BLOOD AND MARROW TRANSPLANT CLINICAL TRIALS NETWORK

BMT CTN 0201 FREQUENTLY ASKED QUESTIONS (FAQs)

A Phase III Randomized, Multicenter Trial Comparing G-CSF Mobilized Peripheral Blood Stem Cell with Marrow Transplantation form HLA Compatible Unrelated Donors

1. **HOW WILL I BE NOTIFIED OF WHICH ARM MY PATIENT HAS BEEN RANDOMIZED TO?**
   Once a suitable donor has been found, both the patient and the donor will be consented for the trial. The transplant center will be responsible for entering the patient randomization eligibility into Advantage EDC[SM]. The NMDP Search Coordinating Unit will enter the donor eligibility information into Advantage EDC[SM]. Once both the patient and donor information has been entered into Advantage EDC[SM], the patient and donor will automatically be electronically randomized. The NMDP will be e-mailed the treatment assignment. The NMDP will then notify the donor center and the transplant center of the treatment assignment and treatment arrangements can be made.

2. **WHICH CONDITIONING REGIMEN SHOULD I USE FOR MY PATIENT?**
   The conditioning regimens are outlined in section 2.6.1 of the protocol. It is up to the discretion of the transplant center medical staff to determine which regimen would be the best for each individual patient. Different conditioning regimens may be used for patients with different diseases as required by institutional protocol. However, the conditioning regimen must be declared prior to randomization in Advantage EDC[SM] and must be used regardless of whether the patient is randomized to receive PBSC or marrow. If cyclophosphamide and TBI is chosen as the conditioning regimen, the order of administration is at the discretion of the transplant center. However, we do ask that within each transplant center, all patients receiving this regimen receive it in the same order. Additional drugs including anti-T cell antibodies may be added at the transplant center's discretion with the exception of Alemtuzumab (Campath-1H).

3. **WHICH GVHD PROPHYLAXIS REGIMEN SHOULD I USE FOR MY PATIENT?**
   The GVHD prophylaxis regimens are outlined in section 2.6.2, 2.6.3, and 2.6.4 of the protocol. It is up to the discretion of the transplant center medical staff to determine which regimen would be the best for each individual patient. The GVHD prophylaxis regimen should be declared in Advantage EDC[SM] prior to randomization and must used whether the patient is randomized to receive PBSC or marrow. It should be noted that full dose methotrexate is being used and mini-methotrexate regimens are not permissible. If the methotrexate dose is reduced for renal, hepatic or mucosal toxicity, these modifications should be documented on the Toxicity Form in Advantage EDC[SM].

4. WHY IS THE VACCINE SCHEDULE NOT CONSISTENT WITH THE SCHEDULE DICTATED BY THE CDC?

The early vaccination schedule is based on a paper[1] published in *Blood* in 2003 by a group at the Dana Farber Cancer Center. Subjects on this study will be receiving the dT and Hepatitis A vaccine at 6 and 12 months post-transplant, the PCV7 vaccine at 7 and 11 months post-transplant, and the PPV23 vaccine at 12 months post-transplant.

5. SHOULD THE VACCINATION SCHEUDULE BE MODIFIED IF THE SUBJECT HAS SEVERE GVHD OR SOME OTHER INFECTION?

The standard practice for use of the vaccine should be followed. For example, if the subject is febrile, the subject should not be vaccinated until they are afebrile. However, every attempt should be made to vaccinate subjects on schedule. Vaccination should take place ± 1 week of the scheduled date. The vaccines being given are not derived from live viruses, thus they pose no risk to immunocompromised patients. There is a risk that the vaccines will not be effective, in which case the subject could be revaccinated at a later date. If for some reason the subject is not vaccinated, the research laboratory samples should still be collected as scheduled.

6. WILL THE RESULTS OF THE RESEARCH LABORATORY ASSAYS BE PROVIDED TO THE TRANSPLANT CENTERS?

The results of the research laboratory assays will not be provided to Principal Investigators at the Transplant Centers for use in patient-care decisions. It is anticipated that the results will be provided to the PI's at the end of the study.

7. WHY CAN'T GROWTH FACTORS BE USED PRIOR TO DAY 21?

Growth factors are often used more frequently for marrow patients than PBSC patients, due to bone marrow engrafting less quickly than PBSC. This variability in the usage of growth factors may introduce a bias that may affect the measure of time to engraftment and other endpoints in a manner that is either unfavorable or not anticipated. Therefore, the center should not begin routine usage of growth factors prior to Day 21 for patients enrolled on this study. However, since patients with infections may benefit from early engraftment, the protocol does permit growth factors to be used prior to Day 21 for patients with a serious infection.

8. WHY MUST I REGISTER ALL OF MY TRANSPLANT PATIENTS INCLUDING THOSE NOT PARTICIPATING IN BMT CTN PROTOCOLS WITH THE IBMTR?

The data collection system and monitoring system for BMT CTN trials builds on the IBMTR data collection system. An essential part of this process is registration of all patients transplanted in each participating center. This requires completion of the

[1] Molrine DC, Antin JH, Guinan EC, et al. Donor immunization with pneumococcal conjugate vaccine and early protective antibody responses following allogeneic hematopoietic cell transplantation. *Blood* 2003; 101: 831-836.

IBMTR "Pre-Reg Form". Registration of transplant recipients (both those entered and those not entered on BMT CTN trials) is a requirement for all Core and non-Core centers. These data will be used to generate recruitment reports that would otherwise be requested of each center monthly to meet NHLBI recruitment monitoring requirements. They will also be used to study the recruitment process and evaluate ways in which we can modify protocols or institute other measures to enhance accrual to BMT CTN protocols.

What forms must I submit to the IBMTR?

- ✓ Pre-Registration Form for all patients (both those entered and those not entered on BMT CTN trials) is a requirement for all Core and non-Core centers. At a minimum these must be batched to the IBMTR monthly.
- ✓ TED Form (including the Core, Graft and Disease inserts) at 100 days post transplant for all patients enrolled in BMT CTN protocols. At a minimum these must be batched quarterly to the IBMTR.
- ✓ Follow-up TED Form (including the Core and Disease follow-up inserts) yearly for all patients enrolled in BMTCTN protocols. At a minimum these must be batched quarterly to the IBMTR.

How do I submit the IBMTR forms?

You may submit "paper" or "electronic" IBMTR forms directly to the IBMTR as you currently do. If you chose to submit paper forms, green "BMT CTN" stickers will be provided to you to place on the forms to notify the IBMTR that the patient is participating in a BMT CTN protocol(s).