Fall 2015

October 6, 2015

Sheraton Hotel

Dallas, Texas
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This is a privileged communication of the PBMTC and not for publication or reference.
October 2015
PIDTC 6901: *A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders v 2.0*

PIDTC 6903: *Analysis of Patients Treated for Chronic Granulomatous Disease Since January 1, 1995 v1.0*

PIDTC 6904: *Analysis of Patients Treated for Wiskott-Aldrich Syndrome Since January 1, 1990*

**Nursing Discipline Committee**

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**Blood and Marrow Transplant Clinical Trials Network (BMT CTN)**

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<thead>
<tr>
<th>Listing of Studies and Proposals</th>
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<tr>
<td>Individual Study Reports</td>
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<td>CTN 1202</td>
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<td>CTN 1204</td>
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<td>CTN 1301</td>
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**Meetings**

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<tr>
<th>Upcoming Meetings</th>
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<tr>
<th>Sheraton Dallas Reunion Floor Plans</th>
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# PBMTC Fall Meeting Schedule

**Tuesday, October 6, 2015**

<table>
<thead>
<tr>
<th>Time</th>
<th>Meeting</th>
<th>Location*</th>
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<tbody>
<tr>
<td>9:45 am – 10:45 am</td>
<td>GVHD Strategy Group Session</td>
<td>Austin 2-3</td>
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<tr>
<td>10:45 am – 12:00 pm</td>
<td>Oncology Strategy Group Session</td>
<td>Austin 2-3</td>
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<tr>
<td>12:00 pm – 1:45 pm</td>
<td>Lunch Symposium (CME)</td>
<td>Austin 2-3</td>
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<tr>
<td></td>
<td>• Food served at 12:00pm;</td>
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<td></td>
<td>• CME program starts at 12:15pm</td>
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<tr>
<td>1:45 pm – 2:45 pm</td>
<td>Cellular Therapeutics Strategy Group Session</td>
<td>Austin 2-3</td>
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<tr>
<td>2:45 pm – 3:30 pm</td>
<td>Supportive Care Strategy Group Session</td>
<td>Austin 2-3</td>
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<tr>
<td>3:30 pm – 4:00 pm</td>
<td>Late Effects Strategy Group Session</td>
<td>Austin 2-3</td>
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<tr>
<td>4:15 pm – 5:15 pm</td>
<td>Non-Malignant Strategy Group Session</td>
<td>Austin 2-3</td>
</tr>
<tr>
<td>5:15 pm – 5:30 pm</td>
<td>Annual General Meeting</td>
<td>Austin 2-3</td>
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* Meeting rooms are subject to change; please check with the PBMTC Registration Desk for updates upon your arrival.
**PBMTC Educational Symposium Agenda**  
**Tuesday, October 6, 2015**  
**12:15 pm – 1:45 pm**

<table>
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<tr>
<th>Time</th>
<th>Topic</th>
<th>Speaker</th>
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<tbody>
<tr>
<td>12:15 – 12:20 pm</td>
<td>Welcome and Introduction</td>
<td>Michael Nieder, MD</td>
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| 12:20 – 1:00 pm | Lessons Learned from opening a CAR-T Clinical Trial                 | John Levine, MD  
Clinical Director, Pediatric Blood and Marrow Transplantation Program  
University of Michigan  
Ann Arbor, MI |
| 1:00 – 1:45 pm  | Implementing Standardization of chronic GVH Evaluation, Grading and Reporting at your Center | Carrie Kitko, MD  
Medical Director, Pediatric Stem Cell Transplantation Program  
Vanderbilt University  
Nashville, TN |

ACCME Sponsor: Cincinnati Children’s Hospital

**Learning Objectives:**

1. To identify the logistical challenges in running a CAR-T protocol  
2. To prepare for managing CAR-T recipients safely  
3. To recognize the differences in CAR-T patient populations  
4. To identify the significant changes to the Diagnosis, Staging and Response criteria based on the 2014 National Institutes of Health Consensus Development Project on Criteria for Clinical Trials in Chronic Graft-versus-Host Disease  
5. To recognize how standardization in clinical documentation can improve data collection  
6. To discuss barriers to performing the full chronic GVHD assessment in a busy clinical setting
## Annual General Meeting

**Tuesday, October 6, 2015**

5:15 pm – 6:00 pm

<table>
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<tr>
<th>Report</th>
<th>Time</th>
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<tbody>
<tr>
<td>Chairman’s Report</td>
<td>5:15 pm – 5:30 pm</td>
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SUPPORTERS

The Pediatric Blood and Marrow Transplant Consortium would like to thank these companies for their generous financial support of the consortium.

St. Baldrick’s
FOUNDATION
Conquer Childhood Cancers
PBMTC Chair’s Report

The past year has been focused on expanding educational collaboration along with obtaining significant new funding for PBMTC Investigator led trials. We have also worked diligently opening and for the initiation of new protocols in both the BMT CTN, PIDTC and the PBMTC. We continue to expand the number of major protocol initiatives we can do by partnering with institutions and industry.

St. Baldrick’s Foundation Core Grant: Ongoing Study Accrual and New Study Development

This year we submitted a formal core consortium grant to the St. Baldrick’s Foundation to continue the support that was established 5 years ago. The St. Baldrick’s Foundation continued their significant support of the PBMTC, awarding us another $500K this year and we expect to receive $500,000 a year for 5 years total to fund innovative trials. The St. Baldrick’s Foundation has been steadfast in their support of the PBMTC and we urge all of our centers to participate in and support St. Baldrick’s fundraising efforts.

Call for Proposals

This year the PBMTC put out two RFA’s, one using the funds granted to us by the St. Baldrick’s Foundation and the other was our first RFA using funds that have been raised by the Pediatric Blood and Marrow Transplant Foundation (PBMTF). We are planning to open the PBMTF RFA as often as funds will allow, if not yearly, every couple of years. Our main goal with the PBMTF RFA is to fund transplant related science that will enhance development of PBMTC/COG and BMT CTN protocols.

St. Baldrick’s Studies Open and Upcoming

Moxetumomab Pasudotox to Decrease Pre-HCT MRD and Reduce Relapse Post HCT. Unfortunately this study has been recently closed due to unexpected adverse events.

Natural History and Biology of Long-Term Late Effects Following Hematopoietic Cell Transplant for Childhood Hematologic Malignancies. LTE1401, is open and accruing at 14 centers. 21 patients have been accrued as of September of this year. The study examines the hypothesis that survivors of pediatric HCT are at risk for late organ toxicity and they will have identifiable biomarkers present within the first two years following HCT which will be predictive for late adverse outcomes allowing for early identification of patients at risk. If you haven’t opened this trial already, please consider it – it is relatively easy for a center to open and run and vitally important to our effort.
Cooperative Trials

Abatacept to Decrease GVHD

We continue to partner with Leslie Kean, MD PhD, at Emory, to run the Abatacept Combined with a Calcineurin Inhibitor and Methotrexate for Graft Versus Host Disease Prophylaxis trial (GVH 1201). Abatacept inhibits T-cell costimulation through the CTLA-4 pathway and pilot studies have shown improved GVHD outcomes without significant toxicity. 81 patients have been enrolled at 12 participating institutions as of September of this year.

Chronic GVHD Biomarkers

The PBMTC has partnered with Kirk Schultz and Geoff Cuvelier in a major effort to define biomarkers that allow prediction of cGVHD. The study is funded through the Canadian Institute of Health Research, as part of a larger grant entitled "ABLE - Applied Biomarkers of Late Effects of Childhood Cancer" for which Kirk is the PI. The study, GVH1202, is opened and accruing. 145 patients have been enrolled at 26 centers, as of September of this year.

Continued PBMTC Initiative in the BMT CTN

The PBMTC is currently leading the development of a protocol for optimizing cord blood and haploidentical aplastic anemia transplantation (CHAMP). The BMT CTN 1502 protocol is currently being finalized and is expected to be released to sites in the next 3 to 6 months.

Scientific Partnerships

BMT CTN

The PBMTC continues to be a successful core center of the BMT CTN. Since becoming part of the BMT CTN in 2005, we have enrolled 358 children in ten clinical trials making PBMTC the third highest accruing core center. We are currently participating in 3 studies:

- 1202 (GVHD biomarkers): The PBMTC has enrolled 135 patients so far. PBMTC sites are urged to continue enrolling patients until enrollment is closed.
- 1204 (RICHI): This trial is expected to change our standard of care for HLH; any sites that wish to participate should contact Laura Hancock.
- 1301 (Calcineurin Inhibitor-Free Interventions for Prevention of Graft-versus-Host Disease): This is a very important study and PBMTC participation is not as high
as it should be, please contact Laura Hancock if your site would like to open this trial.

Our performance in laboratory and data compliance has continued to improve. We continue to give “Welcome Packets” to centers to facilitate the review of eligibility criteria and remember required study observations.

**PIDTC**

We continue to enroll very high numbers of SCID patients on PIDTC trials and have assisted in development of concepts aimed at developing therapeutic trials. We were able to add 10 PBMTC sites as participants this year to the PIDTC, associated with their grant renewal which is aimed at capturing all newborns with SCID diagnosed through state screening programs. Several important publications are coming forward from this group, and an upcoming clinical trial aimed at determining lowest doses of busulfan needed for selected patients with SCID is being planned.

**Late Effects Consensus NIH Conference**

After the success and popularity of the Late Effects conference that was held in 2011 and the generous funding from the St. Baldrick’s Foundation and Jeff Gordon Foundation, the PBMTC is planning another Late Effects Conference that will be held in Minneapolis, MD on May 10-11, 2015. This meeting will be the day before the PBMTC conference at ASPHO and will cover such topics as inherited bone marrow failure syndromes, hemoglobinopathies, and SCID. We urge everyone to attend this exciting conference!

**Educational Partnerships**

**ASBMT – PSIG**

The Pediatric Special Interest Group (PSIG) as a joint endeavor between the PBMTC and ASBMT has now supported 9 highly successful sessions and another great session is planned for Honolulu, Hawaii in February 2016. Dr. Christopher Dvorak chaired the sessions in 2015 in San Diego, and Dr. Peter Shaw has lead the organization efforts of the upcoming meeting in February 2016. The Tandem PSIG meetings are considered to be the finest pediatric blood and marrow transplant educational/scientific forum in the world.

**ASPHO Joint Educational Conference 2015**

The PBMTC continues to hold a joint educational PBMTC/ASPHO Session in conjunction with the ASPHO Meeting every spring. The 2015 meeting was held in
Phoenix, Arizona and led by Dr. Eneida Nemecek. Speakers included experts in several key topics. Several outstanding young investigators presented abstracts and 14 travel awards were given out. The meeting was a great opportunity heme/onc doctors and nurses to update themselves on the latest in BMT by extending their ASPHO trip by a single day. We were awarded an R13 grant from the NCI and had significant foundational sponsorship. The meeting reviews were once again outstanding. In May of 2016, David Jacobsohn is leading another outstanding PBMTC/ASPHO conference planned for the ASPHO meeting in Minneapolis. Our desire is that these joint conferences will continue to improve communication with our Hematology/Oncology colleagues about major advances in BMT and that this will continue as a major forum moving into the future.

**PBMTC Leadership/Operations**

A major change occurred for the PBMTC Operations Center this year as Laura Hancock and the Operations Center moved from the COG Operations Center in Monrovia to Children’s Hospital of Los Angeles. This change included moving contracting with PBMTC centers from CHOP to CHLA, to be done as a stand-alone PBMTC site contract that will now allow us the freedom to contract for other PBMTC studies, not just BMT CTN and PIDTC. It has taken a lot of work to make this transition happen and we are finally getting to a place where things are running smoothly.

**The PBMTC Website**

Dr. Niketa Shah continues to work to make sure the PBMTC website is always up to date and a good source of information for our members. There are constant initiatives to make the website more dynamic. This year we also partnered with a group called Sosido. Sosido provides a weekly newsletter for our members that include current publications, news and events for the PBMTC. Sosido also includes a forum for PBMTC members to ask questions in the PBMTC community, or provide an answer. The website can be found at [www.pbmtc.org](http://www.pbmtc.org).

**Conclusion**

The PBMTC continues to move forward with key initiatives for studies that answer important questions in HCT of malignant and non-malignant disorders. In addition, we continue to organize high quality educational meetings (CME session at PBMTC, PSIG at Tandem, joint ASPHO meeting, etc.). We have continued to promote the educational and scientific efforts of BMT nursing, and we have provided a forum for the development of multi-institutional efforts to improve pediatric BMT Pharmacy. Finally, we continue to provide an opportunity to establish relationships amongst pediatric BMT groups by promoting scientific collaboration in a wide variety of areas.
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PBMTCC Website
www.pbmtc.org

Please contact Laura Hancock for the User ID and Password to access the website.
# Executive Committee Members – 2015

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<thead>
<tr>
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<th>Name</th>
<th>Institution/Address</th>
<th>Contact Information</th>
</tr>
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<tr>
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<tr>
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<td>A. I. Du Pont Hospital for Children&lt;br&gt;1600 Rockland Road&lt;br&gt;Wilmington, DE 19803</td>
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<tr>
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This is a privileged communication of the PBMTC and not for publication or reference. October 2015
### Steering Committee Members – 2015

#### Non-Malignant Diseases Strategy Group

**Chair:**
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#### Graft vs. Host Disease Strategy Group

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Organizational Chart – Fall 2015

PEDiATRIC BLOOD AND MARROW TRANSPLaNT CONSORTIUm (PBMTC)

Executive Committee
Chair - Michael Pulsipher
Vice Chair - Michael Nieder, Secretary Treasurer - Gregory Yanik
Members-at-Large - Ann Woolfrey, E. Anders Kolb, Roger Giller
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<td><a href="mailto:dozeran@childrenscentralcal.org">dozeran@childrenscentralcal.org</a></td>
</tr>
<tr>
<td>Children's Hospitals and Clinics of Minnesota</td>
<td>Richards</td>
<td>Michael K.</td>
<td><a href="mailto:michael.richards@childrensmn.org">michael.richards@childrensmn.org</a></td>
</tr>
<tr>
<td>Colombian Childhood Cancer Parents Organization</td>
<td>Mesa</td>
<td>Mauricio</td>
<td><a href="mailto:mesamauriciomd@yahoo.com">mesamauriciomd@yahoo.com</a></td>
</tr>
<tr>
<td>Floridal Hospital for Children</td>
<td>Shook</td>
<td>David</td>
<td><a href="mailto:david.shook.md@flhosp.org">david.shook.md@flhosp.org</a></td>
</tr>
<tr>
<td>Hasbro Children's Hospital</td>
<td>Grodman</td>
<td>Howard</td>
<td><a href="mailto:hgrodman@lifespan.org">hgrodman@lifespan.org</a></td>
</tr>
<tr>
<td>Helen DeVos Children’s Hospital</td>
<td>Mageed</td>
<td>Aly</td>
<td><a href="mailto:aly.mageed@spectrum-health.org">aly.mageed@spectrum-health.org</a></td>
</tr>
<tr>
<td>Hospital de Clinicas de Porto Alegre</td>
<td>Gregianin</td>
<td>Lauro</td>
<td><a href="mailto:lgregianin@hcpa.ufrgs.br">lgregianin@hcpa.ufrgs.br</a></td>
</tr>
<tr>
<td>Instituto de Oncologia Pediatrica</td>
<td>Seber</td>
<td>Adriana</td>
<td><a href="mailto:adrianaseber@graacc.org.br">adrianaseber@graacc.org.br</a></td>
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<tr>
<td>Maine Children's Cancer Program</td>
<td>Larson</td>
<td>Eric</td>
<td><a href="mailto:larsee1@mmc.org">larsee1@mmc.org</a></td>
</tr>
<tr>
<td>MD Anderson Orlando/Arnold Palmer Hospital for Children</td>
<td>Kelly</td>
<td>Susan</td>
<td><a href="mailto:susan.kelly@orlandohealth.com">susan.kelly@orlandohealth.com</a></td>
</tr>
<tr>
<td>Medical City Children's Hospital</td>
<td>Weinthal</td>
<td>Joel</td>
<td><a href="mailto:Joel.Weinthal@usoncology.com">Joel.Weinthal@usoncology.com</a></td>
</tr>
<tr>
<td>Medical College of Georgia</td>
<td>McDonough</td>
<td>Colleen</td>
<td><a href="mailto:cmcdonough@mail.mcg.edu">cmcdonough@mail.mcg.edu</a></td>
</tr>
<tr>
<td>National Cancer Institute</td>
<td>Mackall</td>
<td>Crystal</td>
<td><a href="mailto:mackallc@mail.nih.gov">mackallc@mail.nih.gov</a></td>
</tr>
<tr>
<td>New York University</td>
<td>Gardner</td>
<td>Sharon</td>
<td><a href="mailto:sharon.gardner@nyumc.org">sharon.gardner@nyumc.org</a></td>
</tr>
<tr>
<td>Ramathibodi Hospital</td>
<td>Hongeng</td>
<td>Suradej</td>
<td><a href="mailto:rashe@mahidol.ac.th">rashe@mahidol.ac.th</a></td>
</tr>
<tr>
<td>St. Christopher's Hospital for Children</td>
<td>Rozans</td>
<td>Marta</td>
<td><a href="mailto:mrozans@yahoo.com">mrozans@yahoo.com</a></td>
</tr>
<tr>
<td>Stollery Children's Hospital</td>
<td>Desai</td>
<td>Sunil</td>
<td><a href="mailto:Sunil.Desai@capitalhealth.ca">Sunil.Desai@capitalhealth.ca</a></td>
</tr>
<tr>
<td>The Children's Hospital at TriStar Centennial</td>
<td>Frangoul</td>
<td>Haydar A.</td>
<td><a href="mailto:Haydar.Frangoul@hcahealthcare.com">Haydar.Frangoul@hcahealthcare.com</a></td>
</tr>
<tr>
<td>Tulane University/Tulane University Hospital &amp; Clinic</td>
<td>Chavan</td>
<td>Rishikesh</td>
<td><a href="mailto:rchavan@tulane.edu">rchavan@tulane.edu</a></td>
</tr>
<tr>
<td>U of Hawaii/Kapiolani Med Ctr for Women &amp; Children</td>
<td>Wada</td>
<td>Randal K.</td>
<td><a href="mailto:randalw@hawaii.edu">randalw@hawaii.edu</a></td>
</tr>
<tr>
<td>University Hospital, Brno</td>
<td>Sterba</td>
<td>Jaroslav</td>
<td><a href="mailto:jsterb@fnbrno.cz">jsterb@fnbrno.cz</a></td>
</tr>
<tr>
<td>University of Arkansas for Medical Sciences</td>
<td>Becton</td>
<td>David</td>
<td><a href="mailto:bectondavid@uams.edu">bectondavid@uams.edu</a></td>
</tr>
<tr>
<td>University of California-Davis School of Medicine</td>
<td>Taylor</td>
<td>Douglas</td>
<td><a href="mailto:douglas.taylor@ucdmc.ucdavis.edu">douglas.taylor@ucdmc.ucdavis.edu</a></td>
</tr>
<tr>
<td>University of Kentucky Markey Cancer Center</td>
<td>Howard</td>
<td>Dianna</td>
<td><a href="mailto:dshowa0@uky.edu">dshowa0@uky.edu</a></td>
</tr>
<tr>
<td>University of Maryland</td>
<td>Munchel</td>
<td>Ashley</td>
<td><a href="mailto:amunchel@peds.umaryland.edu">amunchel@peds.umaryland.edu</a></td>
</tr>
<tr>
<td>Women's &amp; Children's Hospital</td>
<td>Tapp</td>
<td>Heather</td>
<td><a href="mailto:heather.tapp@health.sa.gov.au">heather.tapp@health.sa.gov.au</a></td>
</tr>
<tr>
<td>Yale University</td>
<td>Chirnomas</td>
<td>Deborah</td>
<td><a href="mailto:deborah.chirnomas@yale.edu">deborah.chirnomas@yale.edu</a></td>
</tr>
</tbody>
</table>

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October 2015
Strategy Groups

Non-Malignant Diseases

Graft vs. Host Disease

Oncology

Late Effects

Cellular Therapeutics

Supportive Care
Strategy Group Sign Up Sheet

If you would like to participate as a member of one of the PBMTC Strategy Groups, please fill out the following information and return the completed form to Laura Hancock by person, email, or mail.

Laura Hancock  
Program Administrator  
PBMTC Operations Center  
4650 Sunset Blvd MS #54  
Los Angeles, CA 90027

Name: __________________________________________
Institution: ______________________________________
Address: _________________________________________
City: ___________________________ State: _______ Zip Code: _____________
Phone: ___________________________ Fax: ___________________________
Email: _______________________________________________

Committee you wish to join:

☐ Cellular Therapeutics ☐ GVHD ☐ Non-Malignant

☐ Oncologic Disorders ☐ Supportive Care ☐ Late Effects
Graft vs. Host Disease Strategy Group

Chair

David Jacobsohn, MD
Children’s National Medical Center-DC

Vice Chair

Carrie Kitko, MD
Vanderbilt University
# AGENDA

<table>
<thead>
<tr>
<th>Topic</th>
<th>Presenter</th>
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<tbody>
<tr>
<td><strong>ONGOING STUDIES</strong></td>
<td></td>
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<tr>
<td>Abatacept Combined with Cyclosporine and Methotrexate for Graft Versus Host Disease Prophylaxis: A Randomized Controlled Trial (PBMTC GVH 1201)</td>
<td>Leslie Kean, MD</td>
</tr>
<tr>
<td>A Randomized, Phase II, Multi-Center, Open Label, Study Comparing Sirolimus to Prednisone in Patients with Minnesota Standard Risk, Ann Arbor 1/2 Confirmed Acute Graft-Versus-Host Disease (BMT CTN 1501)</td>
<td>John Levine, MD</td>
</tr>
<tr>
<td>A Randomized, Multi-Center, Phase III Trial of Calcineurin Inhibitor-Free Interventions for Prevention of Graft-versus Host-Disease (BMT CTN 1301)</td>
<td>Carrie Kitko, MD</td>
</tr>
<tr>
<td>GVH 1201: Applied Biomarkers in Late Effects of Childhood Cancer Study Group</td>
<td>Geoff Cuvelier</td>
</tr>
</tbody>
</table>
Graft vs. Host Disease (GVHD) Protocols

<table>
<thead>
<tr>
<th>Study / Proposal Number</th>
<th>Study Status</th>
<th>Study Title</th>
<th>Principal Investigator</th>
</tr>
</thead>
<tbody>
<tr>
<td>BMT CTN 1202</td>
<td>Open</td>
<td>Prospective Multi-Center Cohort for the Evaluation of Biomarkers Predicting Risk of Complications and Mortality Following Allogeneic HCT</td>
<td>John Levine</td>
</tr>
<tr>
<td>GVH 1202</td>
<td>Open</td>
<td>Applied Biomarkers in Late Effects of Childhood Cancer Study Group</td>
<td>Geoff Cuvelier</td>
</tr>
<tr>
<td>BMT CTN 1301</td>
<td>Open</td>
<td>A Randomized, Multi-Center, Phase III Trial of Calcineurin Inhibitor-Free Interventions for Prevention of Graft-versus Host-Disease</td>
<td>Luznik, Perales, Pasquini</td>
</tr>
<tr>
<td>BMT CTN 1501</td>
<td>Not Open</td>
<td>A randomized, Phase II Multi-Center, Open Label, Study Comparing Sirolimus to Prednisone in Patients with Minnesota Standard Risk, Ann Arbor 1/2 Confirmed Acute Graft-Versus-Host Disease</td>
<td>MacMillan</td>
</tr>
</tbody>
</table>
Open Trial

GVH 1202: Prospective Multi-Center Cohort for the Evaluation of Biomarkers Predicting Risk of Complications and Mortality Following Allogeneic HCT v2.0

Study Chairperson: Geoff Cuvelier, MD
Protocol Coordinator: Anat Halevy

Study Objectives:

- To determine in HSCT recipients and donors whether “predictive” cGVHD biomarkers exist in the early peri-transplant time period, including before the start of the conditioning regimen and at day +100 after HSCT
- To determine the relationship between these early “predictive” cGVHD biomarkers and the later development of cGVHD
- To determine and validate whether “predictive” cGVHD biomarkers (either individually or in combination) present before the onset of cGVHD are able to predict a subset of pediatric patients at highest risk for the development of cGVHD in the future.
- To correlate early cGVHD biomarker levels (in patients who do and do not go on to later develop cGVHD), with cGVHD biomarker levels seen at 6 months and 12 months post-transplant in patients not developing cGVHD, and with cGVHD biomarkers present at the onset (diagnosis) of cGVHD.

Study Overview:

- Eligible HSCT recipients are enrolled before the start of the conditioning regimen and followed prospectively for the development of cGVHD.
- It is anticipated that most subjects will not develop cGVHD in the first year post-transplant. These subjects form the control group.
- It is anticipated that approximately 16% of subjects will develop cGVHD. These subjects form the experimental group.
- Collection of clinical data occurs at specified time points post-transplant, through combinations of (1) the Main Case Report Form (all subjects), and (2) the cGVHD case report form – initial onset, which is only completed for patients developing cGVHD in the first year post-transplant (completed at the time of initial cGVHD diagnosis / onset).
- Blood samples are sent to the study laboratory (Dr. Kirk Schultz, Child and Family Research Institute, University of British Columbia, Vancouver, BC, Canada) for all HSCT recipients (regardless of whether the subject does or does not develop cGVHD) before the start of the conditioning regimen (pre-conditioning) and at day +100 (+/- 14 days) post-transplant.

- Subjects NOT developing cGVHD in the first year post-transplant: additional blood samples are sent at 6 months (+/- 1 month) and 12 months (+/- 1 month) post-transplant.

- Subjects developing cGVHD in the first year post-transplant: an additional blood sample is sent at the time of cGVHD onset / diagnosis and a cGVHD case report form is completed at that time. Further blood samples after the cGVHD blood sample are drawn are not required (i.e. do NOT need to send blood at 6 months and 12 months post transplant).
Oncology Strategy Group

Chair
Terry Fry, MD
Mark O. Hatfield-Warren Grant Magnuson
Clinical Research Center

Vice Chair
Susan Kelly, MD
Arnold Palmer Hospital for Children
# ONCOLOGY STRATEGY GROUP SESSION

**October 6, 2015**  
**10:45 am – 12:00 pm**

**CHAIR:** Terry Fry, MD  
**VICE CHAIR:** Susan Kelly, MD

## AGENDA

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<thead>
<tr>
<th>Topic</th>
<th>Presenter</th>
<th>Time</th>
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<tbody>
<tr>
<td>Brief updates, open protocols:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>A Phase II Study of Treosulfan/Fludarabine/Low Dose Total Body Irradiation as a Preparative Regimen for Children with AML/MDS Undergoing Allogeneic Hematopoietic Cell Transplantation</td>
<td>E. Nemecek</td>
<td>5 minutes</td>
</tr>
<tr>
<td>PBMTC ONC1001, CIBMTR 09-MRD: The Role of Minimal Residual Disease Testing before and after Hematopoietic Cell Transplantation for Pediatric Acute Myeloid Leukemia</td>
<td>D. Jacobsohn</td>
<td>5 minutes</td>
</tr>
<tr>
<td>Protocols Under Development:</td>
<td></td>
<td></td>
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<tr>
<td>12-MOXE: A Phase II Study of the Anti-CD22 Recombinant Immunotoxin Moxetumomab Pasudotox (CAT-8015, HA22) in Children with B-lineage Acute Lymphoblastic Leukemia and Minimal Residual Disease Prior to Allogeneic Hematopoietic Stem Cell Transplantation</td>
<td>N. Shah</td>
<td>20 minutes</td>
</tr>
<tr>
<td>Myeloablative Conditioning and Transplantation of Partially HLA-mismatched (Haploidentical) T cell-replete Bone Marrow for Patients with High Risk Leukemia</td>
<td>H. Symons</td>
<td>20 minutes</td>
</tr>
<tr>
<td>Concepts:</td>
<td></td>
<td></td>
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<tr>
<td>AML post transplant intervention concept, PBMTC/COG AML Committee collaborative effort</td>
<td>T. Fry</td>
<td>20 minutes</td>
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October 2015
## Oncology Protocols

<table>
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<tr>
<th>Study/Proposal Number</th>
<th>Study Status</th>
<th>Study</th>
<th>Principal Investigator(s)</th>
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<tbody>
<tr>
<td>ONC032</td>
<td>Closed to Accrual</td>
<td>High Dose Temozolomide, Thiotepa and Carboplatin with Autologous Stem Cell Rescue Followed by Continuation Therapy with 13-cis-retonic Acid in Patients with Recurrent Malignant Brain Tumors.</td>
<td>Sharon Gardner</td>
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<tr>
<td>ASCT0431/ONC051</td>
<td>Closed to Accrual</td>
<td>A Randomized Trial of Sirolimus-Based Graft versus Host Disease (GVHD) Prophylaxis after Hematopoietic Stem Cell Transplantation (HSCT) in Selected Patients with CR1 and CR2 ALL</td>
<td>Michael Pulsipher</td>
</tr>
<tr>
<td>ONC1001</td>
<td>Closed to Accrual</td>
<td>The Role of Minimal Residual Disease Testing before and after Hematopoietic Cell Transplantation for Pediatric Acute Myeloid Leukemia</td>
<td>David Jacobsohn</td>
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<tr>
<td>ONC1101</td>
<td>Closed to Accrual</td>
<td>A Phase II Study of Treosulfan/Fludarabine/Low Dose Total Body Irradiation as a Preparative Regimen for Children with AML/MDS Undergoing Allogeneic Hematopoietic Cell Transplantation</td>
<td>Eneida Nemecek Colleen Delaney</td>
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<tr>
<td></td>
<td>Open</td>
<td>Myeloablative Conditioning and Transplantation of Partially HLA-mismatched (Haploidentical) T cell-replete Bone Marrow for Patients with High Risk Leukemia</td>
<td>Heather Symons</td>
</tr>
</tbody>
</table>
Cellular Therapeutics Strategy Group

Chair
Maarten Egeler, MD
The Hospital for Sick Kids

Vice Chair
Dean Lee, MD, PhD
M.D. Anderson Cancer Center
CELLULAR THERAPEUTICS STRATEGY GROUP SESSION
October 6, 2015
1:45 pm – 2:45 pm

CHAIR: Maarten Egeler, MD       VICE CHAIR: Dean Lee, MD, PhD

AGENDA

<table>
<thead>
<tr>
<th>Topic</th>
<th>Presenter</th>
<th>Time</th>
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<tbody>
<tr>
<td>Novel Targeted Therapy for High Risk Pediatric Myeloid Leukemia</td>
<td>Mitch Cairo, MD</td>
<td>20 minutes</td>
</tr>
<tr>
<td>Update on haplotransplant with infusion of expanded NK cells for myeloid malignancies in adults in preparation for a pediatric trial</td>
<td>Dean Lee, MD, PhD</td>
<td>20 minutes</td>
</tr>
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</table>
Supportive Care Strategy Group

Chair

Chris Dvorak, MD
University of California at San Francisco

Vice Chair

Jeffrey Auletta, MD
Nationwide Children’s Hospital
# SUPPORTIVE CARE STRATEGY GROUP SESSION

**October 6, 2015**  
2:45 pm – 3:30 pm

**CHAIR:** Chris Dvorak, MD  
**VICE CHAIR:** Jeffrey Auletta, MD

## AGENDA

<table>
<thead>
<tr>
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<tbody>
<tr>
<td><strong>Lung Injury Studies:</strong></td>
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<tr>
<td>Parametric Response Marker Retrospective Study</td>
<td>Chris Dvorak, MD</td>
</tr>
<tr>
<td>Bronchoalveolar Lavage Deep Sequencing Prospective Study</td>
<td>Matt Zinter, MD</td>
</tr>
<tr>
<td><strong>PALISI Collaborations:</strong></td>
<td></td>
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<tr>
<td>PALISI Overview</td>
<td>Christy Duncan, MD</td>
</tr>
<tr>
<td>VOD Treatment Consensus Guidelines</td>
<td>Jeff Auletta, MD</td>
</tr>
<tr>
<td><strong>Upcoming Studies:</strong></td>
<td></td>
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<tr>
<td>Nutrition</td>
<td>Leslie Lehmann, MD</td>
</tr>
<tr>
<td>Iron Overload</td>
<td>Alexa Cheerva, MD</td>
</tr>
</tbody>
</table>
Late Effects
Strategy Group

Chair
K. Scott Baker, MD
Fred Hutchinson Cancer Research Center

Vice Chair
Christine Duncan, MD
Dana-Farber Cancer Institute
LATE EFFECTS STRATEGY GROUP SESSION  
October 6, 2015  
3:30 pm – 4:00 pm

**CHAIR:** K. Scott Baker, MD  
**VICE CHAIR:** Christine Duncan, MD

## AGENDA

<table>
<thead>
<tr>
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<th>Presenter</th>
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<tbody>
<tr>
<td>13-TLEC Prospective Late Effects Study</td>
<td>Christy Duncan, MD</td>
</tr>
<tr>
<td>Late Cardiac Outcomes Study</td>
<td>Christy Duncan, MD</td>
</tr>
<tr>
<td>Non-Malignant Disease Consensus Conference</td>
<td>Andrew, Dietz</td>
</tr>
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</table>

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October 2015
Non-Malignant Diseases Strategy Group

Chair
Lauri Burroughs, MD
Fred Hutchinson Cancer Research Center

Vice Chair
Julie Talano, MD
Medical College of Wisconsin
### AGENDA

<table>
<thead>
<tr>
<th>Topic</th>
<th>Presenter</th>
<th>Time</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Concept:</strong> Reduced Intensity Conditioning Haploidentical BMT with Post-Transplant Cyclophosphamide for Pediatric and Young Adult Patients with Non-Malignant Disorders</td>
<td>H. Symons</td>
<td>10 minutes</td>
</tr>
<tr>
<td><strong>New Protocol:</strong> Optimizing Cord Blood and Haploidentical Aplastic Anemia Transplantation (CHAMP) CTN 1502</td>
<td>A. Dietz</td>
<td>15 minutes</td>
</tr>
<tr>
<td>Overview of completed and current/upcoming BMT CTN trials of HCT for sickle cell disease</td>
<td>S. Shenoy</td>
<td>20 minutes</td>
</tr>
<tr>
<td>PIDTC: <strong>New protocol:</strong> A randomized trial of very low- and low-dose busulfan for infants with severe combined immunodeficiency (SCID): A phase II study by the Primary Immune Deficiency Treatment Consortium</td>
<td>M. Pulsipher</td>
<td>10 minutes</td>
</tr>
<tr>
<td>Update: CTN 1204: Reduced Intensity Conditioning for Children and Adults with Hemophagocytic Syndromes and Selected Primary Immune Deficiencies</td>
<td>M. Pulsipher</td>
<td>5 minutes</td>
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<tr>
<td>Study Number</td>
<td>Study Status</td>
<td>Study Title</td>
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<tr>
<td>BMT CTN 0301</td>
<td>Closed to accrual</td>
<td>A Phase I/II Multicenter Study of Fludarabine-Based Conditioning for Allogeneic Marrow Transplantation from HLA-Compatible unrelated Donors in Severe Aplastic Anemia</td>
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<tr>
<td>BMT CTN 0601</td>
<td>Closed to accrual</td>
<td>Unrelated Donor Hematopoietic Cell Transplantation for Children with Severe Sickle Cell Disease Using a Reduced Intensity Conditioning Regimen</td>
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<tr>
<td>BMT CTN 1204</td>
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<td>Reduced Intensity Conditioning for Children and Adults with Hemophagocytic Syndromes and Selected Primary Immune Deficiencies</td>
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<tr>
<td>PIDTC 6901</td>
<td>Open</td>
<td>A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders</td>
</tr>
<tr>
<td>PIDTC 6902</td>
<td>Open</td>
<td>A Retrospective and Cross-Sectional Analysis of Patients Treated for SCID Since January 1, 1968</td>
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<tr>
<td>PIDTC 6903</td>
<td>Open</td>
<td>Analysis of Patients Treated for Chronic Granulomatous Disease Since January 1, 1995</td>
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<tr>
<td>PIDTC 6904</td>
<td>Open</td>
<td>Analysis of Patients Treated for Wiskott-Aldrich Syndrome Since January 1, 1990</td>
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October 2015
PIDTC 6901: A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders v 2.0

***LIMITED INSTITUTION PARTICIPATION***

Protocol PI: Rebecca H. Buckley, MD
Protocol Co-PI: Morton J. Cowan, MD

Hypothesis: In this prospective natural history study, we aim to identify variables contributing to best outcomes for hematopoietic cell transplantation (HCT) or other treatment where applicable (enzyme replacement or gene therapy), which is life-saving therapy for children with SCID, leaky SCID, Omenn syndrome and reticular dysgenesis. Because SCID and related disorders include a spectrum of immunologic presentations, and there have been several approaches to transplant, there are many questions of interest and variables to be explored.

Primary Objectives, Stratum A
The primary objectives of this study are to estimate the 6 month and 2 year overall survival probabilities for subjects after HCT for SCID, and to study risk factors for overall survival in this patient population. A variety of patient, donor and transplant factors will be evaluated for their contribution to the outcomes described above. These are described in detail in Section 10.6 Prognostic Factors of Interest.

Secondary Objectives, Stratum A
Secondary objectives of this study are to determine the effects of donor, recipient and treatment factors on the proportion of subjects having durable engraftment within each blood lineage, and the proportion of subjects having successful/sustained immunologic reconstitution, including T cell function and B cell function, after allogeneic HCT for SCID. Additionally, we will evaluate donor, recipient and transplant factors as well as engraftment and quality of immune reconstitution as contributors to clinical outcome; for example, occurrence of post-transplant infections, GVHD, autoimmunity, growth and development, and quality of life.

Tertiary Objectives, Stratum A
We will assess findings concerning clinical history and presentation and potential biomarkers of ultimate outcome and prognosis with respect to survival and immune reconstitution.

Primary and Secondary Objectives, Stratum B
It is expected that relatively few patients will be available for enrollment in Stratum B, leaky SCID, Omenn syndrome and reticular dysgenesis. Therefore, a descriptive analysis of Stratum B will be performed. Inclusions of individual patients into Stratum A or Stratum B will be based on clinical and immunological features at the time of enrollment. The primary and secondary objectives for patients in Stratum B are the same as for Stratum A; in addition the relative incidence of these SCID variants will be determined as compared to classic SCID.
Objectives for Non-Transplant Therapy, Stratum C, and Subjects Who Expire Prior to Definitive Therapy

Stratum C will include patients who receive alternative therapy; i.e., those with ADA deficiency who are treated with PEG-ADA or patients with ADA deficiency or XSCID or other SCID patients who get gene therapy. This will represent a relatively small group of patients and only descriptive statistics will be available. The objective in studying this group of patients will be to evaluate alternative therapies to HCT that are either supportive or curative in terms of survival, outcome and immune reconstitution. The objective for study of patients who die prior to definitive therapy will be to identify factors contributing to early demise.

Criteria for Evaluation: Note: Endpoints apply to Strata A, B and (as applicable) C, although analyses will be done separately and only descriptive results will be available for Strata B and C. For Stratum C, endpoints and outcomes for ERT or GT will be analyzed similarly to HCT where applicable.

Primary Endpoint
The primary endpoint in this study is overall survival. The event analyzed is death from any cause. The time to this event is the time from HCT or GT or initiation of ERT to death or last scheduled follow-up visit (whichever occurs first). All patients will be followed for a minimum of 6 months from HCT. Overall survival will be estimated at 6 months and 2 years. Patients in Stratum C will be evaluated from the time that therapy begins with either PEG-ADA or infusion of transduced cells. Patients in Strata A, B or C who die or leave the study prior to transplant, initiation of PEG-ADA, or administration of gene-transduced cells will not be included in the analyses, but will be evaluated separately from time of diagnosis to death or discontinuing participation in the study (whichever occurs first).

Secondary Endpoints – Immune Reconstitution and Clinical Outcomes
1. Immune Reconstitution. Immune reconstitution is defined separately for T cells and B cells based on attainment of lab test values at pre-specified time points. Full T cell immune reconstitution at 6, 12 and 24 months post HCT
   • Lymphocyte proliferation to PHA >30% of lower limit of normal control; AND
   • Donor T cell chimerism ≥ 80%

2. Full B cell reconstitution at 12 and 24 months post HCT
   • Three-fold increase in anti-tetanus antibody following tetanus immunization (without replacement Ig administration during the testing period); OR
   • Three fold increase in another specific antibody response following vaccination (without replacement Ig administration during the testing period)

Engraftment at 100 days, 6, 12 and 24 months
1. For whole blood and subsets the following criteria will be used as endpoints: < 5% donor = autologous reconstitution; 5-80% donor = mixed chimerism; ≥ 80% donor = full chimerism.
   • Whole blood
   • CD3
   • CD19

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October 2015
• CD14 and/or CD15 (myeloid cells)
• CD3-CD56+ NK cells

2. Graft failure/rejection is defined as:
   • Less than 5% of donor CD3 cells at 6 months using standard PCR based or in situ hybridization techniques OR
   • Second transplant (unless > 5% CD3 and purpose is to boost immune recovery)

Clinical Outcomes. Clinical outcome will be evaluated for the occurrence and resolution of pre and post-transplant infections, acute and chronic GVHD,50,51 development of autoimmunity, retardation of growth or development, and quality of life. Depending on the particular outcome a minimum follow up of 6, 12 or 24 months will be required for inclusion in this analysis.

1. Infections.
   • Resolution of any pre-HCT infections. Time to resolution (clinically well, off treatment, and/or negative culture/PCR assay) will be measured from the day of HCT. While the specific date will not be requested, this will be asked at each assessment time point post-HCT.
   • Incidence of infection post HCT. These will be reported by site of disease, organism, date of onset, and resolution, if any at 100 days, and 6, 12 and 24 months post HCT.

2. Growth and Nutrition.
   • Z score of weight and height at pre HCT, 1 year post HCT and 2 years post-HCT.
   • Chronic diarrhea, and/or requirement for supplemental nutrition including tube feeding or TPN.

3. GVHD.
   • Occurrence of acute (grade II-IV and grade III-IV) GVHD at 100 days and 6 months post HCT. Any skin, gastrointestinal or liver abnormalities fulfilling the Consensus criteria of Grades II-IV acute GVHD or grades III-IV acute GVHD are considered events. Death is a competing risk, and patients alive without acute GVHD will be censored at the time of last follow-up.
   • Occurrence of chronic GVHD at 6, 12 and 24 months post HCT. Occurrence of symptoms in any organ system fulfilling the criteria of limited or extensive chronic GVHD. Death is a competing risk, and patients alive without chronic GVHD will be censored at time of last follow-up.
   • Therapy given for GVHD.
   • Time to discontinuation of immunosuppression.

4. Autoimmunity.
   Occurrence of autoimmunity requiring treatment with immunosuppression or other therapy. Death is a competing risk, and patients alive without autoimmunity will be censored at time of last follow-up. Date of onset and type of treatment will be collected on the following:
   • Autoimmune hypothyroidism
   • Autoimmune cytopenia (hemolytic anemia, thrombocytopenia, neutropenia)
   • Arthritis
   • Myositis
5. **Neuro/cognitive developmental assessments.**
Evaluation of patients with SCID Spectrum Disorder will be done using two separate approaches (refer to Appendix II for list and description of questionnaires):

- Formal neuro/cognitive testing will be done as part of routine clinical care if available pre, and approximately 12, 24 and 48 months post HCT. Recommended tools are the:
  - Bayley Scales of Infant Development, Third Edition (BSID III)
  - Vineland Adaptive Behavior Scales, Second Edition (Vineland II)
  - Wechsler Preschool and Primary Intelligence Scale of Intelligence, Third Edition (WPPSI III)
  - Wechsler Intelligence Scale for Children, Fourth Edition (WISC IV)
- Assessments conducted by telephone interview will be done pre and approximately every 6 months post HCT. Telephone assessments will take approximately 30-45 minutes at each time point.

6. **Other complications of HCT needing treatment.**
- Veno-occlusive disease
- Thrombotic thrombocytopenic purpura
- Bronchiolitis obliterans / chronic lung disease
- Seizures
- Hypertension
- Malignancy
- Other

7. **Quality of Life.**
QOL testing will be done pre-HCT and at 12, 24 and 48 months post HCT using the following validated tools depending on recipient’s age:

- Peds QL Family Impact Module, Parent Report
- Peds QL Infant Scales Module 1-24 months (Parent Reports)
- Peds QL Generic Core Scales for Toddlers (ages 2-4 yr), Parent Report
- Peds QL Generic Core Scales for ages 5-25 yr), Child/Parent Reports

**Tertiary Endpoints: Biomarkers at 100 days, and 6, 12 and 24 months post definitive treatment (HCT, ERT, GT) that predict outcome**

1. **T cell.**
   - CD3
   - CD4
• CD4 naïve (CD45RA+)
• T cell proliferation to mitogens and antigens
• TREC
• T cell diversity (spectratyping or V-beta usage)

2. B cell.
• IgA, IgM levels; IgG when off Ig supplementation
• Isohemagglutinin levels
• Naïve B cells
• CD19+ CD27+
• For XSCID: c expression on CD19 cells

3. NK cell.
• NK cell number and/or function

4. Engraftment.
• CD3
• B cell
• CD14/15 (myeloid/monocytic)
• CD3- CD56+ NK (limited sites)

Study Design: This protocol is a prospective natural history study in which investigators at 14 centers and additional PBMTC centers caring for patients with SCID Spectrum Disorder in North America will participate. Investigators will: 1) evaluate and treat patients with SCID Spectrum Disorder according to their institutional practice and protocols, and also 2) enroll the same patients as subjects in this prospective natural history protocol. Thus, subjects will receive comparable baseline and follow up evaluations across all transplant centers, irrespective of the transplant (or other treatment) strategy and methods used at an individual site.

Subject Selection: There will be three strata: Stratum A will include patients with classic SCID; Stratum B will include patients with leaky SCID, Omenn Syndrome, or Reticular Dysgenesis; and Stratum C will include patients with ADA-deficient SCID or XSCID receiving enzyme-replacement therapy or gene therapy. Patients with other primary immunodeficiency diseases who do not meet the strict entry criteria or patients with secondary immunodeficiency or HIV infection will be excluded from this study.

Intervention: None (Observational Study)

Subject Assessments: Per Table 7.1 of protocol.

Sample Size: 209 (Estimate: screen 232 with 90% accrual rate) for Stratum A; 50 for Stratum B, and unknown for Stratum C
Protocol PI: Elizabeth M. Kang, MD

Protocol Co-PI: Harry L. Malech, MD, and Luigi D. Notarangelo, MD

Hypothesis: CGD results from mutations in any one of five genes encoding any one of the critical subunits of the phagocyte NADPH oxidase. The result of these mutations is a common phenotype affecting the phagocyte oxidase (phox) activity of neutrophils, monocytes and tissue macrophages. This deficiency of oxidants results in susceptibility to recurrent bacterial and fungal infections as well as granuloma formation and other associated autoimmune complications. CGD can be cured by HCT (cessation of recurrent infections; resolution of gastrointestinal and pulmonary inflammation).

This study will investigate which patients benefit most from HCT, and what types of transplants are optimal for patients with CGD, in the context of overall outcomes in CGD patients with and without transplant. We aim to identify variables contributing to best outcomes of HCT in patients with CGD. Some hypotheses for this study, provided adequate numbers of subjects are available for statistical power, are described in detail in Section 1.2.4.

Primary Objective

The primary objective of this protocol is to estimate the 1-year, 2-year and 3-year (and longer if possible) overall survival probabilities post-HCT of CGD subjects born on or after 1988 who receive HCT on or after 1995.

Secondary Objectives

1. To compare overall survival from birth between patients born on or after 1988 who receive HCT on or after 1995 vs. those born on or after 1988 who receive conventional therapy, after adjusting for differences in year of birth and oxidase activity.

2. To compare the prevalence of recent infections or inflammatory complications (within one year of last contact), between transplanted patients who are at least 3 years post-transplant and non-transplanted controls who are at least 3 years post diagnosis. This analysis will adjust for birth year and oxidase activity. We will also conduct separate analysis of the rates of recent infections as well as the prevalence of inflammatory complications alone, in addition to the combined prevalence estimate.

3. To compare the prevalence of recent infections or inflammatory complications (within one year of last contact), between transplanted patients who are at least 3 years post-transplant vs.
their prevalence of recent infections or inflammatory complications in the one year period prior to transplant. We will also conduct separate analysis of the rates of recent infections as well as the prevalence of inflammatory complications alone, in addition to the combined prevalence estimate.

4. To compare the prevalence of recent infections or inflammatory complications (within one year of most recent myeloid chimerism assessment) between transplanted patients who are at least 3 years posttransplant and who have achieved partial reconstitution (> 5 and < 15% myeloid engraftment) vs. those who have achieved > 15% myeloid chimerism. The question to be answered by this objective is whether stable long term restoration of oxidase normal granulocytes to 15% of cells or higher in HCT transplanted CGD subjects results in reduction or cessation of incidence of infections and prevalence of autoimmune/inflammation complications. We will also conduct separate analyses of the rates of recent infections as well as the prevalence of inflammatory complications alone, in addition to the combined prevalence estimate.

5. To conduct a similar analysis comparing the prevalence of new infections and inflammatory disease of CGD subjects enrolled in a cross-sectional analysis.

6. To estimate the proportion of patients who will resolve pre-existing inflammatory complications after.

7. To describe chimerism in leukocyte lineages (myeloid, T and B cell) resulting from HCT for CGD at Day 100, Month 6, and annually for three years post-transplant.

8. If sufficient patient numbers are available, we will explore the association of clinical outcomes (survival, infection, autoimmune disease, chimerism, GVHD) with the use of regimen type (myeloablative vs. nonmyeloablative), and degree of HLA matching, as well as age, presence of infection and/or inflammatory disease at the time of transplant, and evidence of portal hypertension or thrombocytopenia.

9. We will assess outcomes when the sibling donor is an oxidase normal or is a CGD phenotype mosaic female carrier of X-linked CGD (for patients with X-linked CGD).

10. To determine the level of engraftment in transplanted patients more than two years post-transplant.

11. To compare the quality of life in transplanted patients alive at least two years post-transplant to nontransplanted patients as stratified by oxidase positive or oxidase null residual oxidase activity (and if possible, other pre-transplant conditions impacting on quality of life). For HCT subjects, will evaluate if there is an association between degree of myeloid chimerism post-HCT and quality of life.

**Study Endpoints (Longitudinal Analysis: Retrospective and Prospective Cohorts) (Part 1)**

**Primary Endpoint - Survival**

The primary endpoint in this study is overall survival. The event analyzed is death from any cause. For the analyses of HCT subjects only, the time from HCT to death or last follow up will be analyzed.
Overall survival will be estimated at 1, 2 and 3 years (and longer, numbers permitting) after transplant. Cause of death will also be collected. Surviving patients will be censored at the time of last observation.


Secondary endpoints include: survival of HCT subjects vs. conventional therapy subjects, prevalence of recent infection or inflammatory complication of HCT subjects vs. conventional therapy subjects, event-free survival post-HCT, immune reconstitution, engraftment, and clinical outcomes such as infection, autoimmune disease and inflammatory complications, GvHD, growth, and quality of life.

**Study Endpoints (Cross-Sectional Analysis) (Part 2)**

Primary Endpoint – Infection History for Preceding 1-Year Duration

Infection history (up to 1 year prior to cross-sectional visit and subject at least 3 years post-transplant or postdiagnosis) is the primary endpoint in this study.
pidtc 6904: analysis of patients treated for wiskott-aldrich syndrome since january 1, 1990

***limited institution participation***

protocol pi: lauri m. burroughs, md
protocol co-pi: david j. rawlings, md, luigi d. notarangelo, md and lisa filipovich, md

hypothesis: was is a congenital primary immunodeficiency that results in significant morbidity and decreased overall life expectancy. in this longitudinal and cross-sectional natural history study, we aim to identify variables contributing to best outcomes of hematopoietic stem cell transplantation (hct) in patients with wiskott-aldrich syndrome (was). hct offers curative therapy, however studies are needed to better define the optimal timing for transplantation, donor source, and conditioning regimen(s). we hypothesize that patients with was who undergo hct when they are in relatively good clinical condition (young age, no lymphoma, etc.) who receive marrow or cord blood from a closely hla matched donor after a myeloablative regimen can reliably be cured of their underlying disorder.

study objectives for stratum a of the retrospective and prospective cohorts of the longitudinal analysis (part 1)

primary objectives
the primary objectives of this study are to estimate survival at 6 months and 1, 2, 3, 5, 10, and 15 years post-hct, and to study risk factors for overall survival in this patient population. a variety of patient, donor, and transplant factors will be evaluated for their contribution to the outcomes described above.

secondary objectives
secondary objectives of the study are to determine the effects of donor, recipient and treatment related factors on the proportion of subjects having durable engraftment of t, b and myeloid lineages, and the proportion of subjects having successful immunologic and hematologic reconstitution, including t cell function, b cell function, and platelet numbers at 100 days, 6 months and 1, 2, 3-5, 6-10, 11-15, and > 15 years after hct for was. additionally, we will evaluate donor, recipient and transplant factors as well as engraftment and quality of immune reconstitution as contributors to clinical outcome, including occurrence of post-transplant infections, bleeding episodes, and/or new malignancies, gvhd, autoimmunity, growth and development, and quality of life.

study objectives for stratum a of the cross-sectional analysis (part 2)

primary objectives
the primary objective of this cross-sectional study is to evaluate current survivors of hct for was as to the effects of patient, donor and transplant-related factors on the degree of immune reconstitution of t, b and nk cells, and normalization of peripheral blood platelet counts.

secondary objectives

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october 2015
The secondary objectives of this cross-sectional study are to comprehensively evaluate current survivors of HCT for WAS as to the effects of patient, donor and transplant-related factors on current health, as measured by Karnofsky or Lansky functional scores, frequency, type and chronicity of infections, abnormalities of growth or organ function, presence or absence of graft-versus-host disease, presence or absence of autoimmune disorders, presence or absence of severe bleeding episodes, quality of life, fertility, presence or absence or malignancy, hematological and immune reconstitution.

Study Endpoints for Stratum A of the Retrospective and Prospective Cohorts of the Longitudinal Analysis (Part 1)

**Primary Endpoint** – Overall Survival at Month 6, and Years 1, 2, 3, 5, 10, and 15 Post-HCT.

The primary endpoint in this study is overall survival. The event analyzed is death from any cause. The time to this event is the time from HCT to death or last follow-up. Cause of death will also be collected. Surviving patients are censored at the time of last observation. Overall survival will be estimated at 6 months, 1, 2, 3, 5, 10, and 15 years (numbers permitting).

**Secondary Endpoints** – Hematologic and Immune Reconstitution and Clinical Outcomes

Secondary endpoints include: hematologic reconstitution, immune reconstitution, engraftment, graft failure/rejection, and clinical outcomes.

Study Endpoints for Stratum A of the Cross-Sectional Analysis (Part 2)

Disease response including immune reconstitution, lineage specific chimerism, disease/clinical status and quality of life will be assessed for subjects surviving at least 2 years post-HCT.

**Primary Endpoint**: Proportion of Patients Achieving Full Immune Reconstitution and Resolution of Thrombocytopenia

Study Outcomes for Stratum B

Stratum B will include patients who receive gene therapy and these patients will be followed within the retrospective, prospective and cross-sectional cohorts as appropriate.

Endpoints and outcomes for Stratum B will be analyzed similarly to Stratum A (HCT) as applicable with the exception of chimerism and mechanistic studies.

Statistical analysis will be limited to descriptive statistics as the number of subjects available for study will be small.

**Study Design**: This protocol includes prospective, retrospective and cross-sectional studies in which investigators at 13 centers and additional PBMTC centers caring for patients with WAS in North America will participate. Patients with WAS will have been treated according to institutional practice and protocols. Investigators will enroll these patients as subjects in this protocol.

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October 2015
Nursing Discipline Committee

Chair

Rita Secola, PhD, RN, CPON, FAAN
Children’s Hospital Los Angeles
The PBMTC Nursing Committee is a group of professional nurses dedicated to promoting optimal nursing care for pediatric patients undergoing stem cell transplantation and their families. They provide leadership, education and expertise to hematology/oncology/stem cell transplant nurses to ensure the highest standards of practice and care for this specialty population.

I. Year 2015-2016

Mission

To set the standard of excellence for the care of pediatric patients undergoing stem cell transplantation and to support and advance nursing knowledge and practice in order to optimize patient and family outcomes through education, evidence based practice and research.

Vision

To be recognized as leaders and expert resource in pediatric stem cell transplant nursing.

Goals

- Ongoing recruitment to PBMTC nursing membership
- Disseminate awareness of and education on PBMTC participating protocols to nursing
- Provide continuing nursing education through scheduled nursing workshops
- Promote BMTCN certification.
- Evaluate and develop evidence based standards of nursing care and guidelines in the context of autologous and allogeneic transplants
- Evaluate and develop teaching strategies and tools for patients and families that relate to stem cell transplantation
- Seek funding resource options for PBMTC nursing
- Promote and participate in the exchange of scientific and clinical information about stem cell transplantation through regular medical, nursing and scientific meetings and scholarly publications
- Foster and participate in stem cell transplant nursing and interdisciplinary research
- Liaison with community of interdisciplinary scholars
- Liaison with nursing organizations focused on evidence based practice and nursing stem cell transplantation research
PBMTC Nursing.

**Chair:** Rita Secola PhD RN CPON FAAN; rsecola@chla.usc.edu

**Vice Chair:** TBD

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**Advisory Nurse Member:**

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Yvonne Barnes DNP RN CPNP; yibarnes@charter.net

**2014-2015 Summary of Activities:**


2. November 2014 sent Nursing Recruitment letter to all PBMTC full member Institutions. Letter provided introduction to the nursing committee, its purpose and goals and to seek membership. Favorable response and new members on board and will continue ongoing recruitment over the next year.
3. February 2015 held PBMTC nursing committee meeting in San Diego, CA. Review progress and planning for 2015 Workshop, update on recruitment and brainstorm next initiatives and interests.


5. Leadership committee ongoing email and conference call communications.

6. Planned and Confirmed 2015 PBMTC Nursing Workshop to be held on Oct 5, 2015, Dallas, Texas. (Brochure and Agenda attached).

7. Awarded five $500.00 Poster Presentation/Travel Scholarships for workshop attendees.
Blood and Marrow Transplant Clinical Trials Network  
(BMT CTN)

Studies with PBMTC Participation

<table>
<thead>
<tr>
<th>BMT CTN Study Number</th>
<th>Study Status</th>
<th>Title</th>
<th>Study Chair(s)</th>
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<tr>
<td>0101</td>
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<td>A Randomized Trial of Fluconazole vs. Voriconazole for the Prevention of Invasive Fungal Infections in Allogeneic Blood and Marrow Transplant Patients</td>
<td>John Wingard</td>
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<td>A Phase III Randomized, Multicenter Study Comparing G-CSF mobilized Peripheral Blood Stem Cell with Marrow transplantation from HLA compatible unrelated donors</td>
<td>Claudio Anasetti</td>
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<td>A Phase I/II Multicenter Study of Fludarabine-Based Conditioning for Allogeneic Marrow Transplantation from HLA-Compatible unrelated Donors in Severe Aplastic Anemia</td>
<td>Paolo Anderlini</td>
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<td>Initial Systemic Treatment of Acute GVHD: A Phase II Randomized Trial Evaluating Etanercept, Mycophenolate Mofetil (MMF), Denileukin Diftitox (Ontak), and Pentostatin in Combination with Corticosteroids</td>
<td>Daniel Weisdorf</td>
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<td>0501</td>
<td>Closed</td>
<td>Multi-Center, Open Label, Randomized trial Comparing Single Versus double Umbilical Card Blood (UCB) Transplantation in Pediatric patients with High Risk Malignancy</td>
<td>John Wagner, Joanne Kurtzberg</td>
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<td>Unrelated Donor Hematopoietic Cell Transplantation for Children with Severe Sickle Cell Disease Using a Reduced Intensity Conditioning Regimen</td>
<td>Shalini Shenoy Naynesh Kamani</td>
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<td>0801</td>
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<td>A Phase II Randomized, Multicenter Trial Comparing Sirolimus plus Prednisone, Sirolimus/Extracorporeal Photopheresis plus Prednisone, and Sirolimus/Calcineurin inhibitor plus Prednisone for the Treatment of Chronic Graft versus Host Disease.</td>
<td>Paul Carpenter</td>
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<td>A Multicenter, Randomized, double Blind, Phase III Trial Evaluating Corticosteroids with Mycophenolate Mofetil vs. Corticosteroids with Placebo as Initial Systemic Treatment of Acute GVHD</td>
<td>Javier Bolanos-Meade Vincent Ho</td>
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<td>1202</td>
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<td>Prospective Multi-Center Cohort for the Evaluation of Biomarkers Predicting Risk of Complications and Mortality Following Allogeneic HCT</td>
<td>John Levine John Hansen</td>
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<td>1204</td>
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<td>Reduced-Intensity Conditioning for Children and Adults with Hemophagocytic Syndromes or Selected Primary Immune Deficiencies (RICHI)</td>
<td>Carl Allen Michael Pulsipher</td>
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<tr>
<td>1301</td>
<td>Open</td>
<td>A Randomized, Multi-Center, Phase III Trial of Calcineurin Inhibitor-Free Interventions for Prevention of Graft-versus-Host Disease</td>
<td>Leo Luznik Miguel-Angel Perales Marcelo Pasquini</td>
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**BMT CTN Specific Information:**


Administrative and technical MOPs are also available on this website.

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October 2015
Open Trial
BMT CTN 1202: Prospective Multi-Center Cohort for the Evaluation of Biomarkers Predicting Risk of Complications and Mortality Following Allogeneic HCT v2.0

Study Chairperson: John Levine, MD
Study Co-Chair: John Hansen, MD
BMT CTN Protocol Coordinator: Jason Thompson

Objective: The goal of this protocol is to establish a cohort of biologic samples collected prospectively from patients treated in BMT CTN centers that will be a shared biospecimen resource for conducting future allogeneic hematopoietic stem cell transplantation (HCT) correlative studies.

Accrual Objective: A maximum of 1,500 patients will be enrolled.

Accrual Period: The estimated accrual period is 4 years.

Eligibility Criteria: All U.S. Allogeneic Transplant Donors and Recipients weighing 10 or more kg may participate in the collection of samples.

Treatment Plan: Conditioning regimens, GVHD prophylaxis, and other supportive care will follow institutional guidelines.

Study Duration: Patients will be followed for 24 months post-HCT; long-term follow-up data will be collected through usual procedures of the Center for International Blood and Marrow Transplant Research (CIBMTR).
Open Trial
BMT CTN 1204: Reduced-Intensity Conditioning for Children and Adults with Hemophagocytic Syndromes or Selected Primary Immune Deficiencies (RICH)

Study Chairperson: Carl Allen, MD
Study Co-Chair: Michael Pulsipher, MD
BMT CTN Protocol Coordinator: Alyssa Ramirez

Primary Objective: To prospectively determine the 1-year overall survival in subjects treated for hemophagocytic syndromes or primary immune deficiencies (CGD, HIGM1, IPEX, and severe LAD-I) using a standardized, reduced-intensity conditioning protocol consisting of fludarabine, melphalan and intermediate timing of alemtuzumab (Day -14).

Secondary Objectives: Secondary objectives for the study include measurement of sustained engraftment, incidence of HLH reactivation and death from disease, immune reconstitution and functional immune recovery at 1-year, cumulative incidence (CI) of grade II-IV and III-IV acute GVHD and chronic GVHD, transplant-related complications (VOD, CNS toxicity), infectious complications including reactivation of CMV, adenovirus, EBV, invasive fungal infection or bacterial sepsis, and overall survival and rate of sustained engraftment of specific disease subsets.

Study Design: This study is designed as a Phase II multi-center trial. The study population includes patients with HLH, HLH-related disorders, and selected primary immune deficiencies: CGD, HyperIgM Syndrome (HIGM1), IPEX Syndrome, or severe LAD-I with indications for HCT receiving a bone marrow transplant with a 6/6 HLA-matched related donor at HLA-A, -B, (intermediate or higher resolution) and –DRB1 (at high resolution using DNA-based typing) OR a 7/8 or 8/8 HLA-matched related donor at HLA-A, -B, -C, and -DRB1 (at high resolution using DNA-based typing) OR a 7/8 or 8/8 HLA-matched unrelated donor at HLA-A, -B, -C, and -DRB1 (at high resolution using DNA-based typing).

Accrual Objective: The trial will accrue 35 patients.

Accrual Period: The estimated accrual period is 3 years.

Eligibility Criteria: Eligible patients are ≥ 4 months and ≤ 45 years of age with Lansky/Karnofsky performance status ≥ 50% who have HLH or related disorders or selected immune deficiencies with an indication for HCT. Patients must have adequate organ function (cardiac, renal, hepatic, pulmonary). HLA typing of related donors must be a 6/6 match for HLA–A and –B (intermediate or higher resolution) and –DRB1 (at high resolution using DNA-based typing) OR a 7/8 or 8/8 match for HLA–A, –B, –C, and –DRB1 (at high resolution using DNA-based typing). Unrelated donors must be a 7/8 or 8/8 match at HLA–A, –B, –C, and –DRB1 (at high resolution using DNA-based typing). Only bone marrow donors are allowed on this study.
Treatment Description: All eligible patients undergoing bone marrow HCT will receive reduced-intensity conditioning (RIC) with fludarabine, melphalan and alemtuzumab beginning on Day -14.

Study Duration: Patients will be followed for 1 year post HCT.
Open Trial
BMT CTN 1301: A Randomized, Multi-Center, Phase III Trial of Calcineurin Inhibitor-Free Interventions for Prevention of Graft-versus Host-Disease

Co-Principal Investigators: Leo Luznik, Miguel-Angel Perales, Marcelo Pasquini

Study Design: The study is designed as a three arm randomized Phase III, multicenter trial comparing two calcineurin inhibitor (CNI)-free strategies for GVHD prophylaxis to standard calcineurin inhibitor tacrolimus and methotrexate (Tac/Mtx) in patients with acute leukemia or myelodysplasia undergoing myeloablative conditioning hematopoietic stem cell transplantation.

Primary Objective: The primary objective of the randomized trial is to compare chronic GVHD/relapse-free survival [CRFS] as a time to event endpoint after hematopoietic stem cell transplant (HSCT) between each of the CNI-free interventions and a Tac/Mtx control.

Secondary Objectives: Secondary objectives are: comparison of rates of grade II-IV and III-IV acute GVHD, chronic GVHD, chronic GVHD-free survival, immunosuppression-free survival at one year, neutrophil and platelet engraftment, disease relapse, transplant-related mortality, rates of Grade ≥ 3 toxicity according to the Common Terminology Criteria for Adverse Events (CTCAE) Version 4.0; incidence of CMV and EBV reactivation, incidence of infections; immune reconstitution, quality of life and overall survival.

Eligibility Criteria: Patients > 1 year and < 65 years undergoing HSCT for treatment of acute leukemia in morphologic complete remission or myelodysplasia with <5% blasts in the marrow and no circulating blasts, and who are eligible for a myeloablative allogeneic transplant. Patients must have a related or unrelated donor. Related donor must be an 8/8 match for HLA-A, -B and -C at intermediate (or higher) resolution, and -DRB1 at high resolution using DNA-based typing. Related donor must weigh ≥ 25.0 kg, must have adequate peripheral venous catheter access for leukapheresis or must agree to placement of a central catheter, must be willing to (1) donate bone marrow and (2) receive G-CSF followed by donation of peripheral blood stem cells (product to be determined by randomization post enrollment), and must meet institutional criteria for donation. Unrelated donor must be an 8/8 match at HLA-A, -B, -C and -DRB1 at high resolution using DNA-based typing. Unrelated donor must be medically eligible to donate according to NMDP (or equivalent donor search organization) criteria. At time of enrollment, the donor should not have any known preferences or...
contraindications to donate bone marrow or peripheral blood stem cells.

**Treatment Description:** Patients will be randomized to receive one of the three specified interventions: 1) CD34 selected T-cell depleted peripheral blood stem cell (PBSC) graft; 2) unmanipulated bone marrow (BM) graft followed by cyclophosphamide (Cy) 50mg/kg Day +3 and +4 post HSCT; or, 3) unmanipulated BM graft with Tac/Mtx GVHD prophylaxis. Tac will be maintained at therapeutic doses for a minimum of 90 days. Methotrexate will be dosed at 5-15mg/m² for a maximum of 4 doses post-transplant.

**Accrual Objective:** The clinical trial will enroll 345 patients or 115 per arm, in an adaptive design for futility evaluated at time of interim analysis.

**Accrual Period:** The estimated accrual period is 42 months.

**Study Duration:** Patients will be followed for 2 years following hematopoietic cell transplantation

**Interim Analysis:** No formal interim analyses for efficacy will be used. There is also not included in the design an option for closure of the control group while keeping the two treatment arms open, in the event that at least one of the treatments demonstrate early efficacy. Interim analyses for futility will be conducted at times coincident with regularly scheduled meetings of the DSMB, starting when approximately 45-50% of the targeted number of events have been observed.

**Stopping Guidelines:** Monitoring of a key safety endpoint (mortality) will be conducted monthly up to 100 days post-randomization separately in each of the three treatment arms. At least three events must be observed in order to trigger review.

**Correlative Studies:** Comparison of immune reconstitution using a panel of clinically available tests across all treatment arms. Advanced immune reconstitution assays.
UPCOMING
PBMMC MEETINGS

Winter 2016
BMT Tandem Meetings
February 18-22, 2016
Honolulu, HI

ASPHO/PBMMC
May 11, 2016
Minneapolis, MN

Fall 2016
September 13, 2016
Atlanta, GA