advised she might study the NIH's population with dyskeratosis congenita. She informed the committee that the question being asked in regards to premature aging is specific to patients who underwent allogeneic HCT.

The proposal was not approved.

c. **PROP 1612-07** Evaluation of the impact of changing clinical profile, transplant conditioning regimens and stem cell source on clinical outcome in patients with Thalassemia major (V Mathews/C Li/S Hongeng)

Dr. Mathews presented the proposal. Dr. Mathews described that allogeneic HCT for Thalassemia major has undergone significant changes in the past decade, including increasing age of recipients, more transplants done in developing countries, increasing use of alternative conditioning regimen drugs and alternative donor types, and increasing use of peripheral blood stem cells as a graft source. The study hypothesizes that there have been significant changes in the demographic profile of patients undergoing allogeneic HCT for Thalassemia major over the past 10 years, with significant improvement in clinical outcomes related to changes in conditioning regimens and graft source. The goals of the study include describing the demographics of patients transplanted for Thalassemia in the past 10 years as compared to historical controls, investigating the impact of changes in conditioning regimen on clinical outcomes, and evaluating the impact of differences in choice of donor and graft types on outcomes. Dr. Mathews explained his plans to collaborate on this study with a few non-CIBMTR institutions in China and Thailand in order to gather a much larger study population.

The CIBMTR identified 365 patients in the US and Dr. Mathews identified 443 patients in India that underwent a first allogeneic HCT for Thalassemia from 2000-2016. Dr. Mathews also corresponded with Dr. Chunfu Li and Dr. Suradej Hongeng to determine that there are approximately 129 patients from Thailand and approximately 627 patients in China with sufficient data to be added to the study, bringing the potential total number of patients to 1,564.

The proposal was generally well-received by the committee. Several attendees expressed that the very large potential study population was encouraging. A comment was made that a possible concern was the availability of liver biopsy and MRI results for analysis, which would limit the ability to analyze the patients in terms of risk category. This was also an issue for the last CIBMTR study on thalssemia. Dr. Mathews has confirmed that a subset of patients will have results of liver biopsy and numbers permitting a subset analysis can be undertaken. The earlier CIBMTR study (with a smaller cohort) clearly identified risk factors associated with mortality despite the lack of results of liver biopsy. However, that study was limited to HLA-matched sibling BMT. With increasing numbers of alternative donor HCTs, identifying risk factors for mortality will add to the literature. Another concern expressed was about the combination of patients from different geographic regions and the potential implications of variation in practice by region. Statistically, we check for and if needed adjust for transplant center effect. Additionally we could compare overall survival across the regions to ensure comparability of outcomes. Most CIBMTR studies include international centers so this study is in keeping with other studies. Another question was whether it is known if the datasets from the different institutions can be merged successfully. Dr. Eapen explained that there have been preliminary discussions with Dr. Li and Dr. Hongeng to confirm the compatibility of the data.

The proposal was approved to proceed to protocol development.

d. **PROP 1612-09** Outcomes of Hematopoietic Stem Cell Transplantation in Young Children with Sickle Cell Disease (S Shenoy)

Dr. Eapen presented the proposal on behalf of Dr. Shenoy, who was unable to attend the meeting. The study aims to investigate clinical outcomes for pediatric patients transplanted for sickle cell disease, including engraftment, transplant-related mortality, overall survival, and incidences of acute and chronic GVHD.

The CIBMTR identified 213 patients aged 12 or younger transplanted for sickle cell disease between 2006-2016. A major concern regarding the scientific impact of the study was that these 126 patients are a subset of the recent committee publication (NM14-04, Gluckman et. al). The other concern was how would a subset analysis offer new knowledge? A potential feasibility issue discussed was that the potential study population is heterogeneous with respect to graft source and conditioning regimen intensity. For a study with only 213 cases, this heterogeneity could be problematic in the multivariate analysis.

A question posed by several attendees was about the rationale behind choosing age 12 as the cutoff age for inclusion in the study. Some attendees felt that the uniqueness of this particular study, as opposed to previous sickle cell disease studies, would be to learn about prognostic factors specifically for very young children undergoing BMT. In other words is there something unique about the disease in the very young such as severity and disease cure may be impacted. Given this, some voiced their opinions that the cutoff age of 12 was too high debated the rationale for lowering the age of the study population. That would further limit the study population and perhaps the success of transplantation could be determined by other endpoints and not solely, HCT related endpoints. These were communicated to Dr. Shenoy after the meeting and she was encouraged to work with sickle cell disease experts to design a more compelling/robust study proposal for consideration at the next Tandem meeting.

The proposal was not approved.

e. **PROP 1612-11** Late effects after hematopoietic stem cell transplantation in patients with HLH (A Horne/KS Baker/K Beutel)

Dr. Baker presented the proposal. The goal of the study is to investigate the relationship of HCT to survival and particularly late effects in patients with HLH. Dr. Baker explained that there is no published data at this time regarding late effects and long-term outcomes in patients transplanted for HLH, and that such a study could be valuable in gaining insight into the problems and needs of long-term survivors after transplant. The specific aims of the study would be to describe the frequency and severity of late effects in survivors of allogeneic HCT for HLH, as well as analyzing late mortality and identifying risk factors for development of late effects. The study population would include patients aged < 18 who underwent allogeneic HCT for HLH between 2004-2014, and who survived for at least two years after transplant with complete follow-up data on relevant outcomes. The study would be a collaborative effort between the CIBMTR and the EBMT.

The CIBMTR identified a total of 487 patients transplanted for HLH between 2004 and 2014 (80 sibling, 25 other related, and 382 unrelated donor HCT). Many attendees voiced their opinions that this study would have strong scientific impact. One question was whether the data that exists on HLH includes whether the disease is acquired or genetic in nature. Dr. Baker responded that there is no correlation between this and adverse outcomes, so this would not impact findings of the study. Another attendee asked about the follow-up of the patients, since this type of study has never been done before and there is thus no standard follow-up to adhere to. Dr. Baker responded that there is an approximately 5 year median follow-up for the CIBMTR patients.

It was asked whether there is sufficient CNS data available for the purposes of the study, as this would be important. Dr. Baker and Dr. Eapen responded that there is CNS data collected on the CIBMTR HLH disease-specific form. Dr. Baker explained that the CIBMTR disease-specific data in general for HLH is fairly complete for the purposes of this study. There will need to be collection of some supplemental data, particularly for the EBMT cases, but a grant was submitted to the UK Histiocyte Association, and the same will be done in the US. The US cases will require limited supplemental data collection.

A few attendees asked whether it would be worth considering a comparison to a non-transplant control group or a transplant control group with a different disease. Dr. Baker and others responded that there isn't any such group that would be comparable enough for this to be feasible. One attendee pointed out that the bulk of the CIBMTR cases across all donor types (>70%) were aged 0-9, and suggested considering restricting the study population to this young age group.

The proposal was approved to proceed to protocol development.

6. ***Dropped proposed studies***

a. **PROP 1610-04** Trends and Risk Factors for Transplant-Related Mortality Following Allogeneic Hematopoietic Cell Transplantation in Children and Adolescents with Non-Malignant Disorders *Dropped due to overlap with study NM16-02*

b. **PROP 1610-11** Efficacy and safety of hematopoietic cell transplantation for pure red cell aplasia *Dropped due to feasibility*

c. **PROP 1610-15** Outcomes of allogeneic hematopoietic stem cell transplant in patients with Diamond-Blackfan anemia *Dropped due to overlap with previously published DBA papers*

d. **PROP 1611-23** Outcomes of upfront versus salvage allogeneic hematopoietic stem cell transplant in patients with aplastic anemia *Dropped due to feasibility*

e. **PROP 1611-44** Clinical outcomes of allogeneic stem cell transplantation in patients with Diamond-Blackfan Anemia utilizing sibling or alternative donor sources *Dropped due to overlap with previously published DBA papers*

f. **PROP 1611-88** Impact of age on acute and chronic graft-versus-host disease in children receiving HLA-identical sibling bone marrow transplant for sickle cell disease: a joint CIBMTR/Eurocord-Pediatric Working Party of EBMT proposal *Dropped due to overlap with NM14-04*

g. **PROP 1611-96** Determining the utility and safety of Hydroxyurea as a prophase in stem cell transplantation for patients with sickle cell disease *Dropped due to feasibility*

h. **PROP 1611-140** Can stem cell transplantation improve outcomes in malignancy – associated hemophagocytic lymphohistiocytosis at first remission? *Dropped due to feasibility*

i. **PROP 1611-144** Hematopoietic stem cell transplant outcomes comparing severe aplastic anemia to paroxysmal nocturnal hemoglobinuria clone-aplastic anemia *Dropped due to feasibility*

j. **PROP 1611-160** Analysis of clinical outcomes with post-transplant cyclophosphamide in non-malignant disorders *Dropped due to feasibility*

7. Other Business

Adjourned at 4:17pm.

Working Committee Overview Plan for 2017 - 2018

a. **AA13-01** Correlation of levels of donor cell chimerism after alloHCT for hemoglobinopathy (A Abraham). Submit manuscript June 2017.
Statistical Hours allocated- Through June 2017: 30; To completion: 30

b. **AA13-02** Malignancies in patients with FA (J Wagner). Submit manuscript by September 2017.
Statistical Hours allocated- Through June 2017: 200; to completion: 200

c. **ID12-01** Allo HCT for CID/CVID (G Cuvelier/G Guilcher/N Wright). Manuscript preparation by June 2017, manuscript submission by June 2018.
Statistical Hours allocated- Through June 2017: 80; July 2017-June 2018: 70; To completion: 150

d. **ID13-01** HCT for Congenital Neutropenia/Kostmann Agranulocytosis (J Connelly). Completed date file preparation of transplant data and data integration with Congenital Neutropenia Registry. Exploring the possibility of joining with German registry data. Manuscript preparation by June 2018.
Statistical Hours allocated- Through June 2017: 10; July 2017-June 2018: 140; To completion: 150

e. **NM14-01** An investigation of the long term neurological outcomes of Hematopoietic Stem Cell Transplant (HCT) in boys with X-linked Adrenoleukodystrophy (X-ALD). Discussion among committee leadership required.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 0; To completion: 200

f. **NM14-02** Allo HCT for Shwachman Diamond Syndrome (K Myers). Date file preparation by June 2017, manuscript preparation by June 2018.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 120; To completion: 190

g. **NM15-01** Outcome of allogeneic Hematopoietic Cell Transplant (HCT) in Erythropoietic Porphyria (A Saad). Manuscript submission by September 2017.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 30; To completion: 30

h. **NM16-01** Combined EBMT/CIBMTR retrospective study of allogeneic stem cell transplant outcomes in older patients (age > 50 years) with severe aplastic anemia. (C Rice/J Marsh/V Potter) Manuscript preparation by June 2017, manuscript submission by June 2018.
Statistical Hours allocated- Through June 2017: 80; July 2017-June 2018: 70; To completion: 150

i. **NM16-02** Allogeneic transplantation for primary immune deficiencies: Current patterns of practice and change over the past 10 years. (R Marsh) Manuscript preparation by June 2017, manuscript submission by June 2018.
Statistical Hours allocated- Through June 2017: 50; July 2017-June 2018: 70; To completion: 120

j. **NM16-03** Results of transplants from genetically-identical twin donors in persons with aplastic anaemia. (RP Gale) Analysis by June 2018.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 160; To completion: 310

k. **NM16-04** The effect of conditioning regimen on clinical outcomes of allogeneic transplantation in severe aplastic anemia. (N Bejanyan/N Kekre/D Weisdorf/J Antin) Analysis by June 2018.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 70; To completion: 180

l. **NM17-01** Late effects after hematopoietic stem cell transplantation in patients with HLH (A Horne/KS Baker/K Beutel). Protocol development and preparation of study file by June 2018.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 30; To completion: 310

m. **NM17-02** Evaluation of the impact of changing clinical profile, transplant conditioning regimens and stem cell source on clinical outcome in patients with Thalassemia major (V Mathews/C Li/S Hongeng). Protocol development and preparation of study file by June 2018.
Statistical Hours allocated- Through June 2017: 0; July 2017-June 2018: 60; To completion: 310

Oversight Assignments for Working Committee Leadership (March 2017)

Neena Kapoor	**AA13-01**	Correlation of levels of donor cell chimerism with hemoglobinopathy symptoms following allogeneic hematopoietic cell transplantation
Vikram Mathews	**AA13-02**	Malignancies in patients with fanconi anemia
Vikram Mathews	**ID12-01**	Allogeneic hematopoietic cell transplantation for combined immunodeficiency and common variable immunodeficiency
Neena Kapoor	**ID13-01**	Second and subsequent hematopoietic cell transplants for congenital neutropenia/kostmann agranulocytosis
Jaap Boelens	**NM14-01**	An investigation of the long term neurological outcomes of Hematopoietic Stem Cell Transplant (HCT) in boys with X-linked Adrenoleukodystrophy
Paolo Anderlini	**NM14-02**	Outcomes of allogeneic hematopoietic cell transplant in patients with Shwachman diamond syndrome
Jaap Boelens	**NM15-01**	Outcome of Allogeneic Hematopoietic Cell Transplant in Erythropoietic Porphyria
Paolo Anderlini	**NM16-01**	Combined EBMT/CIBMTR retrospective study of allogeneic stem cell transplant outcomes in older patients (age > 50 years) with severe aplastic anemia
Neena Kapoor	**NM16-02**	Allogeneic Hematopoietic Cell Transplantation for Primary Immune Deficiencies: Current Patterns of Practice and Change over the last 10 years
Vikram Mathews	**NM16-03**	Results of transplants from genetically-identical twin donors in persons with aplastic anaemia
Jaap Boelens	**NM16-04**	The effect of Conditioning Regimen on Clinical Outcomes of Allogeneic Hematopoietic Cell Transplantation in Severe Aplastic Anemia
Jaap Boelens	**NM17-01**	Late effects after hematopoietic stem cell transplantation in patients with HLH
Vikram Mathews	**NM17-02**	Evaluation of the impact of changing clinical profile, conditioning regimens and stem cell source on clinical outcome in patients with Thalassemia major