

From: Tull, Lori
Sent: Friday, October 09, 2015 3:02 PM
To: Wouter Van't Hof (wvanthof@clevelandcordblood.org)
Cc: Gildner, Jean
Subject: Request for information for BLA

Follow Up Flag: Follow up
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Hi Wouter,

Would you please provide the following information by Friday, October 23?

1. A line listing of patients who received a dose of TNCs $\geq 2.5 \times 10^7/\text{kg}$ and who have an HLA match $> 3/6$, with the degree of HLA match and the TNC dose ($\times 10^7/\text{kg}$) listed.
2. A line listing for the degree of HLA match for all patients.
3. A line listing for all patients who experienced primary graft failure.
4. A line listing for all patients who experienced infusion reaction. Please indicate the number of infusions for each patient.
5. Additional information about ethnicity: e.g., breakdown into categories -- Caucasian, Hispanic, African American, Asian, American Indian, Multi-race
6. For items #2-5, please provide patient ID information for all patients and indicate the patients for whom the data for that parameter is not available.
7. A line listing indicating which patients received non-CCBC cord blood units.
8. In order for us to be able to perform statistical analyses on your data, please provide an Excel file(s) for the data that is requested above, and also an Excel file(s) for the information that you submitted in the BLA application in the Section titled, "Listings of Clinical Outcomes".
9. In your summary of the clinical data (section 31.8), it is unclear whether all or only some of the efficacy and safety outcomes that you summarized are based on analyses of the subset of patients who received a minimum dose of 2.5×10^7 TNCs. Please clarify this for each of the efficacy and safety outcomes that are in this Section of the BLA submission. For the outcome measures for which your analyses were not performed on the subset of patients who received a minimum dose of 2.5×10^7 TNCs, please summarize those particular outcomes for this subset of patients.

Best Regards,

Lori Tull
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Regulatory Management Staff
Office of Cellular, Tissue, and Gene Therapies
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